Ethic and health economic evaluation in the context of decision-making about reimbursement of health technologies by the national health insurance

Clémence Thebaut

UNIVERSITE PARIS-DAUPHINE

ECOLE DOCTORALE DE DAUPHINE

Laboratoire d'Economie et de Gestion des Organisations de Santé

Ethique et évaluation économique des interventions de santé en vue d'une définition du périmètre des soins remboursables

Doctorat d'économie, spécialisé en économie de la santé

Thèse présentée et soutenue par :

Clémence THEBAUT

le 31 octobre 2012

Directeurs de thèse :

Professeur Marc FLEURBAEY, Princeton University

Professeur Jérôme WITTWER, Université Paris-Dauphine

JURY :

Professeur Florence JUSOT,	Université de Reims
Professeur Pierre-Yves GOEFFARD,	Paris School of economics
Professeur Lise ROCHAIX,	Université Aix-Marseille (Rapporteur)
Professeur Erik SCHOKKAERT,	Université de Louvain-la-Neuve (Rapporteur)

Remerciements

Mes remerciements vont d'abord à mes directeurs de thèse pour leur soutien sans lequel je n'aurais pu mener à terme ce travail. Je remercie particulièrement Marc Fleurbaey, d'avoir accepté d'encadrer ma thèse. Je lui suis infiniment reconnaissante pour le temps qu'il m'a consacré et pour m'avoir guidé dans l'orientation de mes travaux. Je suis tout aussi reconnaissante à l'égard de Jérôme Wittwer de m'avoir accueilli dans l'équipe du LEGOS de Dauphine. Je dois beaucoup aux échanges que nous avons pu avoir ensemble et à la confiance qu'il m'a accordée pour ce projet de recherche.
J'adresse dans le même temps ma gratitude à l'attention de Mesdames et Messieurs les Professeurs Florence Jusot, Lise Rochaix, Pierre-Yves Geoffard et Erik Schokkaert, pour avoir accepté de participer à mon jury de thèse. Je les remercie également pour les commentaires qu'ils m'ont d'ores et déjà adressés, lors de ma pré-soutenance et lors des Journées des doctorants en économie de la santé, ainsi que dans le cadre des séminaires internes du LEGOS. A ce titre, je remercie la Chaire Santé de Dauphine, et plus particulièrement le Professeur Brigitte Dormont, d'avoir organisé certaines de ces rencontres et de m'avoir permis de participer aux travaux du groupe « Valeur de la santé » qui s'est réuni autour de l'enquête Revenu Equivalent-Santé.
Je souhaite enfin remercier Anne-Laure Samson, Maître de conférences à l'Université de Dauphine, de m'avoir accompagnée dans le projet qui a abouti au troisième chapitre de cette thèse. Son expérience de recherche et ses compétences m'ont permis d'aller plus loin dans ce travail que je ne l'espérais initialement. Je remercie également Stéphane Luchini pour le soutien méthodologique qu'il nous a apporté.

Cette thèse n'aurait pas pu être menée sans le soutien intellectuel et financier de la Haute Autorité de Santé, en la personne du Professeur Jean-Luc Harrousseau, son Président, de Dominique Maigne, son Directeur, et de toutes les personnes qui donnent corps à cette institution. Je remercie le Professeur Lise Rochaix (à nouveau !), François Romaneix et François Meyer d'avoir accepté de se lancer dans l'aventure d'un contrat CIFRE. Enfin, j'ai une dette immense à l'égard de Catherine Rumeau-Pichon qui m'a offert, dans le même temps, ma première expérience professionnelle et la chance de réconcilier mes formations en philosophie et en économie de la santé dans le cadre de cette thèse.

Je dois beaucoup également aux discussions passionnantes que j'ai eu avec mes collèges, amis et Professeurs, en particulier à Cléa Sambuc, Olivier Scemama, Anne-Line Couillerot-Peyrondet, Paul-Loup Weil-Dubuc, Antoine Van den Berg, Adrien Fauve, Pierre Lévy, ainsi qu'avec l'ensemble de mes collègues du Service Evaluation économique et de santé publique de la HAS ; et qui ont de surcroît consacré du temps à lire et commenter les trois chapitres de cette thèse. Je remercie aussi Emmanuelle Blondet et Juliette Chazareng pour leur aide dans mes recherches bibliographiques.

Je remercie mes parents, mes sœurs, Christine et mes amis pour avoir chacun relu attentivement mon travail, ainsi que Maggie Galbraith et Nicolas Sowels.
Enfin, je remercie Armand, de tout mon cœur, de m'avoir accompagné avec ses conseils et sa tendresse.

Sommaire

Introduction générale

«A mesure que se creuse l'écart entre le techniquement possible et l'équitablement accessible, l'éthique consiste de moins en moins à faire ce qu'on peut pour offrir l'éternité à chacun. Elle consiste toujours plus à apprendre à mourir. »

(Van Parjis, 1994, p.7)

«Gouverner c'est choisir ; le Bien commun est construit sur des choix. Accepter la démocratie républicaine c'est faire le choix ensemble que certaines choses peuvent valoir plus que la vie à n'importe quel prix. Le Bien commun n'est-il pas de construire une société juste, qui aura su établir démocratiquement, dans la transparence et avec courage, une hiérarchie et des priorités dans les besoins inhérents à la dignité des hommes, libérés de l'angoisse de la mort. »

(Intervention de Suzanne Rameix, à la Conférence Nationale de la santé, 28 mars 2001, pp.141-42)

A la question que posait Socrate, à la suite de sa condamnation, et qui a donné naissance à la philosophie occidentale, « y-a-t-il des choses qui valent plus que sa propre vie ? »[1], Van Parjis et Rameix s'accordent pour affirmer que la seule chose qui vaut plus que le désir d'éternité pour certains, c'est la justice entre tous face à la maladie[2]. La définition du périmètre des soins remboursables, dans le contexte de systèmes de protection sociale fondés sur la notion de couverture d'assurance maladie universelle, met en jeu en permanence cette interrogation

[1] Lorsque Criton propose à Socrate de fuir de la prison, Socrate lui répond en effet *« le plus important n'est pas de vivre, mais de bien vivre »* avant d'ajouter *« ferons-nous acte de justice en donnant de l'argent à ceux qui nous tirerons d'ici (…) ou bien commettons nous une injustice en faisant tout cela ? Et si nous voyons que ce serait une injustice de le faire, nous n'avons pas à calculer s'il nous faut mourir en restant ici sans bouger ou subir tout autre peine, quand il s'agit d'éviter l'injustice. »*. C'est à l'issue de cet échange que se déroule la célèbre prosopopée des Lois qui accuserait Socrate de vouloir les détruire si d'aventure il en venait à fuir (Platon, Le Criton, 48c, traduction de E. Chambry, pp.71-2).

[2] La notion de « justice » renvoie ici à l'appréciation morale de la répartition des biens entre les membres d'une société. Il est courant en matière de politique de santé d'évoquer la notion d'équité plutôt que celle de justice. L'équité renvoie alors à *« l'absence de différences systémiques et potentiellement remédiables, dans un ou plusieurs aspects de la santé parmi la population qui sont définis socialement, économiquement, démographiquement ou géographiquement. »* (OMS, 2005, in Rochaix L., Tubeuf S., 2007, p.327). Cependant il existe une équivalence entre les notions de justice et d'équité : d'une manière plus générale en effet, l'équité renvoie à la définition du juste et de l'injuste ; *« en tant surtout qu'il se manifeste dans l'appréciation d'un cas concret et particulier »*. (Lalande A., 1926, p.295).

philosophique lorsqu'il s'agit de décider collectivement des interventions de santé que l'on inclut et que l'on exclut de ce périmètre.

En effet, tandis que le progrès technique médical se développe, les bénéfices de santé qu'offrent les interventions thérapeutiques augmentent, si bien que le risque que tous ne puissent pas y accéder équitablement s'aggrave. Ce progrès technique médical favorise la détection précoce des maladies et l'efficacité de leur prise en charge grâce à l'extension de l'arsenal thérapeutique et diagnostique disponible, ce qui *in fine* constitue un facteur d'amélioration de la qualité de vie des individus qui en bénéficient et un facteur d'allongement de l'espérance de vie. Cependant, la montée du chômage en Europe, le ralentissement de la croissance économique et la diminution du pourcentage d'actifs ont un impact sur les recettes des organismes d'assurance maladie (HCAAM, 2010 ; Cornilleau G., et Debrand T., 2011 ; OMS, 2009) [3]. Dans ce contexte, les décideurs publics sont donc conduits à s'interroger sur la juste définition du périmètre des soins remboursables.

En France, comme dans d'autres pays européens, ce périmètre s'est constitué progressivement, *via* des mécanismes d'inscription au remboursement et des outils de régulation de l'accès au panier de soins, au gré de l'évolution de l'offre de soins, des contextes politiques conjoncturels et ceci sous l'influence des professionnels de santé, des associations de patients et des industriels (Ravoux V., 2003)[4]. Il semble dorénavant nécessaire de définir clairement quels sont les critères de sélection des interventions de santé et de s'assurer qu'ils soient cohérents d'un point de vue à la fois économique et éthique[5]. En 2000, le Haut Comité de la Santé Publique soulignait en effet qu'une redéfinition du périmètre des soins remboursables devait reposer sur un double objectif d'efficience et de solidarité : *« Il s'agit pour les responsables de fonder leurs décisions sur une éthique des choix : recherche de l'équité d'accès aux soins, recherche de l'éthique du système qui se rapporte à une conception de la justice sociale, et recherche de l'efficacité et de l'efficience exprimant la capacité d'un*

[3] Sur l'analyse de la part attribuable à l'innovation thérapeutique dans la croissance des dépenses de santé Cf. Huber H. et Dormont (2006). *"Deux mécanismes principaux sont à l'œuvre dans le progrès technique médical : la substitution de traitement, qui permet un gain d'efficacité; la diffusion du traitement, qui correspond à une utilisation croissante de l'innovation. (…) C'est le mécanisme de diffusion qui conduit à une hausse des coûts de la santé : de nouveaux traitements apparaissent continuellement, dont l'usage s'étend plus ou moins rapidement. Pour l'exprimer en termes économiques, de nouveaux biens et services sont offerts et consommés, en plus des biens et services déjà consommés."* (Dormont B., 2009, p.41)

[4] On entend par « périmètre des soins remboursables », la *« liste des services de santé et des biens médicaux faisant l'objet d'une prise en charge par la tutelle et/ou le financement d'un système de santé »* (Haut Comité de Santé publique, 2000, p.5)

[5] L'éthique désigne *« la science ayant pour objet le jugement d'appréciation en tant qu'il s'applique à la distinction du bien et du mal »*. (Lalande A., 1926, p.305).

système de santé à proposer ses produits pour un maximum de satisfaction compte tenu des contraintes économiques» (Haut Comité de Santé publique, 2000, p.2).

Les décideurs publics se heurtent donc désormais à un dilemme moral que Daniels, dans son ouvrage *Just health*, résume en ces termes : *« How can we meet health needs fairly when we cannot meet them all ? More specifically, given moral disagreement about how to meet health needs, how can priority-or limit-setting decisions come to be accepted as fair and legitimate?"* (Daniels N., 2008, pp.3-4). Autrement dit, à quels besoins de santé doit-on répondre quand il est impossible de les satisfaire tous?

Les enjeux que soulève la définition du périmètre des soins remboursables renforcent la nécessité, pour les décideurs publics, d'être en mesure de justifier les choix qui sont effectués. Dans ce contexte, l'évaluation des interventions de santé se développe au sein d'un processus de légitimation des choix collectifs, on parle alors d'institutionnalisation de la démarche évaluative (Benamouzig D., 2005, 2006)[6]. *« L'évaluation apparaît comme un des meilleurs moyens de répondre aux besoins d'information des décideurs qui doivent justifier leurs choix auprès de publics de plus en plus exigeants »* (Contandriopoulos *et al.*, 2000, p.520). Ce phénomène d'institutionnalisation se traduit notamment par la création d'agences nationales d'évaluation des interventions de santé et par l'extension de leurs missions, en particulier en matière d'évaluation économique.

La Grande-Bretagne est le plus souvent désignée comme pionnière avec la création en 1999 du NICE (National Institute for Health and Clinical Excellence) qui dispose dès son origine d'une mission d'évaluation économique. En France, la Haute Autorité de santé, a été créée par la Loi du 13 août 2004 relative à l'assurance maladie, *« afin de contribuer au maintien d'un système de santé solidaire et au renforcement de la qualité des soins, au bénéfice des patients »*[7]. Elle s'est vue confiée depuis 2008 une mission d'évaluation médico-économique[8]. L'institutionnalisation de l'évaluation économique des interventions de santé se développe donc selon des rythmes différents dans les divers pays. On observe toutefois que l'intégration d'un critère économique dans la définition du périmètre des soins se diffuse progressivement. Les pratiques des agences publiques d'évaluation semblent donc converger vers un même

[6] Le terme d' « intervention de santé » désigne toute activité visant à préserver ou améliorer la santé d'une population : procédures chirurgicales ou médicales mobilisant équipements, dispositifs médicaux, médicaments, utilisées dans un objectif de prévention et de soins. Il désigne également les systèmes organisationnels requis pour leur mise en œuvre et, de manière plus générale, l'ensemble des programmes de santé publique.

[7] http://www.has-sante.fr/portail/jcms/c_452559/presentation-de-la-has

[8] Cette mission médico-économique lui est confiée par la Loi de financement de la Sécurité Sociale pour 2008, avant d'être renforcée par la Loi de financement de la Sécurité Sociale pour 2012.

objectif : utiliser le calcul économique pour favoriser une utilisation efficiente des ressources publiques, c'est-à-dire justifier que le périmètre des soins remboursables permet d'obtenir les meilleurs résultats de santé possibles compte tenu de l'enveloppe budgétaire disponible[9].

Le rôle de ces agences publiques d'évaluation est le plus souvent décrit comme étant exclusivement scientifique. D'une part, les avis qu'elles rendent au sujet de l'intérêt d'une prise en charge collective des interventions de santé sont remis au décideur à titre consultatif, ce qui signifie que les décisions finales sont prises par le pouvoir exécutif : le Ministère des Affaires sociales et de la Santé, ainsi que l'Union nationale des caisses d'assurance maladie (UNCAM) en France, le National Health Service (NHS) en Grande-Bretagne ou encore le Gemeinsamer Bundesausschuss[10] en Allemagne (GBA). D'autre part, les agences déclarent préserver le respect d'une neutralité axiologique dans leurs travaux en établissant une distinction entre :

- l' « *assessment* » : phase de l'évaluation consacrée à la mise en lumière des différentes conséquences d'une intervention ;
- l' « *appraisal* » : phase de l'évaluation consacrée à la mise en balance des différentes conséquences de l'intervention en vue de la formulation d'une conclusion ou d'un avis.

Selon ces agences, la première phase relèverait de la stricte évaluation scientifique, tandis que la seconde relèverait davantage de choix politiques, reprenant ainsi la distinction entre les faits (*ce qui est*) et les valeurs (*ce qui doit être*)[11].

Cette distinction ne suffit pourtant pas à prévenir l'immixtion de jugements de valeur normatifs dans les évaluations économiques qui sont réalisées en vue d'aider à la décision publique, notamment dans la phase d'*« assessment »* qui se veut pourtant strictement descriptive. Dans la mesure où les conclusions des évaluations doivent servir à légitimer les décisions publiques qui sont prises sur leur fondement, le choix des critères d'évaluation doit nécessairement être cohérent avec l'objectif du système de santé que visent ces décideurs au nom de la collectivité qu'ils représentent. Si l'efficience désigne une situation dans laquelle un objectif fixé est atteint en utilisant pour cela le moins de ressources possible, y faire référence implique donc qu'un objectif soit défini au préalable. Toute évaluation économique

[9] [9] Sur la place de l'évaluation économique dans les processus de fixation des prix et remboursement des technologies de santé en Europe Cf. Espin, 2009 ; OCDE (2008) ; Dickson M. (2003), Base de données de l'ISPOR (http://www.ispor.org/HTARoadMaps/Default.asp); Lettre du Collège des économistes de la santé, Synthèse du Congrès de l'ISPOR 2008 (2008).

[10] Comité fédéral de l'assurance maladie

[11] Sur la distinction entre ces deux notions : Cf. Hume, *Traité de la nature humaine* (1739-1740); Poincarré, *Dernières pensées* (1913).

suppose donc nécessairement l'adhésion, même implicite, à des valeurs morales et un positionnement sur la question de la vie bonne[12]. *« Les critères permettant d'évaluer le caractère souhaitable des états socio-économiques, et permettant de formuler des objectifs pour la politique économique, ne sont pas des données premières, elles découlent de valeurs morales, ou parfois, de théories de la justice issues de la philosophie morale. »* (Fleurbaey M., 1995, p.3).

L'une des illustrations les plus éclairantes de ces jugements de valeur qui sous-tendent ces choix méthodologiques concerne le choix du critère d'efficacité. Comme nous le verrons plus précisément dans le Chapitre 1, estimer le ratio coût/conséquence implique, pour mesurer et comparer les conséquences des différentes interventions, de choisir un critère. Plusieurs sont envisageables. Il en existe notamment qui se fondent sur des données cliniques ou biologiques et qui renvoient à une conception naturaliste et/ou universaliste de la santé (nombre d'années de vie gagnées, baisse de la pression artérielle, augmentation de capacités fonctionnelles comme l'audition ou la motricité, etc.)[13]. D'autres prennent en compte une valorisation subjective des conséquences par les individus eux-mêmes (QALY ou méthodes d'évaluation contingente). Le choix du critère d'efficacité des interventions de santé met en jeu la caractérisation de l'objet de la redistribution des ressources en santé. *«Tout choix de critères implique une hiérarchisation de valeurs qui ne sont que rarement explicitement définies et discutées lors de la définition des objectifs ou de l'évaluation des procédures.»* (Aymé S., *in* Matillon Y. et Durieux P., 1994, p.88). D'autres exemples de jugements de valeur sous-jacents aux choix méthodologiques pourraient également être évoqués tel que le choix de la perspective de l'évaluation des bénéfices de santé (perspective de la population générale ou perspective des patients) ou encore le choix du degré d'aversion aux inégalités qui est retenu dans l'évaluation[14]. Ces choix méthodologiques dépendent en effet de l'objectif qui est recherché en termes de justice sociale. Ils seront plus précisément examinés dans les Chapitres 2 et 3.

[12] Dans un tel contexte, la notion de « vie bonne » ne renvoie pas à l'idée d'une vie exclusivement orientée vers la recherche de plaisirs (bien que cette thèse hédoniste soit défendue par Calliclès dans *Le Gorgias* de Platon, comme elle le sera plus tard par les utilitaristes classiques), mais elle renvoie à l'idée d'une vie orientée vers le Souverain Bien, lequel peut alors être définit, à la façon d'Aristote, comme la fin en vue de laquelle tous nos actes sont accomplis et qui n'est jamais désirable en vue d'autre chose. (Cf. Aristote, Ethique à Nicomaque, I,1, trad. J. Tricot, Vrin)

[13] L'approche naturaliste considère que la maladie est une déviation par rapport à des normes biostatistiques qui régulent le fonctionnement organique (Schramme T., 2007; Boorse C., 1977). L'approche universaliste tend quand à elle à considérer que les conditions d'une bonne qualité de vie sont identiques pour tous.

[14] Sachant que ne pas intégrer de degré d'aversion aux inégalités consiste à intégrer un degré de 0 ce qui constitue également d'un choix normatif.

Dans l'attente d'un examen plus précis des controverses éthiques qui jalonnent l'évaluation économique des interventions de santé, nous nous contentons donc, dans le cadre de cette introduction générale, d'avancer l'hypothèse selon laquelle l'évaluation économique ne permet pas de se soustraire à un choix en matière de principe de justice sociale. Au contraire, elle y est subordonnée. C'est ce que souligne Amartya Sen lorsqu'il réfute l'opposition entre éthique et économie et qu'il rappelle que le rôle de l'économiste est d'apporter au décideur public des réponses sur la mise en œuvre concrète d'une justice sociale en matière de répartition des richesses.

> *« L'étude de l'économie, bien que liée de façon immédiate à la quête de la richesse, est en liaison, à un niveau plus profond, avec d'autres études, qui consistent à évaluer et à promouvoir des buts plus fondamentaux.* "Quant à la vie de l'homme d'affaires, c'est une vie de contrainte, et la richesse n'est évidemment pas le bien que nous cherchons : c'est seulement une chose utile, un moyen en vue d'autre chose." (Aristote, *Ethique à Nicomaque*, I, 1–I, 5c)[15] *L'économie, en définitive, se ramène à l'étude de l'éthique et de la politique, thèse qu'Aristote approfondit dans sa Politique. Rien de tout ceci ne justifie de dissocier l'étude de l'économie d'une part et l'étude de l'éthique et de la philosophie d'autre part. (…) L'évaluation doit davantage être centrée sur l'éthique et adopter une conception plus large du "bien" »* (Sen A., 2003, p.7).

De même, Walliser précise que les modèles économiques s'appuient sur des critères d'évaluation établis à partir de normes collectives suggérées par la philosophie morale et politique qui dictent l'objectif de justice sociale souhaité (Walliser B., 1994).

Problématique

Il est particulièrement important de souligner la relation qui existe entre l'évaluation économique et les principes éthiques en matière de justice sociale, et ce pour deux raisons. D'une part, être explicite sur l'existence et sur la nature des choix normatifs qui sont effectués en amont de l'évaluation permet d'éviter qu'il soit reproché à l'évaluation économique *« d'embarquer clandestinement des jugements de valeur »* (Benamouzig, 2005, p.345), voire qu'il lui soit reproché de dissimuler derrière une évaluation prétendument

[15] Traduction fançaise de J. Tricot, Paris Vrin, 1959

scientifique, des décisions politiques[16]. D'autre part, la mise en lumière de ce lien constitue le pré-requis de tout travail visant à améliorer la cohérence entre les principes de justice sociale que les décideurs souhaitent appliquer et les méthodes d'évaluation qui sont utilisées en pratique. Nous considérons en effet ici que le calcul économique ne présuppose pas le choix d'un modèle de justice sociale particulier. Au contraire, il est possible d'adapter l'évaluation économique à l'ensemble des modèles de justice sociale envisageables pourvu qu'ils soient clairement identifiés. Pour cette raison, l'évaluation économique reste une discipline positive et non normative : si elle implique qu'un choix soit effectué en matière de jugement de valeur sociale, elle ne limite pas le champ des choix possibles. L'économiste adopte effectivement une position positive lorsqu'il formule ses conclusions de la façon suivante : *« si les valeurs et/ou théories philosophiques retenues sont…, alors les critères et objectifs les plus fidèles à ces valeurs et/ou théories sont… »* (Fleurbaey M., 1995, p.3).

Il est vrai que l'évaluation économique des interventions de santé s'inscrit historiquement dans une tradition utilitariste. Elle vise en effet le plus souvent à évaluer les différentes interventions par rapport à un objectif de maximisation de la production de gains en santé dans la population et elle s'appuie sur les préférences des individus pour juger la valeur intrinsèque de ces gains en santé (Cf. *infra* Chapitre I). « *L'économie normative s'est développée pendant les deux derniers siècles essentiellement à partir de l'utilitarisme, puis de son héritier contemporain l'économie du bien-être parétienne. Les principaux concepts étudiés sont ceux de l'utilité, du bien-être, du bonheur, etc. L'ambition était de chercher des techniques de mesures, des procédures de comparaison, des méthodes d'agrégation.* » (Clément V. *et al.*, 2008). L'éthique utilitariste privilégie alors *« le plus grand bonheur du plus grand nombre »* et impose de considérer que l'amélioration du bien-être de chaque individu a une valeur identique quelle que soit sa situation personnelle - chacun compte pour un et pas pour plus d'un. Selon elle, la légitimité et la moralité d'un acte doivent être évaluées en fonction de la somme totale des satisfactions individuelles (appelées « utilités ») (Gamel C., 2005). Appliquée à la santé, cette maxime renvoie à l'idée qu'une juste répartition des

[16] *« Des décisions politiques risquent d'être prises comme si elles étaient des décisions techniques, non par des responsables politiques ou des instances paritaires, mais par des comités d'experts ou des personnalités ne rendant compte qu'à leur propres compétences. (…) Qu'on la considère du point de vue de l'administration, de la médecine ou de la science, la Haute Autorité de Santé se trouve dans une situation telle que le risque de prises de décisions politiques au nom de la science est palpable. Apparaissant comme l'expression contemporaine d'un ensemble de processus plus anciens, cette situation pourrait rapidement révéler les propriétés des bureaucraties techniques que l'évolution paradoxale des agences laissait depuis quelques temps pressentir. »* (Benamouzig D., Besançon, J., 2005, pp.319-20)

ressources est une répartition qui permet de maximiser la production de « gains de santé » au niveau collectif (Cookson R. et Dolan P., 2000).

Toutefois, la thèse qui est soutenue dans le cadre de ce travail est qu'il est possible de mener des évaluations économiques des interventions de santé fondées sur d'autres modèles de justice sociale et nous nous intéresserons en particulier à la façon de prendre en compte les développements récents en philosophie politique auxquels a donné lieu la parution de la *Théorie de la justice* de Rawls en 1971.

Tandis que l'éthique utilitariste recommande de répartir les ressources publiques de telle sorte qu'elles permettent de maximiser la quantité de bien-être, il convient selon Rawls de prendre en compte la répartition de ces ressources entre les membres de la collectivité. Ainsi la répartition des ressources publiques est considérée comme juste lorsqu'elle permet d'améliorer au maximum le sort des individus les plus défavorisés, même si cela conduit à diminuer la production globale de bien-être au niveau collectif. Il précise par ailleurs qu'une société est juste lorsque les libertés fondamentales des individus sont respectées[17]. Ces principes de justice sont définis par Rawls sur la base d'un consensus virtuel qui aurait été obtenu en plaçant les individus dans une situation originelle, dite « sous voile d'ignorance», c'est-à-dire qu'ils ignorent quelle pourrait être leur situation particulière (leurs capacités naturelles et leur position à l'intérieur de la société). Rawls en déduit alors une liste des biens primaires sociaux qui correspondent aux biens que toute personne désire rationnellement quels que soient ses désirs et ses goûts particuliers, et dont toute personne doit pouvoir bénéficier pour être réellement libre de faire des choix et d'accomplir son projet de vie. Selon lui, il est impératif de garantir la juste répartition de ces biens premiers sociaux, même si cela conduit à diminuer la production globale de bien-être au niveau collectif.

S'il est vrai que Rawls ne se prononce pas sur la place de la santé parmi cette liste des biens premiers sociaux, plusieurs auteurs se sont interrogés explicitement sur les conditions de politiques de santé équitables, en se référant aux principes de justice rawlsiens. D'une part, s'il est vrai que le bien-être qu'un individu tire d'une amélioration de son état de santé peut entrer en concurrence avec d'autres types de consommation (loisirs, éducation, modes de vie pathogènes, etc.), la santé constitue toutefois un bien particulier dans la mesure où elle est également la condition de possibilité pour jouir de tous les autres biens : au-delà d'un certain

[17] Le droit et les libertés fondamentales ; la liberté de mouvement et le libre choix d'une position dans un contexte d'égalité des chances ; les pouvoirs et prérogatives attachés aux différentes fonctions dans les institutions ; les revenus et la richesse ; les bases sociales du respect de soi (Rawls, 1990).

seuil de douleur ou d'incapacité, l'individu n'est plus en mesure de désirer autre chose que le soulagement ou la guérison, le cas extrême étant la mort. Promouvoir une répartition équitable des gains en santé dans la population serait donc justifié dans la perspective de toute théorie de la justice fondée sur une égalisation des chances de bien-être (Roemer J., 1985 ; Cohen G. A., 1989, et dans une moindre mesure Sen A., 1987, 1999; Cf. Fleurbaey M., 1995 ; 1996).

De surcroît, la santé peut également être considérée comme un bien particulier en ce qu'elle est la condition de possibilité des opportunités de fonctionnements, dont Sen recommande la juste répartition dans la population. Comme Rawls, Sen propose de centrer l'analyse économique non plus sur les résultats d'une intervention en termes de satisfactions individuelles (ou utilités) mais sur ce qu'elle représente pour l'individu en tant que moyen d'améliorer le champ de ses possibilités. Néanmoins, à la différence de Rawls, Sen privilégie la notion de capabilités plutôt que de biens premiers. Tandis que les biens premiers sont des moyens d'ordre général et donc également nécessaires pour chacun (libertés, revenu, éducation, bases sociales du respect de soi, etc.), les capabilités ne peuvent pas être définies *a priori* car elles varient en fonction du contexte dans lequel s'insère chaque individu. Si Sen ne dresse pas de liste exhaustive de ces capabilités, Nussbaum en recense onze, parmi lesquelles l'état de santé des individus occupe en effet une place centrale *« (i) Pouvoir vivre autant que possible une vie humaine complète jusqu'à la fin (…) ; (ii) Pouvoir jouir d'une bonne santé, d'une alimentation adéquate, d'un foyer décent, avoir des opportunités de satisfaction sexuelle ; pouvoir se déplacer d'un endroit à un autre (…); (iii) Pouvoir éviter toute douleur inutile et connaître l'expérience du plaisir ; (iv) Pouvoir utiliser nos cinq sens ; pouvoir imaginer, penser et raisonner*» (Nussbaum M., 2008, pp. 120-123).

Enfin, Daniels souligne qu'il est justifié de mobiliser les principes généraux énoncés par Rawls, dans le cadre de sa théorie de la justice comme équité, pour guider les choix publics en matière d'interventions de santé, dans la mesure où des études épidémiologiques ont montré une corrélation entre les inégalités de santé et les caractéristiques socio-économiques des individus telles que le revenu ou l'éducation[18]. Les inégalités de santé peuvent donc être qualifiées d' « injustes », dans la perspective de la *Théorie de la justice comme équité,* car

[18] N. Daniel cite à ce propos, entre autres, les études de Michael Marmot, (*The status Syndrome: How Social Standing Affects Our Health Longevity*, New York, Time Books, 2004), celles de Margaret Whithead, Peter Townsend et Nick Davidson (*Inequalities in Health : The black Report ; The health Divide*, Londres, Penguin Group, 1988) et celles de Goerges Davey-Smith, Martin J. Schipley, Geoffrey Rose, ("*Magnitude and causes of Socioéconomic Differentials in Morality: Further Evidence from Whitehall Study*", Journal of Epidemiology an Community Helath, vol.44, 1990).

elles sont le fruit d'inégalités économiques dont Rawls soutenait qu'elles devaient être réduites à leur minimum (Daniels N., 2008).

L'enjeu que soulève la définition du périmètre des soins remboursables est donc double : il est politique d'un côté et scientifique de l'autre.

- D'un point de vue politique, il est en effet nécessaire d'expliciter les choix normatifs concernant les principes de répartition des ressources en santé et de s'assurer de la légitimité de ces choix. Une réflexion devrait donc être menée sur l'élaboration d'espaces et de processus de délibération adéquats. Les propositions de Daniels sur la justice procédurale appliquée aux politiques de santé constitueraient assurément un point de départ essentiel pour ces réflexions. Daniels s'appuie en effet sur les propositions de Rawls au sujet des délibérations démocratiques pour établir une liste de critères permettant de juger la légitimité de décisions publiques en matière d'allocation des ressources en santé. Pour être justes, les décisions doivent être raisonnables, c'est-à-dire qu'elles doivent répondre aux conditions de publicité, de pertinence, d'appel et de révision et à la condition régulatrice (Daniels, 2008).
- D'un point de vue scientifique, il est nécessaire de développer des méthodes d'évaluation qui permettent de s'adapter à ces choix, de telle sorte que l'évaluateur puisse mettre à disposition de la délibération des informations cohérentes avec les principes de justice qui sont recherchés par la collectivité.

Le présent travail s'inscrit dans le cadre de cet enjeu scientifique et il vise plus particulièrement à s'interroger sur la faisabilité des méthodes qui ont été récemment développés pour pendre en compte les principes égalitaristes dans l'évaluation des interventions de santé, dans un contexte de procédure de fixation de prix et d'admission au remboursement des produits de santé. Hausman, par exemple propose une méthode alternative à celle utilisée dans le cadre de l'évaluation coût/efficacité traditionnelle pour valoriser les gains en santé de façon à prendre en compte l'impact de l'intervention sur les opportunités offertes à l'individu qui va en bénéficier (Hausman D., 2009). De même, Fleurbaey a développé le concept de revenu équivalent-santé qui permet d'introduire dans l'évaluation coût/bénéfice traditionnelle une pondération des résultats en fonction du degré d'aversion aux inégalités que partage la collectivité (Fleurbaey M., 2005, Fleurbaey *et al.*, 2009). Ces méthodes proposent toutes deux d'introduire les principes développés par Rawls dans l'évaluation économique des interventions de santé mais elles s'opposent sur plusieurs points, en particulier sur la place accordée aux préférences individuelles. *« Il n'est pas*

pertinent de toujours séparer la question de la définition de la justice et celle de la mise en œuvre des états justes. La faisabilité est une valeur pratique, qui doit être prise en compte dans la réflexion éthique en vue de garantir l'opérationnalité du modèle décisionnel » (Fleurbaey M., 1996, p.11).

Plus précisément, il s'agira au fil des trois chapitres qui constituent cette thèse, d'examiner quels sont les principes de justice sociale qui sous-tendent les méthodes actuellement utilisées en évaluation économique dans l'aide à la décision publique en santé et d'envisager, au moyen de cas concrets, comment l'évaluation économique est en mesure de s'adapter aux autres principes qui pourraient également être retenus. Nous nous appuierons pour cela sur les travaux théoriques qui sont actuellement menés en économie normative et sur les travaux plus pratiques qui s'intéressent à leur application dans l'évaluation économique des interventions de santé.

Plan

Le premier chapitre de cette thèse propose une revue des méthodes d'évaluation économique auxquelles ont recours trois grandes agences d'évaluation des technologies de santé en Europe (NICE, IQWIG, KCE) afin d'identifier les positions en matière de justice sociale qui en découlent. La méthode de travail s'appuie sur une mise en perspective des guides publiés par les agences avec une grille d'analyse élaborée à partir de la littérature sur les théories économiques de la justice sociale.

Cette grille d'analyse s'articule autour de deux axes :

- Comparaison des choix des agences en matière de critères de définition des gains en santé
- Comparaison des choix des agences en matière de critères de répartition des gains en santé dans la population

Ce premier chapitre constitue une étape liminaire importante pour la suite de la thèse parce qu'il permet d'examiner dans quelle mesure les agences s'écartent du modèle utilitariste traditionnel qui constitue pourtant le modèle de justice sociale sur lequel sont fondées les méthodes classiques d'évaluation économique. Ce constat justifie donc d'étudier dans les chapitres II et III, comment les travaux qui sont menés en économie du bien-être et qui prennent en compte d'autres modèles normatifs que l'utilitarisme sont applicables à l'évaluation des interventions de santé.

Dans le cadre du deuxième chapitre, nous analysons les dilemmes moraux auxquels se heurtent les économistes chargés de mener l'évaluation de deux technologies de compensation du handicap : l'hormone de croissance chez les enfants non-déficitaires présentant une petite taille à la naissance et les implants cochléaires bilatéraux chez les enfants présentant une surdité profonde à la naissance.

L'objectif est d'observer dans quelle mesure les phénomènes d'adaptation des préférences forcent les économistes à rouvrir le débat sur le choix de la perspective de l'évaluation et dans quelle mesure ce débat ne peut être tranché sans prendre position à l'égard des principes de justice sociale. Trois options sont ensuite proposées pour prendre en compte ces phénomènes d'adaptation au moyen de méthodes d'évaluation qui soient cohérentes avec les principes de justice retenus par les décideurs. Elles s'inscrivent toutes les trois dans le cadre des théories égalitaristes de la justice sociale. Nous verrons en particulier que chacune de ces options implique des bases informationnelles différentes[19].

Ce deuxième chapitre permet de montrer comment certains problèmes concrets rencontrés dans l'évaluation des interventions de santé peuvent être levés en mettant en perspective les travaux menés en économie de la santé et les réflexions sur les théories de la justice sociale. La mise en œuvre d'un dialogue entre éthique et économie, en amont de l'évaluation, permet en effet de redéfinir le choix des critères qui sont retenus dans l'évaluation (bien-être subjectif, opportunité de fonctionnement ou réalisations sociales fondamentales), le choix de la perspective (patients vs population générale) et le périmètre de l'évaluation (interventions médicales et extra-médicales).

Enfin, dans le cadre du troisième chapitre, il est proposé de tester l'utilisation du concept de revenu équivalent-santé développé par Fleurbaey (2005) dans l'évaluation des traitements antihypertenseurs. L'objectif de cette approche est d'introduire différents degrés d'aversion aux inégalités dans l'évaluation des interventions de santé de façon à prendre en compte des principes égalitaristes en matière de répartition des ressources en santé. Il s'agit ici de démontrer la faisabilité de cette démarche dans le contexte de l'aide à la décision publique en

[19] *« Toute démarche d'évaluation se caractérise par sa base d'information, c'est-à-dire par l'ensemble des informations dont il est nécessaire de disposer pour formuler un jugement conforme à cette démarche, mais aussi, et ce n'est pas moins important, par l'ensemble des informations* exclues *de l'évaluation directe. (…) De fait, pour isoler le « principe actif » d'une théorie de la justice, il suffit de se pencher sur sa base d'informations et de voir quels éléments celle-ci intègre ou exclut. »* (Sen A., 2003, p.81-82)

santé et ceci compte-tenu des conditions réelles auxquelles elle est soumise, notamment en termes de disponibilité d'information.

Cette évaluation repose sur l'estimation de fonctions de bien-être social permettant de comparer les conséquences des interventions de santé qui sont évaluées de façon extensive. Ces conséquences sont pondérées en fonction de la situation des individus qui les supportent de manière à valoriser les interventions qui permettent d'améliorer la situation des individus les plus défavorisés. Quatre degrés d'aversion aux inégalités sont inclus dans le calcul de façon à laisser au décideur la responsabilité du choix normatif que cela représente. Il s'appuie sur une méthode empirique qui a été élaborée au sein d'une équipe réunissant plusieurs économistes [20] . L'intérêt de ce troisième chapitre est de démontrer que l'évaluation économique est en mesure de s'adapter aux différents modèles de justice sociale qui sont recherchés par la collectivité et qu'elle n'est pas remise en question par le rejet du modèle utilitariste traditionnel. Les données qui ont servi à élaborer le modèle d'évaluation des traitements antihypertenseurs par l'approche revenu équivalent-santé sont issues :

- d'une enquête menée sur 3330 individus interrogés sur leur état de santé, leur revenu et sur leurs préférences en matière d'arbitrage revenu/santé ; cette enquête était financée par la Chaire Santé de l'Université Paris Dauphine ;
- d'un modèle coût/efficacité sur les traitements antihypertenseurs en primo-prescription réalisé par la HAS et externalisé auprès d'IMS Health.

Le travail qui est présenté dans le cadre de ce troisième chapitre a bénéficié du soutien méthodologique des personnes impliquées dans la réalisation du modèle coût/efficacité de la HAS[21].

Nous avons choisi dans cette thèse de partir d'exemples particuliers d'évaluations économiques des interventions de santé qui sont réalisées dans le cadre de l'aide à la décision publique. L'objectif est à la fois d'étudier les implications philosophiques des choix méthodologiques qu'elles sous-tendent et d'examiner comment les réflexions récentes menées

[20] Les co-auteurs de ce travail sont les suivants : Brigitte Dormont (LEDa-Legos, Université Paris Dauphine), Marc Fleurbaey (Woodrow Wilson School, Princeton University), Stéphane Luchini (CNRS, GREQAM), Anne-Laure Samson (LEDa-Legos, Université Paris Dauphine), Erik Schokkaert (CORE, Université de Louvain-la-Neuve), Clémence Thébaut (LEDa-Legos, Université Paris Dauphine, Haute Autorité de Santé), Carine Van de Voorde (Katholieke Universiteit Leuven).

[21] Ont participé à l'élaboration du modèle de la HAS : Aikaterini Vellopoulou, MSc (IMS), Laetitia Gerlier, MSc (IMS), Frédérique Maurel, MSc (IMS), Fabienne Midy (HAS), Emmanuelle Cohn-Zanchetta (HAS), Anne-Line Couillerot-Peyrondet (HAS), Catherine Rumeau-Pichon (HAS), Dr Olivier Scemama (HAS), Anne d'Andon (HAS), Emmanuelle Blondet (HAS), Maud Lefevre (HAS) et Frédérique Pagès (HAS), Dr Michel Lièvre (pharmacologue méthodologiste, Lyon) ainsi que l'ensemble des experts du groupe de travail et du groupe de lecture.

en économie du bien-être pouvaient permettre d'améliorer les méthodes de façon à ce qu'elles soient plus adaptées aux principes de justice sociale que visent les décideurs au nom de la collectivité qu'ils représentent. Par conséquent nous n'identifierons pas, dans cette thèse, l'ensemble des controverses philosophiques que peuvent rencontrer les évaluateurs au moyen d'une démarche systématique et descendante. Notre objectif est plutôt de mettre en œuvre de façon concrète le dialogue entre économie et éthique au sujet de l'évaluation des interventions de santé, dont Sen souligne, dans son essai intitulé *« On Ethics and Economics »*, la nécessité. Nous verrons toutefois que le travail qui est réalisé sur « ces cas pratiques » nous permet de tirer des enseignements dont la portée dépasse leur strict périmètre et dont nous effectuerons un recensement dans le cadre de la conclusion générale.

Chapitre 1 :

*Méthodes d'évaluation économique de trois agences publiques d'évaluation (NICE, IQWIG ET KCE) : Quels fondements en matière de justice sociale ?**

* *Cet article a été accepté pour publication dans la revue d'*Economie publique.

1. Introduction

Le périmètre des soins remboursables s'est constitué, en France et plus généralement en Europe, de façon progressive et conjoncturelle sans que les critères d'inclusion n'aient été clairement explicités. Compte tenu du fait que l'augmentation des dépenses des systèmes d'assurance maladie menace leur viabilité économique, la nécessité d'effectuer des choix en matière d'allocation des ressources en santé s'impose désormais. Il convient donc aujourd'hui de redéfinir les contours de ce périmètre des soins remboursables de façon cohérente avec les contraintes budgétaires.

Cette redéfinition soulève d'importantes questions de justice sociale. La décision qui est prise d'inclure une intervention de santé dans ce périmètre détermine la possibilité pour l'ensemble de la population d'en bénéficier en fonction de ses besoins. Dans le cas contraire, l'accès à cette intervention est limité en fonction des moyens financiers de chaque individu. Ce processus conduit immanquablement à s'interroger sur les critères permettant de définir dans quelle mesure l'accès à l'intervention, sans conditions de ressources, relève d'un principe de justice. Dans la plupart des pays européens, cette évolution s'est accompagnée de la décision de confier aux agences d'évaluation des interventions de santé une mission d'évaluation économique. L'objet de cet article est de souligner que les choix méthodologiques que ces dernières ont effectués pour mettre en œuvre leurs évaluations ne sont pas neutres et qu'ils sont conditionnés par des partis-pris éthiques, délibérés ou non.

L'évaluation économique s'inscrit par principe dans le cadre d'une morale publique de type conséquentialiste[22]. Elle consiste à mesurer les ratios coût/conséquence qui sont associés aux différentes alternatives envisageables. Il s'agit ensuite, grâce à ces ratios, d'identifier les choix qui permettent d'atteindre un objectif donné au moindre coût et de mettre en exergue les ressources minimales que la collectivité doit sacrifier pour atteindre cet objectif. Cette évaluation doit alors servir à justifier que chaque euro dépensé par la collectivité permet d'obtenir les meilleurs résultats possibles.

Néanmoins pour que les conclusions de l'évaluation puissent légitimer les décisions publiques, les critères de cette évaluation doivent logiquement être conformes au modèle de justice sociale qui est recherché par la collectivité[23]. Pour cette raison, il semble légitime de considérer que les économistes sont insidieusement tenus, par le truchement de choix méthodologiques en matière de critères d'évaluation, de prendre position sur des controverses philosophiques majeures, dès lors que différents modèles de justice sociale sont envisageables et ceci à défaut de pouvoir se ranger derrière une doctrine qui soit clairement assumée par les décideurs publics. De tels enjeux philosophiques ne sont pas seulement théoriques : ils influent les résultats des évaluations qui, à leur tour, ont un impact sur les décisions de remboursement et donc sur la répartition effective des gains en santé dans la population.

Au préalable de cette analyse, il convient de souligner qu'en aucun cas il n'est question ici d'affirmer que les agences ont défini leurs méthodes d'évaluation économique à partir d'une conception explicite et cohérente de la justice sociale en matière de répartition des ressources en santé. Il s'agit seulement de mettre en lumière les conséquences philosophiques de choix méthodologiques, qui à première vue, semblent scientifiques et neutres. Il est plus que jamais nécessaire de porter ces questions au sein du débat public en France, compte tenu du renforcement de la mission d'évaluation économique qui a été confiée à la Haute Autorité de

[22] A l'inverse de la morale déontologique qui juge la moralité de l'action à partir d'un principe ou d'une valeur qu'il convient de respecter, la morale conséquentialiste considère qu'une action est juste en fonction de ses conséquences. Nous pourrions à ce sujet évoquer la distinction entre l'éthique de responsabilité, qui selon Weber est le fait d'assumer la conséquence de ses actes à la différence de l'éthique de conviction qui permet de justifier les moyens par le résultat. Dans ce contexte l'éthique de responsabilité relève d'une position déontologique et l'éthique de conviction renvoie, quant à elle à une position conséquentialiste (Weber, N. 1958).

[23] *« Le rôle de l'évaluateur est de réaliser une évaluation cohérente de telle sorte qu'elle puisse servir au débat politique plutôt que de chercher à remplacer le processus démocratique lui-même. Le problème de l'évaluation économique est le suivant : juger les intérêts individuels dans la perspective d'une conception politique particulière ou d'un échantillon de conceptions politiques (proposant chacune une fonction de bien-être social), et non de synthétiser les conceptions politiques des citoyens au sein d'une doctrine prétendument « collective » ».* (Fleurbaey M. *et al.*, 2007, p.6, traduction non officielle) ; *« Le Siècle des Lumières, quant à lui, marque l'avènement de la raison rationnelle. (…) l'aménagement des conditions économiques se présente comme l'un des buts à atteindre. Cet objectif, parfaitement réalisable, doit s'appuyer sur un critère ou une norme permettant d'évaluer les états sociaux. Les conseils, que les économistes se veulent capables de donner, doivent donc se référer à une véritable conception du bien et du juste. »* (Leroux A., Marciano A., 1998, p.47)

Santé (l'agence française chargée de l'évaluation des interventions de santé) par la loi de financement de la sécurité sociale pour 2012 et au vu des choix méthodologiques que cette dernière doit effectuer en conséquence (HAS, 2011).

2. Méthode

Une revue de la littérature a été réalisée pour identifier les recommandations méthodologiques d'agences publiques d'évaluation des interventions de santé appartenant au réseau *European Network for Health Technology Assesment* (EUnetHTA) et publiées en langue anglaise. Les guides des trois agences suivantes ont été sélectionnés :

- le *National Institute for Health and Clinical Excellence* (NICE) en Angleterre et au Pays de Galles ;
- l'*Institute für Quälitate und Wirtschaftlichkeit im Gesundheitswesen* (IQWiG) en Allemagne ;
- le *Centre Fédéral d'expertise des soins en santé* (KCE) en Belgique.

Une grille d'analyse est utilisée pour comparer ces recommandations méthodologiques. Elle repose sur une typologie permettant de comparer les différents modèles de justice sociale en matière de répartition des ressources en santé. Cette typologie a été élaborée à partir de la littérature sur les théories économiques de la justice sociale et sur les réflexions menées dans le cadre de la philosophie politique et morale. La difficulté réside dans la transposition des réflexions générales sur la justice à la question plus spécifique de la répartition des ressources en santé. Pour faire face à cette difficulté, la littérature qui traite des implications éthiques de l'évaluation économique en santé a été mobilisée (Power M., Faden R., 2000 ; Wagstaff A., 1991 ; Moatti J-P. *et al*., 1995 ; Le Pen C., 1996 ; Mooney G., 1998 ; Schneider-Bunnier C., 1998 ; Fleurbaey M. *et al*., 2007, etc.). Enfin une revue de la littérature a été effectuée afin d'identifier les critiques portées par les économistes de la santé aux recommandations méthodologiques des agences, et en particulier celles du NICE (Birch S., Gafni A., 2007 ; Gafni A., Birch S., 2006 ; Schlander M., 2008 ; Drummond M., 2007 ; Gold M. et Bryan S., 2007 ; Culyer A., 2006, etc.).

La démarche adoptée ici pour analyser les méthodes d'évaluation économique des agences est positive et non normative : il ne s'agit pas de juger les recommandations méthodologiques des agences mais d'examiner comment chacune propose de concilier les intérêts particuliers des malades et celui de la collectivité dans son ensemble comme en témoigne leur utilisation du

calcul économique[24]. Notre intention n'est pas ici de confronter les valeurs qui sous-tendent les différents modèles développés par les agences mais de les expliciter et de tenter d'en percevoir la cohérence.

3. Typologie des modèles de justice sociale en matière de répartition des ressources en santé

3.1. Articulation principale de la typologie

Il est proposé d'articuler la typologie des modèles de justice sociale en matière de répartition des ressources en santé autour d'une distinction (relevée par trois auteurs) entre la définition de l'objet qu'il s'agit de répartir de façon équitable dans la population (ex. bien-être, niveau de revenu, avantages socio-économiques, etc.) et les critères de cette répartition (Van Parijs, Sen et Fleurbaey). Les trois auteurs soulignent pareillement que les critères utilisés pour définir le bien qu'il convient de répartir dans la population et les critères de cette répartition sont relativement indépendants.

Van Parijs distingue ainsi le *distribuendum* et le critère de répartition de ce *distribuendum*.

> *« La justice va ici consister à distribuer d'une certaine manière – c'est le critère de répartition – une variable dont la distribution interindividuelle importe directement (pas seulement au titre d'indicateur ou de facteur causal) – c'est le* distribuendum. *Les nombreuses variantes du libéralisme solidariste diffèrent les unes des autres par les choix essentiellement indépendants l'un de l'autre, du* distribuendum *et du critère.»* (Van Parijs P., 1991, p.251).

En d'autres termes, cette distinction est également soulignée par Fleurbaey au sujet des attributs de la répartition et du critère de répartition.

> *« On peut disséquer à l'infini les différences formelles entre les solutions mais cela est d'un intérêt limité. Il est plus important de comprendre les différences essentielles de contenu entre les solutions. Ces différences essentielles portent sur la description jugée pertinente des* situations *individuelles, d'une part, et sur la manière d'*agréger

[24] *« Le véritable débat n'oppose pas l'éthique économique, d'une part à l'éthique médicale, d'autre part. Il oppose plutôt les différentes conceptions de la justice sociale et les approches contrastées qu'elles proposent pour réconcilier les intérêts individuels et les préférences en matière de biens collectifs et de bien-être. »* (Moatti J-P., 1999, traduction non officielle, p.154)

les variables individuelles pour en tirer un jugement global sur les états sociaux, d'autre part. » (Fleurbaey M., 1996, p. 15)

En outre, cette articulation avait déjà été proposée à plusieurs reprises par Sen.

« Les théories substantielles qui constituent le fondement de jugements éthiques portés sur la vie des individus reposent sur une base d'information composée schématiquement de deux types d'informations intrinsèquement pertinentes : 1/les caractéristiques personnelles centrales, *et 2/ les* caractéristiques combinatoires. *Ainsi, les seules caractéristiques personnelles centrales jugées intrinsèquement importantes par la théorie utilitariste classique sont* les utilités individuelles, *et la seule caractéristique combinatoire qu'elle recommande est la* sommation, *qui donne le total des utilités.»* (Sen A., 1993, p.216)

Cette distinction semble tout à fait utile pour classer les modèles de justice sociale en matière de répartition des ressources en santé. D'une part, l'objet de la répartition - en l'occurrence l'amélioration de l'état de santé que nous appelons « gains en santé » - peut être appréhendé de différentes façons : en termes d'années de vie sauvées, d'amélioration de la qualité de vie, d'amélioration de certains indicateurs cliniques (facteurs de risque, prévalences de maladies, cas dépistés) ou, plus généralement en termes de bien-être. D'autre part, plusieurs principes de répartition des gains en santé dans la population sont envisageables. Certes la santé ne peut pas être répartie en tant que telle entre les individus de la même façon que l'on peut répartir les revenus : elle est une caractéristique inhérente aux individus et ne peut pas être transférée de l'un à l'autre, entre « pauvres » et « riches » en santé. En revanche, les gains en santé peuvent être répartis différemment dans la population dès lors qu'ils résultent de la mise en œuvre d'interventions de santé financées collectivement. Ces interventions constituent des biens privés exclusifs[25]: la décision de financer collectivement une intervention implique que certains individus bénéficient de gains en santé, au détriment d'autres individus qui auraient pu bénéficier de ces mêmes ressources pour un usage alternatif. Il est donc nécessaire de s'appuyer sur un principe de justice en matière de répartition de ces gains en santé pour pouvoir justifier que la collectivité consacre davantage de ressources à ces individus.

[25] A l'exception des certaines interventions telles que les campagnes de vaccination et de prévention.

3.2. Articulation secondaire de la typologie

Pour distinguer les différents critères de mesure des gains en santé, il est possible de s'appuyer sur la classification des théories économiques de la justice sociale proposée par Fleurbaey qui différencie les objets de répartition décrits par l'emploi d'attributs objectifs et ceux décrits par l'emploi d'attributs subjectifs.

> *« Un première famille de solutions se concentre sur les attributs subjectifs, au premier chef l'utilité ou satisfaction. (…) Une seconde famille de solutions ne retient que certains attributs objectifs. Ces attributs peuvent être la jouissance des droits, comme les libertés de base ou le droit de propriété ; des consommations de biens et de services (y compris loisirs) ; ou de réalisations jugées fondamentales (par exemple la santé). »* (Fleurbaey M., 1996, p.15-16)

Distinguer ces deux catégories est utile pour classer les critères de définition des gains en santé. Cela permet de mettre en évidence l'écart qui existe entre des critères de mesure de l'amélioration de l'état de santé qui relèvent d'une conception de la santé comme pouvant être décrite objectivement et ceux qui s'appuient sur la perception subjective qu'ont les individus de leur santé.

Enfin, on peut également s'appuyer sur les travaux de ces trois auteurs pour distinguer les différents critères de répartition des gains en santé dans la population. Van Parijs, Sen et Fleurbaey distinguent chacun à leur manière les critères de répartition dans la population selon qu'ils sont de type agrégatif ou de type prioritariste. Van Parijs oppose les principes agrégatifs, qui ont comme objectif de maximiser la somme ou la moyenne de la variable choisie comme *distribuendum*, aux principes distributifs qui cherchent au contraire à minimiser la dispersion interindividuelle du *distribuendum*. Fleurbaey distingue les critères de répartition qui reposent sur une sommation directe des résultats et ceux qui recherchent leur égalisation. Sen évoque également la notion de sommation qu'il différencie de celle de maximin, où cette dernière consiste à juger la qualité d'une situation en fonction des ressources dont bénéficie la personne la plus mal lotie (Sen A., 1993).

Pour résumer, la typologie élaborée est la suivante :

I.	**Les modèles de justice sociale varient en fonction des critères de mesure des gains en santé**
	1. Critères objectifs de mesure des gains en santé
	2. Critères subjectifs de mesure des gains en santé
II.	**Les modèles de justice sociale varient en fonction des critères de répartition des gains en santé**
	1. Critères de répartition de type agrégatif
	2. Critères de répartition de type prioritariste

En utilisant à cette typologie, nous distinguons d'emblée deux dimensions du modèle de justice sociale dite « utilitariste »:

- la dimension « préférentialiste » qui concerne le critère de mesure du gain qui est distribué dans la population
- la dimension « maximisation d'un indice quelconque » qui concerne le critère de répartition de ce gain dans la population.

Pour plus de clarté nous n'utiliserons donc pas le terme d' « utilitarisme » pour évoquer ni l'une ni l'autre de ces deux dimensions car ce terme implique nécessairement la combinaison des deux dimensions.

4. Choix méthodologiques des agences en matière de critères de mesure des gains en santé

4.1 Grille d'analyse

4.1.1 Critères objectifs versus critères subjectifs

La grille d'analyse qui est utilisée pour comparer les choix méthodologiques des agences conduit à distinguer les méthodes d'évaluation économique selon qu'elles proposent de mesurer les gains en santé :

- au moyen de critères objectifs faisant référence, par exemple, à des normes biologiques ou statistiques (ex. années de vie sauvées, nombre de cancers dépistés, baisse du LDL-cholestérol, gain de centimètres par rapport à la taille prédite à l'âge adulte pour les

enfants traités par hormones de croissance, etc.). Ces critères sont utilisés dans le cadre d'études dites « coût/efficacité »[26].

- au moyen de critères subjectifs, tels que le bien-être ressenti par les individus grâce aux gains de santé. Ces critères sont utilisés dans le cadre d'études dites « coût/bénéfice ». C'est ce à quoi les économistes font référence lorsqu'ils proposent de mesurer l'intérêt que représentent pour les individus les effets d'un traitement au moyen de méthodes de révélation des préférences[27]. Parmi celles-ci, les méthodes d'évaluation de la disposition-à-payer cherchent à simuler un marché hypothétique dans l'objectif d'identifier la valeur attribuée par les individus à un gain en santé en observant la façon dont ils effectuent un arbitrage entre leur revenu et leur santé. Il est donc demandé aux individus d'estimer le prix maximum qu'ils seraient prêts à payer pour un gain en santé particulier[28].

Il semble important de souligner que cette grille d'analyse se différencie de l'habitude acquise en économie de la santé de distinguer les méthodes d'évaluation économique selon qu'elles permettent :

- de comparer l'allocation de ressources au sein d'un même domaine thérapeutique ou de domaines thérapeutiques comparables (études coût/efficacité)
- de comparer l'allocation de ressources entre différentes pathologies (études coût/utilité)
- de comparer l'allocation de ressources de façon plus large entre différents secteurs économiques, tels que la santé, les transports, l'éducation, etc. (études coût/bénéfice)[29].

Ces deux grilles d'analyse semblent en premier lieu se superposer. En effet, pour effectuer ces comparaisons interpathologies ou intersectorielles, les économistes s'appuient habituellement sur les préférences individuelles afin de disposer d'un étalon commun pour évaluer les conséquences des différentes allocations de ressources envisageables. Pourtant, il serait théoriquement envisageable d'effectuer des comparaisons interpathologies ou intersectorielles

[26] Il est clair alors que cette prétendue objectivité pose question au regard des travaux de G. Canguilhem sur la limite entre le normal et le pathologique. Selon l'auteur, celle-ci est en effet davantage qualitative (l'anomalie n'est pathologique que si elle est ressentie comme telle), que quantitative (standard défini par rapport à une ou des valeurs moyennes).

[27] *« Les théories morales et politiques ne sont pas souvent explicites au sujet des critères de bien-être qu'elles font prévaloir. (…) Par critères subjectifs, j'entends les critères selon lesquels le niveau de bien-être dont jouit une personne suite à un bénéfice ou à un sacrifice particulier doit être mesuré en évaluant soit les circonstances matérielles, soit le bénéfice ou le sacrifice du point de vue des goûts et des intérêts de cette personne uniquement. L'utilitarisme hédoniste repose sur un critère qui, en ce sens, est subjectif (…) au même titre que le nouvel utilitarisme de l'économie du bien-être. »* (Scanlon T.M., 1975, p. 656, traduction non officielle)

[28] Dans le cadre de ces études coût/bénéfice, les valeurs monétaires sont utilisées comme des étalons servant à évaluer les besoins ressentis par les individus. L'argent est un substitut conventionnel ; notion que l'on retrouve dans les racines étymologiques du mot « monnaie » (*nomisma*), issue du grec *nomos* (la règle, la convention) (de Boyer J., 2003). Il serait donc envisageable de faire des études coût/bénéfice en utilisant un autre critère que le revenu, pourvu qu'il soit numérique (Fleurbaey M. et *al.*, 2007).

[29] Sur cette distinction Cf. Drummond M. *et al.*, 2005 ; Brazier J. *et al.*, 2007.

au moyen de critères objectifs comme le proposent Sen, Daniels et Hausman (Sen, 1999 ; Daniels, 2006, Hausman, 2009). Et il serait aussi possible de recourir à des critères subjectifs tout en refusant d'effectuer de telles comparaisons. Les deux grilles d'analyse sont donc bien distinctes.

4.1.2. Les QALYs : un critère intermédiaire entre critères objectifs et subjectifs

Les QALYs (Quality Adjusted Life Years), qui sont mobilisés dans le cadre des études coût/utilité peuvent être considérés comme un critère intermédiaire sur l'axe « critères objectifs *versus* critères subjectifs de mesure des gains en santé ». En effet, en utilisant les QALYs, les économistes proposent de pondérer le nombre d'années de vie gagnées par la qualité de vie. Cette dernière est mesurée de façon subjective, à partir des préférences individuelles mesurées au moyen des arbitrages qu'effectuent les individus entre qualité de vie et longévité (technique dite de *« time trade off »*) ou entre qualité de vie et probabilité de survie (technique dite de *« standard gamble »*) au sein d'enquêtes (Drummond M., 2005 ; Brazier J. *et al.*, 2007). Les QALYs proposent donc d'associer un critère objectif de mesure des gains en santé (le nombre d'années de vie gagnées) et un critère subjectif (satisfaction individuelle associée à la qualité de vie). En outre, les pondérations d'utilités qui sont associées aux différents états de santé dans le cadre de la mesure des QALYs sont soumises à des hypothèses très restrictives au point que l'on en vient finalement à douter qu'elles rendent véritablement compte des préférences individuelles (Dolan P., 2008 ; Dolan P. *et al.*, 2009)[30].
Il est difficile de déterminer si l'ambivalence des QALYs sur l'axe « critères objectifs *versus* critères subjectifs » s'explique par des contraintes techniques ou si elle traduit plutôt la volonté de trouver un compromis entre l'utilisation de critères objectifs et subjectifs pour mesurer les gains de santé. D'une part il semble effectivement possible d'interpréter la position intermédiaire qu'occupent les QALYs sur l'axe « critères objectifs de mesure des gains en santé *versus* critères subjectifs de mesure des gains en santé » comme le fruit des écarts opérés avec la théorie économique du bien-être pour faciliter la production de données scientifiques.

[30] Les conditions sous lesquelles les QALYS peuvent être considérés comme des utilités sont restrictives (Pliskin J. *et al*. 1980) :

- *« independance utility »* : indépendance entre l'utilité associée à un état de santé et le temps passé dans cet état de santé ;
- *« constant proportional time trade-off »* : la proportion d'années de vie qu'un individu est prêt à échanger contre une amélioration donnée de sa qualité de vie est constante et indépendante du nombre d'années de vie passées dans cet état de santé ;
- *« risk neutrality over life years »* : pour un état de santé fixe, l'utilité est directement proportionnelle au temps.

En effet, la réalisation d'enquêtes visant à évaluer le niveau d'utilité associé aux différents états de santé est un travail complexe et coûteux. Or les procédures de régulation et de fixation de prix et de remboursements impliquent de produire régulièrement de nouvelles données scientifiques sur le coût et l'efficacité de l'ensemble des interventions prises en charge par les systèmes d'assurance maladie. Les évaluateurs sont donc contraints de prendre des dispositions pour améliorer l'opérationnalité des QALYs au moyen, par exemple, de systèmes de classification des états de santé multi-attributs pré-scorés[31]. L'utilisation de ces échelles suppose toutefois que des hypothèses supplémentaires soient ajoutées par rapport à celles posées par l'économie du bien-être[32].

Néanmoins, au-delà du fait que l'on puisse expliquer la position intermédiaire qu'occupent les QALYs sur l'axe « critères de mesures objectifs *versus* critères de mesures subjectifs » par les difficultés techniques que pose l'intégration des préférences individuelles dans le cadre de l'évaluation des interventions de santé dite « de routine », il est possible d'interpréter l'ambivalence des QALYs comme le fruit d'enjeux disciplinaires, voire politiques et philosophiques. Les QALYs sont en effet plus facilement acceptés par la communauté médicale car celle-ci est habituée à utiliser des critères objectifs (ex. nombre d'années de vie, gagnées, qualité de vie mesurée au moyen d'échelles psychométriques) (Culyer A., 2006). En utilisant les QALYs, les économistes s'opposent moins frontalement à la tradition médicale qu'en recourant à des critères strictement subjectifs tels que les dispositions à payer. Il est donc possible que les QALYs soient privilégiés justement parce qu'ils représentent un compromis entre les deux termes de l'alternative « critères objectifs *versus* critères subjectifs ».

L'ambivalence des QALYs sur l'axe « critères objectifs versus critères subjectifs » semble confirmée à la lumière du débat qui oppose C. Phelps et A.I. Mushlin à S. Birch et C. Donaldson au sujet du statut à accorder aux QALYs par rapport à l'analyse coût/bénéfice traditionnelle, en particulier lorsque les études coût/utilité sont associées à une valeur seuil tutélaire (Wagstaff A., 1991 ; Phelps C., Mushlin A.I., 1991 ; Birch S. et Gafni A., 1992 ;

[31] Ces échelles telle que « Euro-Qol-5D » et « Health Utility Index 1, 2 et 3 », permettent aux évaluateurs de mesurer l'efficacité d'un traitement sur un échantillon de patients au moyen d'une échelle de qualité de vie, puis d'associer aux différents états de santé que les patients ont décrits, des valeurs d'utilité prédéterminées et disponibles grâce aux enquêtes antérieurement réalisées dans la population générale (Torrance G. *et al.*, 1992 ; Dolan P. *et al.* 1996 ; Feeny D. *et al.* 2002 ; Tsuchiya A. et Dolan P., 2005).

[32] L'utilisation de ces échelles multi-attributs est critiquée notamment par P. Dolan pour qui ces échelles sont inadéquates pour rendre compte des expériences vécues par les patients et leur entourage. En outre P. Dolan souligne que pour utiliser ces échelles, il convient d'adhérer à des partis-pris sur lesquelles elles reposent et de justifier explicitement les choix qui sont ainsi effectués (le choix des cinq dimensions de la santé qui sont privilégiées dans le cadre de l'EQ-5D par exemple ; l'importance accordée à certaines de ces dimensions ; le fait de privilégier les préférences de la population générale plutôt que celle des patients) (Dolan *et al.* 2009).

Johannesson M. et Meltzer D., 1998). Les premiers considèrent qu'il existe une quasi-équivalence entre les études coût/utilité et coût/bénéfice, ce que réfutent les seconds. Tous s'accordent néanmoins à reconnaître qu'il existe des racines théoriques communes entre les outils de mesure des QALYs et les méthodes welfaristes de révélation des préférences (ex. dispositions à payer) et qu'elles partent d'un constat commun : la nécessité de prendre en compte, au moins pour partie, les préférences individuelles pour valoriser les gains en santé (Birch S. et Donalsdson C., 2003 ; Dolan P., 2000)[33].

4.2. Choix méthodologiques des agences en matière de critères de mesure des gains en santé

4.2.1. Choix méthodologiques du NICE

Le NICE recommande de mesurer en priorité les résultats des interventions de santé en termes de QALYs *via* l'échelle EuroQol-5D. L'amélioration de la qualité de vie est estimée en interrogeant les patients au moyen d'une échelle générique de qualité de vie et la valeur d'utilité associée à ces différents états de santé est mesurée à partir des préférences du public. Le NICE évoque également la possibilité d'avoir recours de façon complémentaire à la mesure des dispositions à payer.

4.2.2. Choix méthodologiques de l'IQWiG

Les recommandations de l'IQWiG en matière de critères de mesure des gains en santé sont plus ambigües. Les avantages et les inconvénients des indicateurs cliniques sont décrits et il est décidé que le choix de l'indicateur doit être effectué, au cas par cas, pour chaque domaine thérapeutique. Bien que les QALYs présentent l'avantage de synthétiser plusieurs dimensions d'un état de santé, l'IQWiG souligne qu'ils font l'objet de vives critiques, tant du côté des cliniciens, que de certains économistes. D'après l'IQWiG, les cliniciens sont en désaccord avec les fondements conceptuels des QALYs : ils contestent notamment le fait que cinq ans en bonne santé puissent être jugés équivalents à dix années passées dans des conditions de vie

[33] L'ambivalence des QALYs à l'égard de l'analyse coût/bénéfice traditionnelle reflète l'ambivalence de la doctrine extra welfariste à l'égard de la doctrine welfariste en général : *« L'extra welfarisme transcende donc le welfarisme : il n'exclut pas le bien-être individuel de ses critères de jugement sur les états sociaux, mais il les complète par la prise en compte d'autres caractéristiques pertinentes pour évaluer la situation des individus. »* (Culyer A. , 1989, traduction non officielle).

jugées deux fois plus médiocres. L'IQWiG rappelle également que de nombreux économistes mettent en cause leur validité empirique en raison de la variabilité des résultats obtenus selon les méthodes utilisées pour révéler les préférences liées à l'état de santé (*standard gamble* ou *time-trade-off*). L'agence allemande conclut que tant que les problèmes soulevés par les QALYs ne seront pas résolus, ils ne peuvent pas être utilisés comme seul indicateur d'efficacité. C'est donc aux *« cliniciens et aux autres experts »* de définir, pour chaque domaine thérapeutique, les critères de résultats permettant d'estimer au mieux l'intérêt d'une intervention de santé. Dans ce contexte il est possible d'utiliser ponctuellement les QALYs. Cette position est relativement ambigüe. S'il est envisageable que les perfectionnements de l'outil permettent à terme de lever certaines critiques méthodologiques, les fondements philosophiques resteront identiques et continueront donc de faire l'objet de critiques de la part des cliniciens dans la mesure où l'IQWIG ne propose aucune méthodologie de résolution des désaccords.

4.2.3. Choix méthodologiques du KCE

Le KCE recommande d'utiliser des critères cliniques de résultats lorsque l'augmentation de l'espérance de vie représente le principal objectif du traitement ou s'il existe un résultat clinique dominant. Les QALYs peuvent être mobilisés si le traitement a un impact sur la qualité de vie ou s'il apparaît nécessaire d'évaluer de façon multi-dimensionnelle son efficacité. En revanche, le KCE rejette l'utilisation des mesures de dispositions à payer utilisées dans le cadre d'études coût/bénéfice, arguant du fait qu'il existe d'importantes *« divergences d'opinion sur la méthodologie »* (Cleemput I. *et al.*, 2008, p.16).

En conclusion de cette revue de la littérature, il est possible de représenter ainsi les choix méthodologiques des agences en matière de critères de définition des gains en santé :

	Critères objectifs de mesure des gains en santé	Position intermédiaire	**Critères subjectifs de mesure des gains en santé**
	Critères d'efficacité clinique	QALYs	Mesures de la disposition à payer
	Coût/Efficacité	Coût/Utilité	Coût/Bénéfice
NICE		——	- - - - - - - - - - - - - - -
IQWiG	——	- - - - - - - - - - - -	
KCE	——	——	

—— Utilisation en routine

····· Utilisation ponctuelle

4.3. Implications philosophiques des choix méthodologiques en matière de critères de mesure des gains en santé

Le NICE et le KCE ont recours à des critères intermédiaires pour mesurer les gains en santé tandis que l'IQWiG les rejette presque systématiquement au bénéfice de critères objectifs. En revanche aucune des trois agences ne recommande l'utilisation de critères complètement subjectifs. En définitive les choix méthodologiques qu'adoptent les trois organismes ont des conséquences philosophiques importantes.

En effet, en utilisant des critères subjectifs pour mesurer les gains en santé, ce sont les individus qui composent la collectivité qui sont chargés de quantifier la valeur de gains en santé obtenus grâce à une intervention. La mesure des gains en santé au moyen de critères subjectifs consacrerait donc la prééminence d'un principe préférentialiste : les individus constituant ici la source essentielle des jugements sur lesquels l'action publique doit se fonder (Wolfelperger A., 2001)[34]. Les individus sont libres d'apprécier les conditions de leur bien-être et d'effectuer un arbitrage entre les différentes dimensions de leur vie qu'ils souhaiteraient privilégier (santé, loisir, revenu etc.). Ce qui peut être jugé bon pour l'un, ne l'est pas nécessairement pour un autre, le bien-être est ressenti subjectivement et n'est pas soumis à des normes valides *a priori.*

Au contraire, l'utilisation des critères objectifs permet de connaître le coût d'une intervention pour une unité de résultat de santé gagnée (coût/année de vie gagnée, coût/cas dépisté, coût/baisse du LDL-cholestérol par exemple, etc.). Par conséquent le décideur est implicitement mandaté par la collectivité pour déterminer quelle est la valeur de cette unité de résultat de santé gagnée. Il lui incombe la responsabilité de déterminer quelles sont les conditions d'une « vie bonne » auxquelles aspire l'ensemble de la population. En utilisant des critères objectifs pour évaluer les gains en santé¸ on soutient donc que certaines conditions de vie sont souhaitables pour tous et qu'elles peuvent donc être identifiées par le décideur à qui il appartient d'effectuer des arbitrages concernant l'allocation des ressources publiques. Le refus plus ou moins radical des agences publiques d'utiliser des critères strictement subjectifs pour définir les gains en santé implique donc, de fait, le rejet du principe de préférentialisme qui

[34] Ce préférentialisme n'est pas nécessairement synonyme d'égoïsme. Les préférences peuvent en effet être influencées par le souci de répartir équitablement les ressources, en vertu d'une aversion aux inégalités par exemple. On distingue donc les préférences des individus pour eux-mêmes et celles des individus en tant que membres d'une collectivité *« On voit que cette théorie officielle est étroitement liée à une définition formelle de l'utilité, qui, à travers les préférences individuelles, peut de fait intégrer quelque chose comme des normes sociales si celles-ci s'exercent sur les préférences des acteurs. »* (Demeulenaere P., 2002, p.43)

était pourtant l'apanage de l'économie du bien-être dans laquelle s'inscrit l'évaluation économique en santé orthodoxe.

A ce titre, un rapprochement pourrait être établi entre le choix du critère de définition des gains en santé et les controverses philosophiques plus générales sur le thème du statut à accorder aux jugements individuels. Doit-on fonder les décisions publiques sur les opinions des individus en acceptant que celles-ci puissent être influencées par l'intérêt personnel et par des circonstances particulières ? Doit-on au contraire mettre de côté ces opinions potentiellement arbitraires au bénéfice de décisions prises au terme d'un raisonnement autonome et fortes de l'ambition d'avoir une portée universelle ? Ce débat opposait déjà au XVIIIe siècle les philosophes utilitaristes anglo-saxons et les libéraux français, tels que de Staël et Constant, qui dans la lignée de Kant, reprochent à la notion d'utilité de n'être qu'un *«calcul variable, une opinion du moment que chacun peut envisager de la façon la plus relativiste (…). L'arithmétique des plaisirs et des peines, la compensation du gain et de la perte est sophistique en ce qu'elle fait de l'individu* « la mesure de toute chose » *(Protagoras). L'utilité n'est pas susceptible d'une démonstration précise. C'est un objet d'opinion individuelle et par conséquent de discussion, de contestation indéfinie »* (Jaume L., 1997, p. 94). On pourrait donc interpréter les polémiques soulevées par le choix des critères de mesure des gains en santé comme le fruit de l'affrontement entre deux traditions en philosophie politique. Nous irons ici jusqu'à faire l'hypothèse qu'elles sont elles-mêmes influencées par des divergences épistémologiques en établissant un rapprochement entre le préférentialisme et la tradition empiriste par opposition à une philosophie davantage rationaliste de type kantien. En effet, la tradition empiriste met en doute le fait que des idées, telles que le bien, le vrai et le juste, puissent être définies objectivement par la raison humaine ; les seules sources de la connaissance sont les sensations et les perceptions qui nous viennent du monde extérieur par l'intermédiaire de nos sens. Par conséquent, le seul moyen d'éviter aux individus de subir une autorité arbitraire consiste à imposer au décideur de soumettre son action à un calcul strict et reproductible permettant de comparer la somme des plaisirs et celle des peines qu'elle engendre conformément à une doctrine de type préférentialiste. A l'inverse, si l'on adhère à une conception épistémologique de type kantien, il est plus légitime de considérer que le décideur peut définir son action en conformité avec des principes moraux universels établis rationnellement et sur lesquels tous les individus ont la capacité de s'accorder (par le biais

d'un processus de délibération)[35]. L'adhésion, consciente ou non, à l'une ou à l'autre de ces traditions en matière de théories de la connaissance pourrait donc bien influencer le choix des instruments mobilisés dans l'aide à la décision publique. L'ancrage de la philosophie empiriste en Grande-Bretagne pourrait donc bien expliquer que le NICE adopte une position davantage préférentialiste que l'IQWiG et le KCE.

Il est intéressant de constater que les choix méthodologiques des agences suscitent, en aval, des controverses éthiques différentes lorsqu'elles sont mises en pratique. L'utilisation de critères objectifs appelle une nécessaire hiérarchisation des priorités en matière de besoins de santé, ce qui engendre naturellement de nombreux débats éthiques[36]. Fonder une décision publique sur une mesure prétendument objective du gain en santé soulève immanquablement des interrogations sur le concept de « pathologie ». Doit-on considérer que la définition d'un état pathologique et l'évaluation de sa gravité relèvent toujours d'une construction normative relative selon les lieux et les époques[37] ou peut-on s'appuyer sur une conception naturaliste centrée sur la maladie ? Le décideur est dans ce cas forcé de se positionner.

A l'inverse, la mesure subjective des gains en santé fondée sur les préférences des individus permet au décideur d'éviter de se confronter aux dilemmes posés par la concurrence de systèmes de valeur en matière d'évaluation des besoins de santé puisque ces jugements sont laissés à la charge des individus. Cette position ouvre toutefois la voie à d'autres catégories de controverses à la fois techniques (ex. éventualité de biais dans la mesure des préférences individuelles) et conceptuelles (ex. phénomène d'adaptation des préférences).

[35] Dans le cadre des philosophies contemporaines, cette approche a donné naissance aux théories constructivistes qui visent la « construction » d'un accord au moyen de procédures de délibération. Cf. Rawls J., 1971 ; Habermas J., 1986).

[36] Ainsi le débat sur la légitimité d'une prise en charge collective des soins de réassignation sexuelle en cas de transsexualisme reste ouvert (HAS, 2009). Parallèlement il existe des controverses sur la prise en charge des traitements par hormones de croissance pour les enfants de petites tailles non déficitaires d'un point de vue hormonal. L'administration du même traitement aux enfants déficitaires fait pourtant consensus au motif qu'il vise à traiter un dysfonctionnement physiologique. Bien que l'on puisse observer un risque de handicap psychosocial lié à la petite taille, est-il légitime de consacrer d'importantes ressources pour traiter des enfants pour lesquels aucune maladie n'a été diagnostiquée ? Le décideur doit-il valoriser le gain de plusieurs centimètres sur le seul critère de l'existence de stéréotypes sociaux ? (HAS, 2012).

[37] *"Ce que l'on dit injustifiable se donne généralement comme un mal radical, voire absolu: le mot intolérable lui-même suppose ce franchissement d'un extrême. Pourtant, le regard vers un passé encore proche nous apprend qu'il s'agit toujours d'une norme et d'une limite historiquement constituées, et donc frappées d'une relativité temporelle – nul ne sait aujourd'hui ce que seront les intolérable de demain. – et, de surcroît, l'attention portée à la diversité de ces transgressions nous suggère que toutes ne se situent pas sur une même échelle de valeurs, incitant cette fois à une hiérarchie morale. »* (Fassin D., in Fassin D. Bourdelais P., 2005, p.7)

Pourtant, au-delà de ces controverses philosophiques qui apparaissent en amont et en aval du choix du critère de définition des gains en santé, il n'est pas établi que le fait de privilégier une mesure objective plutôt que subjective des gains en santé conduise à des décisions publiques significativement différentes. Il est même probable que la valorisation des gains en santé par le décideur sur le fondement de critères objectifs et leur valorisation par les individus sur le fondement de critères subjectifs soient relativement proches. C'est bien ce que soulignent les théories conventionnalistes lorsqu'elles évoquent la *« possibilité d'une fonction d'utilité standard typique de la société environnante »* (Fleurbaey M., 1996, p.128 au sujet de Scanlon T.M., 1975).

5. Critères de répartition des gains en santé dans la population

5.1. Grille d'analyse

La deuxième partie de la grille d'analyse conduit à comparer les choix méthodologiques des agences en fonction des critères de répartition des gains en santé qui semblent prévaloir au travers de leur utilisation du calcul économique. L'évaluation économique s'inscrit en effet dans un objectif de maximisation de la somme des gains en santé lorsqu'elle vise à classer les interventions en fonction des ratios coût/conséquence qui leur sont associées et que cette évaluation s'accompagne du principe selon lequel les gains en santé ont la même valeur quelle que soit la situation personnelle des patients[38]. Dans cette perspective, on qualifiera donc d'« efficiente » l'intervention dont le ratio coût/conséquence est plus faible que les ratios coût/conséquence associés aux autres interventions disponibles. Dans le cas où il ne serait pas possible de comparer toutes les interventions entres elles, une frontière dite d' « efficience » peut être fixée pour guider la décision. Elle correspond au montant maximum que la collectivité est prête à dépenser pour un gain en santé donné (Johannesson M. et Meltzer D., 1998). Sur ce fondement, il est recommandé d'inclure dans le périmètre des soins remboursables les interventions dont le ratio coût/conséquence se trouve en deçà de cette frontière, et d'exclure les autres. Cette méthodologie d'évaluation implique que l'objectif du système de santé est l'amélioration globale maximum de l'état de santé de la population,

[38] Il serait possible en revanche de s'appuyer sur des théories davantage prioritaristes selon lesquelles la notion d'efficience prendrait en compte l'aversion aux inégalités de la collectivité.

c'est-à-dire la production du plus grand nombre possible de gains en santé (Wagstaff A., 1991).

Les politiques de redistribution fondées sur un critère d'efficience économique, tel que défini ci-dessus, visent donc en priorité les patients susceptibles de bénéficier au maximum des traitements qui leur sont offerts. Le principe qui est sous-tendu par ce critère de répartition des ressources est le suivant : chacun a le droit de bénéficier d'un traitement, à condition qu'il ait été démontré que les ressources consommées ne peuvent pas être utilisées à meilleur escient en produisant davantage de gains en santé. L'amélioration du bien-être de chaque individu a une valeur identique quelle que soit sa situation personnelle : « chacun compte pour un et pas pour plus d'un ».

Par opposition à ce critère de répartition fondé sur un objectif de maximisation de la somme des gains en santé, la répartition des ressources en santé peut s'appuyer sur des principes prioritaristes visant à améliorer en priorité la condition des plus défavorisés, c'est-à-dire ceux qui sont victimes d'inégalités de santé. Deux types de principes prioritaristes peuvent être distingués : ceux qui visent une répartition égalitaire en termes de résultats et ceux qui visent une répartition égalitaire en termes de moyens ou d'opportunités[39]. Les premiers invitent à réduire l'écart entre les états de santé des individus en privilégiant notamment les traitements qui s'adressent aux patients souffrant des maladies les plus graves, tandis que les seconds conduisent plutôt à agir sur les déterminants socio-économiques des inégalités de santé.

5.2. Choix méthodologiques des agences en matière de critères de répartition des gains en santé

5.2.1. Choix méthodologiques du NICE

Dans son document sur les valeurs sociétales, le NICE reconnaît explicitement s'appuyer sur un principe de maximisation de la somme des gains en santé pour fonder ses avis[40] comme le traduit la fixation d'une valeur seuil identique pour toutes les pathologies. Cette valeur seuil correspond au coût maximal que la collectivité est prête à dépenser pour gagner un QALY supplémentaire au moyen d'une intervention innovante en comparaison de l'intervention de

[39] Cette distinction est établie dans une perspective plus générale par M. Fleurbaey (Fleurbaey M., 1996).

[40] *« Malgré le fait que le NICE comprenne que les usagers du NHS s'attendent à recevoir les traitements en réponse à leurs besoins, cela n'impose pas au comité de décision du NICE de recommander des interventions dont l'efficacité ou l'efficience n'est pas suffisamment prouvée, et ce dans l'objectif de garantir l'intérêt du NHS dans son entier. »* (NICE, 2008, p.20).

référence (ratio coût/QALY incrémental ou ICER). Lorsque le coût pour obtenir un QALY supplémentaire est inférieur à la fourchette de £20 000-£30 000, l'intervention est *a priori* jugée efficiente, tandis qu'elle est *a priori* jugée inefficiente si le coût est supérieur à cette même fourchette. Le NICE conserve toutefois une certaine marge de manœuvre par rapport à cette valeur seuil. Il peut en effet émettre un avis négatif pour une intervention dont le ratio se situerait au dessous du seuil ou au contraire émettre un avis positif pour une intervention dont le ratio se situerait au dessus du seuil, dans les deux cas néanmoins, il est contraint de justifier sa décision. Il peut par exemple arguer du fait que les données disponibles ne sont pas assez robustes pour démontrer l'efficacité réelle de l'intervention ou démontrer que le questionnaire grâce auquel sont obtenues des données sur l'impact d'une intervention sur la qualité de vie ne permet pas de capter la spécificité de l'intérêt thérapeutique de l'intervention.

En vertu de ce principe de maximisation de la somme des gains en santé, le NICE conteste le fait qu'il relève d'un impératif moral de mettre en œuvre tous les moyens disponibles pour secourir les individus dont la vie est en danger (« *rule of rescue* »). Selon le NICE, cette règle de sauvetage s'exerce au détriment des patients qui pourraient bénéficier d'interventions préventives ou thérapeutiques qui permettraient à long terme d'éviter davantage de décès. L'agence semble donc ici adhérer au principe formulé par A. Williams, selon qui *« 1 QALY = 1 QALY, quel que soit l'individu »*. L'efficacité d'un programme de santé doit être mesurée et évaluée de la même façon, quelle que soit la condition des malades susceptibles d'en bénéficier. Il n'y a pas de raison de consacrer davantage de ressources à efficacité égale pour une catégorie spécifique de malades[41]. *" Il n'y a pas de place pour les interventions de santé inefficientes, car les ressources mobilisées pour prendre en charge des interventions inefficientes, ne sont plus disponibles pour atteindre l'objectif de maximisation de la santé »*. (Culyer A., 2006, traduction non officielle p.308)

En revanche la position du NICE à l'égard des critères de répartition des gains en santé dans la population est ambigüe lorsque l'agence propose de pondérer le coût d'une intervention pour un QALY supplémentaire au sujet des traitements de fin de vie comme l'agence dans son rapport *« Appraisal life-expending, end of life treatment »*. Cette dérogation concerne uniquement les traitements permettant d'augmenter la survie de trois mois pour des patients dont l'espérance de vie est inférieure à 24 mois, dans le cadre de pathologies touchant

[41] *« Les économistes ont souvent considéré que l'objectif des services de santé était la maximisation du nombre de QALYs gagnés, sans considération pour la façon dont les gains étaient répartis. Une telle recommandation est fondée sur la présomption que les QALYs sont valorisés de la même façon par la société quelle que soit la personne qui en bénéficie. Nous avons qualifié cette présomption de neutralité en termes de distribution."* (Nord E. *et al.*, 1995, traduction non officielle, p.1429)

seulement de petites populations[42]. Il est alors suggéré d'attribuer à ces patients le même niveau de qualité de vie que celui dont bénéficient les individus du même âge dans la population générale en bonne santé[43]. Etant donné que les QALYs impliquent de mesurer le nombre d'années de vie gagnées ajusté sur la qualité de vie, en augmentant artificiellement le niveau de qualité de vie de ces patients, on augmente effectivement la probabilité que le ratio coût/efficacité du traitement soit inférieur à la frontière d'efficience.

Cette mesure révèle que le NICE prend en compte une certaine aversion de la collectivité à l'égard des inégalités pures de santé. En particulier, elle favorise les traitements de fin de vie à l'intention des populations jeunes. On suppose en effet que, dans la population générale, plus les individus sont jeunes et plus leur qualité de vie est bonne. Par conséquent, l'impact de cette pondération artificielle sur les résultats de l'évaluation varie selon l'âge moyen de la population cible. Il est difficile de quantifier *a priori* le nombre d'interventions de santé pour lesquelles il est possible d'appliquer cette dérogation. Les conditions requises sont relativement restrictives car elles excluent les interventions visant uniquement une amélioration de la qualité de vie. Néanmoins, cette dérogation vise des interventions dont le coût unitaire est le plus élevé (médicaments anticancéreux, médicaments orphelins, dispositifs médicaux implantables). Le critère de maximisation de la somme des gains en santé est donc ici mis de côté au profit de critères prioritaristes. Cette entorse à la doctrine utilitariste est légitimée au nom de la justice procédurale puisque le NICE s'appuie sur la réunion du comité de Citoyens pour justifier cette décision[44].

---

[42] La première version de cette recommandation proposait une restriction plus précise, i.e. « traitements indiqués pour les populations inférieures à 7000 patients ». A la suite des réactions dans le cadre de la consultation publique, critiquant notamment le caractère arbitraire de cette frontière, la notion de « petite population » a été privilégiée.

[43] Ces modalités précises de dérogation ne sont pas précisément justifiées. Elles ont fait l'objet de discussions au sein du Citizen Council sur le fondement de propositions, puis elles ont été soumises à une consultation publique (Cf. note précédente).

[44] Le NICE propose de considérer que lorsqu'il n'est pas possible de trancher un débat sans faire intervenir de jugement de valeur, le caractère raisonnable d'une décision publique est déterminé par quatre conditions :

- la condition de publicité,
- la condition de pertinence,
- la condition d'appel et de révision,
- la condition régulatrice.

Ainsi lorsque l'agence est confrontée à des choix méthodologiques qui font intervenir des jugements mettant en jeu des valeurs sociales, le NICE fait appel au Citizen Council et soumet l'avis qui est rendu à une consultation publique. C'est seulement au terme de ce processus qu'il considère que certains choix sont justifiés au titre d'un principe de « justice procédurale ». (Cf. *Social Value Jugements*, NICE, 2008) Ces quatre conditions ont initialement été définies par Daniels dans le cadre de ses travaux sur l'adaptation des réflexions rawlsiennes sur la justice à la répartition équitable des ressources en santé. *« Ensemble, ces conditions soustraient le processus décisionnel sur la satisfaction des besoins de santé de cette boîte noire mystérieuse (…) Ces conditions relient les décisions, à quelque niveau institutionnel que ce soit, à un processus démocratique plus large, éducatif et délibératif »* (Daniels, N., 2009, p. 28)

Les méthodologies du NICE ont fait l'objet de critiques et de discussions abondantes entre les économistes de la santé. On distingue plusieurs catégories de discussions :

(i) les discussions sur les modalités de définition et d'utilisation de la notion de frontière d'efficience (Birch S., Gafni A., 2007 ; Gafni A., Birch S., 2005 ; Schlander M., 2008 ; Drummond M., 2007 ; Gold M., Bryan S., 2007) ;

(ii) les discussions sur les méthodes de prise en compte des dimensions d'équité (Drummond M., 2007 ; Culyer A.J., 2006 ; Gold M., Bryan S., 2007) ;

(iii) les discussions sur la cohérence entre les méthodes utilisées et les préférences de la population (Schlander M., 2008) ;

(iv) les discussions sur la transparence du NICE et son respect d'une déontologie procédurale (Culyer A.J., 2006 ; Schlander M., 2008 ; Drummond M., 2007 ; Gold M., Bryan S., 2007).

5.2.2. Choix méthodologiques de l'IQWiG

Contrairement au NICE, l'IQWiG refuse d'emblée de fixer une frontière d'efficience qui proposerait un seuil identique pour toutes les pathologies. L'agence déclare souhaiter éviter de se confronter aux conflits de valeur qui apparaissent dès lors que l'on cherche à comparer l'allocation des ressources mobilisées pour traiter différentes pathologies. L'IQWiG considère en effet qu'il doit se limiter à évaluer les interventions dans des indications comparables. Il est préférable de laisser le décideur déterminer la disposition à payer de la population pour des gains en santé pour chaque pathologie en fonction des valeurs que leur accordent les citoyens qu'il est censé représenter. L'IQWiG soutient donc qu'il est possible que la disposition à payer des citoyens pour un gain de santé identique puisse varier d'un contexte thérapeutique à un autre. En conclusion, la méthodologie d'évaluation qu'il adopte implique la fixation d'une frontière d'efficience différente pour chaque domaine thérapeutique, où chacune est déterminée à partir des interventions déjà disponibles sur le marché, considérées comme ayant valeur de référence.

5.2.3. Choix méthodologiques du KCE

Le KCE conteste plus radicalement la pertinence d'une frontière d'efficience, qu'elle soit identique ou variable selon les contextes thérapeutiques. L'agence préfère présenter les données économiques pertinentes de façon désagrégée. L'objectif est de laisser libre le décideur de pondérer entre elles les différentes dimensions (économiques, politiques,

sociétales etc.) que soulève la décision d'inclure/exclure une intervention dans le périmètre des soins remboursables. Le KCE souligne que la décision de prise en charge par la collectivité d'une intervention est *« politique par nature »* et qu'elle ne peut se limiter à un objectif de maximisation de la santé.

L'efficacité réelle et la rentabilité économique ne sont alors que deux considérations parmi tant d'autres pour effectuer des choix politiques. Il convient de laisser le décideur arbitrer entre les valeurs et les intérêts des différentes parties prenantes.

5.3. Implications philosophiques des choix méthodologiques en matière de critères de répartition des gains en santé

Si le NICE peut émettre une recommandation qui conduirait à consacrer davantage de ressources pour certains patients dans le cadre de mesures dérogatoires, l'IQWiG et le KCE refusent catégoriquement de fixer une frontière d'efficience identique pour toutes les pathologies. Les méthodologies d'évaluation des trois agences n'impliquent donc pas de recourir à un critère de répartition des gains en santé qui soit systématiquement fondé sur un objectif de maximisation ; *a fortiori* l'IQWiG et le KCE s'en écartent plus explicitement que le NICE.

L'analyse de ces méthodologies révèle que les agences prennent en compte le fait que la disposition à payer sociétale pour l'amélioration des conditions de vie peut varier selon les patients qui en bénéficient. Dans le cadre de certains domaines thérapeutiques, il serait justifié de consacrer davantage de ressources pour un résultat quantitatif équivalent par exemple en termes d'années de vie sauvée. Il est regrettable que cet élément ne soit pas davantage explicité et justifié par les agences alors qu'il paraît motiver les écarts qui sont opérés par le NICE, l'IQWiG et le KCE par rapport aux critères de répartition qui sont habituellement mobilisés dans le cadre de l'évaluation économique traditionnelle. Nous en sommes donc réduits au stade des hypothèses. En définitive, ces dérogations semblent avoir comme objectif de privilégier les patients considérés comme étant plus les défavorisés en termes d'état de santé effectif[45]. Plus la souffrance de certains patients est perçue comme injuste par la

[45] Sur le fondement de la typologie proposée par M. Fleurbaey, il est possible de considérer que le recours à un critère prioritariste par les agences viserait davantage à réduire les inégalités de santé en termes de résultats d'état de santé plutôt qu'en termes d'opportunité d'état de santé. En effet l'objectif de réduction des inégalités sociales de santé est rarement abordé en tant que tel. Il est évoqué par le NICE mais il n'est pas précisé dans quelles proportions le seuil d'efficience peut être dépassé au motif que l'intervention permet de réduire leur impact.

collectivité et plus il paraît justifié de dépenser davantage de ressources pour y « remédier » (au sens propre et figuré). Toutefois et contrairement à ce qu'on pourrait en déduire, ces choix méthodologiques ne paraissent pas relever d'un positionnement éthique déontologique qui justifierait que des réponses thérapeutiques soient apportées aux patients quelles que soient les conséquences à long terme pour la collectivité.

L'IQWiG et le KCE imposent en effet de réaliser systématiquement une analyse d'impact budgétaire parallèlement à l'évaluation économique. Or cette analyse s'inscrit là assurément dans une perspective conséquentialiste. L'objectif d'une analyse d'impact budgétaire est en effet de mesurer les conséquences financières pour la collectivité induite par l'inclusion d'une intervention dans le périmètre des soins remboursables. Il s'agit d'estimer la quantité totale des ressources qui seront consacrées à la mise en œuvre d'une intervention. L'influence que peut avoir une analyse d'impact budgétaire sur la décision publique doit être soulignée. Si la collectivité semble disposée à consacrer davantage de ressources pour l'amélioration de l'état de santé de certaines populations de patients, le rôle de l'analyse d'impact budgétaire est de mettre en lumière le sacrifice qui est ainsi consenti et qui s'effectuera au détriment des autres individus. La gravité de certains handicaps et leur impact sur la qualité de vie, par exemple, peut inciter la collectivité à financer des dispositifs très coûteux au titre d'une prise en charge qualifiée de compassionnelle. Néanmoins, le volume de la population cible conditionne l'impact budgétaire. Elle risque donc d'influencer la décision finale au sujet du remboursement du dispositif[46].

Un tel raisonnement va parallèlement à l'encontre des deux doctrines, celle qui privilégie l'option déontologique et celle que nous qualifions de strictement utilitariste (Schlander M, 2008). Il apparaît davantage comme une tentative de compromis entre deux critères de répartition :

- un critère visant à maximiser la somme totale de la production des gains en santé dans la population ;

[46] L'évaluation d'un dispositif de stimulation phrénique peut illustrer ce propos. Cette intervention est destinée à des patients tétraplégiques souffrant de lésions médullaires hautes causées par des traumatismes (ex. chutes). Il s'agit souvent d'individus jeunes, voire d'enfants. Elle permet à ces patients d'être débarrassés partiellement d'une machine respiratoire externe qui fonctionne par l'intermédiaire d'une trachéotomie, générant une importante détérioration de la qualité de vie (en limitant la mobilité et les capacités gustatives et en induisant une forte nuisance sonore). Le coût du stimulateur phrénique implantable s'élève à plus de 20 000€. En revanche, le nombre de patients pour lequel ce dispositif est indiqué est inférieur à 20 par an. Bien que le prix à payer pour cette amélioration de l'état de santé soit supérieur au seuil de référence, il est possible que la collectivité soit prête à le financer. En effet, la somme des ressources consommées au total peut sembler relativement modeste par rapport à la compassion ressentie à l'égard de ces patients. Cet arbitrage pourrait toutefois être remis en question par une croissance de la population cible qui conduirait mécaniquement une augmentation de l'impact budgétaire.

- un critère prioritariste visant à favoriser la production de gains en santé au bénéfice des patients les plus défavorisés en termes d'état de santé effectif.

Selon cette hypothèse, la position des agences pourrait se résumer de la façon suivante : la dérogation au principe de maximisation de la somme des gains en santé dans la population, quoique légitime dans certaines circonstances, ne doit pas s'effectuer au prix d'un sacrifice trop important pour la collectivité. Reste à savoir sur quel fondement effectuer un tel arbitrage. Tandis que l'IQWiG et du KCE préfèrent le confier au décideur au cas par cas ou par domaine thérapeutique, le NICE s'appuie en premier lieu sur des algorithmes décisionnels définis *a priori*. Malheureusement, ces processus sont externes au processus évaluatif et restent relativement peu transparents sur l'explicitation des valeurs qui sous-tendent les décisions.

Dans l'hypothèse où il serait confirmé que la collectivité rejette partiellement l'application visant à maximiser la somme totale de la production des gains en santé dans la population, il serait utile que les économistes adaptent leur méthode de calcul de telle sorte qu'ils puissent prendre en compte de façon plus explicite et plus rationnelle ces oppositions de principe. En restant dans un modèle normatif conséquentialiste où l'évaluation économique a toute sa place, il s'agit d'attribuer une valeur différente à certaines conséquences, définies objectivement ou subjectivement, en fonction des circonstances. Or comme l'indiquent de récentes recherches méthodologiques, il est envisageable de prendre en compte ces considérations en appliquant des taux de pondération sur les résultats de l'évaluation (Wagstaff A., 1991 ; Cookson R. *et al.*, 2009 ; Cookson R., *et al.* 2008 ; Cookson R., Dolan P., 2000 ; Nord E., *et al.* 1995). Ces méthodes ont l'avantage de mettre en lumière les choix éthiques qui sont effectués et de les soumettre au débat public. Le problème réside alors dans les processus employés pour déterminer les taux de pondération de telle sorte qu'ils puissent être cohérents et légitimes par rapport au modèle de justice sociale souhaité par la population.

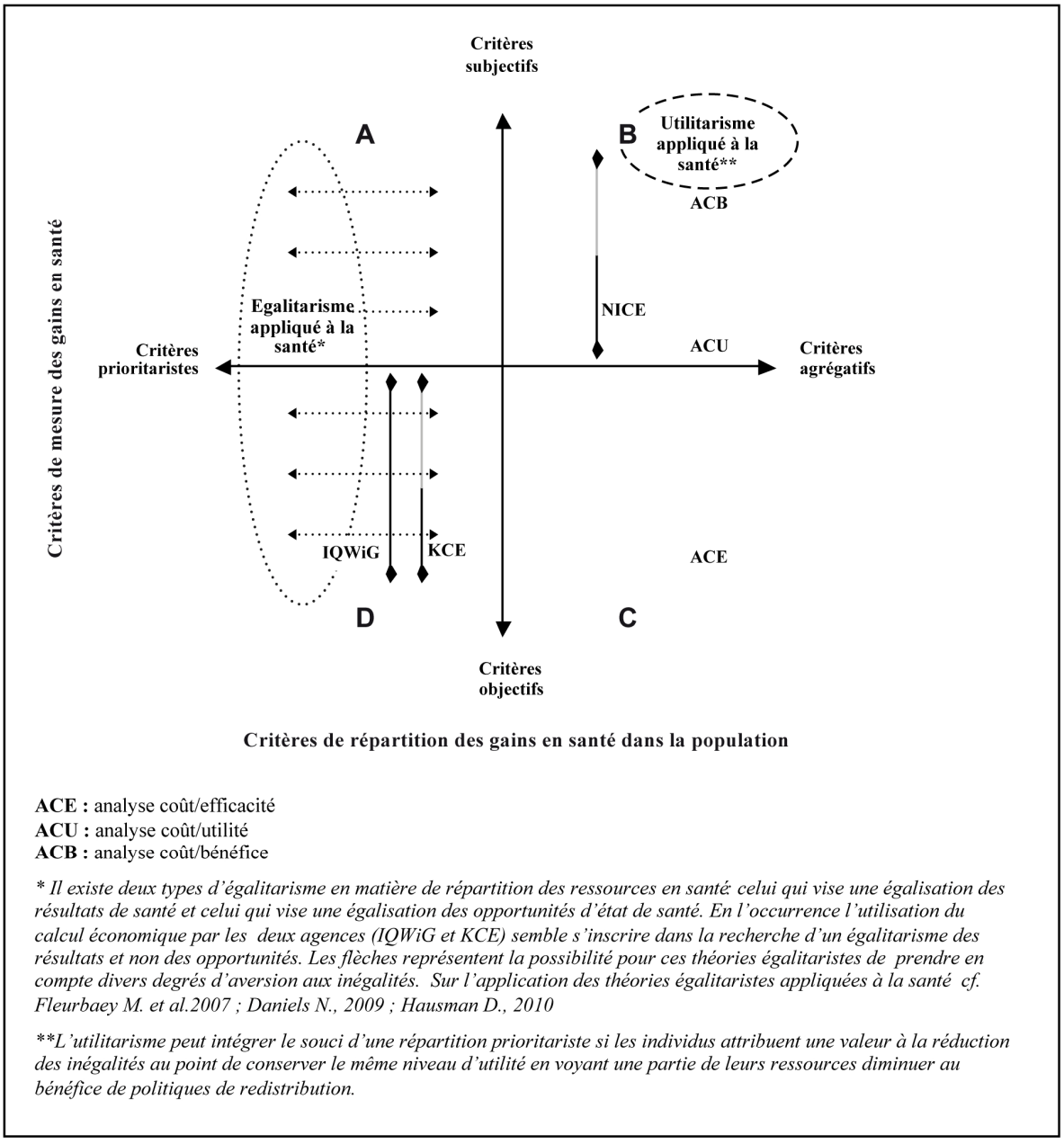
Une première possibilité consiste à définir *a priori* les populations de patients que la collectivité estime comme plus défavorisées en santé. On s'inscrit là, d'après Daniels, dans une démarche proche de celle adoptée par Rawls lorsqu'il définit une liste de « biens premiers » en s'appuyant sur l'allégorie du voile d'ignorance. Il s'agit d'identifier objectivement les besoins de santé pour lesquels il convient d'apporter une réponse thérapeutique de façon prioritaire, ce qui revient à définir des conditions de vie souhaitables pour tous. La légitimité de ces choix réside alors dans le respect de principes de justice procédurale (Daniels N., Sabin J.E., 1998 ; Daniels N., 2009 ; Goold S., 1996 ; Hausman D., 2010).

Une seconde possibilité consiste à s'appuyer sur les préférences de la population pour établir ces poids de pondération en s'appuyant sur le modèle des travaux de Fleurbaey *et al.* (Fleurbaey M. et al. 2007). Pour identifier les inégalités de santé vers lesquelles orienter les politiques de répartition, il est proposé de mesurer le revenu équivalent-santé des individus, *i.e.* le revenu pondéré par la valeur attribuée par les individus à leur santé par rapport aux autres dimensions de leur vie. En effet, le revenu brut ne suffit pas pour identifier les patients les plus défavorisés en santé et il convient de prendre également en compte la gravité de la pathologie et la valeur individuelle portée à la santé. La méthodologie classique d'évaluation coût/bénéfice doit s'adapter de telle sorte qu'elle puisse pendre en compte ces dimensions et afficher ainsi clairement une prise de position concernant les critères de répartition des gains en santé dans la population.

6. Conclusion

L'analyse des méthodes d'évaluation des trois agences montre qu'il existe un écart entre le modèle de justice sociale qui est sous-entendu dans les méthodes classiques d'évaluation économique en santé issues de la branche utilitariste de l'économie du bien-être et celui qui semble prévaloir à travers les choix méthodologiques des agences publiques chargées de l'évaluation des interventions de santé en Europe, à savoir le NICE, l'IQWiG et le KCE. D'une part, les individus ne sont pas les seuls à apprécier la valeur de l'amélioration de l'état de santé ; cette responsabilité incombe de façon partielle ou exclusive au décideur. D'autre part, elles refusent de fonder leurs avis sur le seul critère d'un ratio coût/conséquence avantageux. Les agences s'écartent donc bien d'une doctrine utilitariste appliquée à la santé qui aurait comme objectif de maximiser la somme des satisfactions des préférences individuelles liée à la santé comme l'illustre le graphique ci-dessous.

Représentation graphique des choix méthodologiques des agences et mise en perspective avec les différents modèles de justice sociale proposés par l'économie du bien-être



ACE : analyse coût/efficacité
ACU : analyse coût/utilité
ACB : analyse coût/bénéfice

** Il existe deux types d'égalitarisme en matière de répartition des ressources en santé: celui qui vise une égalisation des résultats de santé et celui qui vise une égalisation des opportunités d'état de santé. En l'occurrence l'utilisation du calcul économique par les deux agences (IQWiG et KCE) semble s'inscrire dans la recherche d'un égalitarisme des résultats et non des opportunités. Les flèches représentent la possibilité pour ces théories égalitaristes de prendre en compte divers degrés d'aversion aux inégalités. Sur l'application des théories égalitaristes appliquées à la santé cf. Fleurbaey M. et al.2007 ; Daniels N., 2009 ; Hausman D., 2010*

***L'utilitarisme peut intégrer le souci d'une répartition prioritariste si les individus attribuent une valeur à la réduction des inégalités au point de conserver le même niveau d'utilité en voyant une partie de leurs ressources diminuer au bénéfice de politiques de redistribution.*

Il reste cependant à déterminer si les implications philosophiques des choix méthodologiques des agences sont conformes à l'objectif de justice sociale qui est recherché par les décideurs anglais, allemands et belges. Le choix des critères d'évaluation a un impact sur les résultats qui sont soumis au décideur. L'existence d'un écart entre les valeurs qui sous-tendent les choix méthodologiques des agences et les valeurs que supportent les décideurs nuirait à la légitimité des choix d'allocation des ressources en santé. Il est donc essentiel d'éclairer les articulations qui existent entre les outils mobilisés et les fondements éthiques sur lesquels ils reposent afin de les soumettre aux décideurs publics pour s'assurer de leur adhésion.

Il est particulièrement important de mener cette réflexion en France car il est souhaitable que les choix qui seront finalement retenus en matière d'évaluation économique soient cohérents avec le modèle de justice sociale qui est visé par la collectivité. En particulier, il semble exister en France une méfiance à l'égard de l'adoption d'une doctrine utilitariste pour guider l'allocation des ressources en santé[47]. Cette méfiance pourrait d'ailleurs expliquer pourquoi il est encore difficile d'intégrer explicitement un critère économique dans les choix en matière de périmètre des soins remboursables. Il serait donc nécessaire de vérifier si cette méfiance est réelle ou si elle est seulement présumée par les acteurs chargés de l'aide à la décision. Si elle est clairement établie, alors il serait nécessaire d'aller plus loin dans les choix méthodologiques qui sont actuellement effectués en s'appuyant sur les réflexions récentes dans le cadre des théories économiques de la justice sociale, et qui proposent des outils inscrits dans d'autres modèles normatifs que l'utilitarisme, tels que ceux qualifiés d'égalitaristes (Fleurbaey M. *et al*. 2007). Ces outils sont figurés dans les quadrants A et D du graphique parce qu'ils proposent d'évaluer les gains en santé au moyen de critères objectifs, subjectifs ou mixtes (en termes de ressources ou de probabilité de bien-être) et ils prennent en compte des degrés d'aversion aux inégalités variables.

Des travaux doivent cependant encore être réalisés pour déterminer de quelle façon ils peuvent être concrètement utilisés dans l'aide à la décision publique en matière de définition du périmètre des soins remboursables compte tenu des contraintes auxquelles sont soumises les agences publiques d'évaluation des interventions de santé.

[47] Cette méfiance a d'ailleurs été explicitée et discutée par la HAS dans le cadre d'un document stratégique de l'agence. *« Traditionnellement, la culture française en général et la culture médicale en particulier nourrissent une crainte implicite à l'égard des raisonnements utilitaristes. De fait, jusqu'à présent, la qualité des soins a été le plus souvent traitée dans notre pays comme un objectif en soi. (…) Il est donc nécessaire de clarifier les craintes qu'inspire encore aujourd'hui un argumentaire utilitariste qui fonderait la recherche de la qualité sur ses bénéfices en termes d'efficience durable pour notre système de financement solidaire des soins. »* (HAS, 2007, p.13)

Chapitre 2 :

*Dealing with Moral Dilemma Raised by Adaptive Preferences in Health Technology Assessment: the Example of Growth Hormones and Bilateral Cochlear Implants**

** Cet article est en cours de soumission pour publication.*

1. Introduction

1.1. Context

From 2007 to 2011, the French National Authority for Health (HAS) was asked to assess recombinant growth hormone (GH) treatment for non-GH-deficient short children,[48] and bilateral cochlear implants for children with severe to profound deafness, in order to provide evidence about the relevance of their coverage by French national health insurance.[49] Both health technologies have to be introduced as early as possible in patients' lives to improve their effectiveness (from the first year for cochlear implants and the age of 4 for growth hormones),[50] and both have irreversible consequences on the development of an individual's character.

These result from:

[48] The scope of this article is restricted to children "born small for gestational age, with persisting growth failure at 4 years of age or later". Issues raised by growth hormone treatment for other conditions such as growth hormone deficiency, Turner syndrome, Prader-Willi syndrome, or chronic renal insufficiency are not considered.

[49] Till now, GH treatments were entirely reimbursed for non-GH-deficient children defined as being "born small for gestational age, with persisting growth failure at 4 years of age or later". Besides, if unilateral cochlear implants are entirely reimbursed by French national health insurance, bilateral implants are not.

[50] Regarding cochlear implants, the age at which the second implant is performed was found to affect the speed and final amount of improvement. A person whose bilateral cochlear implant was placed in adulthood does not experience the same effects as a person whose implant was placed at a very young age. Regarding growth hormones, it should also be noted that the treatment is no longer effective after puberty.

(1) the medicalisation of the child's life induced by daily injections for growth hormone treatment, or surgery and intensive speech therapy for cochlear implants;
(2) structural consequences on the person him/herself: physical appearance, functional capacities, tastes, skills or life projects are likely to be affected after being treated with the technology.

If the decision to implement the two technologies has consequences on an individual's personality and life, there are also consequences when deciding to not administer treatment. Due to social stereotypes, short stature may induce psychological suffering in children and future adults through complexes about their physical appearance.[51] Therefore, parts of medical community consider that by increasing the height of non-deficient short children, growth hormone treatment will actually reduce their suffering.[52] However, the causal relationship between increasing adult height and improvements in quality of life is not really demonstrated by clinical studies. Similar issues arise about the assessment of bilateral cochlear implants. Bilateral cochlear implantation has no incremental effectiveness on oral language acquisition compared with unilateral cochlear implantation. However, bilateral cochlear implants provide deaf children with better listening comfort through an ability to hear sounds in stereo which enhances their capacities to understand speech in noisy conditions and to perceive better where sounds are coming from. The question that still remains is to what extent these enhanced capacities give them better access and better ease in activities and situations which require a good hearing performance (e.g. transport, schooling and education, leisure or employment), and to assess what impact this better access and ease has on their life quality.

Deciding whether or not to reimburse these technologies is a very important choice because it determines accessibility of children to them whatever parents' income level. Yet, as the financial resources of national health insurance are not unlimited, needs of children with short size as well as needs of children with deafness compete with the needs of other individuals for treatment. The total cost per patient of growth hormones treatment, over several years, ranges from €30,000 to €33,200 and the effectiveness is about 2 cm gained in adult height (HAS,

[51] An American study including 166 short children referred for consultation showed that these children have some behavioural disorders, lower educational achievement and lower social integration (Stabler B. *et al*., 1994). Other studies showed that men with a high stature are associated with greater qualities related to social and professional success. Meanwhile, persons whose jobs are socially best valued tend to consider themselves taller (Voss L. *et al.* 2006). Moreover, marriage is less common among short men, and tall men are less likely to be single (Herpin N., 2003). A systematic review has been made by HAS (HAS, 2011).
[52] Issues raised by adverse effects are not considered in this article, although they are currently being questioned. This article considers that the risk/benefit balance was previously judged to be favourable.

2011). Costs related to unilateral cochlear implants have been evaluated to average €35,000 per child for the first year (including the surgical implementation and the cost of the device), while costs of bilateral cochlear implantation are about twice as high (HAS, 2007). Therefore, in order to decide whether it is relevant or not for the national health insurance to cover these two health technologies, it is important to be able to assess whether their benefits are worth the costs. However, the evaluation of outcomes is very controversial among stakeholders, and some of the features of both health technologies induce methodological issues for the evaluator when it comes to applying traditional preference elicitation methods.

1.2. Problematic

Economists used to measure the outcome of health technologies in terms of their impact on individuals' well-being through preference elicitation methods. Individuals are asked to what extent they are willing to trade survival chances (standard gamble), life span (time-trade-off) or financial income (contingent evaluation) in order to benefit from better health that is provided by the relevant technology.[53] As children cannot be considered as fully rational agents, it is not possible to consult them directly to elicit their preferences. Therefore, proxy respondents speaking on the children's behalf need to be interviewed. Nevertheless, empirical research conducted to study the shaping of individual's preferences conclude that values assigned to health improvement may vary, among individuals, in regard with their age and their experiences in terms of health states. As shown in the Section 3 of this article, the assumption of variations of health related preferences seems particularly relevant in the context of growth hormone and bilateral cochlear implant assessments. Significant and systematic variations may be anticipated depending on which proxy respondents are interviewed to evaluate the outcomes of growth hormones and bilateral cochlear implants: parents, individuals with an experience of the same disability, or representative samples of the general public, etc.[54] Therefore, the choice of proxy respondents may generate systematic bias.

[53] The scope of this article is limited to the issues raised by a direct evaluation of the health benefits induced by growth hormone treatment and bilateral cochlear implants, using the three main individual preference elicitation methods: standard gamble, time-trade-off and willingness-to-pay. Issues raised by indirect evaluation of health benefits through a pre-scored multi-attribute health status classification system, which are commonly used in the measurement of QALYs, have not been examined. Whether the impact of the two health technologies on welfare can be accurately evidenced by Euroqol-5D or HUI remains a relevant question. Nevertheless, this question is the same for every health technology assessment and it refers to more general discussions about the gap between welfare economics and operational tools in health technology assessment aimed at facilitating data production.

[54] Representative samples of the general public may comprise individuals of short size or individuals with deafness in proportion to the extent of the prevalence rate of these disabilities in the general population.

As the gap between values reported by the different proxy respondents tends to affect the results of the assessment, methodological choice regarding the selection of proxy respondents implies a moral dilemma for the economists.

To begin with, it seems consistent to base the assessment on the preferences of individuals who actually have a personal experience with a disability because preferences of healthy individuals are more a matter of belief and are not based on real experience of disability. Nonetheless, able-bodied individuals do enjoy greater autonomy because they do not suffer any handicap. As consequence, they are offered opportunities which disabled individuals cannot experience. In this respect, able-bodied individuals are better equipped/placed to gauge the extra-welfare resulting from those opportunities. Therefore, there is no reason to give priority to the former (patients) or the latter (healthy individuals) in evaluating outcomes of growth hormone treatment and bilateral cochlear implants. Indeed, all of them may claim an accurate experience: the experience of the disability *versus* the experience of opportunities provided by health (i.e. with no disability). Economists who are in charge of the evaluation of these two technologies, which will have a real impact on the coverage decision, cannot avoid making a normative choice.

Under present practices this choice is made implicitly. Indeed it is usually recommended to base the assessment on the preferences of individuals in the population as a whole. Patients are asked to describe their state of health, but they are not the ones who are asked to value their potential health improvements (Brazier *et al*., 2007). The reasons for this are the following: i) it is preferable to base the assessment on preferences of tax payers,[55] and ii) it is better to base the assessment on preferences expressed by individuals behind "the veil of ignorance", i.e. who have no knowledge of their future state of health (Gray *et al*., 2011)[56]. The principal argument against these justifications, especially when looking at the two above-mentioned technologies, concerns the reference to the veil of ignorance. Non-GH deficient shortness and severe to profound deafness of birth are two congenital disabilities. Individuals drawn from a representative sample of the general population are certain they will never be affected by these disabilities. This is obvious for shortness, but it is also true for deafness. The

[55] *"A major argument deployed in favour of obtaining valuations from the general public is that, as tax or insurance payers, they bear the costs of health care decision"* (Gray *et al.* 2011, p.97).
[56] *"It could also be argued that members of general public are all potential patients. Asking the public also correspond more closely to a Rawlsian approach to distributive justice, which strives to blind individuals to self-interest by asking them to make fair choices from behind 'veil of ignorance'"* (Gray *et al.* 2011, p.97)

lives of individuals who become deaf are not the same as persons born deaf. The ability to use oral language varies strongly in particular between these two situations. The specificities of the two interventions require the debate on the choice of the perspective of the assessment to be reopened. Actually, both the choice of the perspective and consequently the position taken with respect to the problem raised by adaptive preferences depend on the principles of justice which the community strives to implement.[57]

In this article, it is assumed that the community seeks to apply an egalitarian conception of justice in terms of allocating health resources. Three options have been identified, each of which is based on a different choice in terms of the *equalisandum* (i.e. the object whose distribution across the population is to be guaranteed): opportunity of functioning, welfare chances, and fundamental social outcomes.[58] The three options have been distinguished with the help of the typology of egalitarian theories of social justice put forward by M. Fleurbaey (1995) in his article *Equal opportunity or equal social outcomes.*
In this article I show that each of these options proposes distinct ways of treating preference adaptation phenomena and that they have very different consequences on the methods for evaluating the two technologies.

These two concrete examples have been chosen in order to study how theoretical approaches to social justice can help solve a dilemma which assessors experience in their current practices. In the conclusion of this article, I nevertheless try to gauge to what extent the lessons learnt from these two examples can be applied to other subjects of assessment. The difficulties raised by the phenomenon of adaptive preferences in fact also apply to evaluation of more generally speaking assistive technologies,[59] and even to evaluation of health technologies as a whole.

[57] *"The role of the evaluator is to make a consistent evaluation that can be useful in the political debate, not to replace the democratic political process itself. The problem of economic evaluation is to adjudicate individual interests according to one particular political view or a sample of political views (with a different social welfare function for each view), not to synthetize the citizens' political views into a 'collective' doctrine."* (Fleurbaey M. *et al.*, 2007, p.6).
[58] *"An equalisandum claim specifies what ought to be equalized, what, that is, people should be rendered equal in."* (Cohen G. A., 1989, p.908)
[59] Assistive technology includes assistive, adaptive, and rehabilitative devices for people with disabilities and also includes the process used in selecting, locating, and using them

2. Structure of the paper

The third section of this article reviews the main studies which have demonstrated the impact of adaptative preferences in evaluating the outcomes of health technology (Section 3). Indeed it does seem that, due to adaptation phenomena, preferences linked to health actually change over time (3.1) and that they change depending on individuals' experience of states of health (3.2). I examine to what extent these two childhood disabilities (non-GH deficient shortness and severe to profound deafness of birth) are especially likely to lead to conflicting preferences between different proxy respondents asked for assessing the outcomes of the two technologies. It is then asked if these conflicting preferences can be avoided by using an ordinal measure of preferences rather than a cardinal measure. However, an extreme case of the adaptation of preferences does show that a reversal of preferences (a preference for disability) is not to be excluded. Thus, the preference adaptation phenomena do lead to conflicting preferences which cannot be resolved by using an ordinal measure (3.3).

Section 4 identifies several options on the basis of theories of egalitarian justice for resolving the dilemma raised by the conflict of preferences. The first option follows on from a theory of social justice based on the equal opportunity of functionings or capabilities (Section 4.1). The second follows on from a theory of social justice based on equal chances of welfare (4.2). The third follows on a theory of social justice centred on equal social outcomes (4.3). The main arguments which can be put forward in criticism of these three options are discussed in Section 5 of the article.

3. Adaptive Preferences: Why Are They Likely to Rise in Growth Hormone Treatment and Cochlear Implants Assessments?

3.1. Life-long variations of health related preferences

According to Sackett and Torrance, the time-trade-off elicitation method shows that values given to quality of life improvements decrease all along patients' lives, compared to the values given to increased life expectancy (Sackett D. and Torrance G., 1978). These findings

are challenged by Dolan's study, according to which individuals, until the age of 40, are more and more willing to accept a decrease of quality of life in exchange of increased life expectancy. Then, trade-offs between increased life expectancy and quality of life reverse. Beyond the age of 40, individuals give more and more value to their quality-of-life compared to the value given to their life expectancy (Dolan P., 1996).[60] Consequently when groups comprising people with every age are interviewed to value health improvement, preference variation spanning lifetimes cancel out (e.g. health related utility values of the EQ-5D scoring function are assessed within a representative sample of the general public over 18 years old). In contrast, if a group of persons interviewed is more homogeneous in terms of age, then health-related, life-long preference variations are assumed (e.g. health related utility values of the HUI2 Canadian scoring function are assessed within representative samples of schoolchildren's parents).[61] Such normative issues are likely to be particularly acute in the context of economic assessments of paediatric technologies (like growth hormone treatment and bilateral cochlear implants) because they both aim at improving the children's quality of life.

(i) First, it is well-known that short stature induces stronger psychological suffering in adolescents who attach more importance to their physical appearance (Visser-van Balen H. *et al.*, 2008). When choosing to interview proxy respondents over 18 years old, extra-value given by adolescents to centimetres gained with growth hormones, compared to the value given by adults, may not being taken into account.

(ii) Secondly, consistent with Sackett and Torrance (1978) and Dolan *et al.* (1996), values given to the improvement in quality of life progressively decrease from 18 years old to 40 years old (compared with the value given to the lengthening of life expectancy). Thus, it may be speculated that these lifetime variations about health-related preferences follow the same patterns for individuals under 18: the younger individuals are, the greater is the value they give to their quality of life. As a result, value given by an adult to the improvement in quality of life could be lower than the value he/she would have been given to the same degree of health improvement as a child.

[60] Starting from that age, the value given to improvement of the quality of life gradually increases until 60 years old and more quickly thereafter.

[61] As such, health related preferences for the HUI2 Canadian scoring function are based on a random sample of parents of schoolchildren in the City of Hamilton. This sample is a more age-homogenous group than the representative sample of the public on which preferences were measured for British and French scoring function. Thus it is implicitly admitted that health related preferences are more relevant in middle age rather than old age or youth.

When it is decided to interview adult proxy respondents to value outcomes of paediatric technologies that aim to improve quality of life, the cost/effectiveness ratio associated with such technologies tends to decrease compared with the cost/effectiveness ratio which would be obtained if the preferences of the same individuals were included for when they were children. Excluding the preferences of children in the assessment is not challenged here: there is no way to consider that children may be rational agents. However it is regrettable that an assessment of the impact of the technology on subjective well-being does not take into account the impact of a better quality of life on children's well-being. To avoid this issue, it is possible to imagine interviewing proxy respondents on what they would have preferred during their childhood. However, it cannot be decided if these retrospective preferences match the evaluation systems of their childhood or if these retrospective preferences match their current values. It could even been argued that such preferences do not have any equivalent, as descriptions of past values change over time, reflecting current preferences. In the end, following Bernheim and Rangel, it seems that the aggregation of several "selves" across time raises thorny problems similar to problems raised by aggregating interpersonal preferences, which are a well-known challenge in welfare economics (Bernheim D. and Rangel A., 2007).

3.2. Variations of health related preferences across experiences of health states

The variation of health-related preferences over time is all the more problematic as there are also variations of health-related preferences between health states experienced. Indeed, empirical studies comparing values assigned to health improvement by patients themselves with values assigned to the same extent of health improvement by a representative sample of the general public conclude that health-related preferences may vary depending on an individual's experience of the disease (Dolan P., 1999 ; de Wit G., *et al.*, 2000).[62] According to Sackett and Torrance, when 0 represents death and 1 represents perfect health, values assigned by a representative sample of the general population to quality of life of patients treated at home by haemodialysis is equivalent to 0.36, while the value assigned to the same quality of life by patients themselves is equivalent to 0.56 (Sackett D. et Torrance G., 1978). Were it possible to cure these patients and bring them to perfect health, the utility gain would be about 0.44 when valued by patients *versus* 0.61 when valued from the point of view of the

[62] Patients were treated for chronic renal failure, paraplegia, colostomy etc.

public. Such a gap between "expected utility", "decision utility" and "experienced utility" has been largely discussed in literature (Kahneman D., Wakker P. and Sarin R., 1997).

Drawing on research in behavioural economics conducted by Tversky and Kahnemann, followed by Dolan and White, it is now quite common to explain the gap between preferences of healthy individuals and disabled individuals as being the result of adaptive preference phenomena (Tversky A. and Kahneman D., 1991; Dolan P. and Kahneman D., 2008).[63] Research in behavioural economics shows that individuals develop coping strategies to adapt themselves to new living conditions. Menzel *et al*., for example, highlight different adaption processes like "*skills enhancement"*, "*activity adjustment"* and "*substantive goal adjustment*". These processes could also include some phenomenon such as *"hedonic adaptation"* (Menzel P. *et al*., 2002, p.2151).[64] Reciprocally, it has been observed that the impact of cheerful events on welfare tend to decrease over time, as it shown in an empirical study on lottery winners (Brickman P., Coates D. and Janoff-Bulamn R., 1978).[65] However behavioural economics research also shows that healthy individuals interviewed to value the quality of life related to a disease they have not experienced focussing on emotional feelings immediately associated with the degradation of their own abilities and their own health. By doing so, they overlook the gradual change of their initial feelings over time. Healthy individuals tend to underestimate their capacity to go through living worsening conditions *via* more or less conscious processes involving compensating activities, changes in tastes and interests or even changes in sensitivity. Indeed, according to Tversky and Kahneman and Dolan and White, it seems that individuals do not think about health states in an absolute way, but they think about them as improvements (gains) or declines (losses) from their specific baseline (Tversky A., Kahneman D., 1991; Dolan P. and White M., 2006). *"Consequently, intuitive predictions about the state of* being *a paraplegic will be dominated by thoughts about the event of* becoming *a paraplegic"* (Kahneman D., 2000, p.11).[66]

[63] *Adaptation is the process of adjustment to new or changed circumstances. It occurs "at different levels and in different ways, ranging from molecular changes at the cellular level that diminish the perceived or experienced intensity of an objective stimulus to overt behaviour that reduces exposure to the stimulus"*. Hedonic adaptation occurs when there is a *"reduction in the affective intensity of favourable and unfavourable circumstances*" (Frederick S. and Lowenstein G., 1999, p.302).

[64] For a review of the history of research about preference reversals and its impact on the rational choice theory reliability, see P. Slovic (1995).

[65] "Hedonic treadmill" is the name commonly used to qualify the observation that effects of extreme changes in life circumstances are transient and, ultimately small (Kanheman D., 2000).

[66] It is interesting to note that, according to Tversky and Kahnemann, individuals are more affected by losses than gains in equal proportions due to a phenomenon of loss aversion (Tversky A. and Kahneman D., 1991).

The relevance of adaptive preferences phenomena in the context of short stature or deafness is even more likely given the congenital nature of both disabilities. Children with short stature and children with severe deafness at birth develop their personality, interests, hobbies and aspirations taking into account their disabilities. They never experience other living conditions, and thus they do not undergo a deterioration of their quality of life. On the other hand, individuals who do not suffer from these disabilities obviously never develop coping strategies in order to overcome suffering and/or constraints induced by a short stature or deafness. Therefore such adaptive preferences raise issues in the economic assessment of growth hormone treatment and bilateral cochlear implants. Indeed, they are likely to have an impact on the evaluation of welfare loss induced by short stature or deafness, and consequently on the evaluation of outcomes of both technologies, which itself determines their cost/effectiveness ratio.

3.3. Adaptive preferences as an issue when using cardinal or ordinal measures for health-related preferences

At first glance, it may be considered that adaptive preferences only raise issues when cardinal measures are used to assess the impact of a disability on an individual's welfare. Indeed, adaptive preferences are likely to bring variations, within the measurement of the quantity of welfare loss, according to the proxy respondent's baseline (age and/or health status). However, it is possible that the rise of adaptive preferences does not only lead to disagreement about how much worse it is to suffer from a health state compared with other health states, but also leads to disagreement about the classification of health states. If this is confirmed, a reversal in the ranking order of health states may be seen. And this is precisely what can be observed in the evaluation of cochlear implant outcomes, when taking into account testimonies collected within the deaf community within a public consultation conducted by the French National Authority for Health (HAS). Actually, part of the deaf community denies that hearing loss involves a deterioration of the quality of life. Deafness is according to them *"a linguistic specificity. It is an identity transmitted through sign language, an identity which is both shaped and articulated around the deaf community's set of values"*[67] . In short, it is an identity, and not a disease, a deficiency, nor a handicap. From this point of view, unilateral

[67] The historical and sociological context should be highlighted: the deaf community was victim of oppression for several centuries (prohibition of sign languages, unsuccessful attempts to treat deafness, eugenism) before the educational approach undertaken by Charles-Michel de l'Épée.

and bilateral cochlear implants should not be implemented because they result from "*an exaggerated and outrageous medicalization* (...) *dominated by a medical conception of deafness focused on audiocentrism"* guided by the *"fantasy of finding a cure for deafness"*[68] . Given these testimonies, it can be assumed that deaf individuals, who have not had implants, may give a null or negative value to the opportunity of hearing in stereo with bilateral implants, because they design their lifestyles and aspirations exclusively within the "non-implanted" deaf community. In contrast, deaf individuals with unilateral implants may give it a positive value, especially as they are often excluded from the deaf community. In this case, adaptive preferences are so strong that non-implanted deaf individuals may not declare any welfare loss due to deafness: in fact, they may even declare preferring to stay deaf rather than being treated. Thus, some individuals would assign positive values to an improved overall quality of sound provided by bilateral cochlear implants, leading to a lower cost/effectiveness ratio (deaf individuals with a unilateral implant, deaf individuals with bilateral implants or non-deaf individuals)[69]. Others would assign negative values leading to an increase in this ratio (non-implanted deaf individuals). Using ordinal preferences does not solve the moral dilemma raised by adaptive preferences: choosing to interview one or another of these proxy respondents could have a direct impact on the public-decision making about the coverage of the device by the national health insurance.

4. Dealing with the Moral Dilemma Raised by Adaptive Preferences

It does not seem possible to solve the dilemma arising from conflicting preferences in the evaluation of these technologies, without recourse to a normative foundation (set of values) based on a theory of justice. Indeed each group of proxy respondents (parents, adult individuals affected by these disabilities or representative samples of the whole population) can legitimately claim to possess experience that is useful in assessing the benefits of these

[68] An anonymous respondent to the public consultation of the HAS guidelines "Deaf children: family support and follow-up of children aged 0 to 6 years" (HAS, 2009, p.8): http://www.has-sante.fr/portail/upload/docs/application/pdf/2010-02/surdite_de_lenfant_-_0_a_6_ans_-_consultation_publique.pdf

[69] The group of persons with bilateral implants is necessarily small as the spread of the treatment depends on the results of economic assessments. An evaluation of the outcomes of bilateral cochlear implants, which are questioned in this paper, is needed for the economic assessment. However, data may be collected through clinical experimental surveys.

two technologies: experience of disability on the one hand and experience of the opportunities provided by good health on the other hand.

For this reason, in Section 4, I identify three options for taking into account adaptive preferences phenomenon in evaluating growth hormones and bilateral cochlear implants, while acknowledging that each option is associated with a specific theory of justice. For the results of evaluation to be used in deciding whether health insurance should fund these technologies, it is necessary that evaluation criteria are coherent with the ethical principles which the community seeks to apply when allocating health resources. Each of the three options fits in with egalitarian theories of justice. As we will see below, it is possible to find points of agreement between them. Nevertheless, they are assumed to draw on different informational bases when evaluations are being run. The research here uses the typology set out by Fleurbaey to classify the egalitarian theories of social justice and the notations he uses to highlight the differences between them (1995). I try here to draw attention to the specific problem of adaptive preferences when evaluating the opportunity of public funding for assistive technologies.

- O_i is used to indicate individual outcomes that are subject to redistribution policies, given that such outcomes may be measured using different criteria such as individual subjective well-being W_i or the range of capabilities C_i, as defined by Sen for example. His concept of capability relates to the capacity to achieve functionings, which are "*the different things a person can aspire to do or to be. Depending on their situation, a person will privilege various functionings, ranging from the most elementary – feeding oneself correctly, enjoying the freedom of escaping from inevitable diseases – to very complex activities or states – participating in community life, or having high self esteem…*" (Sen A., 2003, p.105-106)[70].
- Here I distinguish three factors for individual outcomes: resources r_i to which individuals have access, talents t_i with which they are endowed, and

[70] Sen does not give a list of those functionings. However, Nussbaum elaborated a commonly mentioned list naming eleven opportunities of functionings: *"(i) Being able to live to the end of a human life of normal length (...) ; (ii) Being able to enjoy a good health, including reproductive health, being adequately nourished, having access to an adequate dwelling (…) ; (iii) Being able to move freely from place to place (…) having opportunities for sexual satisfaction and for choice in matters of reproduction (iv) Being able to use one's senses, being able to imagine, to think, and to reason (…) ; (v) Being able to have attachments to things and persons outside ourselves (…); etc."* (Nussbaum M., 2008, pp. 120-123).

will/willpower w_i, so that individual outcomes can be described as a specific function of these three variables $O_i (r_i, t_i, w_i)$.

Resources and talent are two distinct entities. The former represents goods which social institutions control and redistribute directly among individuals (incomes, education, public services, etc.).[71] The second concerns an individual's aptitudes over which neither he/she, nor social institutions have any influence (physical characteristics, social background, etc.). As for will/willpower, it relates to a set of elements for which an individual is responsible. However, as Fleurbaey (1995) has pointed out, the perimeter of individual responsibility varies according to theories of justice (effort, willpower, personal aspirations). For the sake of simplicity, it is assumed here that public policies do not have an impact on this variable. Will is therefore left aside in the analysis of possible options for solving the dilemma raised by conflicts of preferences. This choice however is discussed in Section 5.

Public policies can use resources as an adjustment variable in order to equalise the basket of goods among individuals endowed with different talents. A differential in talents is compensated by a supplementary allocation of resources. *"Some of the resources which concern us are non-transferable. 'Talent' is the generic name for such resources. There are also, of course, some transferable resources, like money and food. We cannot physically and literally equalize all resources, and therefore a proposal to "equalize resources" requires specifying a mechanism which assigns transferable resources in such a way as to compensate people appropriately for their bundles of non-transferable resources"* (Roemer, 1985, p. 155). However, it is the ability to "buy" talent which is at stake when evaluating the opportunity of public funding for growth hormone treatment or bilateral cochlear implants. The issue is one of determining the maximum amount of resources the community or government should spend in reducing the inequality of talents which affects the living conditions of certain individuals. The answer to this question is all the more complicated given the emergence of adaptive preferences phenomena: who can legitimately judge the injustice which individuals with short stature or unilateral deafness may suffer, and to what extent should such handicaps be compensated? The answers vary depending on the nature the object whose a fair distribution in a population is to be guaranteed (*equalisandum)*. They are discussed as

[71] The notion of resources is defined differently by various authors. For Dworkin, for examples, resources include talents. He therefore distinguishes private and public resources (Dworkin, 2000). However this is not the typology used here as talents constitute a separate category in the following analysis.

follows: capabilities (Section 4.1), the chances of well-being (4.2), or basic social achievements (4.3).

4.1. The first option: improving capabilities in order to equalize the range of opportunities

4.1.1. Philosophical justifications

The first option to solve the moral dilemma raised when assessing conflicting preferences in growth hormone treatment and bilateral cochlear implants is to exclude adaptive preferences resulting from a process of resignation or habituation to impaired living conditions (Elster J., 1982; Sen A., 1991). Not excluding them would limit the chances for individuals from benefiting from policies that could improve their autonomy. This option is justified by the discussions of Sen and Elster, who both advocate giving priority to the improvement of individual autonomy even if it means giving up some welfare gains in the process.[72] *"It cannot be true that the smallest loss in welfare always counts for more than the largest increase of autonomy. There must be cases in which the autonomy of wants overrides the satisfaction of want"* (Elster J., 1982, p.233). This option fits in with social justice theories, focussed on the equality of capabilities, rather than utilitarian social justice aimed at maximizing subjective individual well-being. To highlight the philosophical issues raised by adaptive preferences phenomenon in economic assessments based on utilitarian goals, Elster recalls the fable by Jean de La Fontaine *The Fox and the Grapes*, in which a hungry fox stares cravingly at grapes in a tree which he cannot reach. To cope with this situation, the fox convinces himself that the grapes are not quite ripe and that their taste is certainly sour. So he concludes that they are not worthy of his attention. Consequently, when focusing on subjective utility, there would be no reason to help the fox to reach the grapes and calm his hunger.[73] Indeed, from a utilitarian point of view there is no welfare loss due to the grapes' inaccessibility, since the fox is now pretty sure that they are not to his liking. This hungry fox could be compared to *"the hopeless beggar, the precarious landless labourer, the dominated*

[72] J.S. Mill gives precedence to liberty and he discussed the different qualities of pleasure, in which he explained that "complete satisfaction" of desires is not in itself a goal for intelligent human beings. It leads M. Qizilbash to discuss the possibility of an agreement between J.S. Mill and A. Sen about the capability approach. According to Mill's version of utilitarianism, there would be no inconsistency in excluding adaptive preferences from the assessment while keeping a utilitarian normative framework (Qizilbash M. 2006).

[73] "*For the utilitarian, there would be no welfare loss if the fox were excluded from the consumption of the grapes, since he thought them sour anyway*" (Elster J., 1982, p.233).

housewife, the hardened unemployed or the over-exhausted coolie" that A. Sen brings to mind to discuss issues raised by adaptive preferences (Sen 1993, p.44). According to him, it would be deeply mistaken to attach a small value to the loss of their well-being because each of them manages to suppress intense suffering through survival strategies.[74] The rationale of Elster and Sen about the paradox raised by adaptive preferences seems relevant in the context of adaptive preference in health technology assessments of growth hormones or bilateral cochlear implant. As it underlined by Menzel *et al*., it would be a complete nonsense, if not morally inacceptable, that individuals suffering from a debilitating disease, who had the strength to cope and accept their fate, were made responsible for reducing their chance of benefitting from public resources covering health technology aimed at reducing their disabilities. *"While the need to adapt may be sad, the adaptation itself may often be highly admirable. In light of such laudable effort and achievement, it would be ironic, or even perverse or unjust, if disabled persons lost competitive advantage in the race for scare resources because their adaptation diminished the estimated value of curative and rehabilitative services for them."* (Menzel P. *et al*., 2002, p.2155)

4.1.2. Methodological consequences for growth hormone treatment and bilateral cochlear implant assessments

Two proposals may be put forward to adapt the assessment method to this philosophical position: a first proposal aims to adapt the traditional assessment method minimally (i.e. a minimalist version) and a second proposal aims to modify more substantially the assessment method (i.e. a maximalist version). The minimal version of the first option involves excluding from the health technology assessment preferences that would decrease the range of patients' opportunities. This miniminal version is very similar to Goodin's recommendation of "laundering" economic assessments of perverse preferences or from preferences resulting from addictive processes (Goodin R., 1986). If A is a situation where a health technology is implemented in order to limit the impact of a disability and B a situation in which the health technology is not implemented, this minimal version of the first option leads to excluding preferences of individuals who adapt themselves to the disability from the economic assessment. Here it is assumed that an individual adapts him/herself to a disability when he/she has the same level of subjective well-being $W_1(r_1, t_1, w_1)$ as an another individual W_2

[74] *"Valuing is not the same as desiring, and the strength of desire is influenced by considerations of realism in one's circumstances. Nor is valuing invariably reflected by the amount of pain if the valued object is not obtained."* (Sen A., 1987)

(r_2, $t_{2,}$ w_2), even when $r_1 = r_2$, $w_1 = w_2$ and $t_1 > t_2$. This minimalist version conforms to Lowenstein and Ubel's conclusions about the necessity of excluding preferences associated with the *"malfunction of sensory, informational or computational brain processes at the time of choice"*, or deciding to de-bias individuals' preferences in providing them accurate information (Loewenstein G. and Ubel P., 2008, p. 1805).[75]

The maximalist version of the first option involves modifying the evaluation more substantially, in accordance with the conclusions by Elster and Sen. It means evaluating the impact of the two health technologies in terms of the range of capabilities rather than in terms of subjective well-being: $C_i(r_i, t_{i,} w_i)$ is evaluated rather than $W_i(r_i, t_{i,} w_i)$, with C_i referring to the range of capabilities offered to individuals. The impact a technology has on capabilities can be assessed *via* deliberation process as suggested by Daniel's (2008),[76] or by using synthetic indicators as proposed by Hausman (2009). According to Hausman, it would be possible to build a health state classification system in which health states would be valued in terms of consequences on feelings and activity limitations within groups encompassing representative samples of the general population. *"Rather than asking respondents 'Do you prefer H_1 to H_2 , or simply 'Is H_1 better than H_2 ?' the question to ask is 'Does H_1 constrain the possibilities of living well pursuing valuable objectives more than H_2 does?' The evaluative criterion 'G' in terms of which health states should be compared and ultimately quantified is something like capacity enhancement' or put negatively 'capability constriction'"* (Hausman D., 2009, p.287).[77]

[75] Loewenstein and Ubel take the example of the distortion of want experienced by an alcoholic. *"Suppose that immediately after satisfying his craving for alcohol, an alcoholic expresses a desire to be deprived of the future opportunity to drink, but as craving returns, the individual expresses a strong desire for a drink. If one could demonstrate that the alcoholic's thinking processes were distorted by craving, one might argue that we should honor the earlier satiated, alcoholic's stated preference."* (Loewenstein G. and Ubel P., 2008, p.1805)

[76] In *Just Health*, N. Daniels proposes relying exclusively on deliberative processes to determine the impact of disability on the range of capabilities and the fairness of health care coverage. Decisions that result from such a deliberative process can be accepted as fair and legitimate when four conditions are satisfied: i) there is publicity of rationales, ii) search for relevant reasons that are properly vetted by those affected by the decisions, iii) opportunity for the revisiting decisions in the light of new evidence and arguments, and iv) assurance that these conditions are uniformly enforced. Health economics is an input in the discussions (Daniels N., 2008).

[77] Activities are classified in two groups: instrumental of daily living (IADL), which encompasses light housework, doing the laundry, preparing meals, grocery shopping, outside mobility, travel, money management and telephoning; and activity of daily living which encompasses eating, getting in/out of bed, inside mobility, dressing, bathing and toileting. However D. Hausman acknowledges that this list may vary across societies and over time.

4.1.3. Limits of the first option

It is important to note that this first option is based on a relatively paternalist ethical stand. In the minimalist version, it is about giving precedence to the preferences of some individuals (able-bodied individuals) to the detriment of preferences of other individuals (disabled individuals). In the maximalist version, individuals' preferences are no longer considered.[78] Actually, approaches proposed by Daniels and Hausman are based on a naturalistic definition of health.[79] Indeed, the last ones consider that the aim of the health care system is to improve individuals' normal functionings in order to maximise the range of opportunities that are offered to them without taking into account contingencies or specific circumstances, e.g. impairments and disability, particular life goals, particular skills. *"For the purposes of assisting the public evaluation of health states, activity limitations must be classified without references to the previous choices of individuals concerning how to live and what to try to accomplish."* (Hausman D., 2009, p.292). In the same way, Lowenstein and Ubel emphasize that decision-makers should give precedence to maintaining functioning and to maximise people's range of opportunities, independently of the particular circumstances they are experiencing. This proposal could then be open to the same type of criticism evoked by Dworkin concerning resource fetishism. *"If we decide on equality, but then define equality in terms of resources unconnected with the welfare they bring, then we seem to be mistaking means for ends, and indulging a fetishistic fascination for what we ought to treat only as instrumental"* (Dworkin R., 2000, p.14). It is possible here in fact to talk about fetishism of capabilities in as far as such a proposal consists of promoting their extension with no regard for the final well-being this may provide an individual with. As Dworkin has stressed, even though he himself takes a so-called "resourcist" approach, egalitarian theories centred on the equalisation of resources and opportunities are ultimately striving to promote individual well-being. "*If we want genuinely to treat people as equals (or so it may seem) then we must contrive to make their lives equally desirable to them or give them the means to do so, not to make figures in their bank accounts the same"* ((Dworkin R., 2000, p.14). Yet as we shall see

[78] *"In the debate about how preferences should figure in social choice, we can identify two extreme positions, between which I shall situate my own. The first position can be called* subjective welfarism*. This position holds that all existing preferences are on a part for political purposes, and that social choice should be based on some aggregation of all of them. (…) The second position can be called* platonism*. According to this view, the fact that people desire or prefer something is basically not relevant, given our knowledge of how unreliable desires and preferences are as a guide to what is really just and good."* (Nussbaum, M. 2000, p. 117).

[79] The naturalist approach holds that illness is a subnormal deviation from a bio-statistical norm of organismic functional ability (Schramme T., 2007; Boorse C., 1977). Yet, it can be argued that the the capabilities approach, put forward by Sen, is more pluralist/liberal. On the distinction between Sen and Daniels about a naturalistic definition of health see Weil-Dubuc (2012).

in the second option, taking into account the impact on well-being does not necessarily mean adopting a utilitarian approach.

The second criticism, which can be levelled at the first option in its maximalist version, concerns the difficulty of establishing (through deliberation as Daniels and Hausman in particular propose) the impact of an improvement in functional capacities with assistive technologies in terms of capabilities. Given the current model of therapeutic innovation, evaluations relate nearly always too small technical improvements rather than therapeutic revolutions. However, evaluating the impact on capabilities is far easier when the differential in the improvement between strategy A and strategy B is large. This differential could be important, for example, when evaluating the impact of bilateral cochlear implants versus no implants at all. Nevertheless, the issue here focuses on evaluating bilateral cochlear implants versus unilateral cochlear implants. Furthermore, there is a continuity in the evaluation of functional capacities which may also raise problems. For growth hormones for example, it is difficult to define the threshold in terms of a predetermined adult height, beyond which height really has an impact on the range of opportunities given to an individual. Evaluation through deliberation about the impact of a reduction in incapacity with the two technologies in terms of extension of the range of capabilities, seems difficult in practice given their marginal comparative effectiveness.

4.2. Second proposition: promoting adaptive preferences phenomena with fundamental resources allocations in order to equalize chances of welfare

4.2.1. The philosophical justification

The second option to resolve the dilemma raised by conflicting preferences over growth hormones and bilateral cochlear implants is to focus the evaluation on welfare determinants for individuals with disabilities, either *via* medical interventions (which diminish the scope of disabilities) or *via* non-medical interventions (which favour the development of coping strategies): skills enhancement, activity adjustment and substantive goal adjustment. This option relates to theories of justice which continue to place strong emphasis on subjective individual welfare as a goal of public policy, though without adopting a utilitarian ethical

position.[80] Indeed, such theories do not seek to maximise the sum of utilities but aim more at their equalisation. Moreover, they do not exclude objective evaluation of the good life, in as far as they assert that there is a link between welfare and the achievement of life goals. Scanlon, for example, puts forward the hypothesis that individual preferences are affected by a standard utility function which is based on the concept of urgent needs (Scanlon, 1975). For his part, Roemer stresses the interaction between resources to which individuals have access and the shaping of life projects whose achievement affects subjective welfare (Roemer, 1985).[81] To summarise these theories and indicate the interest they represent in solving the dilemma raised by conflicting preferences concerning the two technologies discussed here, it is useful to return to the notation put forward by Fleurbaey. If G_i stands for these life goals, then:

$$W_i(r_i, t_i, w_i) = W'_i(G_i(r_i, t_i, w_i))$$

an individual's welfare depends on the satisfaction obtained from achieving personal life goals, achievement which is conditional on the resources available to the individual, his/her talents and effort.

This philosophical position is especially interesting in dealing with the problems raised by adaptive preferences phenomena because it envisages the existence of interactions between resources, talents and life goals. The evidence-based correlation between the education level of parents and the importance given to their children's short stature may help to highlight the relevance of such extension (Singh J. *et al*.1998). According to these authors, parents with high education levels seem to have greater confidence in their children's ability to cope with physical weakness. It may then be speculated that parents' confidence will actually boost the development of these compensating strategies among children with short stature. Alternatively, it could be said that the opposite is quite intuitive: children whose parents have no confidence in their children's ability to overcome their physical characteristics are likely to have more difficulty in developing coping strategies and being happy despite their disability.[82] Following this example, it is to be expected that other determinants increase the development of compensating strategies and, thereby, increase the chances of welfare for

[80] For instance, Cohen speaks about "advantage" which "*is understood to include, but to be wider than, welfare*" (Cohen G. A., 1989, p.907).

[81] On the comparison of the different authors whose reflexions refer to these ethical framework (Arneson, Roemer, Cohen, and in a lesser extent, Sen), Cf. Fleurbaey (1995, 1996).

[82] In contrast, scientific literature indicates that every parental attempt to change the appearance of their child is interpreted by the latter as a tacit disapproval, which would affect the child's ability to cope with its situation (Sandberg D.E., 2002 ; Diekema D.S. 1990).

individuals who have suffered from disability like short stature or deafness. Maybe practicing some sport, playing a musical instrument or even travelling around the world are likely to increase the probability that children would succeed in coping with their disabilities. Indeed, such activities may promote the development of tastes and the building of life goals for which children's short size or deafness is no longer a barrier. It could also promote the development of some skills that would compensate disability. If so, it may be asked whether it is justified to allocate public resources toward extra-medical interventions rather than medical treatments once it is proved that they promote compensating strategies and contribute more efficiently to the children's welfare than medical strategies. In this prospect, it could be justified to use public funding for tutoring programs for disabled children, to provide psychological support, assistance for entering working life, or even leisure and artistic activities. By drawing on the views of Roemer, it is possible to qualify these resources, which modify individuals' life goals to the point of changing their perception of their disabilities, as "fundamental resources". "*Suppose two people have different preferences over a list of* n *goods. There must be a reason. Perhaps that reason takes the form of another good, or several goods, which the two people are consuming in different amounts. Thus if you and I have different preferences, is it not because we have different levels of endorphins, different patterns of synaptic connection, exposures to different families (which is a kind of external resource), and so on? (…) The consistency axiom I have discussed forces the resource-equalising mechanism to take into account these 'hidden resources' in deciding how to allocate the resources we see"* (Roemer J. E., 1985, pp.145-146).

This second proposition has the advantage of allowing disabilities to be identified which individuals are not able to adapt to, either because they prevent other factors of welfare from developing, or because they lead to social discrimination, so that individuals are unable to develop coping strategies that are sufficiently strong to overcome their disabilities. Actually, it has been observed that individuals adapt less easily to the degradation of their quality of life when they are suffering from specific diseases like chronic pain, mental disease (depression and anxiety) and evolving diseases (e.g. multiple sclerosis) (Dolan P. and Kahneman D., 2008). Levels of welfare of individuals with such disabilities remain low and do not tend to increase over time. *"People can never adapt to chronic pain or to mental illness – feelings that come from inside themselves rather than limitations on their external activities. The control of such suffering must be one of our top priorities."* (Layard, 2005, p. 69, cited by Qizilbash M., 2006, p. 15). The first position, in its minimalist version, leads to drawing on

individuals' preferences for good health to promote the impact of healthcare interventions which diminish disabilities. In its maximalist version, it means no longer considering the impact of technology on subjective welfare. Neither of these versions allows identification of variations in adaptive preferences concerning disabilities. Yet identifying illnesses to which individuals do not adapt is of major importance as the welfare of these individuals is *de facto* the lowest and they should be a priority for public health.

4.2.2. Methodological consequences for assessing growth hormone treatment and bilateral cochlear implants

The assumption according to which some resources would increase the occurrence of adaptive preference phenomena means that some resources like $r\alpha$, wealth, $r\beta$,, education level, $r\gamma$ access to leisure activities, etc. could help to offset the impact of less talent t_i on the subjective well-being of an individual via a change in their life goals.

$$W'_i(G_i(r_i, t_i, w_i)) = W'_i(G_i(r_i(r\alpha_i, r\beta_i, r\gamma_{i,\dots}), t_i, w_i)$$

For the economists who are in charge of the assessment, the issue is about increasing the scope of the evaluation in order to assess the impact of different resources and talents on the individual well-being. To begin with, it would be possible to assess overall well-being of disabled individuals (through recent methods of the subjective appreciation of life, which come from "happiness economics")[83] or to assess the impact of the disability in terms of welfare losses (through classical preference elicitation methods like time trade-off, standard gamble or the discrete choice method). In the meantime, general questions could be put to the same individuals about their lifestyles. The objective would be to conduct a regression analysis in order to estimate the impact of each variable $r\alpha_i$, $r\beta_i$, $r\gamma_i$, t_i, etc. (independent variables) on the dependent variable, W_i. The assessment question would be the following: given an equivalent amount of resources, is it more effective to allocate public resources toward $r\beta_i$, for instance, or is it more effective to buy an increase in talents, via the coverage of health technology by the national health insurance?

[83] Cf. World Database of Happiness ; Directed by R. Veenhoven ; http://www.worlddatabaseofhappiness.eur.nl/

4.2.3. The limits to the second option

This proposition allows the dilemma raised by conflicts of preferences to be solved by identifying extra-medical resources which favour the development of compensation strategies. It also compares the impact on individuals' welfare of these extra-medical resources, with the impact of an improvement in their functional capacities. The proposition therefore avoids "resource fetishism", as the assessment is focused on welfare. It cannot either being confused with a utilitarian approach because it takes into account objective factors involved in the genesis of preferences formation. However, this philosophical option does not justify the moral responsibility of the community dedicating resources to improving the welfare of these individuals in particular. If levels of initial well-being are similar between individuals suffering from disability and the population as a whole, for instance due to adaptive preferences phenomena, then why should differences in size or partial deafness lead to resources being allocated to disabled persons, when other people could also benefit from greater welfare with the same extra-medical resources? If the community for example finances extra support and schooling for children whose adult size is predicted to the below a certain threshold, then parents of the children whose growth is on a normal trend could also hope that their children would benefit from extra school support too. This example is quite close to the problem raised by Dworkin concerning individuals with a passion for playing the violin. One of the individuals is paraplegic and when offered the possibility of benefiting from a very expensive wheelchair, he/she prefers to be offered a Stradivarius instead of the wheelchair. He/she could argue that this would provide higher welfare than having a wheelchair. Now, if the level of initial welfare between the two individuals is equivalent and both are likely to obtain the same extra welfare by acquiring a Stradivarius, then what justifies the community offering a Stradivarius this to the first person and not the second? *"The paraplegic treats the transfer, not as the occasion to remove or mitigate his handicap, but simply as an opportunity to increase his welfare in other ways, and the other violin-lover would seem to have, in his low state of welfare, as much claim to do that as the paraplegic has."* (Dworkin R., 2000, p.62)

4.3. Third proposition: compensating disabilities in order to equalize fundamental social outcomes

4.3.1. Philosophical justifications

The third option to resolve the dilemma raised by the conflict of preferences in the evaluation growth hormone treatment and bilateral cochlear implants concerns evaluating the impact of the disability on social outcomes. This option draws on the social justice theory put forward by Fleurbaey (1995, 1996), according to which the community has a responsibility for reducing inequalities in terms of resources and/or talents, as soon as these affect the results an individual can achieve in areas of his/her life which are collectively judged to be important that Fleurbaey named "social outcomes": respect for privacy, health, education and information, wealth, power of collective decision, social integration (Fleurbaey M., 1995).[84] This theory is different from the previous ones, as it proposes an *ex post* assessment of inequality. It looks not just at life goals but also at their are effective achievement, denoted here by $A_i(G_i)$, (where A_i indicates the achievement of life goals G_i discussed above). This $A_i(G_i)$ category covers two distinct types of life goals. Indeed, Fleurbaey distinguishes between the achievement of so-called social goals R_i (SG_i), that he calls "social outcomes", and the achievement of goals which lie in the private sphere R_i (PG_i), that he calls "private outcomes". *"A boundary would be drawn between individual outcomes submitted to egalitarian care, and individual outcomes of purely private interest. (…) The precise location of such a boundary may partly be a matter of public debate"* (Fleurbaey M., 1995, p.45). Evaluating individual welfare must therefore take into account two variables: A_i (SG_i) and R_i (PG_i), such that W_i ((A_i (SG_i), R_i (PG_i)). In as far as the achievement of so-called private life goals is not the responsibility of the community, and given that private life goals affect individual welfare, then this option implies only concentrating on the equalisation of A_i (SG_i (r_i, t_i, w_i)).

The distinction between social and private goals may raise questions about the two technologies examined here. Indeed, short stature and deafness may have consequences on

[84] *"If cheerfulness is deemed a private matter, the person who is naturally sad but otherwise leads a perfectly normal life, will not be compensated with any social resources. (…) But if their social outcomes (job, income, family life, for instance) are affected, then social institutions may intervene. (…) The case of expensive tastes can be dealt with in a similar way. It is not because Louis has chosen to cultivate a taste for plover eggs and old claret that he will not receive a subsidy (…) The reasons why he develops such tastes are not to be scrutinized, unless they interfere with social outcomes (for instance, not to be threatened or manipulated would probably be a social outcome)."* (Fleurbaey M., 1995, p.52).

areas of a person's private life, such as sexual and emotional fulfilment, family life and self-esteem. Therefore it seems possible to adopt an approach based on the notion of a "defined interval" of outcomes to evaluate the impact of the two disabilities in these private dimensions as suggested by Fleurbaey. "*An intermediate degree of responsibility might also be assumed by society with respect to some private outcomes. For these outcomes, society would not guarantee equality, but would only check that their individual levels belong to some defined interval. The level they have within the interval would be a private matter, but they would give rise to a social concern, as soon as they move outside the bounds of the interval"* (Fleurbaey M., 1995, pp.45-46).

4.3.3. Methodological consequences for assessing growth hormones and bilateral cochlear implants

When following this third option, the economists in charge of the assessment would begin by evaluating the impact of short stature and unilateral deafness on social outcomes by individuals suffering from one of these disabilities. Proxies could be used: it would be possible for example to measure the impact of short stature or unilateral deafness on individuals' careers, by calculating correlation between height and individual's income or unemployment. It would also be possible to evaluate the impact of disabilities on achievements in the private sphere, by comparing marital status, the number of sexual partners, the number of children which individuals suffering shortness or deafness may have, and comparing these results with an average interval obtained for the same indicators in the population as a whole. The advantage of this option is that it allows disabilities to be distinguished according to whether compensation is an issue of justice and those which are not, without having to draw on a naturalistic conception of health. If some disabilities have no demonstrated impact on fundamental achievements, this would indicate that the community is not responsible for limiting their scope. Similarly, this option makes it possible to deal with the problem raised by the continuity of functional aptitudes. The impact of disability on inequalities in fundamental achievements according to different degrees and severities can indeed be measured empirically.[85] Short stature could thus appear as a factor of inequality only below a certain threshold, which would justify restricting the use of growth hormones when adults' size is predicted to fall below the threshold. Once such inequalities are identified,

[85] The severity of short size could be estimated with the height standard deviation score. The severity of deafness could be estimated depending on the ability to hear sounds with and without the devices: tonal loss < 20, between 21-40, between 41-70, between 71-90, between 91-119 or > 120.

then medical and non-medical interventions can be assessed, according to the impact on the reduction of inequalities in fundamental achievements. The reduction of inequalities could occur either *via* improving functional capacities or by resources favouring the development of compensation strategies. Social welfare functions could be used to evaluate the impact of different interventions on social outcomes with the use of equivalent income approach (see the work of Fleurbaey, 2005, and Fleurbaey *et al.* 2009). "*First, fix a reference talent (an endowment-insensitive solution). Second, for every individual define her 'equivalent' budget that would give her current satisfaction exactly if her talent were equal to the reference level and if she were submitted to no other tax or subsidy than a lump-sum transfer. (…). Then one may apply the leximin (i.e. lexicographic maximin) criterion to the equivalent budgets, and this is the criterion retained here"* (Fleurbaey M., 2002, p.102).

4.3.3. The limits of the third option

Two main limits can be identified. First, the choice of proxies and their ability to fully estimate impact of disability on the complex realities underlying social outcomes may be questioned. But this problem is not different to what health economists used to face: the five dimensions of Eq-5d, which is the scale used to calculate QALYs, are also proxies for the complex reality that is "perfect quality of life". Second, the statistical approach of this third option could be accused of failing to take into account individual specificities. Indeed, this third approach proposes justifying financing for medical and non-medical interventions to compensate the lesser talents of certain individuals, by basing itself on a statistical demonstration that the individuals affected by a certain diseases/conditions have a higher risk than other individuals of not attaining social outcomes, and to a lesser extent, private outcomes because of lesser functional capacities. However, some individuals may actually be disadvantaged by their small size, even though their size is greater than the threshold at which a significant impact on employability in the French population is proved statistically. Subgroups of the population could be especially affected in so far as they do jobs requiring a minimum height or because they grow up in milieus in which discrimination against small individuals is greater than within the overall population. Nevertheless, the assessment of health technology generally meets this type of criticism, as it most often requires adoption of a statistical approach. The effectiveness and adverse effect of treatments are mainly evaluated from a population perspective. The benefit/risk balance is judged to be favourable for all individuals. Yet, the risks may turn out to be greater than the benefits for a group of

individuals with specific characteristics. To assess the impact of disabilities on social outcomes, one need, as for medical evaluation to identify as precisely as possible homogenous groups of persons, so as to limit inter-individual variability within groups.

5. Discussion

These options are open to three principle criticisms. The first criticism concerns the transversality of the results that would be obtained with assessment undertaken with the methods set out above (4.1). The second criticism concerns the simplification which is carried out for the interaction between variables t and w, in the analysis of adaptive preferences phenomena (4.2). Lastly, a third criticism may be put forward concerning the risks of interference in individuals' private lives by public institutions, when the financial opportunity of interventions which favour the development of compensation strategies through the evolution of life goals is put forward (4.3). These criticisms are examined within this general discussion, and are related to the three options.

5.1. The transversal nature of evaluation results

The objective of economic assessment is to compare the use of public resources in the health sector and beyond. It is the search for such transversality which leads health economists usually to favour the use of QALYs (Quality-Adjusted-Life-Years) as effectiveness criteria in health technology assessment. This is not just about determining which health strategy is the most efficient in a particular therapeutic area (e.g. bilateral cochlear implants versus school support programmes), it also entails being able to fix priorities between different interventions between different therapeutic areas. For instance, given their respective costs and effectiveness, is it more justified for the community to pay for bilateral cochlear implants, growth hormone treatment, or for an innovative treatment for diabetes? The traditional methods based on utility assessment appear to be particularly relevant to this type of comparison, in as far as the measure for effectiveness criterion (individual subjective well-being) is common to all interventions.

Nevertheless, the three options presented above could also allow comparisons to be made between different disabilities in terms of their impact on the range of capabilities, chances of well-being and social outcomes. Indeed, the minimalist version of the first option involves

excluding from the cost/effectiveness ratio preferences expressed by individuals who have adapted to incapacity, because of the priority given to increasing their autonomy. Nevertheless, the results of the evaluation do clearly show up well in the form of a cost/QALY ratio or in the form of a numerical balance in the case of cost/benefit analysis. Such result can therefore be put into perspective with results of other health technologies assessment run in a similar way. The first option in its maximalist form consists of evaluating the impact of assistive technology for individuals with a disability in terms of their impact on the range of capabilities. The use of a composite index, as put forward by D. Hausman, makes it possible to compare different assistive technologies for different disabilities. As far as the comparability of results in the second and third options are concerned, it seems possible to envisage using social welfare functions to evaluate the impact of variations in resources and talents, *via* medical and non-medical interventions on subjective welfare (Proposition 2); or to evaluate the impact of interventions in terms of equivalent incomes corresponding to social outcomes (Proposition 3).

5.2. The interaction between talents and will/willpower in the preference adaptation phenomena

The second criticism that could be put forward concerns the simplification which was made as part of this research concerning the distinction between resources r, talents t, and will w. It has indeed been assumed that public policies cannot have an impact on individuals' will or willpower. This variable has therefore been put aside in the analysis of options which allow the dilemma raised by conflicting preferences in evaluating growth hormones or bilateral cochlear implants to be resolved. Three limits of this implication may however be underlined.

(i) First, it is possible to explain the adaptive preferences phenomena, as a result of the effort and willpower an individual may use to adapt to worsening living conditions. In other words, if two individuals have the same level of welfare, when the resources at their disposal are equivalent (r1 = r2) and individual 1 has more talent than Individual 2, who is affected by disability, *ceteris paribus*, so that W'1(G1 (r1, t1, w1)) = W'2(G2 (r2, t2, w2)), this can be explained by the difference in terms of effort and willpower between the two individuals in pursuing life goals G1 and G2, so that w1<w2. Yet, assuming this hypothesis when evaluating assistive technology does seem to be debatable from a moral

point of view, because it leads to considering individuals suffering from disability and whose level of welfare has remained weak to be responsible somehow for not adapting themselves. There is no reason to allocate public resources to them, given such a lack of effort and willpower.

(ii) Second, the adaptative preferences phenomena may be explained as the fruit of developing new talents or improving existing talents in reaction to the deterioration of certain functional capacities. Talents can indeed evolve when influenced by new circumstances, other things being equal in terms of resources and will or willpower. The enhancement of certain sensory abilities among people with disabilities is indeed a well-known phenomenon (the improved auditory skills of blind people, for example). This implies considering that if Individual 2's welfare is identical to Individual 1's, despite disability, this is, in fact, because his/her talents are not inferior to Individual 1, i.e. t1 = t2. There is then a real equivalence between W1(r1, t1, w1) and W2 (r2, t2, w2). In this case again, there is then no reason to compensate individuals suffering from incapacity, as it is assumed that they have themselves compensated for their incapacity by improving their initial endowment of talent.

(iii) Lastly, it is also possible to examine the interaction between will/willpower and resources, drawing on the work of Roemer (1985) and Cohen (1989). The latter dispute the distinction made by Rawls (1971) and Dworkin (1981, 2000) between individuals' aspirations and the resources they have, in which the formers (aspirations) only follow from individuals' responsibility and the latters (resources) follow from the community (for a discussion on this see Fleurbaey, 1996, Chap. 6). According to Roemer and Cohen, individuals are not entirely responsible for their aspirations, which determine their well-being, as they may be influenced by the consumption of certain goods. Ultimately, this hypothesis is identical to that set out in the second option concerning the impact of resources on the chances of well-being, via the evolution of life goals (4.2). The only difference is that the previous variable G_i, was introduced, representing life goals such as $W'_i(G_i(r_i, t_i, w_i)) = W'_i(G_i(r_i(r\alpha_i, r\beta_i, r\gamma_{i,\dots}), t_i, w_i)$, whereas G_i could be considered as one of the elements of variable w_i, such that $w_i(G_i,\dots)$. In this case, it would be possible to state that $W'_i(G_i(r_i, t_i, w_i)) = W'_i((r_i(r\alpha_i, r\beta_i, r\gamma_{i,\dots}), t_i, w_i(G_i,\dots))$, given that G_i may be influenced by resources $r\alpha_i$, $r\beta_i$, $r\gamma$.

5.3. The interference of the public institutions in individuals' private lives

Lastly, the final criticism which can be put forward concerns the interference of public institutions in individuals' private lives when it comes to considering the promotion of compensation strategies via certain resources (education, leisure, psychological support, programmes to help with employability). This criticism may in particular be levelled at strategies aimed at promoting social integration, emotional and sexual fulfilment and self-esteem. However, it may be asked whether there is a difference in terms of intruding in an individual's private sphere between financing medical interventions which are invasive, such as daily growth hormone injections or operations for cochlear implants, and financing non-medical interventions. Both types of intervention interfere partly with an individual's physical, psychological and/or emotional intimacy. In reality, it is individuals' free and informed consent concerning the interventions which are proposed to them – be they medical or otherwise – which prevent the intrusion of public institutions in the private sphere when it comes to compensating inequalities due to disability. Thus, if it is demonstrated that bilateral cochlear implants – when compared to unilateral implants – do clearly improve the capabilities offered to children who are deaf at birth and hence improve their chances of well-being or reduce inequalities in terms of fundamental achievements, then it would be justified to include such measures within the perimeter of health care funded by insurance. But, the decision to carry out implants on a child is made by the parents. It is the same for non-medical interventions. It is true that such liberty of choice may constitute a cost to the community.[86] Nevertheless, this leads to the classical question concerning social justice, i.e. the cost which the community is ready to incur in order to defend rights.

6. Conclusion

The analysis of the difficulties raised by interference of adaptive preferences phenomena in the growth hormone treatment or bilateral cochlear implants assessments and the identification of three options drawing on arguments developed in philosophy and normative economics about egalitarian theories of social justice may be useful in guiding the economic

[86] For example, the refusal of some parents to allow an implant following diagnosis may affect the cost/effectiveness ratio of neonatal testing for deafness at birth when such testing is evaluated in terms of improving oral language acquisition..

assessment of the two technologies discussed here. It may even help provide lessons which extend beyond the scope of these two technologies.

On the one hand, it does not seem possible to resolve the problem of conflicting preferences brought about by adaptive preferences phenomena without making normative choices. There is in fact no reason to favour the preferences of individuals suffering from a disability or the preferences of the population as a whole, because the preferences of any person are the result of adaptation phenomena: adaptation to disability or adaptation to a lack of ability. For the point of view of the assessment, the choice depends on the goals of policy redistributing health resources. As for egalitarian theories, it depends on the *equalisandum* to be studied. If the assessment is centred on the equalisation of opportunities, then the view of individuals not suffering from disability should be favoured. If the assessment is centred on the equalisation of chances of well-being, then the assessment should be based on the point of view of people suffering from the disability.

On the other hand, the analysis of the third option indicates that disabilities whose scope the community has a moral obligation to limit with medical or non-medical interventions (via the development of compensation strategies) are disabilities which have an impact on the effective social outcomes. This criterion makes it possible to identify adaptive preferencs phenomena which raise problems of social justice and those which do not, though without drawing on a naturalist conception of health. The reference to this naturalist conception of health is in fact one of the main limits of justice theories centred on the *a priori* assessment of the impact of disability on the range of capabilities depending on their functional capacities. Health technologies rarely allow persons to go from abnormal functioning to normal functioning – even when it is possible to establish a frontier between them (Canguilhem, 1966). The question to be addressed concerns the value accorded to a marginal reduction in an abnormality whose effect depends on complex variables (talents, will/willpower, resources, the capacity of an individual to develop new resources, the cultural and economic context, etc.). Moreover these variables interact with each other to such an extent that it is difficult to distinguish clearly what follows from individual responsibility (effort and willpower) and what depends on external circumstances which an individual cannot control and which may vary according to context (discrimination, socio-economic inequality, institutions) as it is found necessary in resourcist approaches (Rawls and Dworkin) or in capability approaches (Daniels). An empirical evaluation of the consequences of a reduction in abnormality individuals' social outcomes would seem to be the only real way to take into account the interaction of all variables in order to identify individuals who are truly disadvantaged by a

disability, without making an *a priori* value judgement of factors relating to their individual responsibility (effort, willpower, life goals), as well as the circumstances they have to face (cultural context, discrimination, etc.). On the basis of this criterion of justice, it thus becomes possible to determine which adaptive preferences raise moral problems that need to be taken into account by the assessor, especially by using the three options identified, and those adaptive preferences which do not raise problems.

Chapitre 3 :

*Fair Cost-Benefit Evaluation of Health Care: A Case Study of Blood Pressure Lowering Drugs in France**

**Co-authors: Brigitte Dormont (LEDa-Legos, Université Paris Dauphine), Marc Fleurbaey (Woodrow Wilson School, Princeton University), Stéphane Luchini (CNRS, GREQAM), Anne-Laure Samson (LEDa-Legos, Université Paris Dauphine), Erik Schokkaert (CORE, Université de Louvain-la-Neuve), Carine Van de Voorde (Katholieke Universiteit Leuven)*

1. Introduction

1.1. The Context

Health economic assessments are produced in order to justify fairness of public decision-making in resource allocation for types of health care. However, this goal implies that the assessment methodology is consistent with the social justice theory, which is desired by the community. Traditional economic assessment methods are based on utilitarian social justice theories according to which the aim of public resource allocations is to maximize the sum of subjective utilities ("utility" here refers to subjective individual well-being). However, since the publication of Rawls' theory about justice as fairness in 1971, new discussions about the moral principles that should guide public resource allocation have taken place. Many philosophers now agree that liberal communities, which promote a pluralistic conception of the Good, are justified in guarantying equal access to some minimal fundamental resources to every citizen while respecting their rights[87]. Egalitarian social justice theories are centred on this goal and they depart from utilitarian ones that aim to maximize the sum of subjective

[87] Rawls named his minimal fundamental resources "primary goods" comprising (i) basic rights and liberties (freedom of thought and liberty of conscience), (ii) freedom of movement and free choice of occupation, (iii) powers and prerogatives of the offices of responsibility are needed to give scope to various self-governing and social capacities of the self (Rawls J., 1971).

utilities, whatever the distribution of these subjective utilities is within the population. The development of economic assessment methods which would be consistent with this egalitarian goal is quite a challenge in the area of health care assessment as many actors (public decision makers, physicians, and patients) seem reluctant to adopt a utilitarianism ethical stand on health policies[88]. Indeed, it would be relevant for the economists to adapt their assessment methods so that they can take into account explicitly and rationally philosophical oppositions to utilitarianism and provide information to public decision-makers that would be appropriate with the value system they claim to respect.

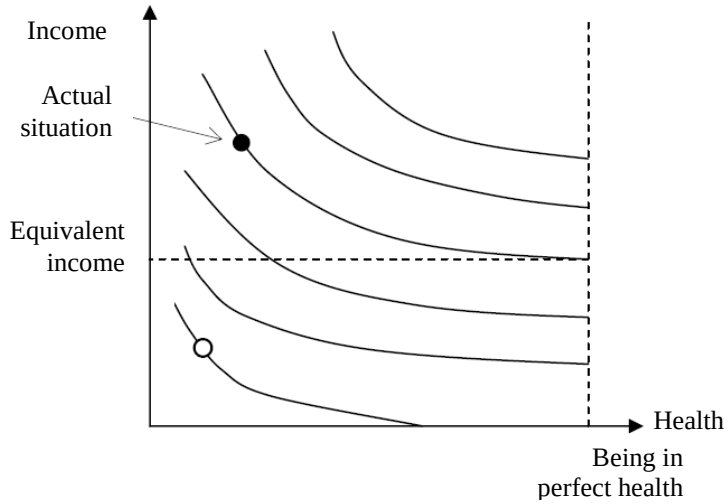
There is a growing literature about methods for setting quantitative 'equity weights' in health technology assessment. Their objective is to give more value to health gains benefiting individuals who are worst-off. However, as underlined by Cookson *et al.*, *"these methods remain at a developmental stage and have not yet been applied in practice"* (Cookson *et al.*, 2009). Most of them propose applying a set of equity weights on the results of cost/QALY studies. Those sets of equity weights may be defined on the grounds of deliberative processes aimed at determining the impact of health improvements on the range of individual's capabilities (Hausman, 2009), or they may be defined on the grounds of empirical surveys aimed at estimating the preferences of the general population about distributive concerns (Nord, 1995; Nord *et al.*, 1999; Dolan *et al.*, 2004, 2008;). Another approach has been put forward by Fleurbaey. It relies on the equivalent income concept. In contrast to previous approaches, this equivalent income approach has the merit of respecting individual preferences while including explicit distributional judgments without determining equity weights by veil of ignorance arguments (Fleurbaey, 2005). The objective of this article is to provide some evidence about the feasibility of using this equivalent income approach to highlight health technology efficiency in the context of public decision-making. We will see that real conditions under which such kinds of decision-making are submitted to do not limit the possibility to use this innovative assessment method.

1.2. The equivalent income approach

The equivalent income approach proposes assessing the impact of health policies on the distribution of both individuals' income (y_i) and individuals' health (h_i). "Equivalent income"

[88] See review of guidelines edited by the national health technology assessment agencies in Europe (NICE, KCE, IQWIG and HAS) in Chapter I.

is a notion referring to the level of income that would put the individuals in an equivalent situation, from their point of view, if they were in perfect health rather than in their actual health states. Equivalent incomes are estimated through empirical surveys where individuals are asked how much of their income they would accept to renounce in exchange of being in perfect health rather than being in their actual health states. The equivalent income is an individual welfare index $b_i(h_i,y_i)$ which encompasses two dimensions – health (h) and income (income). These two dimensions are combined according to the importance each individual gives to their health when compared with other dimensions of their lives.



Source: Fleurbaey et al. (to be published)

Unlike traditional cost-benefit analysis, the equivalent income approach are not limiting to measures the variation of incomes individuals are willing to give up, in exchange of health improvements, it compares the level of individuals incomes. With this information about the level of individuals' incomes:

(i) it is possible to compare consequences of each scenario by the means of the computation of social welfare function which is a numerical representation of different states of social ordering (see. Bergson, 1938 and Samuelson, 1947).

$$SW(b_1(h_1,y_1), \ldots, b_n(h_n,y_n))$$

(ii) it is possible to give different value to the consequences on health and incomes of each scenario, depending on the situation of each individual. Indeed, we can apply a concave function to the sum of the $b_i(h_i,y_i)$, as it is proposed by Atkinson in order to give more value to positive consequences that affect individuals who are the worst-off (Atkinson, 1970).

$SW(x) = x^{1-\rho}/ (1-\rho)$, with ρ, the degree of inequality aversion and x, the individual welfare index $b_i(h_i, y_i)$.

Applying this concave function means that an increase of $b_i (h_i,y_i)$ has less weight in the sum $b_1(h_1,y_1)+ \ldots.+ b_n (h_n,y_n)$ when $b_i (h_i,y_i)$ is high than when $b_i (h_i,y_i)$ is low. In other words, the more the individuals are worse-off, the more the improvements of their situation have weight in the aggregation of benefits induced by each scenario in terms of social welfare. The priority which is given to the worst-off depends on the degree of inequality aversion (ρ) that is chosen by the evaluator.

- when ρ=0, there is no inequality aversion included in the social welfare function assessment;
- when ρ=2, the inequality aversion is already significant;
- when ρ=3, the inequality aversion is really strong.

As the choice of the degree of inequality aversion is a normative choice, it is for the public-decision makers to decide on it as they are designated by the community to bear such kind of responsibilities. However, economists may test the impact of the different degrees of inequality aversion on the result of the assessment and present all of them to public-decision makers.

1.3. Specificities of equivalent income compared with traditional approaches

1.3.1. Using monetary valuation of health improvements

Like traditional cost/benefit analysis, the equivalent income approach implies the use of a monetary value to determine the value of health gains resulting from health technology assessment, i.e. individual's willingness-to-pay to be in perfect health. The use of monetary value in health technology assessment differs from the use of clinical criteria (life years gained, decrease of LDL-cholesterol, cancer screening rate, etc.) or quality adjusted life year criterion (QALY) that are commonly used in cost/effectiveness analyses. Oppositions to the use of monetary value in health care assessment are well-known. *"Many decision-makers might object to monetary values being placed on something as fundamental as health"* (Oliver *et al*. 2002, p. 1774). However, the choice of using a monetary value in the equivalent income approach is justified for two reasons.

(i) Firstly, the use of a monetary value is justified because it allows taking into account individual's preferences about trade-off between health and other dimensions of life. Indeed, monetary value is a common metric that is able to convert all dimensions of welfare into one dimension. It is therefore very useful to compare different kinds of public resource allocations within a health area and beyond this area. When adopting a perfectionist ethical stand is ruled out, it is then necessary to take into account these individual's preferences.[89] According to the ethical stand, what is good for one person is not necessarily good for the others. Everyone has to be free to decide what is best for them, according to their own life goals. For this reason, we cannot avoid relying on individual's preferences, i.e. to ask them what is the value they give to health improvements like an increase in mobility, a decrease physical pain, an increase of life expectancy, etc. compared with the other dimensions of their lives. *"In particular, it is hard to see how the trade-off between health and other types of consumption can be decided without relying on a particular conception of the good life, a conception that may vary from individual to individual. An individual whose plans involve a lot of physical performance is in greater need of a healthy body, someone who wants to exert authority over others is in greater need of a healthy mind, whereas someone who wants to have a Nobel prize in physics may very well operate from a wheel-chair"* (Fleurbaey, 2006). Monetary values are used in the equivalent income approach only because they are a common criterion to compare individuals' situations, according to their own preferences. However, as it is more precisely explained in Fleurbaey *et al.* (2009), it would be possible to produce cost/benefit analyses or equivalent income based analyses with another criterion than income, provided that it is a numerical criterion.

(ii) Secondly, the use of monetary value in the equivalent income approach is justified by the introduction of inequality aversion in the assessment. This introduction resolves one of the most important criticisms that are usually addressed towards the use of willingness-to-pay to value health improvements, namely its propensity to strengthen economic inequalities. Indeed, when

[89] A perfectionist ethical stand implies that it is possible to determine *a priori* what the conditions of a good life are and to set priorities between these conditions. The rejection of a perfectionist ethical stand and the adoption of a pluralistic conception of the condition of the good life are advocated by the liberal ethical stand which underlies the Rawlsian theory of justice as fairness.

inequality aversion is not taken into account, individuals with lower incomes may be penalized by the use of willingness-to-pay because they have less available income, and therefore, they have statistically smaller willingness-to pay than individuals with higher incomes. As a result, technologies aiming to treat diseases affecting specifically poorer individuals are less likely to be included in the perimeter of health care reimbursed by the national health insurance compared to technologies that would aim to treat diseases affecting richer individuals. Such consequences are, of course, unacceptable especially when the objective of universal health care systems is to provide equal access to health care whatever their incomes are. However as explained previously, the equivalent income approach aims to give priority to health and/or income increases that affect individuals who are the worst-off. The inclusion of these equity principles offsets inequalities between the willingness-to-pay of individuals depending on their incomes. Consequently, one of the most important criticisms of the use of money values no longer holds when using an equivalent income approach.

1.3.2. The equivalent income approach respects preferences but is not a utility index

It is necessary to stress that equivalent income relies on individual's preferences but it is not a utility index. Its objective is not to measure and to compare levels of subjective individual welfare across people. To assess health technology assessment with equivalent incomes aims at comparing individuals' situations regarding one of the most important dimensions of their lives (their health), and not assessing how much welfare they benefit from their health. Thus, equivalent income avoids the criticism formulated by Rawls about the monistic conception of the Good which underlies utilitarianism.[90]

However, the equivalent income approach respects the Pareto principle according to which:

- two situations yield the same social welfare function if every individual is indifferent between them,
- a situation yields greater welfare if at least one individual prefers it, while everyone else's welfare remains unchanged.

[90] Indeed, utilitarian social justice theory considers that there is only one criterion to assess a situation's goodness: the subjective happiness of individuals.

When using an equivalent income approach, an individual with bad health would always be considered as worse-off compared to a healthy individual when the latter has, at least, the same income than the former (Fleurbaey 2007, 2009).

1.3.3. Ex ante versus ex post approach

The equivalent income approach advocates using an *ex post* perspective in the assessment of inequalities which has consequences on the modelling method. It is needed to assign randomly risky events, through several draws, rather than limiting ourselves to aggregate individual risk expectations. This difference in the methodology has some impact on the results. When we use the *ex ante* perspective, individuals who present the same risk are equal, whatever their actual situation when the risky events actually occur. In contrast, when we use an *ex post* perspective, inequalities that result from pure chance are also taken into account.

> "*Suppose that only two scenarios are considered possible. In one scenario, the extreme latitudes gain and the low latitudes suffer, whereas the reverse occurs in the opposite scenario. Suppose that in either scenario the distribution of well-being is ultimately much worse than in absence of climate change. Therefore one is sure that such climate change is harmful. However, if individual expected utilities are not diminished* ex ante*, because everyone may gain or lose depending on which scenario is realized,* ex-ante *egalitarianism considers that climate change is harmless. This is strange as the same criterion considers that the change will ultimately be catastrophic"* (Fleurbaey, 2009).

Besides, in consistence with this *ex post* perspective, the impact of one or several diseases on the individuals' equivalent incomes is assessed on the grounds of preferences expressed by individuals who have a real experience with the disease. Indeed, to assess individual's equivalent income, they are asked how much of their income they would be willing to renounce in exchange of being in perfect health, rather than being in their actual health states. Usually, it is an *ex ante* perspective that prevails in health care assessment. In traditional cost/QALY or cost/benefit analyses, representative samples of the population are asked how many survival chances, how much life span or income they would be willing to exchange not to suffer from hypothetical health states (i.e. health states they have not necessarily or would not necessarily undergo). The two main arguments are as follows: 1) it is more relevant to include preferences of tax-payers to evaluate the results of health policies because they bear the costs of health care decisions; 2) it is more relevant to include preferences of members of

the general public because they are all potential patients and, as consequence, they are blind to self-interest: this would correspond more closely to the Rawlsian argument of 'veil of ignorance' (Gray *et al.* 2011). Nevertheless it is generally admitted that less informed preferences do not deserve as much respect as more informed preferences. From this point of view, *ex post* preferences would be more relevant because they result from a real experience of a disease and a more accurate knowledge about the impact the disease may have on individuals' daily lives[91].

1.3.4. The evaluation of death prevention

Due to the fact that the equivalent income approach implies using *ex post* preferences, the evaluation used for death prevention raises some methodological difficulties. Indeed, the value given by individuals to death prevention is not comprised in the answers they give to the following question (which is used to assess their equivalent income): *"how much of your income would you have been willing to give up in exchange to having perfect health during the last 12 months?"*. Actually, *ex post* preferences are preferences of individuals who survive the occurrence of medical events. Individuals who died following a medical event are not here to declare the decrease of their equivalent income. Yet, following the theoretical framework underlying the equivalent incomes approach, the decrease of their equivalent income may be easily estimated: to be dead implies that their health and their income are both equal to 0. As equivalent income is an index resulting from two dimensions, health and income, when both are equal to 0, the equivalent income is also equal to 0. It is what we implicitly do for the non-born and for individuals who died before the day of the survey aimed at assessing individual's equivalent income was conducted. It is important to underline that, because inequality aversion coefficients are included in the assessment, the fact that an individual has a higher income has no impact on the valuation of his death prevention compared with the evaluation of death prevention. The impact of different levels of income on death evaluation is off-set.

[91] It is true that *ex post* preferences may be affected by adaptive preferences phenomena because individuals tend to develop coping strategies to face deteriorations in the quality of their lives. Then we have to be particularly careful that such adaptive preferences phenomena do not bring systematic bias leading to undervaluation of the impact of some health technologies. That could happen if it turns out those adaptive preferences phenomena are more correlated with some diseases compared to other diseases. Such bias would be very counter-effective as it is likely to conceal precisely the inequalities we are trying to highlight by the means of the equivalent income approach.

2. Aim of the paper

2.1 Objectives

This study is the first empirical application of the equivalent income approach in health technology assessment. The objective is to prove its feasibility in the context of public health decision-making and to compare results obtained with this method against results obtained with more traditional assessment methods (cost/effectiveness, cost/utility, cost/benefit). For this first application, we chose to assess antihypertensive treatments for patients with essential hypertension. Patients with essential hypertension are patients with high blood pressure (over 150 mmHg) but without history of cardiovascular events (*i.e.* stroke, myocardial infarction, angina or heart failure). Prescribing antihypertensive treatments to these patients is considered as primary prevention. Indeed, these treatments aim at controlling arterial blood pressure (under 140 mmHg, blood pressure is considered as controlled, according to French scientific guidelines) and therefore at decreasing the probability of occurrence of further cardiovascular events. If blood pressure is not controlled with the first-line treatment (i.e. if blood pressure is still over 140 mmHg after three months of treatment), other treatments are prescribed untill blood pressure is controlled. In this study we will assess first-line, second-line and third-line treatments.

There are nine antihypertensive drug classes currently available on the French market. However, their effectiveness in terms of cardiovascular morbi-mortality reduction has been demonstrated for only five of them:

- diuretics (DIU),
- beta-blockers (BB),
- calcium antagonists (CA),
- angiotensin converting enzyme (ACE inhibitors),
- angiotensin II receptor antagonists (ARA II).

For the other classes of drugs (alpha-blockers, vasodilators, alpha-2 agonists, renin inhibitors) the impact on the control of arterial blood pressure has been demonstrated, but there is no evidence in terms of decrease of cardiovascular events and mortality.

To 2012, French scientific guidelines do not distinguished between these five classes of drugs for use in primary prevention for patients with no specific comorbidity (HAS, 2005).

Physicians are free to prescribe one of them or to prescribe bitherapy or tritherapy (*i.e.* a combination of two or three classes of drugs). There is just one recommendation: to prescribe a monotherapy as first-line treatment.

In this assessment, it was not possible to assess every combination of drugs in first, second and third line treatment and therefore, we chose to focus on the comparison of four strategies:

- Strategy A is the current situation. The choice of the class of antihypertensive treatments is left to the physicians.
- Strategy B is the placebo comparator. Patients are not treated with any antihypertensive treatment in primary prevention. Physicians wait for them to experience a cardiovascular event in order to prescribe an active antihypertensive treatment.
- Strategy C is, according to available data, the cheaper strategy when both the cost of the treatment and the cost of avoided medical care are taken into account (HAS, 2012). With this strategy, every patient with essential high blood pressure is treated with ACE inhibitors in first-line treatment, with a bitherapy combining ACE inhibitors-diuretics in second-line treatment and finally with a tritherapy in third-line treatment.
- Strategy D is, according to available data, the most effective strategy in terms of life years gained. Every patient with essential high blood pressure is treated with calcium antagonists in first-line treatment, with a bitherapy combining calcium antagonists-ACE inhibitors in second-line treatment and with tritherapy in third-linetreatment.

2.2 General assessment method

The comparison between the four strategies A, B, C and D relies on the comparison of four social welfare functions (SW_A, SW_B, SW_C and SW_D) in terms of individuals' equivalent incomes, each one corresponding to the situation when strategy A, B, C or D is implemented. The social welfare function SW_A, SW_B, SW_C or SW_D that dominates the three others is considered as being welfare improving: the associated strategy is therefore efficient. In order to compute each social welfare function, we need to produce an extensive assessment of the consequences of each strategy (A, B, C and D) on each individual's health, income and equivalent income within the French population.

Strategy A is already implemented for individuals with essential hypertension because clinical guidelines in France already recommend prescribing antihypertensive treatments to patients with essential high blood pressure. As a result, the five classes of antihypertensive treatments are already freely prescribed in primary prevention in France. Therefore, in order to assess equivalent incomes when strategy A is implemented, we need only to observe the current distribution of equivalent income in a representative sample of the French population.

In this study, we then have to measure the impact of implementing strategies B, C and D, rather than strategy A. Thus, we need to measure:

- the impact of different antihypertensive treatments in terms of occurrence of cardiovascular events and on mortality prevention;
- the impact of cardiovascular events on individual's self assessed health;
- the impact of costs related to each strategy on individuals' income (patients as well as taxpayers), through out-of-pocket expenses and increase or decrease of taxes due to the coverage, by the national health insurance, of antihypertensive treatments and medical care following cardiovascular events,
- the impact of cardiovascular events on individual's careers and income.

The horizon of this assessment is one year.

More precisely, given that we have adopted an *ex post* perspective, it is necessary to randomly assign risky events and assess their consequences (compare individuals' equivalent incomes with strategies A, B, C or D) rather than to compute individual risk expectations as it would be the case if we adopted an *ex ante* perspective. The methodology will therefore consist in processing to repeated draws (n= 500) aiming to:

- randomly assign cardiovascular events to all individuals with hypertension according to the probability of occurrence of these events with strategies B, C or D;
- for each draw, measure the consequences of these events for all individuals of the sample (those with and without hypertension) in terms of health and/or income and estimate each individual's equivalent income with strategies B, C or D;
- for each draw, estimate the value of the social welfare function and compare the distributions of these social welfare functions over the different draws among the different strategies B, C and D.

To process to this assessment, we use two databases:

- *The equivalent income survey* is a representative sample of the French population. It is used to estimate the impact of strategy A, B, C and D on individuals' health, income and equivalent incomes.
- *The HAS model* gives cardiovascular events risk expectations and costs. This model is a cost-effectiveness model about antihypertensive treatments in essential hypertension (cost/life years gained) funded by the French National Authority for Health (HAS).

These two databases are described more precisely in the following section.

3. The Data

3.1. The "equivalent income" survey

3.1.1. Presentation of the survey

3,331 individuals were interviewed using face-to-face interviews in November and December 2009. These individuals are representative of the French population by gender and age group (according to the quota sampling). The survey is divided into three main parts, but this study focuses on the two first sections that provide the following information.

The first part of the survey gives details about income and health status of the respondents. We have information about individual's socio-demographic characteristics (gender, age, marital status, level of education, profession, ….), level of the monthly income (before taxes) and level of the household income (before taxes). Because the household composition is known, we were able to compute individuals' equivalised income, using the OECD scale.[92] Several questions were also asked about the individual's health status. Each respondent was presented a detailed list of 45 diseases grouped in 15 categories (*e.g.*, respiratory diseases, cardio-vascular diseases, …..).

> *We will propose you a list of diseases and health problems. For each disease, you will tell us if you were affected by this disease or health problem during the last 12 months,*

[92] With this method, the equivalised income is the household income divided by the number of household members converted into equivalised adults. The conversion is made using the OECD scale. This scale gives a weight of 1 to the first adult in the household, a weight of 0,5 to the second adult and each subsequent person aged 14 and over, a weight of 0.3 to each child aged under 14.

how many times this disease or health problem affected you during the last 12 months and did you receive a treatment for this disease (or health problem), again during the last 12 months?

In addition, open-ended questions were also proposed to the respondents for each of the 15 groups of diseases, in order to identify if the respondent had suffered from another disease, not specifically mentioned on the list. Then the respondents were asked to evaluate their current health status and their health status during the last 12 months, using a visual analog scale graduated from 0 to 100 (where 100 is the best possible health status and 0 is death). Respondents were asked about their health care utilization during the last 12 months (number of visits to a GP or a specialist, number of hospitalizations,…). We also know if is the respondent has "ALD" (long-term disease) status and if so, for which disease[93]. Questions were also asked about the presence of a complementary insurance coverage (through employers or an individual voluntarily purchased insurance) and whether the individual benefits or not from the "universal medical coverage" (Couverture Maladie Universelle, named CMU and CMU-C) which provides an extensive coverage for individuals with low incomes were also asked. Finally, individuals were asked about their lifestyles (smoking habits, alcohol consumption, BMI, …)

The second part of the survey concerns retrospective health-related preferences. Respondents were asked the amount of income they would have accepted to renounce in exchange for perfect health during the last 12 months. Interviewers began by presenting them a hypothetic scenario:

Imagine that you would have no health problems during the last 12 months. In this case, you would have been in perfect health and you would have had a better quality of life.

Considering the health problems you experienced during the last 12 months, would you have prefered to avoid these health problems in exchange for a decrease of your personal income?

For individuals who answered "yes" to this question, they were asked the following question:

[93] In the French health care system, patients who suffer from one disease which is classified as being a " long-term disease" (Affection de longue durée), are 100% covered by the national health insurance for all cares related to this disease. These ALD diseases are for example diabetes, heart failure, stroke, Alzheimer's disease, …

What is the maximum monthly amount of money you would have accepted to live without in this condition (i.e. in exchange for perfect health during the last 12 months)?

Individuals who answered "no" or "I don't know" to this question were asked the following questions:

You answered "no" because:

1. *My level of personal income is already so low that I cannot imagine having less, even with perfect health.*
2. *There are other dimensions of my life that are more important than my health.*
3. *The question is too difficult to be answered.*
4. *It is not up to me to pay for health.*
5. *Other.*

For individuals who answered 3, 4 or 5, the purpose of the questions about willingness-to-pay were explained again and the question was asked once again. For individuals who answered 1 or 2, their willingness-to-pay for perfect health was considered to be 0.

The remaining parts of the survey were about prospective health-related-preferences. For instance, individuals were asked about their chances of living until 60, 70, 80 or 90 years old, about their future income and how much they would accept to exchange in order to increase their chance to live till 60, 70, 80 or 90 years old. These parts of the survey were not used in our study.

3.1.2. Some descriptive statistics

Table 1 gives the distribution of the 45 diseases declared by the individuals of our sample. Hypertension is the third disease in terms of prevalence: 19 % of individuals in the sample declare having hypertension. This figure seems much lower than the prevalence in the French population: in 2006 and 2007, according to the "Étude nationale nutrition santé", 31% of the population was diagnosed with hypertension (Godet-Thobie H., *et al.*, 2008). Data provided by MONA LISA cohort study also show that 47% of men and 35 % of women in the French population aged between 35-74 years old were diagnosed with hypertension ((MONA LISA, 2008). This under estimation of hypertension is, however, usual in surveys: many individuals are not aware that they have hypertension. For example, Dauphinot *et al.* (2006) compare the prevalence of hypertension, diabetes and cholesterol using a sample of individuals who

answered both the “Enquête décennale santé (INSEE, 2002-2003)” (in which they are asked whether they have hypertension, diabetes and/or cholesterol or not) and underwent medical exams to diagnose them for these three diseases. They show that nearly half of individuals who were diagnosed with hypertension did not declare having hypertension when they were interviewed. Prevalence of hypertension is therefore 19.1 % according to the medical exams, but only 11.4 % according to the survey data. Dauphinot *et al*. attribute this underestimation to individual’s ignorance of the disease or to involuntary omission.
Some cardiovascular diseases that can be a consequence of hypertension are also declared by individuals in the survey: 2.6 % of individuals declare a myocardial infarction over the last 12 months, 2.07 % declare an angina and 1.3 % declare a stroke. However, the presence of other diseases also identified as a consequence of hypertension (such as heart failure, renal failure or end-stage renal failure) were not asked in the survey.

Note that individuals can declare one or more diseases. Indeed, only 14 % of individuals don’t declare any disease over the last 12 months, 16 % declare only one disease and 70 % declare more than one disease (Figure 1).

Table 2 provides the basic features of the data and distinguishes three samples that will be used in the assessment: i) individuals with no hypertension (column 1); ii) individuals with hypertension and no cardiovascular event (column 2); iii) individuals with hypertension and already a cardiovascular event (column 3). Individuals with hypertension and no cardiovascular event represent about 15 % of the sample. Among them, 95 % declare being treated for their hypertension. We decided to exclude from the sample of individuals with high blood pressure (for the descriptive statistics as well as for the whole assessment), those who do not declare taking any treatment: they are considered as having no hypertension (in table 2, they therefore appear in column 1). This choice was made because, given the actual guidelines, every individual whose blood pressure is over 140 mmHg is prescribed a treatment. Thus, we considered that individuals who declare hypertension but do not declare being under treatment are under 140 mmHg and therefore they are not considered to have essential high blood pressure.

There are no significant differences between the three columns as concerns gender and income (personal or equivalised income). However, individuals with hypertension are older (64.8 and 67.2 years old *versus* 50.1 for individuals without hypertension), they also declare a

much lower self-assessed health (65.1 and even 52.2 if these individuals also have one or more cardiovascular event(s), against 72.2 for individuals without hypertension) and declare a higher willingness-to-pay. Note that the self-assessed-health is quite high in the sample of individuals without hypertension: the value of the first decile is 50, which means that only 10% of individuals declare a self-assessed-health lower than 50. In contrast, 10% of individuals declare a self-assessed-health higher than 95.

All respondents did not answer the question concerning the willingness-to-pay to be in perfect health and this non response has consequences for our whole study. Only 1,224 respondents (36.7% of the sample) answered positively to the question "*would you have preferred to avoid these health problems in exchange for a decrease of your personal income?"* and gave a positive amount of money. 797 (23.9% of the sample) answered "*I don't know*" or "*I refuse to answer"*. And among the 1,310 respondents who answered negatively (39% of the sample), 628 (18.9%) answered "No" because "*My level of personal income is already so low that I cannot imagine having less, even with perfect health"* and 201 (6%) because "*There are other dimensions of my life that are more important than my health"*. These two kinds of answers were considered as being true zeros; their willingness-to-pay is set to be 0. For those who answered "*It is not up to me to pay for health"*, the question was explained again and after that, 80 of them (2.4%) gave a positive willingness-to-pay. Finally, we analysed the open-ended questions of the 495 individuals who answered *"Other reason"* and were able to distinguish, among their answers, that the willingness-to-pay was 0 for 442 of them (13.3%).

Overall, the willingness-to-pay is defined (and is positive or null) for 2,575 individuals of the initial sample (77.3%). As the equivalent income cannot be defined for individuals who do not declare any willingness-to-pay to be in perfect health, 756 individuals were excluded from our sample. As a consequence, the sample used for the whole assessment comprises 2,575 individuals and not 3,331.

Note that this selection of individuals could create a potential bias in our assessment, and all the more so as the individuals who chose not to answer to the question about the willingness-to-pay have specific characteristics. They are more likely to be male than in the whole sample (49% against 44%, p=0.025), they are also a bit older (55 years old against 53 years old, p=0.0008) and have a higher self-assessed health (73 against 71, p=0.03). However, there is no significant difference (p=0.95) concerning the level of equivalised income. There is also no significant difference concerning the prevalence of hypertension in the sample of individuals who declare a willingness-to-pay (18.6%) and in the sample of those who do not declare any (19.9%) (p=0.42). Therefore, the selection of the only individuals who declare

willingness-to-pay should not create too much bias in our assessment. Note however that the methodology we rely on (Fleurbaey et al., 2012) to compute individuals' equivalised income (and that is explained later on), is based on an estimation that takes into account this potential selection bias.

Using this restricted sample of 2,575 individuals, we see that the average level of willingness-to-pay is quite low. Indeed, for individuals who declare no hypertension, their average willingness to pay is 79.1€ and the median is 0: 50.8% of these individuals have a willingness-to-pay to be in perfect health equal to 0. On the subsample of individuals with hypertension, this willingness-to-pay is a bit higher (91.6€ - but the difference is non significant) and it is even higher on the subsample of individuals with more health problems, i.e. for those with hypertension who experienced a cardio vascular disease over the last 12 months (142.7€). Those quite low levels of willingness-to-pay could be explained because the level of income is already low in the sample.

Table 1 Occurrence of the diseases in the sample

Disease	% of individuals	Disease	% of individuals
Anxiety	26.48	Cataract	4.65
Lumbago	26.39	Malfunction of thyroid	4.80
Hypertension	**18.97**	Urinary infection	4.26
Caries	18.97	Psoriasis	3.75
Cholesterol	16.75	Menstrual disorders	3.03
Nasopharyngitis	14.32	Menopause troubles	3.03
Arthrosis of the knee	14.11	Cancer	2.73
Gastralgia	13.06	Ulcer	2.64
Migraine	11.56	Earache	2.61
Deafness	11.38	**Myocardial infarction**	**2.58**
Acid Reflux	10.75	Handicap	2.58
Allergic rhinitis	10.42	Arteritis	2.13
Sinusitis	10.06	**Angina**	**2.07**
Depression	8.77	Throat infection	2.07
Diabetes	8.47	Overgrowth of the prostate	1.86
Heart rhytm disorder	8.35	Glaucoma	1.77
Bronchitis	8.14	**Stroke**	**1.29**
Arthrosis of the hip	7.75	Infirmity	1.11
Asthma	7.57	Epilepsy	0.75
Varicose vein	7.11	Hepatitis	0.66
Colitis	6.75	Parkinson	0.30
Hemorrhoids	5,64	Alzheimer	0.18
Eczema	5.25		

Figure 1 Number of diseases listed by the individuals in the sample

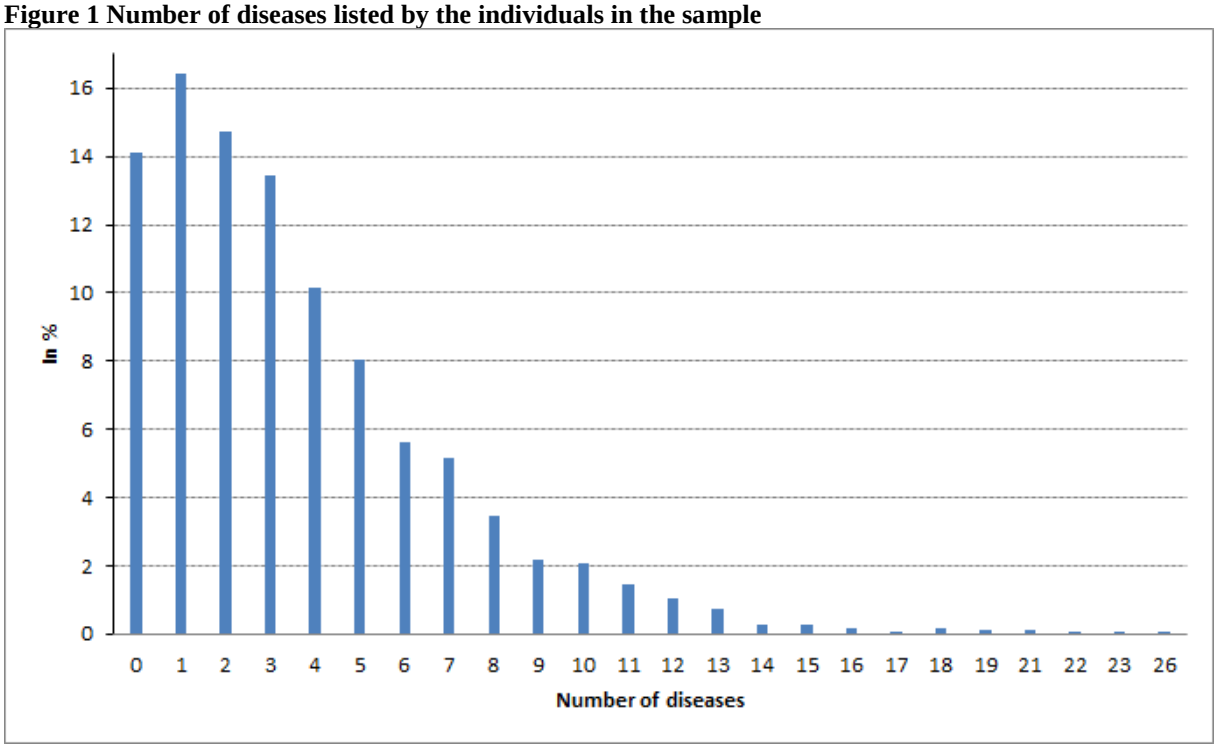


Table 2 Basic features of the data

	No hypertension (col 1)	Hypertension and no CVE (col 2)	test for signif. diff (col 1 & 2)	Hypertension and CVE (col 3)	test for signif. diff (col 1 & 3)	test for signif. diff (col 2 & 3)
% female	44.6%	41.1%	NS	60%	NS	**
Age (mean) **[D1-D9]**	50.1 [26 - 75]	64.8 [49 - 81]	***	67.2 [51 -83]	***	NS
Pers. Income (mean) **[D1-D9]**	1 335 € [500€ - 2 400€]	1 322 € [600€ - 2 300€]	NS	1 375 € [627€ - 2 250€]	NS	NS
Equivalised Income (mean) **[D1-D9]**	1 449 € [600€ - 2 400€]	1 459 € [670€ - 2 333€]	NS	1 304 € [520€ - 2 000€]	NS	**
WTP (mean) **[D1-D5-D9]**	79.1 € [0€ - 0€ - 200€]	91.6 € [0€ - 12 € - 250€]	NS	142.7 € [0€ -29€ - 500€]	**	*
SAH (mean) **[D1-D9]**	72.9 [50 - 95]	65.1 [40 - 90]	***	52.2 [20 - 80]	***	***
Equivalent income (mean) **[D1-D9]**	1 106 € [268€ - 2 027€]	1 042 € [246€ - 1 971€]	NS	655 € [187€ - 1 474€]	***	***
Number of obs (%)	2 116 (82.2%)	399 (15.5%)		60 (2.3 %)		

Notes: ***, ** and * indicate that the averages are statistically significant at the 1%, 5% and 10% levels; NS indicate that the averages are not statistically different.

3.2 The HAS model

The HAS cost/effectiveness model has been produced by IMS Health and funded by HAS. It is based on systematic literature review and consultation of a working group and a review group organized by HAS. It is validated by the HAS committees. This cost/effectiveness model provides data about the natural history of the disease, risk expectations of cardiovascular events without treatment depending on the individual's characteristics, data about antihypertensive treatment effectiveness, cost of antihypertensive treatments and costs of medical care induced by cardiovascular events.

The history of antihypertensive treatment prescription is presented in Figure 2. Three kinds of treatment can be prescribed. The first-line treatment is the first kind of treatment prescribed by physicians in order to control high blood pressure. It is by regulation a monotherapy. If blood pressure is not controlled after three months of treatment, another treatment is prescribed (second-line treatment), which is a bitherapy. If blood pressure remains uncontrolled, a third-line treatment is then prescribed. Data on the probability of controlling high blood pressure with each treatment are described later. It should be noted that "tritherapy" refers to a broad category that encompasses all tritherapies available according to their distribution in prescribing practices as it is observed in the French market share:

- beta-blockers+ARAII+diuretics;
- calcium antagonists+ARAII+diuretics;
- ACE inhibitor+ beta-blockers + diuretics;
- calcium antagonists + beta-blockers + diuretics;
- calcium antagonists + beta-blockers +ARAII

In the present study, the different kinds of tritherapies are not distinguished depending on the classes of drugs which are combined. This hypothesis is a methodological choice made in the HAS model, and it is justified by the lack of data on individual's probabilities of occurrence of cardiovascular events with each combination of tritherapy.

Figure 2 Basic model of antihypertensive treatment prescription depending on the probability of controlling high blood pressure (Source: HAS, 2012)

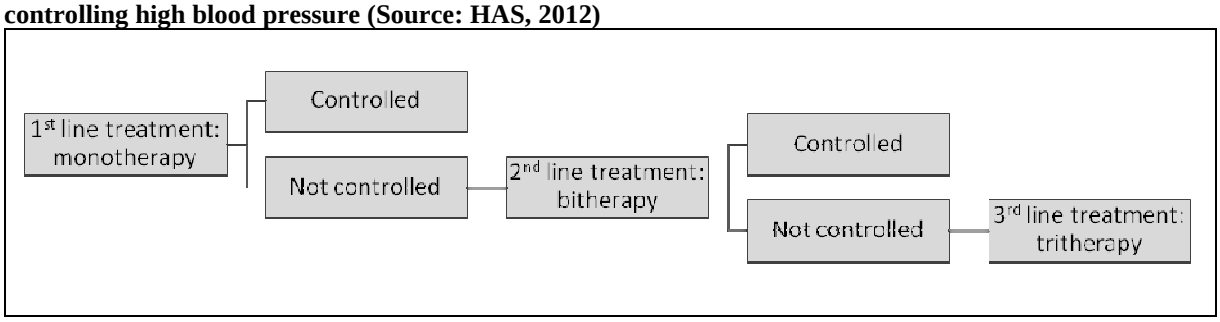
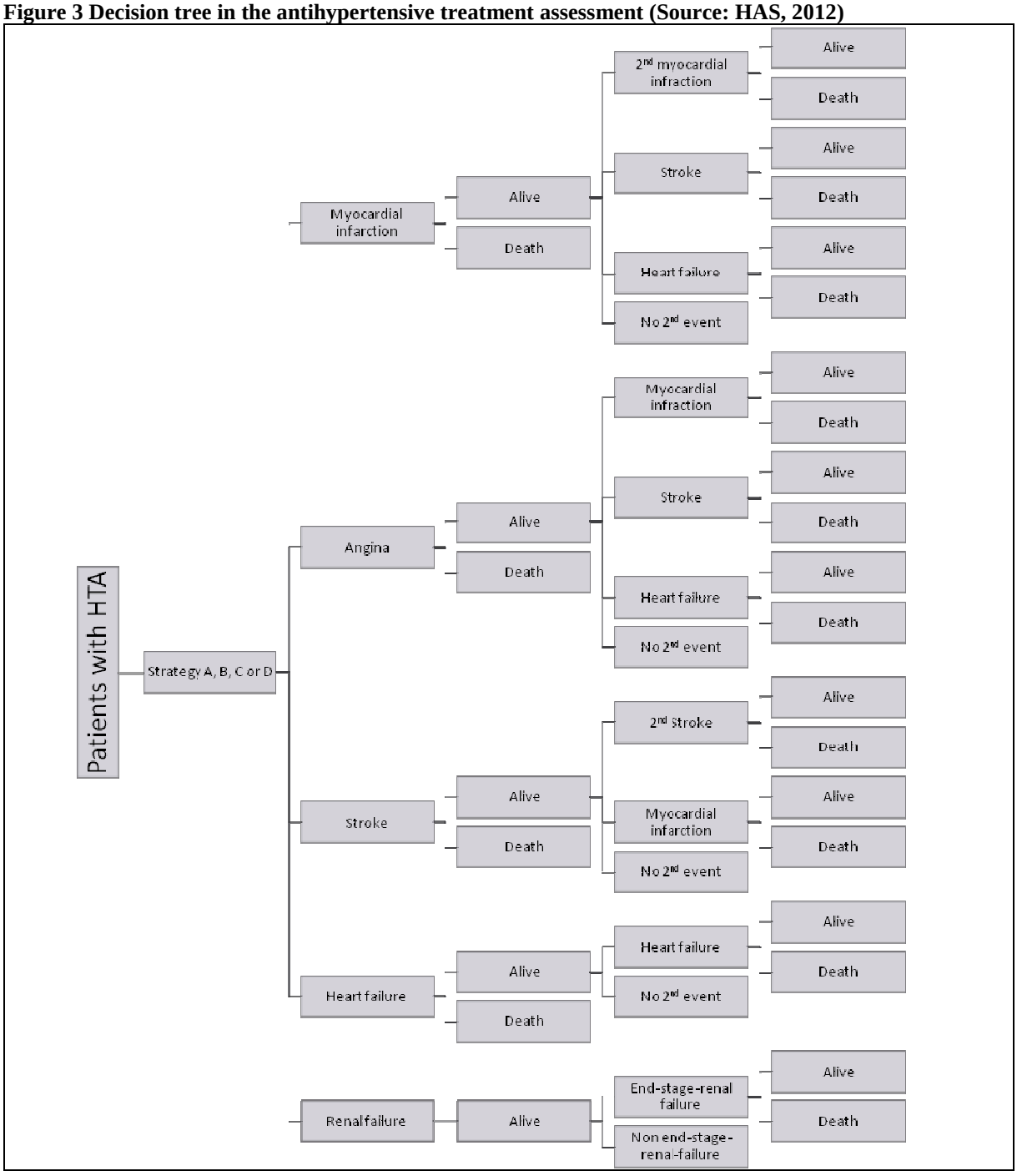


Figure 3 Decision tree in the antihypertensive treatment assessment (Source: HAS, 2012)



The HAS model gives individual's probabilities of occurrence of cardiovascular events, according to the decision tree with strategies B, C and D (Figure 3). This decision tree shows the different kinds of events that may occur for individuals with high blood pressure:

- angina and myocardial infarction, commonly encompassed in a broad category named "coronary heart disease"
- stroke
- heart failure
- renal failure
- end-stage renal failure

Figure 3 distinguishes between the occurrence of first and second events. When a first event occurs, the risk of occurrence of a second event increases. Therefore, treating patients before the occurrence of a first event, in primary prevention, has an impact on the occurrence of events in secondary prevention. Moreover, risk expectations for the occurrence of a second event depend on the nature of the first event (coronary event and/or stroke and/or heart failure).

Each event, primary or secondary, is also associated with a specific risk of death. Furthermore, risk expectations of each event (including death) vary depending on age, gender, and other individuals characteristics (being a smoker, being overweight, having diabetes,…). They also vary according to the strategy used (A, B, C or D) and for strategies C and D, according to the classes of antihypertensive treatment that is prescribed. Data about individual's risks expectations, provided by the HAS model, are synthesised in Table 3 (more details are given in the Appendix, Table 23 to Table 26).

Note that the occurrence of a first event does not only have an impact on the occurrence of a second event, but also has an impact on the treatment that is prescribed following this event. With strategy B (no treatment), a treatment will be automatically prescribed after the occurrence of a first event.[94] With strategies B, C and D, after a coronary heart disease or heart failure occurs, the treatment prescribed is automatically a combination of beta-blockers and ACE Inhibitors (except when the patient is prescribed tritherapy before these events, in that case, he stays with tritherapy that will now include beta-blockers and ACE Inhibitors).

[94] The data given by the HAS model for second cardiovascular event in the placebo arm take into account the fact that patients are now under antihypertensive treatment.

However after a stroke, the choice between the five classes of drugs is still open. For instance, an individual who was under second-line treatment before experiencing a stroke, stays under the same second-line treatment after this event.

Table 3 Collection of risk expectations of cardiovascular events with strategy B, C and D (source: HAS, 2012)

	Individual characteristics that are taken into account to measure risk expectations	**Hypotheses and limits**
Risk of cardiovascular events with strategy B	Probabilities are adjusted on age, gender, smoking habits and diabetes Data and source are presented in Appendix, Table 23 and Table 24	Probabilities are not adjusted on individual's characteristics in terms of overweight, level of cholesterol and level of arterial blood pressure because: - Information about precise level of cholesterol (total cholesterol (mg/100mL) and HDL cholesterol (mg/100mL)) and precise level of arterial blood pressure (systolic arterial blood pressure (mmHg, 60-300) was not available in the sample. - Individual's risk expectations depending on being overweight were not available in the HAS model A unique value has been used for these three variables (overweight, level of cholesterol and level of arterial blood pressure) based on the median value within the French population (Mona Lisa, 2011). It is the same value that has been used in the baseline scenario of the HAS model.
		Risk expectations have been modelled by HAS for patients between 35 and 74 years old. In our sample, individuals who declared high blood pressure are between 19 and 90 years old. To get risk expectations for individuals aged between 19-34 and aged between 75-90: - we proceed to an interpolation to estimate risks expectations before 35 years old and after 74 years old; - or we apply the probability observed at 35 years old for all individuals below 35 and the probability observed at 74 years old for all individuals above 75. The robustness of the results to these two alternative strategies is tested.
Risk of cardiovascular event with strategies C and D	Probabilities of controlling high blood pressure depend on the classes of antihypertensive treatment. Data and source are presented in Appendix, Table 26	The initial arterial blood pressure of individuals in the survey was unknown, therefore we choose to consider it is 150 mmHg for everybody (as it is the case for the baseline scenario in the HAS model). We also consider that the arterial blood pressure target is the same for all individuals in the sample: 140mmHg, which is the target when high blood pressure is below 180 mmHg (as in the baseline scenario of the HAS model). Probability of controlling high blood pressure is the same for all classes of drugs in monotherapy (Law 2003). Probabilities of controlling high blood pressure with bitherapy and tritherapy are estimated following the evidence-based hypotheses about the additive effectiveness of each class of drug (Wald *et al.* 2009). The probability of controlling high blood pressure is applied to each individual for each of the 500 draws. - once the arterial blood pressure is controlled, the patient stays with the

		same prescription over the 12 months. - therefore the cost of the treatment prescribed to each individual corresponds to the line of treatment that has been attributed to the individual in each draw to control his high blood pressure during the 12 months. In the present study, it is assumed that high blood pressure is always controlled with the third line treatment. This hypothesis is justified by the lack of data about effectiveness of quadritherapies in the HAS model.
	Probabilities of occurrence of cardiovascular events are adjusted on age, gender, smoking habits and diabetes. Data and source are presented in Appendix, Table 23, Table 24and Table 25	Data about compliance and persistence of individuals towards the antihypertensive treatments are not included in this study even if it has been included in the HAS model. This choice is justified by the time horizon of the present study which is one year. In the HAS model, data about compliance and persistence were only taken into account after 12 months of treatments.
Risk of dying following a cardiovascular event	Probabilities of dying following a coronary heart disease or a stroke are the same with strategies A, B, C and D, they are adjusted on the gender. Data and source are presented in Appendix, Table 23 Probability of dying following a heart failure depends on the elapsed time being with heart failure (Ho *et al.*,1993). In this study, they are based on risk of death for individuals who are suffering from heart failure from one year. Probabilities depend on the gender and depend on the age (30, 60, 70 or 80 years old). Absolute risk of death following end-stage renal failure is 0.13% for men and women (UKPDS 38, 1998).	These probabilities are not adjusted on the age of individuals or any other characteristics. They are the same following a first or a second event.

4. Methodology

Our methodology consists of:

- random assignment of cardiovascular events for all individuals with hypertension, according to the probability of occurrence of these events in each strategy B, C or D
- measurement of the consequences of these events in terms of health and income for all individuals in the sample, and measure the individual equivalent income in each strategy
- estimation of the total welfare change for society, with each strategy B, C and D.

These 3 steps are described in details hereafter.

4.1. Random assignment of cardiovascular events to all individuals with high blood pressure with strategy B, C and D

Given the decision tree associated with strategies A, B, C and D (Figure 3), we may distinguish 25 groups of individuals in the sample (see table 4).

Table 4 Description of different situations faced by individuals in the sample with strategies A, B, C and D

	Situations faced by individuals				Proportion of individuals concerned	
	Having HTA	Get a first cardiovascular event	Get a second cardiovascular event	Die		
Strategy A					A1[95]	$1-p_{HT}$
	X	X	(X)[96]		A2	$p_{HT}*p^{A}_{CVE}$
	X				A3	$p_{HT}*(1-p^{A}_{CVE})$
	X	X	(X)	X	A4	0
Strategy B					*B1*	$1-p_{HT}$ *(=A1)*
	X	*X*	*(X)*		*B2*	$p_{HT}*p^{A}_{CVE}$ *(=A2)*
	X	X			B3	$(p_{HT})*(1-p^{A}_{CVE})*(p^{B}_{CVE\_First})*(1-p_{DeathCVE})$
	X	X		X	B4	$(p_{HT})*(1-p^{A}_{CVE})*(p^{B}_{CVE\_First})*(p_{DeathCVE})$
	X	X	X		B5	$B3*(p^{B}_{CVE\_Second})*(1-p_{DeathCVE})$
	X	X	X	X	B6	$B3*(p^{B}_{CVE\_Second})*(p_{DeathCVE})$
	X				B7	A3- (B3+B4+B5+B6)
Strategy C					*C1*	$1-p_{HT}$ *(=A1)*
	X	*X*	*(X)*		*C2*	$p_{HT}*p^{A}_{CVE}$ *(=A2)*
	X	X			C3	$(p_{HT})*(1-p^{A}_{CVE})*(p^{C}_{CVE\_First})*(1-p_{DeathCVE})$
	X	X		X	C4	$(p_{HT})*(1-p^{A}_{CVE})*(p^{C}_{CVE\_First})*(p_{DeathCVE})$
	X	X	X		C5	$C3*(p^{C}_{CVE\_Second})*(1-p_{DeathCVE})$
	X	X	X	X	C6	$C3*(p^{C}_{CVE\_Second})*(p_{DeathCVE})$
	X				C7	A3-(C3+C4+C5+C6)
Strategy D					*D1*	$1-p_{HT}$ *(=A1)*
	X	*X*	*(X)*		*D2*	$p_{HT}*p^{A}_{CVE}$ *(=A2)*
	X	X			D3	$(p_{HT})*(1-p^{A}_{CVE})*(p^{D}_{CVE\_First})*(1-p_{DeathCVE})$
	X	X		X	D4	$(p_{HT})*(1-p^{A}_{CVE})*(p^{D}_{CVE\_First})*(p_{DeathCVE})$
	X	X	X		D5	$D3*(p^{D}_{CVE\_Second})*(1-p_{DeathCVE})$
	X	X	X	X	D6	$D3*(p^{D}_{CVE\_Second})*(p_{DeathCVE})$
	X				D7	A3-(D3+D4+D5+D6)

Notes: i) Individuals with strategies C and D are previously sorted in first, second and third-line treatment according to the probabilities of controlling high blood pressure. Therefore, there is, in fact not 25 groups but 41 groups of individuals depending on the situations faced in our simulations. However, in order to facilitate the reading of this table, it has been decided not to include the sorting of the lines of treatment;
ii) Groups B1 and B2, C1 and C2, D1 and D2 are presented in italics because they are excluded from the draws. Individuals who declare high blood pressure and cardiovascular event in the equivalent income survey (individuals belonging to group A2 and therefore B2, C2 and D2) cannot be considered as being in a primary prevention because they have a history of cardiovascular event and therefore antihypertensive treatment are still prescribed to them according to clinical guidelines. Treatment is not removed in B as it is the case for individuals

[95] Those who do not have hypertension (neither with strategy A, nor with strategies B, C and D).
[96] Those individuals may or not having experience a second cardiovascular event, there are comprised in the same group, which correspond to the individuals who are not considered to have a cardiovascular event history.

with high blood pressure but no history of cardiovascular events and treatment is not changed in C and D. Individuals belonging to group A1 are also excluded from the draws because they do not declare high blood pressure.

Given that:

- Individuals in B1, C1 and D1 do not have hypertension (neither with strategy A, nor with strategies B, C and D).
- Individuals in A2 have hypertension and they have experienced one or two (or even more) cardiovascular events.
- Individuals in A3 have hypertension but they do not declare cardiovascular event with strategy A (i.e. in the equivalent income survey).
- Individuals in A4 are dead in the sample. The proportion of people who die from cardiovascular event in state A in not observable.

Where:

- p_{HT} : proportion of individuals who have hypertension
- p^k: proportion of individuals concerned by strategy K=A, B, C or D
- p_{CVE} : proportion of individuals who get cardiovascular event(s)
    - p_{CVE_First} : proportion of individuals who get a first event
    - p_{CVE_Second} : proportion of individuals who get a second event
- The denomination CVE refers to "cardiovascular event". For simplification in the notations, we consider that all events that happen to individuals with high-blood pressure are cardiovascular ones, even if, in theory, renal failure and end-stage renal failure are not considered are cardiovascular diseases.
- p_{Death}: proportion of individuals who are dying following the event
- p_{CVE_First} includes 6 different events :
    - p_{angina}: proportion of individuals who get an angina
    - $p_{Myocardial\ infarction}$: proportion of individuals who get a myocardial infarction
    - p_{Stroke}: proportion of individuals who get a stroke
    - $p_{heart\ failure}$: proportion of individuals who get a heart failure
    - $p_{Renal\ failure}$: proportion of individuals who get renal failure
- p_{CVE_Second} includes 6 different events :
    - p_{angina}: proportion of individuals who get an angina
    - $p_{Myocardial\ infarction}$: proportion of individuals who get a myocardial infarction
    - p_{Stroke}: proportion of individuals who get a stroke
    - $p_{heart\ failure}$: proportion of individuals who get a heart failure
    - $p_{End\ stage\ renal\ failure}$: proportion of individuals who get end-stage renal failure
- P_{Death} includes:
    - p_{Death_angina}: proportion of individuals who die from angina
    - $p_{Death\_Myocardial\ infarction}$: proportion of individuals who die from myocardial infarction
    - p_{Death_Stroke}: proportion of individuals who die from stroke
    - $p_{Death\_Heart\ failure}$: proportion of individuals who die from heart failure
    - $p_{Death\_End\ stage\ renal\ failure}$: proportion of individuals who die from end stage renal failure
    - Note that $p_{Death\_renal\ failure}$ is not defined because individuals cannot die from renal failure.

- p_{HT}, $p^{A}_{CVE\ (}$= p^{A}_{angina}, $p^{A}_{Myocardial\ infarction}$ or p^{A}_{Stroke}) are observed in the equivalent income survey. We do not observe $p^{A}_{Heart\ Failure}$, $p^{A}_{Renal\ Failure}$ or $p^{A}_{end\ Stage\ renal\ Failure}$ in the sample. This point is explained later.
- Note that the probability of dying when a cardiovascular event occurs is the same in state A, B, C and D but it varies according to the nature of the cardiovascular event (Cf. infra).

Each individual in the equivalent income survey sample must be randomly assigned, for each strategy K (K=B, C or D), to one of the 5 groups (K3, K4, K5, K6 or K7), in respect with the natural history of the disease and accordingly his own specific probability (adjusted for age, gender, diabetes, being smoker). These probabilities are given by HAS's model (Cf. Table 3). Individuals are never randomly assigned to group K1 (individuals with no hypertension) and group K2 (individuals with hypertension who already experienced a cardiovascular event in the sample): we know with certainty that individuals belong to these two groups and this cannot be modified with the strategy considered.

For each strategy, to randomly assign individuals to one of the groups, we use the following methodology:

i) We draw a random variable x, that follows a uniform distribution and takes values between 0 and 1.

ii) For each strategy, the probabilities of occurrence of the events are cumulative so that they cover the [0 1] interval; the events are ranked in an arbitrary fixed order.

iii) We pick the event corresponding to the interval of probability x belongs to. For example, if $B_3^{stroke,no\ sec}+B_3^{angina,no\ sec}+\ldots+ B_5^{stroke+stroke}\leq x\leq B_3^{stroke,no\ sec}+B_3^{angina,no\ sec}+\ldots+ B_5^{stroke+stroke}+ B_5^{stroke+angina}$, then then events "non fatal stroke and non fatal angina (as a secondary event)" occur.

iv) This procedure is repeated 500 times and for each strategy.

For strategies C and D, the event that is picked gives both the line of treatment that is prescribed and the cardiovascular event that may happen.

4.2. Compute the consequences of each draw in terms of health and income and measure the individual equivalent income

Given the group each individual is assigned to, we can measure the consequences on his health (h_i) and income (y_i) in order to estimate what would be his equivalent income y^*_i. $y^*_i = b_i (h_i, y_i)$, where b is the welfare index for each individual that depends on his level of income y_i and health h_i.

The levels of health (h_i) and income (y_i) with strategy B, C and D must be simulated, using the following methodology. Firstly, the impact of the occurrence of a cardiovascular event on health is computed in terms of decrease/increase of self-assessed-health (DIFSAH). Secondly, the impact on income takes into account:

- the increase/decrease of the cost of insurance (C_I): this increase/decrease depends on the coverage of antihypertensive treatments and medical care following cardiovascular events,
- out-of-pocket payment for the antihypertensive treatment (C_T) and for medical care following cardiovascular events (C_{CVE})
- the impact of each cardiovascular event on individual's equivalized income ($DIFINC_{CVE}$).

Table 5 describes the individual level of health and income in each strategy, depending on the group the individual has been assigned to. Table 5 Computation of individual's income and heath with strategies B, C and D

	Group to which the individual belongs to:	Individual's income with strategies B, C or D	Individual's health with strategies B, C or D
Strategies B, C and D	B1, B2, C1, C2, D1, D2	$y^B_i = y^A_i - \Delta C^B_{Ii}$ $y^C_i = y^A_i - \Delta C^C_{Ii}$ $y^D_i = y^A_i - \Delta C^C_{Ii}$	$h^B_i = h^A_i$ $h^C_i = h^A_i$ $h^D_i = h^A_i$
Strategy B	B3	$y^B_i = y^A_i - \Delta C^B_{Ii} + \Delta C^B_{Ti} - DIFINC_{CVE,\_First\ i} - C_{CVE\_first,i}$	$h^B_i = h^A_i - DIFSAH_{CVE,\_First,i}$
	B4	$y^B_i = 0$	$h^B_i = 0$
	B5	$y^B_i = y^A_i - \Delta C^B_{Ii} - \Delta C^B_{Ti} - DIFINC_{CVE,\_First\ i} - DIFINC_{CVE\_,Second,i} - C_{CVE\_first,i} - C_{CVE\_Second,i}$	$h^B_i = h^A_i - DIFSAH_{CVE,\_First,i} - DIFSAH_{CVE,\_Second,i}$
	B6	$y^B_i = 0$	$h^B_i = 0$
	B7	$y^B_i = y^A_i - \Delta C^B_{Ii} - \Delta C^B_{Ti}$	$h^B_i = h^A_i$
Strategy C	C3	$y^C_i = y^A_i - \Delta C^C_{Ii} - \Delta C^C_{Ti} - DIFINC_{CVE,\_First\ i} - C_{CVE\_first,i}$	$h^C_i = h^A_i - DIFSAH_{CVE,\_First,i}$
	C4	$y^C_i = 0$	$h^C_i = 0$
	C5	$y^C_i = y^A_i - \Delta C^C_{Ii} - \Delta C^C_{Ti} - DIFINC_{CVE,\_First\ I} - DIFINC_{CVE\_,Second,i} - C_{CVE\_first,i} - C_{CVE\_Second,i}$	$h^C_i = h^A_i - DIFSAH_{CVE,\_First,i} - DIFSAH_{CVE,\_Second,i}$
	C6	$y^C_i = 0$	$h^C_i = 0$
	C7	$y^C_i = y^A_i - \Delta C^C_{Ii} - \Delta C^C_{Ti}$	$H^C_i = h^A_i$
Strategy D	D3	$Y^D_i = y^A_i - \Delta C^D_{Ii} - \Delta C^D_{Ti} - DIFINC_{CVE,\_First\ i} - C_{CVE\_first,i}$	$h^D_i = h^A_i - DIFSAH_{CVE,\_First,i}$
	D4	$y^D_i = 0$	$h^D_i = 0$
	D5	$y^D_i = y^A_i - \Delta C^D_{Ii} - \Delta C^D_{Ti} - DIFINC_{CVE,\_First\ i} - DIFINC_{CVE\_,Second,i} - C_{CVE\_first,i} - C_{CVE\_Second,i}$	$h^D_i = h^A_i - DIFSAH_{CVE,\_First,i} - DIFSAH_{CVE,\_Second,i}$
	D6	$y^D_i = 0$	$h^D_i = 0$
	D7	$y^D_i = y^A_i - \Delta C^D_{Ii} - \Delta C^D_{Ti}$	$h^D_i = h^A_i$

Notes: y^A_i and h^A_i for individuals who belong to A1, A2 and A3are given in the equivalent income survey. They do not need to be simulated.

As it as been explained earlier, individuals who belong to group A2, i.e. individuals who declare high blood pressure and cardiovascular event in the equivalent income survey, are excluded from the draws because antihypertensive treatment is not removed for them with strategy B, nor changed with strategies C and D. Therefore, their income is affected by the increase or decrease of taxes and their health status remains unchanged. The consequences of this methodological choice are analysed in the section "Results".

The table can be read as following: For instance, individuals who belong to groups B1, C1 and D1 do not have high blood pressure. Therefore, when strategies B, C or D are implemented, their income is only affected by an increase/decrease of taxes due to the removal of antihypertensive treatments for patients with high blood pressure in primary prevention and due to costs induced by medical care following cardiovascular events. Their health is the same with strategy A than with strategy B, C and D.

Individuals who belong to groups B3-B7, C3-C7 and D3-D7 have declared high blood pressure but have not declared any cardiovascular event in the equivalent income survey. They are considered as being in primary prevention, and therefore they are not prescribed antihypertensive treatment anymore with strategy B. As a consequence, when they do not die form the first or the second events, their income is affected by an increase or decrease of taxes (like all individuals in the survey) and it is also affected by the removal of out-of payment induced by antihypertensive treatment and medical care provided after a cardiovascular event. Finally, their

income may also be impacted by the occurrence of the disease due to alteration of their career. Their health is affected by the occurrence of cardiovascular events. This impact is estimated with the method described in section 4.2.2.c).

Where:

- y^K_i is the level of individual income with strategy K (K=B, C or D) and h^K_i is the level of individual health status with strategy K (K=B, C or D). These values may vary for the individual according to the group he belongs to.
- $\Delta C^K_I = C^K_I - C^A_I$ is the differential (increase or decrease) of the cost of insurance between strategy K and strategy A. Note that the cost of insurance C_I varies for each individual according to the quantile of his equivalised income;
- $\Delta C^K_T = C^K_T - C^A_T$ is the differential (increase or decrease) of the cost of antihypertensive treatment (out-of-pocket expenses) between strategy K and strategy A.

Note that the cost of antihypertensive treatment varies with the class of drugs used (cost of insurance and out-of-pocket) and therefore depends on:

  - the strategy implemented;
  - the line of treatment for strategy C and D (i.e. monotherapy, bitherapy or tritherapy).

- C_{CVE} represents the out-of pocket expenses induced by cardio-vascular events.
- DIFINC measures the impact of the cardio vascular event on the individual income.
- DIFSAH measures the impact of the cardio vascular event on the individual SAH.
- ΔC^K_I, C_{CVE}, $DIFINC_{CVE}$ and $DIFSAH_{CVE}$ vary according to the kind of cardiovascular event that occurs (angina, myocardial infarction, stroke, heart failure, renal failure or end-stage renal failure).

In the following sections, we explain in more details the computation of the different values of health and income that are listed in Table 5.

The equivalent income approach relies on *ex post* preferences about health care. Therefore, in this study, the impact of cardiovascular events that can be prevented with antihypertensive treatment was assessed *via* the level of health and income expressed by individuals who actually experienced these diseases (myocardial heart disease, angina, stroke, heart failure and/or renal disease) in the last 12 months. More precisely, data provided by the equivalent income survey was used to compute the level of health (h_i) and income (y_i) that individuals would have with strategy B, C or D.

4.2.1. Estimation of hi with strategy B, C, and D

Individuals who declared high blood pressure with no history of cardiovascular events in the equivalent income survey (group A3) are either no longer prescribed antihypertensive treatment with strategy B (groups B3-B7), or they are prescribed different classes of drug with strategies C and D (groups C3-C7 and D3-D7). Depending on the strategy, they may experience one or two cardiovascular events, which has an impact on their health. We first have to measure the impact of cardiovascular events on individual's health status, measured by the level of the self-assessed health (SAH). To do this, we faced two difficulties.

1) First, only 16.4 % of individuals in the sample declared having only one disease during the last 12 months (Figure 1). Individuals who declare diseases related with high blood pressure very often declare other diseases (like cancer, backache, depression, etc.). As their SAH reflects the impact of all diseases they declared (cardiovascular event and other diseases), we can not obtain straightforwardly the level of SAH specifically related with myocardial infarction and/or angina and/or stroke and/or heart failure and/or renal disease.
2) Second, there were only 45 closed-ended questions about diseases experienced by the individuals during the last 12 months. Neither heart failure nor renal failure and end-stage renal failure, which are diseases associated with high blood pressure, were listed in the questionnaire. Consequently, information about the decrease in the level of SAH for patients affected by one of these diseases was not available in the survey.

In order to overcome both difficulties, we elaborated an *ad hoc* methodology.

- Methodology to overcome the first difficulty

In order to compute what would be h_i with strategies B, C and D, we estimate the impact of each disease on the SAH (visual analogic scale graduated from 0 to 100) declared by the individuals in the survey. Then we use the estimated coefficients in order to simulate the SAH that individuals would have declared if they had all their current diseases as well as an extra cardiovascular event. For example, suppose an individual declares having 2 diseases: hypertension and diabetes. The estimated equation of SAH, obtained for all individuals i=1,…,N, is the following:

$$\hat{h}_i = \hat{a}_0 + \hat{a}_1 * \text{hta}_i + \hat{a}_2 * \text{stroke}_i + \hat{a}_3 * \text{angina}_i + \hat{a}_4 * \text{infarct}_i + ... + \hat{a}_{15} * \text{diabete}_i + \hat{a}_{16} * \text{athma}_i + ... + \hat{b}X$$

where X is a vector of different control variables and diseases (hta, stroke, angina,…) are dummy variables that equal 1 if the individual declared the disease and 0 otherwise.

i) Suppose the first draw assigns the individual a non-fatal stroke and no secondary event.

His SAH h_i with strategy B, C or D will be defined as:

$$\tilde{h}_i^K = \hat{a}_0 + \hat{a}_1 * \text{hta}_i + \hat{a}_2 * \text{stroke}_i + \hat{a}_{15} * \text{diabete}_i + \hat{b}X + \hat{u}_i$$
$$i = 1,..., N; K = \text{strategy } B, C \text{ or } D$$

Which is equivalent to adding the coefficient associated to stroke to his initial SAH:

$$\tilde{h}_i^K = h_i + \hat{a}_2 * \text{stroke}_i$$
$$i = 1,..., N; K = \text{strategy } B, C \text{ or } D$$

ii) Suppose now that the first draw assigns him a non-fatal stroke and then a non-fatal myocardial infarction as secondary event.

His SAH h_i with strategy B, C or D will be defined as :

$$\tilde{h}_i^K = h_i + \hat{a}_2 * \text{stroke}_i + \hat{a}_4 * \text{infarct}$$
$$i = 1,..., N; K = \text{strategy } B, C \text{ or } D$$

iii) Suppose now the first draw assigns him a non fatal stroke and another non fatal stroke as second cardiovascular event. We only count once the impact of this disease on the SAH. His SAH h_i with strategy B, C or D will be defined as :

$$\tilde{h}_i^K = h_i + \hat{a}_2 * \text{stroke}_i$$
$$i = 1,..., N; K = \text{strategy } B, C \text{ or } D$$

Note that once the draw assigns the individual a fatal event (as it is the case for individuals who belong to groups B4, B6, C4, C6, D4 and D6), the individual is dead and his health status is 0.

- Methodology to overcome the second difficulty

To make up for the lack of information about the impact of heart failure, renal failure and end-stage renal failure on SAH[97], we propose to use proxies to simulate the impact of these three diseases that are not included in the closed-ended questions. The coefficient obtained for angina is used as a proxy for the impact of heart failure and renal failure on SAH and the coefficient obtained for stroke is used as a proxy for the impact of end-stage renal failure on SAH. These hypotheses are made by comparing the utility levels associated with all cardiovascular events reviewed in technology assessment reports produced by the National Institute for Clinical Excellence (NICE) about antihypertensive treatment and renal disease management. These hypotheses are however pessimistic because the utility levels associated with heart failure and renal failure are weaker than the utility level associated with angina and

[97] Note that the same strategy will be used to estimate the impact of these 3 diseases on income.

the utility level associated with end-stage renal failure is weaker than the utility level associated with stroke (Cf. Appendix, Table 27)

4.2.2 Estimation of y_i with strategies B, C and D

In contrast with health status, when strategies B, C and D are implemented, all individuals in the sample experience a variation of income, and not only those who have hypertension. All individuals are affected, firstly, by the increase/decrease of tax due to the removal of treatment (strategy B) or the change of the treatment (strategies C and D) and, secondly, by the increase/decrease of medical care induced by the occurrence of cardiovascular events. Moreover individuals with high blood pressure and belonging to groups B3, B5, C3, C5, D3 and D5 experience cardiovascular event(s) which induce out-of-pocket payment(s). They may also experience a decrease in their income due to the impact of the cardiovascular event on their career.

To simulate the value of y_i with strategies B, C or D, we need to take into account these three quantities, that influence all individuals' incomes: a) variation of the costs of insurance C_I; b) variation of the amount of out-of–pocket payments (C_T and C_{CVE}) ; c) the impact of cardiovascular events on individuals' equivalised incomes ($DIFINC_{CVE}$).

a) The costs of insurance and the amount of out-of-pocket payments

To estimate the cost of insurance for each individual, the total cost induced by antihypertensive treatments and medical care induced by cardiovascular events, that are reimbursed by the national health insurance, have been divided between all individuals within our sample according to their participation to the national health insurance expenditures. A literature review has been conducted in order to put forward the most accurate hypotheses about the percentage of taxes related to health expenditures according to the level of household income. On the ground of this review, we decided to use data given by Caussat et al (2005) (Cf.Appendix,Table 31). The decomposition of all the costs is detailed in Table 6.

In this study, we consider that out-of-pocket expenses are directly supported by individuals without taking into account the possibility of coverage by private complementary insurance. We assumed this hypothesis because we consider that:

- individuals covered by a private complementary insurance would, in fine, support the consequences of the implementation of strategy B, C or D, *via* an evolution of their insurance premiums;
- the extent of the redistributive mechanisms depends on the nature of the insurance organization (mutual insurance *versus* stock insurance company) which is unknown in the survey. However, as redistributive mechanisms are much less important regarding expenses covered by the private complementary insurance than by the national health insurance, not to take redistributive mechanisms regarding the coverage of out-of-pocket payment *via* private complementary insurance into account will not be a bias of our results.

Table 6 Cost of insurance (CI) and out-of-pocket payments (CT and CCVE) with strategies B, C and D

		Estimation of total costs	**Repartition between costs covered by national health insurance and out-of pocket payment**
Strategy B	**Costs of antihypertensive treatments**	These costs are no longer supported by patients and tax payers in primary prevention, compared with strategy A, because the treatment is no longer prescribed: - To assess the avoided costs due to the removal of treatment, given that treatment prescribed to individual in the sample (i.e. in strategy A) is unknown, we compute a mean cost of antihypertensive treatment based on the current distribution of each class of drugs in the market share in France (Cf. Appendix, Table 28 and Table 29). - However, when a cardiovascular event occurs, individuals are prescribed an antihypertensive treatment. The cost depends on the class of drugs used, which is determined by the nature of the cardiovascular event: - after a coronary heart disease or heart failure occurs, a combination of beta-blockers and ACE Inhibitors is prescribed: these classes of drugs are associated with a specific cost of treatment (Cf. Table 29) - after a stroke, the choice of the class of drug is open: the cost of treatment, in that case, is the mean cost of antihypertensive treatment based on the current distribution of each class of drugs in the market share in France - after renal disease, no antihypertensive treatment are prescribed with strategy B.	Repartition between costs supported by tax payers and out-of-pocket payment is based on actual rate of reimbursement of antihypertensive treatment: - it is assumed that individuals with high blood pressure in primary prevention are not under ALD status for this disease: cost of treatment is reimbursed with a rate of 65% by the national health insurance; - it is assumed that individuals with high blood pressure in secondary prevention (i.e. after the occurrence of a first cardiovascular event, except if it is a renal failure) are under ALD status : the total cost of treatment is covered by the national health insurance; - it is assumed that individuals who declared being under CMU-C status do not support out-of-pocket expenses. The out-of-pocket payments for these individuals are supported by tax payers[98].
	Costs of follow-up of the treatment	The costs induced by follow-ups of antihypertensive treatment prescription are given in the HAS model and include the current cost of physician consultations, blood pressure measure, laboratory test, blood test, etc. given the conventional health care delivery recommended in HAS's clinical guidelines (HAS, 2005). Data and source are presented in Appendix, Table 30. The costs induced by follow-ups are no longer supported by patients and tax payers in primary prevention because the treatment is not prescribed	Repartition between costs supported by tax payers and out-of-pocket payment is based on actual rate of reimbursement of antihypertensive treatment. Out-of-pocket payments are estimated in the HAS's model, it takes into account extra fees paid by individuals when they consult a physician who overbills: - it is assumed that individuals with high blood pressure in secondary prevention are under ALD status : the total

[98] In the survey sample, only 8 individuals declared being covered by CMU-C and declared high blood pressure and no cardiovascular event (groups 6, 8, 12, 14, 18 and 20), i.e. 0.24% of individuals in the sample.

		anymore in primary prevention. However, these costs are supported by indivdiuals when antihypertensive treatments are prescribed in secondary prevention, i.e. after a first cardiovascular event occurred.	cost of follow-up is covered by the national health insurance - it is assumed that individuals who declared being with CMU-C do not support out-of-pocket expenses. The out-of-pocket payments for these individuals are supported by tax payers.
	Cost of cardiovascular events	Costs of medical care induced by cardiovascular events are estimated with data given in the HAS model. Data and sources are presented in Appendix Table 30. They include: - costs for fatal and no fatal hospitalizations for each event; - costs of follow-up for each event. When an individual experiences two identical cardiovascular events on the time horizon of the study (e.g. two myocardial infarction), the total cost encompasses: - costs of the two hospitalizations, - cost related to follow up associated with the medical event on the 12 months. - cost of antihypertensive treatment and cost of follow up. When an individual experienced two different kinds of cardiovascular event (e.g. one myocardial infarction and one stroke), the total cost encompasses: - cost of hospitalization for the first and for the second event - six months of follow ups associated with the first event and six months of follow ups associated with the second event. When an individual dies after the first cardiovascular events (e.g. after a stroke), the total cost encompasses only the cost of hospitalization for this event.	The total costs of medical care induced by cardiovascular events are supported by tax payers because it is considered that individuals who experience such event are systematically under ALD status.
Strategy C and strategy D	**Cost of antihypertensive treatments**	The total costs of antihypertensive treatments with strategies C and D are estimated with data given by HAS model about daily costs of each classes of antihypertensive treatment C and D. Data and source are presented in Table 29. - To estimate the increase/decrease of individual's income due the change of treatment from strategy A to strategy C and D, we estimate the differences between the mean cost of antihypertensive treatment according to the market share in France (Cf. Table 28 and Table 29) and the cost of antihypertensive treatment with strategies C and D. - The costs of antihypertensive treatments with strategies C and D depend on the line of treatment by which high blood pressure is	Idem strategy B

		controlled (1st, 2nd and 3rd line). As it has been explained for costs of treatment with strategy B: - when an individual experiences myocardial infarction, angor or heart failure, he is systematically prescribed betablockers and ACE inhibitors: - when an individual experiences a stroke, he keeps the same treatment he was prescribed before the event; - Besides, when an individual experience myocardial infarction, angor or heart failure and was prescribed tritherapy before the event occurs, he keeps the same treatment: ▪ we just consider that the tritherapy now includes betablockers and ACE inhibitors. ▪ however, as we do not distinguish tritherapy depending on the combination of classes of drug, this change of tritherapy do not have an impact on total costs of antihypertensive treatment.	
	Cost of follow-up	Idem strategy B	Idem strategy B
	Cost of cardiovascular event	Idem strategy B	Idem strategy B

c) The impact of cardiovascular event(s) on individuals' equivalized incomes ($DIFINC_{CVE}$).

We suppose that cardiovascular events do not have an identical impact on all individuals. Individuals who are retired (28 % of the sample) already receive a pension: an extra cardiovascular event should not have any impact on the amount of their pension. For them, we suppose that $DIFINC_{CVE}=0$.

Unemployed individuals (9.7 % of the sample) also receive a pension. We assume that the extra cardiovascular event does not help them to find a job. Therefore they will keep receiving the same amount of pension. For them, we also suppose that $DIFINC_{CVE}=0$.
Housewives/househusbands (6 % of the sample) also receive a pension. We suppose that the extra cardio vascular event does not have any impact on their work status and that they will continue to receive the same amount of pension. As a consequence, we also suppose that $DIFINC_{CVE}=0$. Therefore, we suppose that the cardiovascular event only affects individuals who are employed: those individuals may experience a decrease in the level of their income, following one or two cardiovascular events. For these individuals, $DIFINC_{CVE}$ has to be measured.

We choose to assess the impact of cardiovascular events on equivalized income (according the OECD scale) rather than on personal income for consistency with Fleurbaey *et al.* (2012), as we use their methodology to compute the level of individual's equivalent income. They use the equivalised income in their estimations as this is a better indicator to take into account inequalities in the standard of living.

To estimate the impact of cardiovascular events on individuals equivalised incomes, it would seem natural to proceed in the same way as for the impact on health status: estimate the impact of each disease on the level of equivalised income and then use the estimated coefficients to simulate the level of income that the employed individuals would have if they had all their diseases and an extra cardiovascular event. However, in such estimation the different diseases have no significant impact on the level of income, which is quite surprising. We therefore use a more indirect approach.

Our methodology is the following:

- First, we estimate the impact of SAH on individuals' equivalised incomes (on the subsample of employed individuals) :

$$\hat{\log}(equiv.income) = \hat{b}_0 + \hat{b}_1 * h_i + \hat{c} * Z + \hat{u}_i$$
$$i = 1,..,N(employed)$$

Where h_i is the level of SAH and Z is a vector of control variables.

- Secondly, we use the simulated SAH associated with strategy K (K=B, C or D), defined in section 4.2.1 and denoted $\tilde{SAH}_i^K$, to estimate the impact of this SAH on individual's equivalized incomes.

$$\tilde{\log}(equiv.income) = \hat{b}_0 + \hat{b}_1 * \tilde{h}_i^K + \hat{c} * Z + \hat{u}_i$$
$$i = 1,..,N(employed); K = B, C \text{ or } D$$

Such strategy has important limitations. First, this methodology does not allow us to take possible early retirements into account. In our estimation, we consider that all employed individuals stay employed during the year and keep receiving a salary (even if this salary is reduced). However, an individual close to the legal retirement age could choose to retire earlier following his cardiovascular event(s). In that case, he would receive a pension and not a salary anymore. Panel data would allow us to analyse retirement decisions after a cardiovascular event and randomly assign early retirement to some employed individuals of the sample. However, given that we use cross-sectional data, we cannot overcome this limitation. Second, due to sample size, we cannot use a more flexible specification that would include interactions terms (between SAH and gender or age for example). Thirdly, we must keep in mind that the estimated equation has no causal interpretation: we are only interested in measuring the correlation between SAH and income and we cannot interpret it as a relation of causality between these two variables. To do so, we should have instrumented the SAH variable, which is not straightforward (we have no natural instruments) and seemed useless (as this is not the scope of the paper).

4.2.3. Estimation of y^*_i with strategy B, C, and D

On the ground of the computation of y_i and h_i , we will be able to compute the equivalent income of each individual in the survey. To do so, we use the semi parametric estimation of equivalent income made by Fleurbaey and al. (2012) which has been performed on the same sample. In this paper, they estimate the functional form between SAH and income that

underline the indifference curves. We directly use the estimated coefficients (that can be found in their table 1): for each individual, given his SAH and his level of equivalised income (in each situation A, B, C or D), we would obtain the level of his equivalent income.

For example, for males, the equivalent income in situation A can be computed as follows:

$$\hat{y}_i^{*A} = (0.657 + 0.0079 * age) * \left(h_i^{'A} - \left(h_i^{'A}\right)^2\right) + (4.059 - 0.0161 * age) * \left(\left(h_i^{'A}\right)^3 - \left(h_i^{'A}\right)^2\right) +$$
$$(-1.368 + 0.020 * age) * \left(y_i^{'A} - y_i^{'A}\left(h_i^{'A}\right)^2\right) + (0.601 - 0.009 * age) * \left(\left(y_i^{'A}\right)^2 - \left(y_i^{'A}\right)^2\left(h_i^{'A}\right)^2\right) +$$
$$(0.024 - 0.001 * age) * \left(\left(y_i^{'A}\right)^3 - \left(y_i^{'A}\right)^3\left(h_i^{'A}\right)^2\right) + (8.749 - 0.087 * age) * \left(y_i^{'A} h_i^{'A} - y_i^{'A}\left(h_i^{'A}\right)^2\right) +$$
$$(-2.401 + 0.035 * age) * \left(\left(y_i^{'A}\right)^2 h_i^{'A} - \left(y_i^{'A}\right)^2\left(h_i^{'A}\right)^2\right) + y_i^{'A}\left(h_i^{'A}\right)^2$$

where $y_i^{'A} = y_i^A / 1000$

and $h_i^{'A} = h_i^A / 100$

This formula is very flexible as it is a polynomial function of health and income and coefficients vary according to the age and gender of the individual. We use the same formula to calculate the equivalent income in situations B, C and D. In that case, $h_i^{'A}$ and $y_i^{'A}$ are replaced by, respectively, $\tilde{h}_i^{'B}, \tilde{h}_i^{'C}$ or $\tilde{h}_i^{'D}$ and $\tilde{y}_i^{'B}, \tilde{y}_i^{'C}$ or $\tilde{y}_i^{'D}$.

4.3. Estimate and compare, for each draw, the total welfare change for society

4.3.1. Method used to assess and compare social welfare functions

In order to measure social welfare functions with each strategy and include inequality aversion, we rely on an Atkinson function (Atkinson, 1970), such as:

$$SW_K = \frac{1}{1-\rho} \sum_i (y_i^{*K})^{1-\rho}$$

where:

- ρ is the degree of this inequality aversion and we choose ρ={0, 1, 2, 3};
- y^*_i is individual's equivalent income;
- K indicates the strategy (K=A, B, C or D).

For ρ=1, the value of the social welfare function is computed using the following formula[99]:

$$SW_K = \sum_i \ln\left(y_i^{*K}\right)$$

We are able to compute the value of the social welfare function with strategy A but no unique value of the social welfare function for strategies B, C and D as we only have, for these strategies, the distribution of the equivalent income over the 500 draws. Therefore, we can only study the distribution of the values taken by the social welfare functions for the three strategies B, C and D.

We compare the distribution of the social welfare functions (obtained on the 500 draws) of strategy B to the one of strategy C, the one of strategy B to the one of strategy D but also the one of strategy C to the one of strategy D, with different degrees of inequality aversion. We use tests of stochastic dominance in order to test the efficiency of the different strategies. If we have two distributions X and Y, we consider that X is efficient (or welfare improving) if the cumulative distribution function of X first order stochastically dominates the cumulative distribution function of Y. To implement these non parametric stochastic dominance tests, we follow the methodology used by Lefranc *et al.* (2004), based on Davidson and Duclos's work (2000). The way we test for stochastic dominance could be improved. For the moment, we consider that the different cumulative distribution functions of social welfare functions obtained with the different strategies are independent from each others. But they are not, as we draw observations for the same underlying distribution. Therefore, we should rather apply a test that tests for stochastic dominance between two correlated distributions, which we have not yet performed.

4.3.2. Specificities about the comparison of the different strategies

In this assessment, we choose to compare the different strategies A, B, C and D in this way:

- We compare strategy A to strategy B;
- We compare strategy B to strategies C and D;
- We compare strategy C to strategy D.

But we choose not to compare strategy A to strategies C and D.

The justification is the following. As it had been explained earlier, we excluded from our simulations individuals with hypertension and history of cardiovascular event(s) because we

[99] When y_i^{*K} equals 0, we simply compute : $SW_K = \sum_i \ln\left(y_i^{*K} + 1\right)$

considered that they were not in primary prevention. We applied risk expectations to individuals who do not have cardiovascular events with strategy A so that we obtained extra events. At the same time, we kept the number of events that had been declared by individuals with strategy A. By doing this, we systematically have more cardiovascular events in the whole sample with strategies B, C and D than with strategy A. This is a big bias because perhaps strategy A is less effective than strategy C and D and it is not possible, following our method, to obtain such conclusion.

For this reason, we cannot compare strategy A to strategies C and D without performing the simulations in a different way. We would need to randomly assign cardiovascular events among all individuals with hypertension rather than among the subsample of individuals with hypertension but no cardiovascular events in the last 12 months. In that way, we could observe that some individuals do not experience a cardiovascular event with strategy C or D even if they declared one with A. This is the only way to compare the social welfare functions associated with A, B, C and D.

In contrast, it is consistent to compare social welfare functions associated with strategies B, C and D when cardiovascular events are randomly assigned among individuals with hypertension but without history of cardiovascular events. Indeed, the number of cardiovascular events that has been declared by individual in the equivalent income survey (i.e. with strategy A) does not matter for the comparison of B, C and D as it remains the same with these three strategies.

It is also consistent to compare social welfare functions with strategies A and B because we know that individuals with placebo have an increased risk of experiencing a cardiovascular event. The question is: how many more events will they experience with the placebo? However the comparison between strategy A and strategy B is less robust than the comparison between strategies B, C and D for two main reasons:

- Firstly, in the sample (strategy A), diseases are declared by individuals whereas; with strategy B, diseases are based on objective probabilities and are not self-declared. Conversely, when comparing strategies B, C and D, we only compare occurrence of diseases based on objective probabilities.
- Secondly, when excluding from the drawing sample individuals who already declare a cardiovascular event with strategy A, we implicitly exclude individuals who presented

the biggest cardiovascular risks due to clinical determinants and/or due to socioeconomic determinants (individuals with low socioeconomic status or with smoking/drinking habits may be the same than those who have less adherence to the treatment). Indeed, these individuals presented a much bigger cardiovascular risk as they experienced an event even when they were treated with antihypertensive treatments.

For this reason, we will here compare strategy A to strategy B but we are aware that there are possible biases. In particular, we see that there are significant socioeconomic differences between the two samples (the one composed of individuals with hypertension and no cardiovascular event and the one composed of individuals with hypertension and a cardiovascular event in state A). As shown in table 2, individuals who have a cardio vascular event in state A are more likely to be female, they also have a lower level of equivalised income, a lower self assessed health and a higher willingness-to-pay.

The reason why the restrictive assessment has been favored, at least at the state of this work, is that randomly assigning events among all individuals who declared hypertension raises some methodological issues. Indeed, this means that some people who declared a cardiovascular event in the sample (*i.e.* with strategy A) may not experience this event in B, C or D, or may experience another event. As a consequence, some hypotheses need to be assumed to proceed to the global comparison between strategies A, B, C and D:

- We would need to take into account that some individuals could have heart failure, renal failure or end-stage renal failure in A (and, have it, or not, in B, C or D). As these diseases are not declared in the survey (*i.e.* in A), we face the same bias explained above: the number of events with B, C and D can only increase compared with A. Therefore we would have to simulate the number of individuals who would have had heart failure, renal failure or end-stage renal failure with strategy A, which is not an easy task as epidemiological data are lacking[100].
- We cannot estimate the number of people who died with strategy A. It is again the same bias as there would necessarily be more death with B, C and D than with A.

[100] Moreover we would need to simulate the number of individuals who would have declared health failure, renal failure or end-stage renal failure in the sample if the disease was included in the detailed list of diseases and not to simulate the number of individuals who would have one of the three diseases.

- As it has been explained earlier, when comparing strategy A with strategies B, C and D we would compare a distribution of event that have been declared by individuals (strategy A), with a distribution of event that would objectively happen (strategies B, C and D) .

Finally, the problem met in the comparison of strategy A with strategies B, C and D can be summarized as follows. When comparing A with the other strategies, we produce original effectiveness data based on the equivalent income survey, when such data have not been collected on that purpose. When comparing only B, C and D, we just are using data coming from validated scientific literature in order to perform statistic simulations. The data about effectiveness of treatments may not be questioned.

Given the huge number of hypotheses that should be put forward in order to evaluate all strategies (strategy A *versus* strategy B *versus* strategy C *versus* strategy D), we chose, first, to produce results that only compare strategies B *versus* C *versus* D as well as strategy A *versus* B. The comparison of all strategies is left for future research.

5. Results

5.1. Random assignment of cardiovascular events for all individuals with hypertension, according to the probability of occurrence of these events in each strategy B, C or D

Figure 4 to Figure 6 show the distribution of cardiovascular events that occur with each of the three strategies. Overall, cardiovascular events are less frequent with strategy C than with strategy D (the median is 10 with strategy C but 12 with strategy D) and with strategies C and D than with strategy B (for which the median equals 18), in accordance with the relative risks of cardiovascular events associated with the different classes of drugs (Cf. Appendix, Table 25).

Figure 4 Distribution of the number of cardiovascular events with strategy B

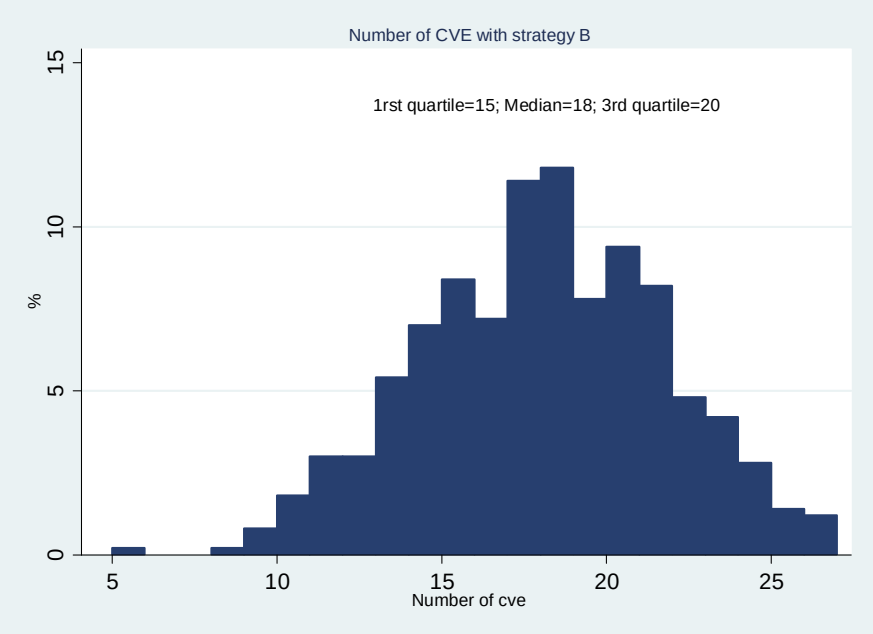


Figure 5 Distribution of the number of cardiovascular events with strategy C

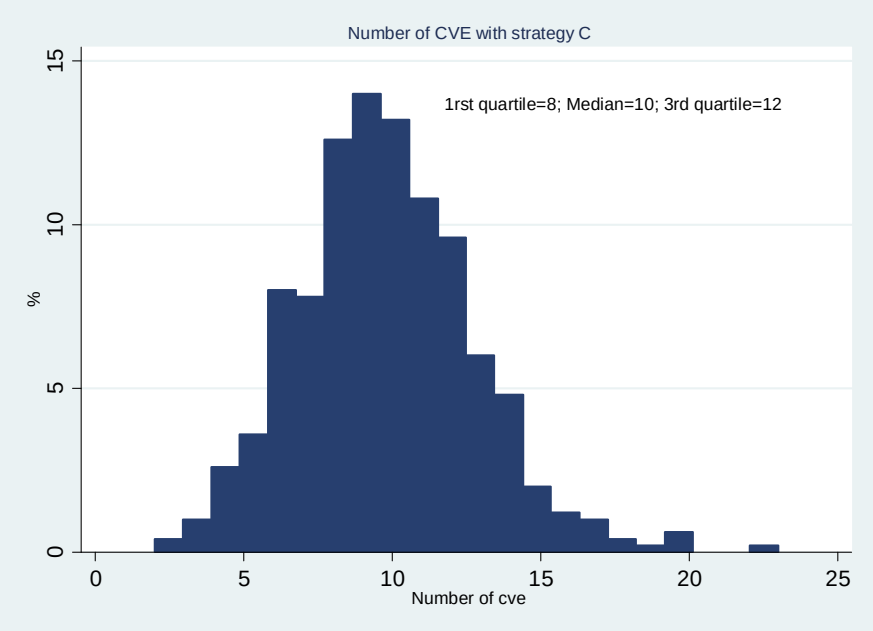
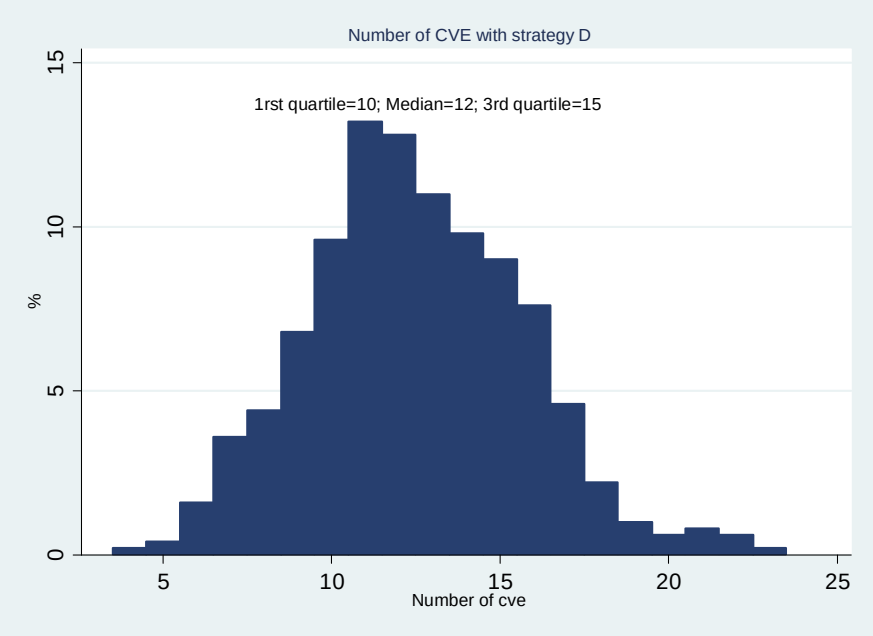


Figure 6 Distribution of the number of cardiovascular events with strategy D



In the appendix, we present figures that decompose the number of cardiovascular events for each strategy: they represent the distribution of each cardiovascular event that occurs at least once during the 500 draws with strategy B (Table 16), strategy C (Table 17 to Table 19) and strategy D (Table 20 to Table 22). Note that for strategies C and D, the different draws assign to the individuals both a line of treatment and one (or zero or two) cardiovascular events. Therefore, three figures are drawn for each strategy C and D because the number of cardiovascular events depends on the line of treatment: monotherapy in first-line, bitherapy in second-line and tritherapy in third-line treatment. Table 18 and Table 19, as well as Table 21 and Table 22 indicate that cardiovascular events are less frequent with bitherapy and tritherapy for two reasons.

- Firstly, the association of classes of drugs increases the effectiveness of antihypertensive treatments (Cf. Appendix, Table 25).
- Secondly, individuals who are prescribed 2nd line, and even more 3rd line treatment are less numerous, which has a mechanical impact on number of events.

Moreover, some cardiovascular events are less frequent with strategy C than with strategy D according to the relative risks of cardiovascular events (Cf. Appendix, Table 25). This is the case with renal failure, for example. On the contrary, some events are also more frequent with C than with strategy D.

These figures also show that some combinations of cardiovascular events never occur. For instance, in the 500 draws, no individuals were assigned two successive non-fatal strokes, nor an angina followed by non-fatal heart failure with the strategy B. This is due to the relatively weak probabilities of experiencing these combinations of cardiovascular events.

5.2. Consequences of these events in terms of health and/or income and estimation of each individual's equivalent income with strategies B, C or D

5.2.1. Estimation of hi with strategies B, C and D: the impact of cardiovascular events on the SAH

In order to measure the impact of cardiovascular events, with strategies B, C and D, on the self-assessed health of individuals, it was necessary to first estimate the impact of each disease on individual's self-assessed health for all people in the sample. The estimation is performed using ordinary least squares, on the whole sample. Note that individuals who declare a self-assessed health lower than 20 (i.e. 97 individuals, 3% of the sample) are excluded from the estimations for consistency with the results of Fleurbaey *et al.* (2012) that have been used to compute the level of the equivalent income.

The results of these estimations are presented in Table 7. Three successive regressions are presented:

- the first one only includes the different diseases;
- in the second one, we add socio-demographic variables (age, gender, equivalised income, marital status, number of children, education, complementary insurance) ;
- in the third one, we add lifestyle variables (smoking and alcohol behaviour, BMI).

We are interested in analysing how the coefficients associated with the disease variables change when we add the different control variables.

Note that in a more flexible approach, the impact of each disease would vary according to age, gender or income. Indeed, we know that poor individuals are more subject to certain diseases (for example diabetes or hypertension). Our estimations suppose that the impact of each disease on the self-assessed health is the same for all individuals. This simplification is necessary as we do not have enough observations in the sample: when the disease variables

are crossed with age and/or gender, they are not significant anymore. The different disease variables could also be crossed with certain lifestyle variables (for example obesity), but for the same reason of lack of power, we cannot present such a specification; all the disease variables would be unsignificant.

Table 7: Impact of the diseases and additional control variables on the self-assessed health (SAH) – estimated coefficients using three different models

	SAH (Model 1)	SAH (Model 2)	SAH (Model 3)
Diseases			
Angina	-7.546***	-6.293***	-5.821**
	(2.376)	(2.363)	(2.341)
Myocardial infarction	-6.747***	-5.563**	-5.748***
	(2.242)	(2.224)	(2.198)
Heart rhythm disorder	-4.190***	-3.681***	-3.276***
	(1.229)	(1.217)	(1.204)
Stroke	-8.704***	-7.684***	-7.851***
	(2.897)	(2.855)	(2.824)
Bronchitis	-4.727***	-3.635***	-3.038***
	(1.174)	(1.165)	(1.168)
Asthma	-2.916**	-3.353***	-3.078***
	(1.185)	(1.171)	(1.162)
Sinusitis	-3.349***	-4.084***	-4.035***
	(1.028)	(1.017)	(1.007)
Gastralgia	-2.541***	-2.773***	-2.574***
	(0.931)	(0.918)	(0.908)
Hepatitis	-9.571***	-8.800***	-7.665**
	(3.397)	(3.372)	(3.338)
Lumbago	-4.848***	-4.312***	-4.167***
	(0.728)	(0.722)	(0.715)
Arthrosis of the knee	-4.533***	-2.824***	-2.375**
	(1.031)	(1.048)	(1.041)
Arthrosis of the lip	-7.564***	-6.451***	-6.296***
	(1.383)	(1.371)	(1.355)
Menstrual disorders	-4.328**	-5.869***	-5.370***
	(1.723)	(1.741)	(1.726)
Menopause troubles	-4.953**	-3.905**	-3.978**
	(1.931)	(1.933)	(1.912)
Diabetes	-6.852***	-5.344***	-4.090***
	(1.231)	(1.226)	(1.229)
Malfunction of the thyroid	-7.062***	-6.540***	-6.133***
	(1.569)	(1.562)	(1.547)
Cholesterol	-4.048***	-2.886***	-2.575***
	(0.919)	(0.929)	(0.921)
Depression	-10.327***	-9.724***	-9.359***
	(1.137)	(1.129)	(1.117)
Anxiety	-3.897***	-3.704***	-3.682***
	(0.771)	(0.763)	(0.758)
Handicap	-11.874***	-11.065***	-10.470***
	(2.027)	(2.005)	(1.986)
Infirmity	-7.316***	-6.484**	-5.614**
	(2.742)	(2.706)	(2.693)
Cancer	-11.484***	-10.516***	-11.391***
	(2.029)	(2.005)	(1.986)
Socio-demographic characteristics			
Age	-	-0.329***	-0.225**
		(0.100)	(0.101)

Age²	-	0.002**	0.001
		(0.001)	(0.001)
Male	-	0.294	0.507
(ref: female)		(0.648)	(0.658)
Equiv.Inc ≤ 875€	-	-3.152***	-2.479***
(*ref: € [1290-1800])*		(0.914)	(0.908)
Equiv.Inc *€ [875-1290]*	-	-1.830**	-1.608*
(*ref: € [1290-1800])*		(0.873)	(0.863)
Equiv.Inc ≥ 1800	-	0.595	0.650
(*ref: € [1290-1800])*		(0.884)	(0.875)
Marital life	-	-0.180	-0.399
(ref: single)		(0.684)	(0.681)
Children	-	0.605	0.616
(ref: no child)		(0.727)	(0.721)
No diploma	-	-3.546***	-2.739**
(ref: baccalauréat)		(1.272)	(1.261)
Primary school certificate	-	-1.587	-1.312
(ref: baccalauréat)		(1.298)	(1.284)
"Brevet" (=GCSE)	-	-2.282**	-2.092**
(ref: baccalauréat)		(0.924)	(0.915)
University (≤ 3 years)	-	-0.391	-0.339
(ref: baccalauréat)		(1.154)	(1.141)
University (≥ 4 years)	-	-1.193	-1.897*
(ref: baccalauréat)		(1.104)	(1.095)
Other diploma [+]	-	-17.467*	-18.412*
(ref: baccalauréat)		(9.606)	(9.499)
National Health Ins. only	-	-1.894	-1.355
(ref: Complem. Insurance)		(1.271)	(1.261)
CMU only	-	-0.883	-0.399
(ref: Complem. Insurance)		(1.389)	(1.380)
Lifestyle variables			
smoker	-	-	-2.608***
(ref: no smoker)			(0.693)
Alcohol – no risk [+]	-	-	2.751***
(ref: no alcohol)			(0.699)
Alcohol–risky behaviour [+]	-	-	-0.987
(ref: no alcohol)			(1.470)
Underweight	-	-	-0.087
(ref:normal BMI)			(1.669)
Overweight	-	-	-1.712**
(ref:normal BMI)			(0.728)
Obese	-	-	-5.205***
(ref:normal BMI)			(0.943)
Severely obese	-	-	-6.723***
(ref:normal BMI)			(1.919)
Constant	82.744***	95.155***	92.744***
	(0.427)	(2.379)	(2.397)
Number of observations	2,513	2,513	2,513
Adjusted R^2	0.28	0.31	0.33
Fisher statistic	46.47	30.58	27.95
RMSE	15.24	14.98	14.79

Notes: i) Standard errors are presented in parentheses; ii) ***, ** and * indicate that the coefficients are statistically significant at the 1%, 5% and 10% levels; iii) for variables followed by "+",their definitions are the following: "other diploma" means that the individual obtained a diploma abroad, that could not be translated during the interview ; For the alcohol variables, a variable of *weekly* consumption was created using the different answers of the respondents, in order to create a categorical indicator of alcohol consumption (inspired from Com-Ruelle *et al.*, 2008). Individuals belongs to the category "alcohol – no risk" if they drink less than 14 drinks a week (for female) or 21 (for male) and drink less than 4 times a week. Individuals belongs to the category "alcohol –risky behavior" if they drink more than 15 drinks a week (for female) or 22 (for male).

We concentrate on results obtained with the full model (model 3) even if coefficients of the diseases are not much affected by the inclusion of the control variables, except for four diseases: hypertension, deafness, arthritis of the knee and diabetes. For these diseases, that mostly concern older individuals, this is the inclusion of the age variable that influences the most the estimated coefficients.

The estimated coefficients are easy to interpret: they measure the variation in self-assessed health (SAH) consecutive to the occurrence of the disease. For example, individuals who have experienced a stroke during the last 12 months declare a lower SAH (-7.8 points of SAH over the 0-100 scale) than the others, all other things being equal. All disease have a negative coefficient: they all contribute to decrease the level of SAH. However, some diseases have more impact on SAH, such as cancer (-11.4 points), handicap (-10.5 points) or depression (-9.4 points). On the contrary, other diseases have much less impact, such as cholesterol (-2.5 points). Note that, to improve readability, only diseases that have a significant impact on SAH in model 3 are kept as explanatory variables for the three models. However, Table 15 in the Appendix presents the results of the full estimation containing all diseases variables, even those which are not significant. We see that the non-significant variables are mostly variables that are not life threatening (caries, otitis, urinary infection, nasopharyngitis,,…), except for Parkinson's and Alzheimer's (but the non significance is probably due to the small number of individuals affected by these diseases in our sample).

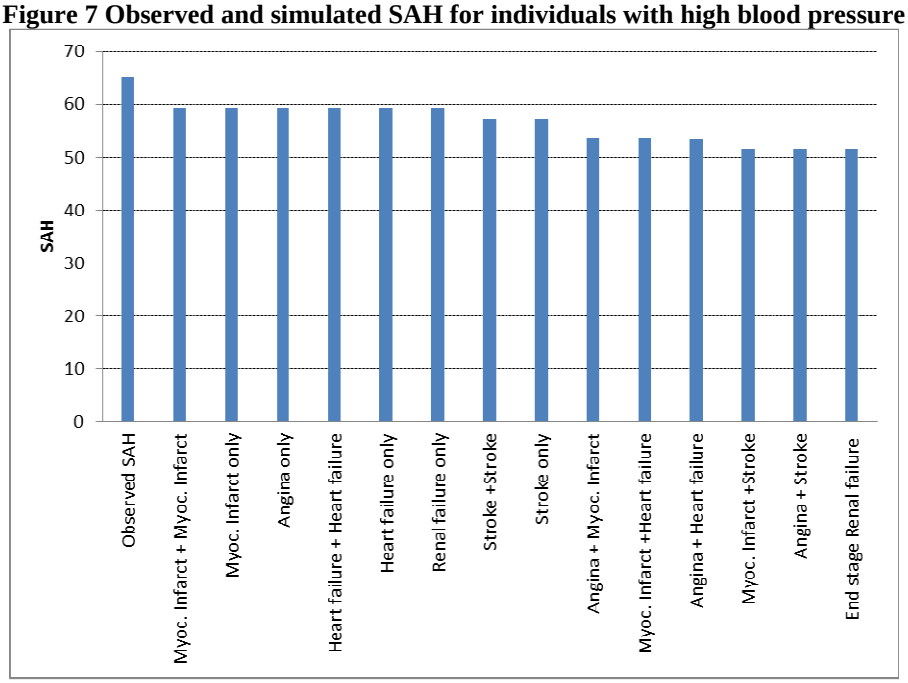
Control variables have the expected signs. Individuals whose equivalent income is the lowest (in the first and second quartiles of the distribution of income) declare a lower level of SAH (-2.5 and -1.6 points) than individuals belonging to the third quartile. Note that this effect is only a correlation and we do not take into account a possible relation of causality between these variables (a lower SAH may cause a lower level of equivalised income and the reverse causality is also true). Education variables also influence significantly the level of SAH: individuals with no diploma or a low one (lower than the high school, *i.e.* "baccalauréat") declare a lower SAH than the others. We did not include variables linked to the professional status of the respondent as these variables are not significant[101].

[101] They also remain unsignificant when education variables or income variables are removed.

Surprisingly, we do not find any impact of the coverage of the individuals (national health insurance or CMU versus complementary insurance), probably because we already control for the level of income. Concerning lifestyle variables, we find that individuals who smoke, who are overweight, obese or severely obese declare a lower SAH than the others. Surprisingly, drinking alcohol moderately (i.e. without any risky behaviour) has a positive impact on the individual's SAH (+2.7 points).

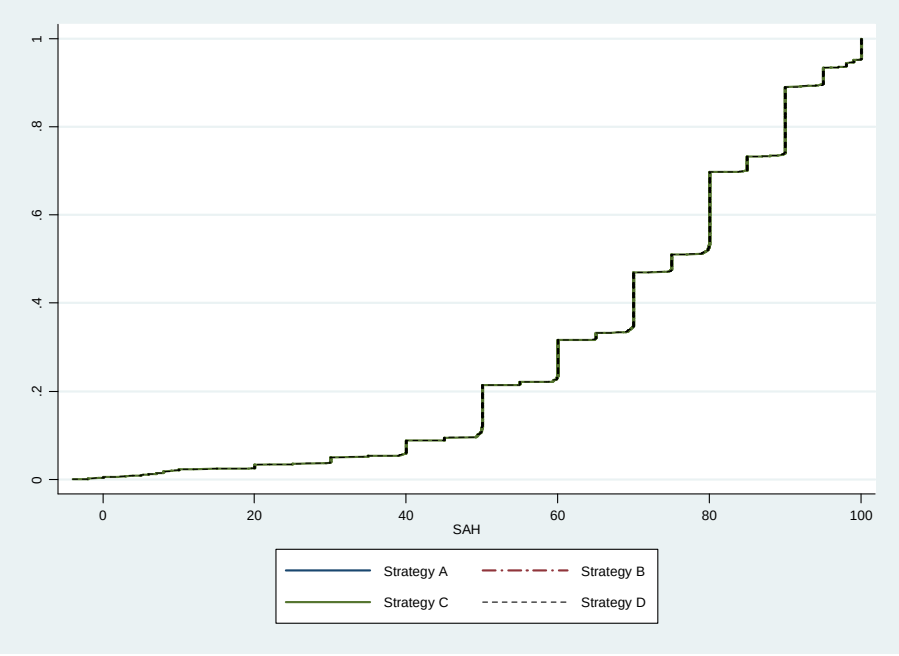
Using the results of these estimations and the methodology described in the section 4.2.1, we then simulate the SAH that individuals would have with strategies B, C and D. Remind that only individuals with hypertension with strategy A and who get a non-fatal cardiovascular event with these strategies B, C or D experience a variation of their SAH. For those who get a fatal event, their SAH is 0 and for the others, with no cardiovascular event, or even no high blood pressure, their SAH remains identical. Figure 7 compares the average value of the SAH observed in the sample for individuals with high blood pressure (65.1) with the simulated value of the SAH estimated using the coefficients of the regression (Table 7) for individuals who got an extra cardiovascular event. Given that the estimated coefficients associated to the diseases are quite low, the impact of a cardiovascular event on the SAH is also relatively low. Suppose an individual experiences 2 infarctions, he would declare a SAH of 59. An individual who gets end-stage renal failure would declare a SAH of 51.4, which is the lowest level of simulated SAH.

Figure 7 Observed and simulated SAH for individuals with high blood pressure



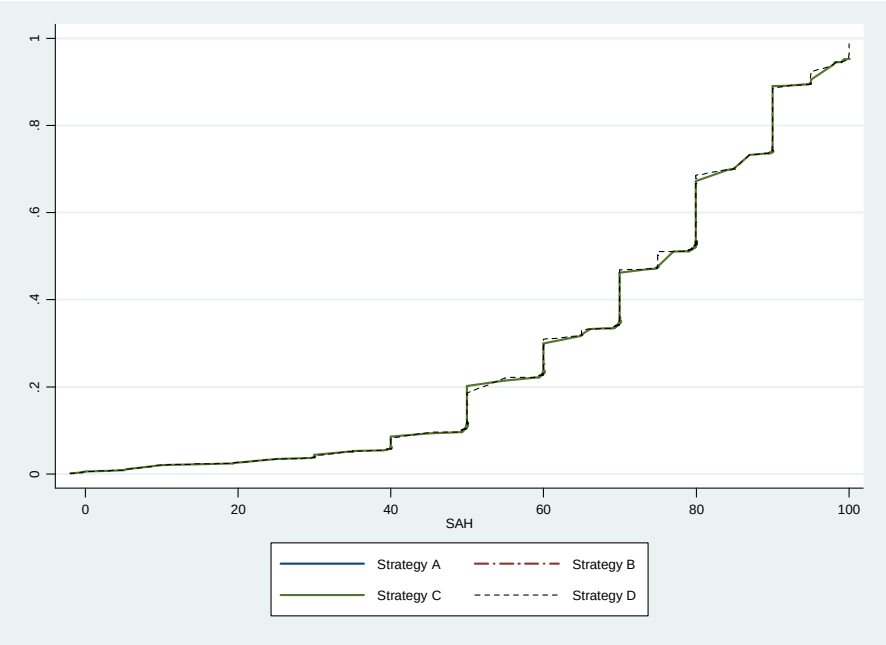
A very small number of individuals being concerned by a change in the level of their SAH (Figure 4 to Figure 6), the cumulative distribution functions of SAH on the whole sample of individuals (N=3,331) are very close between the different strategies A, B, C and D, as shown in Figure 8. Only very small differences can be seen on Figure 9 that is drawn using the sub sample of individuals with high blood pressure. On average, Table 8 confirms these results. Using the whole sample, individuals declare an average level of SAH of 71.2; given the occurrence of some cardiovascular events, this average level of SAH decreases with strategies B (71.15), C (71.18) and D (71.17) but this decrease is very small. As shown in this table, the decrease is only due to the small number of individuals who get a CVE in state B, C or D and for whom the SAH decreases (52.4 in strategy B, 51.8 in strategy C and 52.7 in strategy D). Note that the distribution of cardiovascular events in each strategy is such that strategy D seems more effective than the others: the average SAH of individuals who get a cardiovascular event with D is a bit higher than with the other strategies.

Figure 8 Distribution of SAH for all individuals in the sample with strategies A, B, C and D



Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of SAH for each individual calculated over the 500 draws.

Figure 9 Distribution of SAH for individuals with high blood pressure with strategies A, B, C and D



Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of SAH for each individual calculated over the 500 draws.

Table 8 Synthesis of the consequences of strategies B, C and D

	Situation A	Situation B (mean & sd over the 500 draws)	Situation C (mean & sd over the 500 draws)	Situation D (mean & sd over the 500 draws)
All individuals (N= 3 331)				
Income, including :	1 447 (1 070)	1 450 (1 071)	1 446 (1 070)	1 445 (1 069)
Equivalised income	*1 447 (1 070)*	*1 447 (1 071)*	*1 447 (1 071)*	*1 447 (1 070)*
cost of insurance	-	*-1.29 (0.94)*	*0.642 (0.467)*	*1.18 (0.85)*
out-of-pocket payment	-	*0.06 (0.74)*	*2.11 (4.98)*	*2.38 (5.64)*
SAH	71.2 (20.6)	71.15 (20.6)	71.18 (20.6)	71.17 (20.6)
Equivalent income	1 085 (990)	1 087 (991)	1 085 (989)	1 084 (989)
Individuals with hypertension and no CVE in state A (N= 399)				
Income, including :	1 459 (858)	1 471 (860)	1 456 (859)	1 454 (858)
Equivalised income	-	*1 456 (859)*	*1 457 (859)*	*1 457 (858)*
cost of insurance	-	*-1.33 (0.93)*	*0.661 (0.46)*	*1.21 (0.84)*
out-of-pocket payment	-	*0.36 (1.87)*	*13.63 (1.77)*	*15.34 (2.51)*
SAH	65.11 (19.28)	64.74 (19.6)	64.74 (19.5)	64.86 (19.5)
Equivalent income	1 042 (840)	1 048 (843)	1 038 (839)	1 036 (838)
Individuals with hypertension and a CVE in state B, C or D				
Nb of observations	-	Between N=5 and N=27 (mean=17.6)	Between N=2 and N=23 (mean=9.7)	Between N=4 and N=23 (mean=12.5)
Income, including :	-	1 339 (817)	1 324 (835)	1 349 (800)
Equivalised income	-	*1 344 (816)*	*1 331 (835)*	*1 358 (800)*
cost of insurance	-	*-1.30 (0.92)*	*0.66 (0.42)*	*1.20 (0.82)*
out-of-pocket payment	-	*8.24 (3.79)*	*17.93 (9.7)*	*20.74 (9.07)*
SAH	-	52.4 (22.5)	51.8 (22.4)	52.7 (22.1)
Equivalent income	-	746 (635)	736 (633)	761 (624)

5.2.2. Estimation of y_i with strategies B, C and D: the impact of cardiovascular events on the level of equivalised income

In order to measure the impact of cardiovascular events on the equivalised income of individuals who declare hypertension in the sample, when strategies B, C and D are implemented, we use the indirect methodology described in 4.2.2. We need, first, to estimate the impact of self-assessed health on all individuals' equivalised income (Table 9). Then, we

use the estimated coefficients as well the simulated self-assessed health (table 7) obtained with strategies B, C and D to simulate the impact of cardiovascular events on equivalised incomes with strategies B, C and D.

The impact of self-assessed health on the logarithm of individual's equivalised income is estimated using ordinary least squares and results are presented in Table 15. Recall that these estimations are performed on employed individuals, i.e. retired, unemployed individuals and housewives/househusbands are excluded from the sample. As it is shown in Table 7, three models are estimated: they differ according to the number of control variables included. We mainly comment the full model (i.e. model 3). Note that the coefficient of the self-assessed health variable is slightly modified between columns 1 and 3. This coefficient is 0.0052 in model 1, which means that a rise of 10 points in self-assessed health leads to a 5.2 percentage point rise in the equivalised income. In column 3, when we use additional control variables, the increase in income is lower, 3.5 percentage point, mostly due to the inclusion of the variables linked to the professional status of the individual.

Control variables have the expected signs. Not surprisingly, all other things being equal, top executives and middle-class profession earn, respectively, 17.6 % and 13.7 % more than employees. We also observe that employed men earn 6.5 % more than women. When we control by the professional status, education has a limited impact on the level of equivalised income. Only the absence of diploma has a negative and significant impact on income (-13.8 %). As we consider equivalised income, the spouse's characteristics matter for non-single individuals. We observe a positive impact on income of having a top-executive or middle-class profession spouse (rather than an employee spouse) and a negative impact on income of having a spouse with no diploma or a low diploma. Given the formula used for the computation of the equivalised income, living in a marital life has a positive impact on the level of equivalised income whereas having children has a negative impact, all other things being equal.

Table 9 Impact of SAH and other control variables on the level of equivalised income – estimated coefficients using three different models

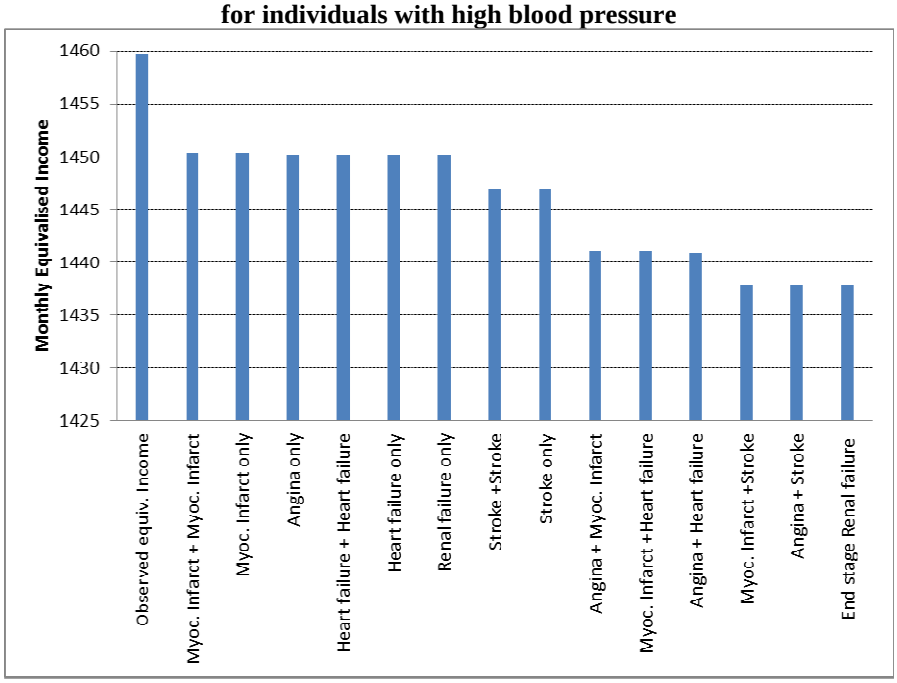
	Log(Equiv. Income) Model (1)	Log(Equiv. Income) Model (2)	Log(Equiv. Income) Model (3)
SAH			
SAH of the last 12 months	0.0052***	0.0042***	0.0035***
	(0.001)	(0.001)	(0.001)
Socio-demographic variables			
Male		0.084***	0.065**
(ref: female)		(0.029)	(0.028)
Age		0.002	0.008
		(0.005)	(0.005)
Age^2		0.00005	-0.00002
		(0.00005)	(0.00004)
No diploma		-0.252***	-0.138**
(ref: baccalauréat)		(0.063)	(0.060)
Primary school certificate		-0.119	-0.050
(ref: baccalauréat)		(0.075)	(0.069)
"Brevet" (=GCSE)		-0.100**	-0.035
(ref: baccalauréat)		(0.041)	(0.038)
University (≤ 3 years)		0.072	0.037
(ref: baccalauréat)		(0.048)	(0.045)
University (≥ 4 years)		0.167***	0.056
(ref: baccalauréat)		(0.050)	(0.049)
Other diploma [+]		-0.379	-0.270
(ref: baccalauréat)		(0.311)	(0.284)
Farmer		-0.524***	-0.464***
(ref: employee)		(0.107)	(0.101)
Artisans/self-employed		-0.046	-0.085
(ref: employee)		(0.056)	(0.053)
Top executive		0.252***	0.176***
(ref: employee)		(0.055)	(0.051)
Middle-class profession		0.131***	0.137***
(ref: employee)		(0.044)	(0.040)
Workmen		-0.116***	-0.056
(ref: employee)		(0.039)	(0.036)
Variables related to the household			
Marital life			0.339***
(ref: single)			(0.049)
Spouse - No diploma			-0.241***
(ref: baccalauréat)			(0.070)
Spouse - Primary school certificate			-0.263***
(ref: baccalauréat)			(0.092)
Spouse - "Brevet" (=GCSE)			-0.167***
(ref: baccalauréat)			(0.046)
Spouse - University (≤ 3 years)			0.022
(ref: baccalauréat)			(0.057)
Spouse - University (≥ 4 years)			0.020
(ref: baccalauréat)			(0.060)
Spouse - Other diploma [+]			-0.377**
(ref: baccalauréat)			(0.155)
Spouse - Farmer			-0.082
(ref: employee)			(0.102)
Spouse - Artisans/self-employed			0.095
(ref: employee)			(0.071)
Spouse - Top executive			0.248***
(ref: employee)			(0.061)
Spouse - Middle-class profession			0.114**
(ref: employee)			(0.051)
Spouse - Workmen			-0.002

(ref: employee)			(0.046)
Spouse – Non-working			-0.339***
(ref: employee)			(0.086)
Live in the Paris area			0.056
(ref: elsewhere)			(0.035)
Children			-0.232***
(ref: no child)			(0.029)
Constant	6.792***	6.650***	6.511***
	(0.067)	(0.139)	(0.130)
Number of observations	1,183	1,183	1,183
Adjusted R^2	0.03	0.22	0.35
Fisher statistic	37.922	23.486	21.881
RMSE	0.522	0.468	0.426

Notes: i) Standard errors are presented in parentheses; ii) ***, ** and * indicate that the coefficients are statistically significant at the 1%, 5% and 10% levels; iii) For variables followed by "+",their definitions are the following: "other diploma" means that the individual obtained a diploma abroad, that could not be translated during the interview.

Using these estimated coefficients and the simulated self-assessed health obtained in the previous section, we can compute a simulated level of equivalised income that individuals would earn if strategies B C or D were implemented. As for self-assessed health, only individuals with hypertension in state A and who get a non fatal cardiovascular event with these strategies B, C or D experience a variation of their income. For those who get a fatal cardio vascular event, their income is 0 whereas for those with no cardio vascular event or even no hypertension, the equivalised income, before taking into account the different costs, remains unchanged. Table 8 compares the average value of the equivalised income observed in the sample for individuals with high blood pressure (1 459 €) with the simulated value of equivalised income, estimated using both the simulated SAH (Figure 7) and the coefficients of the regression (Table 9) for individuals who got an extra cardiovascular event. The impact of these cardio vascular events on the level of income symbolizes the effect that the disease would have on the individuals' careers. For example, an individual who experiences 2 infarctions would earn, on average, 1 450€ per month (a drop of income of 9€); an individual who gets end-stage renal failure would earn 1 438€ per month (a drop of income of 21€). The impact of the diseases on the level of equivalised income is much lower than what could be expected. This is due to the fact that: i) the impact of the diseases on the SAH is quite low; ii) the impact of the SAH on the level of equivalised income is also very low. But note that, as stated in section 4.2.2, we probably don't have the appropriate data to assess the impact that a disease has on income.

Table 10 Observed and simulated level of equivalised income for individuals with high blood pressure



As for the self-assessed health, because a very few number of individuals are concerned by this change in the level of their equivalised income, we do not see much difference in the average level of equivalised income between the four strategies (Table 8).

5.2.3. Estimation of y_i with strategies B, C and D: the impact of the cost of insurance and out-of-pocket payments with strategies B, C and D

Costs of insurance and out-of-pocket payments related with strategies B, C and D include cost of antihypertensive treatments and cost of medical care induced by cardiovascular events. They have been computed to estimate the impact of each strategy on individuals' incomes when the strategy is implemented for all individuals in the sample. All these costs are deducted from the individual's level of income. Therefore, i) for individuals with hypertension and who get a cardiovascular event, they are deducted from the level of income simulated in the previous section ; ii) for individuals who do not get a cardio vascular event or for those who have no hypertension, they are deducted from the declared level of equivalised income.

If we consider only the costs of insurance, Table 11 indicates that:

- strategy B is cheaper than strategy A (the total cost is 3 363 € lower),
- strategy C is more expensive than strategy A (the total cost is 1 669 € higher) and more expensive than strategy B,

- strategy D is more expensive than strategy A (the total cost is 3 058 € higher) and more expensive than strategy B and strategy C.

These insurance costs are shared by all individuals in the sample, and not only those who have high blood pressure. Every individual contributes to the funding of antihypertensive treatments and medical care induced by cardiovascular events, according to his rate of participation to the national health insurance expenditures (Caussat *et al.*, 2005; Cf. Appendix, Table 31).

However, out-of-pocket payments due to treatment and follow-ups are only paid by individuals with high blood pressure and/or a cardiovascular event in strategies B, C or D. For these individuals, out-of-pocket payments are higher with strategies C and D than with strategy B. This is explained by the fact that costs of antihypertensive treatments and costs of follow-up in primary prevention are not completely covered by the national health insurance: the rate of coverage is 65%. The difference in terms of out-of pocket payments between strategies C and D is due to an increase of the number of renal failures with strategy D compared with strategy C (Cf. Appendix, Table 17 to Table 22). This increase is mainly explained by an evidence-based reduction of renal failure with ACE inhibitors (Cf. Appendix Table 25). As strategy C includes ACE inhibitors in all three lines of treatment, it is more effective in terms of decreasing of renal failure than strategy D which includes ACE inhibitor only in the second and third-line treatment. In the present study, patients with renal failure are not considered as being under ALD status, therefore they support out-of-pocket payments for care induced by this disease. Consequently, it is consistent that strategy D induces more out-of-pocket payments than strategy C.

Note that out of pocket payments of CMU-C patients are shared by all individuals like other expenses funded by the national health insurance. Table 11 indicates that there is no out-of-pocket payment for individuals with CMU-C with strategy B. This means that no individuals with CMU-C experienced renal failure in the 500 draws.

Table 11 Average of costs induced by strategies B, C and D for all individuals in the sample and for individuals who declared high blood pressure over the 500 draws

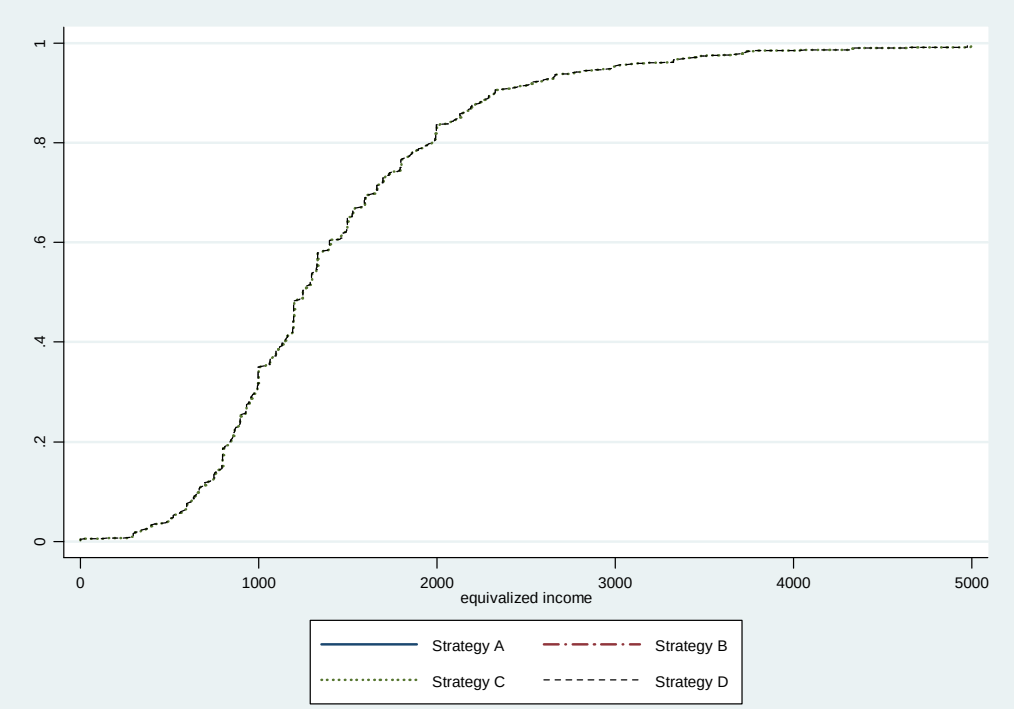
	Strategy B	Strategy C	Strategy D
Monthly costs paid by all individuals (=cost of insurance)			
Hospitalization + Follow ups due to ECV (+)	3 190 € (1 420 €)	2 060 € (1 115 €)	2 081 € (1 040 €)
Cost of treatment & follow-ups in B, C or D (+)	229.5 € * (66.2 €)	6 292 € (35.7 €)	7 659 € (56.6 €)
Cost of treatment & follow-ups in A (-)	6 699 € (=399*(7.423+(112.4/12)))		
Cost of CMU patients in A (-)	84.7 € (=6*((121.4/12)+3.9972))		
Cost of CMU-C patients in B, C or D (+)	0 €	100.9 €	
Total of the costs	**-3 363 € (1 477 €)**	**1 669 € (1 134 €)**	**3 058 € (1 059 €)**
Total per ind., according to the tax rate	**-1.29 € (0.94 €)**	**0.6 € (0.46 €)**	**1.18 € (0.85 €)**
Monthly <u>individual</u> costs paid by individuals with hypertension & a cve or not in B, C or D (=out of pocket payments) **			
Out of pocket due to treatment & follow-ups in A (-)	14.11		
Out of pocket due to treatment & follow-ups in B, C or D (+)	4.67 (9.55)	13.71 (0.07)	15.6 (0.10)

Notes : In parentheses, (-) or (+) means that it is subtracted or added to the sum of the costs of each strategy.
* in B, the cost of treatment and follow-ups (consults,…) is only >0 for individuals for whom a first CVE occurs
** Once a cardiovascular event occurs, individuals do not have to pay any out-of-pocket payments anymore (because they are now classified as ALD), except for individuals who have renal failure

The distributions of income with strategies B, C and D, after taking into account the impact of cardiovascular events on both the level of equivalised income and the costs, are presented on Figure 10 and

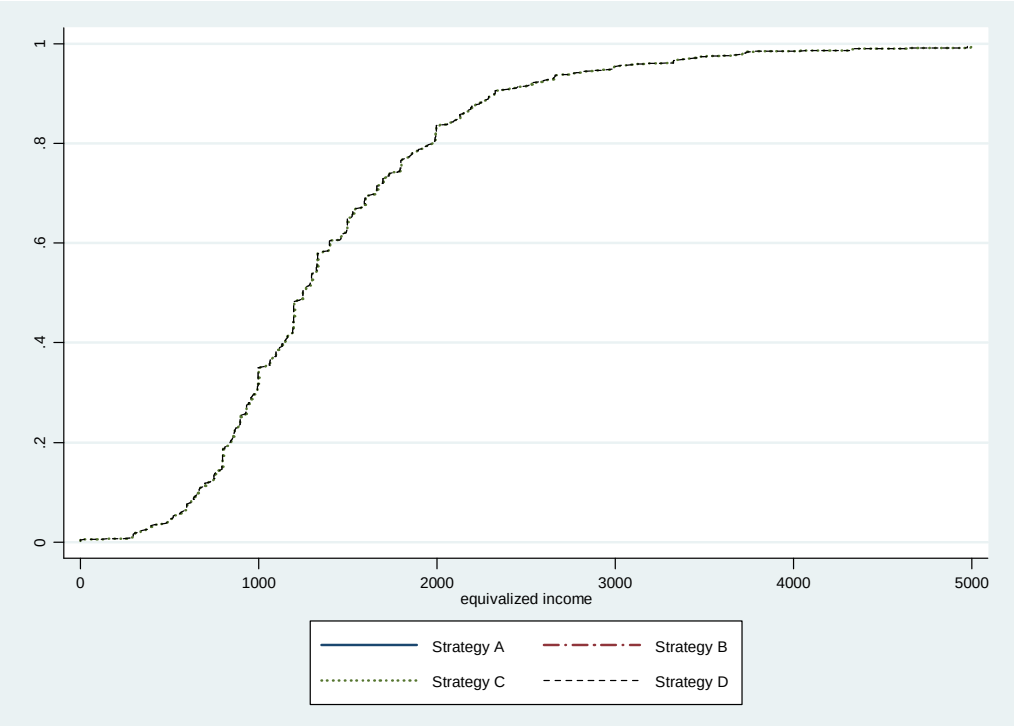
Figure 11. Even more than for the distribution of individuals' self-assessed health, the distributions of incomes among all individuals in the sample (Figure 10), but also among individuals with high blood pressure (Figure 11) are very similar, due to i) the small number of individuals who experience a decrease of their equivalised income and the small variation of income for those individuals; ii) the small level of costs paid by all individuals. On the average, Table 11 shows very small differences between the strategies, except if we consider the sub sample of individuals with high blood pressure and a cardiovascular event in B, C or D. For example, for individuals who get a cardiovascular event with the strategy B, their income decreases, on average, to 1 339€ per month, compared to the initial level of 1 459€.

Figure 10 Distribution of equivalised incomes for all individuals in the sample with strategies A, B, C and D



Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of equivalised income for each individual calculated over the 500 draws

Figure 11 Distribution of equivalised income for individuals with high blood pressure with strategies A, B, C and D



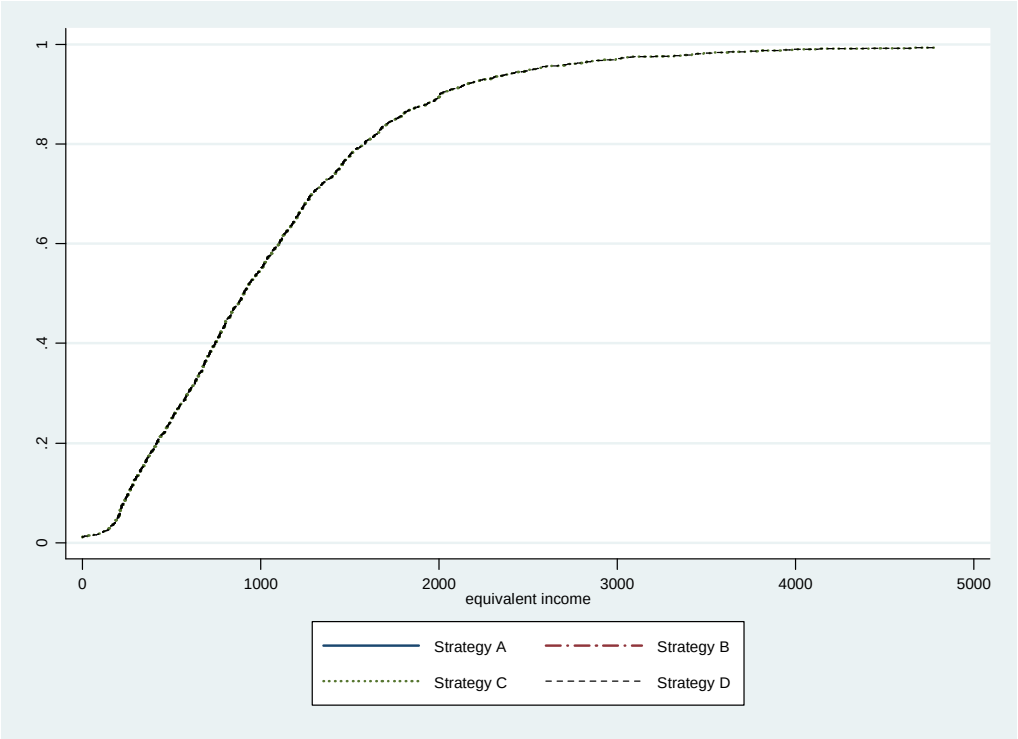
Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of equivalised income for each individual calculated over the 500 draws

5.2.4. Estimate of individual equivalent incomes y^*_i with strategies B, C and D

Now that we have simulated individual's self-assessed health and individual's incomes with strategies B, C and D, we can compute individual's equivalent incomes for each of the four strategies. In case of strategies B, C and D, we can only compute the distribution of individual's equivalent incomes for the 500 draws.

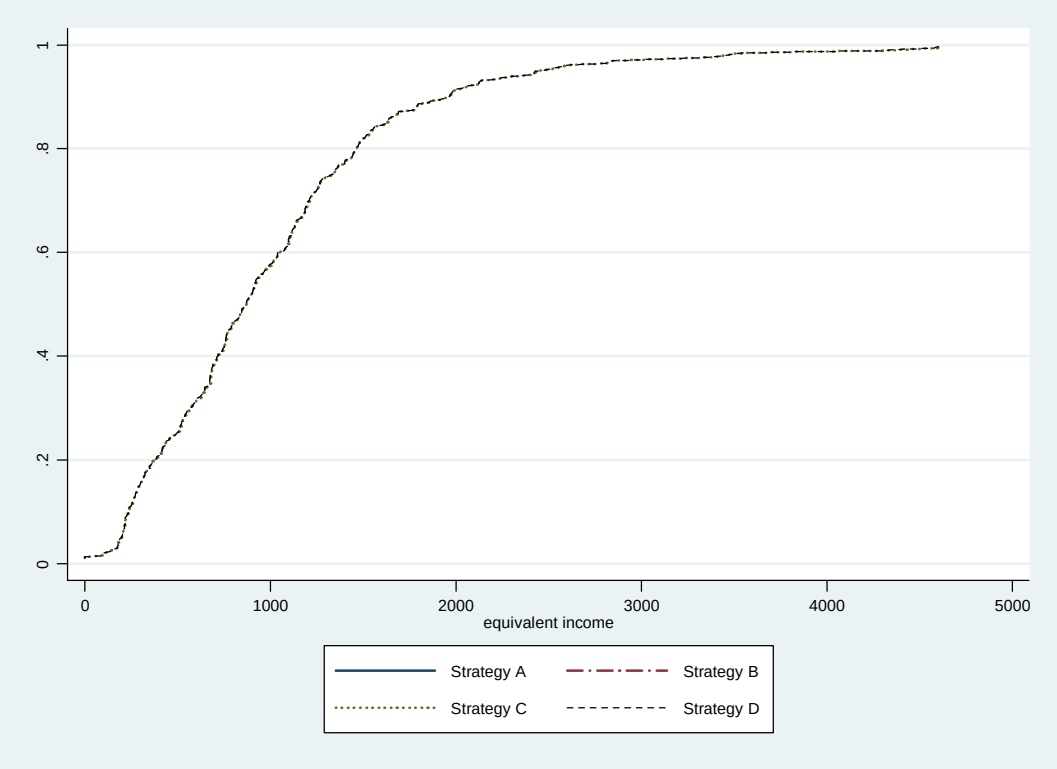
Figure 12 and Figure 13 shows the distribution of equivalent incomes among all individuals in the sample and specifically among individuals with high blood pressure. As for the distribution of individuals' self-assessed health and individuals' equivalised incomes, the differences between strategies B, C and D are more visible in the graphic where only individuals with high blood pressure are considered. But overall (Cf. Table 8), there are not many differences between the strategies.

Figure 12 Distribution of equivalent incomes for all individuals in the sample with strategies A, B, C and D



Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of equivalent income for each individual calculated over the 500 draws.

Figure 13 Distribution of equivalent incomes for individuals with high blood pressure with strategies A, B, C and D



Note: For strategies B, C and D, the cumulative distribution function represents the distribution of the average level of equivalent income for each individual calculated over the 500 draws.

On the whole sample, the average equivalent income is higher with strategy B than with strategies C or D (Table 8). Therefore, strategy B is preferred overall by individuals of the sample. The average equivalent income among individuals with high blood pressure is also higher with strategy B. Like all individuals in the survey, individuals with high blood pressure pay less tax with strategy B than with strategies C and D. They also support lower out-of-pocket payments than with strategies C and D because, with strategy B, they no longer pay 35 % of the cost of antihypertensive treatment in primary prevention.

However different results can be observed when we consider the subsample of individuals with high blood pressure who experienced at least one cardiovascular event with strategies B, C and D in at least one of the 500 draws considered. Their average equivalent income is higher with strategies D than with strategy B. But, as for the self-assessed health and the level of equivalised income, because they are very few (between n=5 and n=27 in strategy B), the decrease of their equivalent income with strategy B has no significant impact on the average level of equivalent income among all individuals in the sample. Overall, given individuals' level of preferences for health in the sample, the extra costs induced by strategies C and D are

not compensated by the reduction of cardiovascular events occurrence with antihypertensive treatment.

Table 12 presents the same information as Table 8 but the consequences for “winners” are distinguished from the consequences for “losers”. If we compare two strategies X versus Y, “winners” are individuals whose equivalent income is better with X than with Y and “losers” are individuals whose equivalent income is worse with X than with Y. This information is useful to understand the impact that the inclusion of inequality aversion may have in the computation of the social welfare functions associated with each strategy (which are presented in the following section).

This table shows that, whatever pair of strategies is considered:

- “winners” are clearly middle age people with good health. They are probably employed and tax payers. The distribution between men and women is balanced. They have smaller incomes than the loosers but have good health and, therefore, they have higher equivalent incomes than the loosers. Less than 20% of them have declared high blood pressure.

- “losers” are older people. Nearly all of them are men. It is a coherent result because men present more risk of experiencing cardiovascular events. They have higher incomes (which may be correlated with being mostly men) but their health is worse than winners’: therefore they have much lower equivalent incomes.

Table 12 Synthesis of the consequences of strategies A, B, C and D for the winners and for the losers

	(Eq Income in A - Eq Income in B)	(Eq Income in C - Eq Income in B)	(Eq Income in D - Eq Income in B)	(Eq Income in C - Eq Income in D)
% (and Number) of « loosers »	4.27 % (N=110)	3.34 % (N=86)	2.68 % (N=69)	4.43 % (N=114)
For the losers				
Average (median) level of loss	-9.70 € (-1.30 €)	-3.90 € (-1.07 €)	-1.78 € (-0.67 €)	-1.01 € (-0.44 €)
Average (median) level of income	In A: 1 823 € (1 433 €) In B:1 826 € (1 436 €)	In B: 1 892 € (1 436 €) In C: 1 886 € (1 432 €)	In B: 1 912 € (1 401 €) In D: 1 905 € (1 388 €)	In C: 1 781 € (1 496 €) In D: 1 782 € (1 497 €)
Average level of SAH	In A: 52.8 (50) In B: 52.2 (49.7)	In B: 48.6 (49.2) In C: 48.9 (49.7)	In B: 43.10 (48.4) In D: 43.26 (48.9)	In C: 53.49 (50) In D: 53.60 (50)
% of Hypertension	70.9 %	62.7 %	53.6 %	71.9 %
Average level of equivalent income	In A: 735 € (282 €) In B: 725 € (279 €)	In B: 641 € (253 €) In C: 644 € (255 €)	In B: 552 € (214 €) In D: 554 € (215 €)	In C: 833 € (364€) In D: 834 € (364 €)
Average (median) age	65.4 (64)	64.4 (64)	64.6 (64)	65.5 (66)
% male	93.6 %	97 %	100 %	79 %
For the winners				
Average level of gains	2.39 € (1.03 €)	3.06 € (1.51 €)	3.73 € (1.96 €)	0.84 € (0.41 €)
Average level of income	In A: 1 430 € (1 250 €) In B:1 431 € (1 251 €)	In B: 1 432 € (1 251 €) In C: 1 429 € (1 249 €)	In B: 1 435 € (1 251 €) In D: 1 431 € (1 249 €)	In C: 1 429 € (1 249 €) In D:1 428 € (1 249 €)
Average level of SAH	In A: 72.03 (80) In B: 72 (79.5)	In B: 71.93 (78.7) In C: 71.94 (79.2)	In B: 71.93 (78.64) In D: 71.94 (78.97)	In C: 72 (79.3) In D: 72 (79.1)
% of Hypertension	15.4 %	16.3 %	16.8 %	15.3 %
Average level of equivalent income	In A: 1 120 € (934 €) In B: 1 120 € (934 €)	In B: 1 119 € (933 €) In C: 1 117 € (930 €)	In B: 1 118 € (933 €) In D: 1 115 € (929 €)	In C: 1 113 € (924 €) In D:1 112 € (923 €)
Average (median) age	52.2 (53)	52.4 (53)	52.5 (54)	52.22 (53)
% male	42.3 %	42.6 %	42.9 %	42.8 %

5.3. Estimate the total welfare change for society with strategies B, C and D

In accordance with section 4.3.2, we compare the following pairs of strategies, taking into account different degrees of inequality aversion in the computation:

- strategy A versus B
- strategy B versus C
- strategy B versus D
- strategy C versus D

To compare strategy A to strategy B, we evaluate the position of the value of the welfare function in A in the distribution of the different values taken by the social welfare function in B (Figure 14 to Figure 17). We consider that strategy B is efficient (or welfare improving) if more than 95 % of the values taken by the social welfare function in B are higher than the value of the social welfare function in A.

Comparing strategy B to strategy C, or strategy B to strategy D or strategy C to strategy D is less immediate as we have to deal with three distributions of values taken by the social welfare functions in these strategies. As explained in section 4.3.1, we use tests of stochastic dominance in order to test the efficiency of the different strategies.
If we consider two distributions X and Y, X is efficient (or welfare improving) if the cumulative distribution function of X first order stochastically dominates the cumulative distribution function of Y.

5.3.1. Comparison of the social welfare functions obtained with strategies A and B

The comparison of strategies A and B show that strategy B is always preferred as strategy A: whatever the degree of inequality aversion is considered, more than 97 % of the values taken by the social welfare function in B are higher than the value of the social welfare function in strategy A.

Figure 14

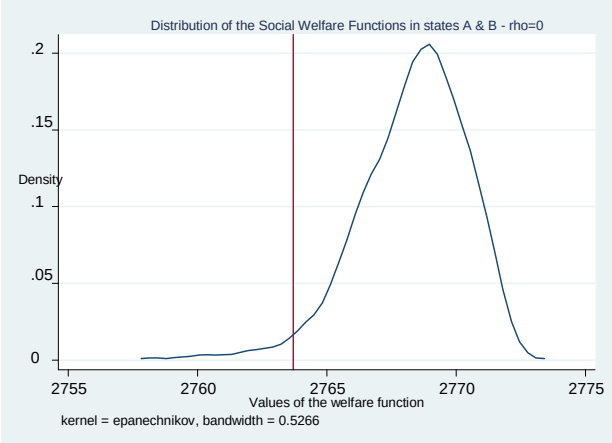


Figure 15

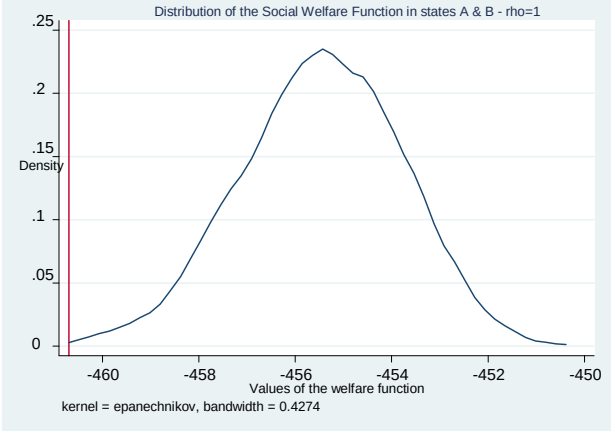


Figure 16

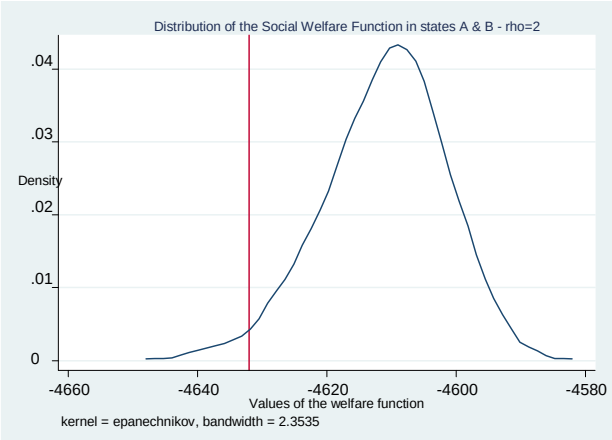
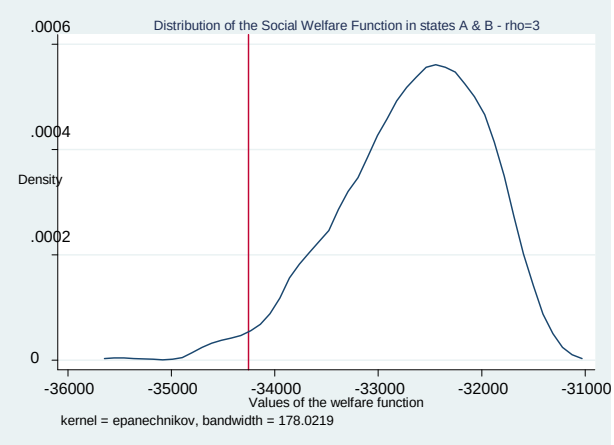


Figure 17


Distribution of the Social Welfare Function in states A & B - rho=3
.0006
.0004
.0002
0
Density
-36000
-35000
-34000
-33000
-32000
-31000
Values of the welfare function
kernel = epanechnikov, bandwidth = 178.0219

5.3.2. Comparison of the social welfare functions obtained with strategies B and C, B and D and C and D

Figure 18 to Figure 21 show that, whatever the degree of inequality aversion that is considered, strategy B always first-order stochastically dominates strategy C and strategy D. Moreover, strategy C always first-order stochastically dominates strategy D. This first-order stochastic dominance can be seen because the cumulative distribution function of B is everywhere to the right of C and D, and the one of C is everywhere to the right of D. The distributions never cross. Tests of first-order stochastic dominance (Table 13) were performed to confirm these results. As a consequence, strategy B is always preferred than the others, and C is also preferred than D terms of social welfare.

Figure 18

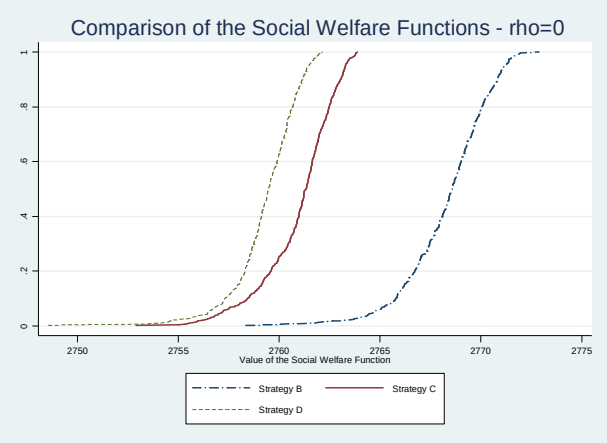


Figure 19

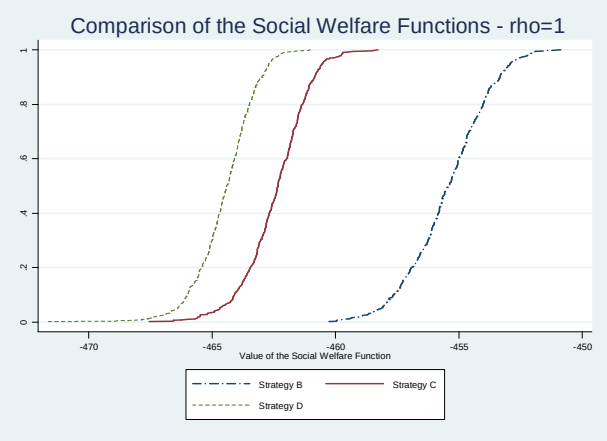


Figure 20

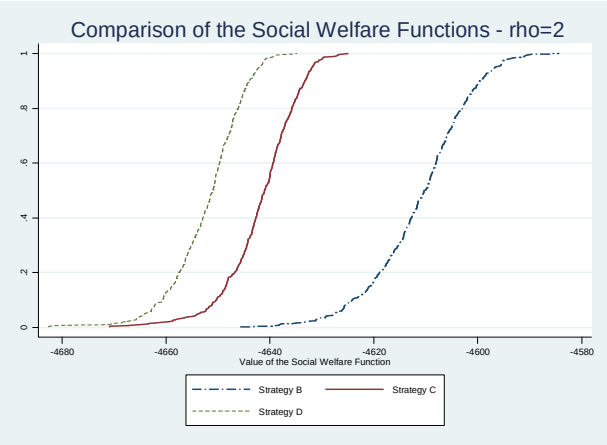


Figure 21

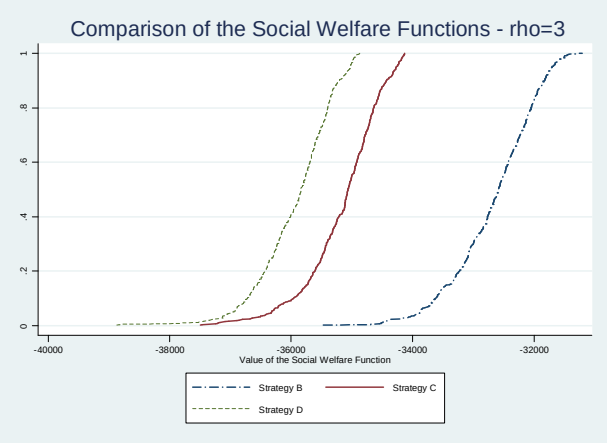


Table 13 Stochastic dominance tests for different pairs of strategies and different values of ρ

	ρ=0	**ρ=1**	**ρ=2**	**ρ=3**
Baseline case				
B and C	B SD1 C	B SD1 C	B SD1 C	B SD1C
B and D	B SD1 D	B SD1 D	B SD1 D	B SD1 D
C and D	C SD1 D	C SD1 D	C SD1 D	C SD1 D
Change of the rate of coverage of antihypertensive treatments				
B and C	B SD1 C	B SD1 C	B SD1 C	B SD1C
B and D	B SD1 D	B SD1 D	B SD1 D	B SD1 D
C and D	C SD1 D	C SD1 D	C SD1 D	C SD1 D
Change of the rate of participation to expenses of the national health insurance				
B and C	B DS1 C	B DS1 C	B DS1 C	**B DS2 C**
B and D	B DS1 D	B DS1 D	B DS1 D	**B DS2 D**
C and D	C DS1 D	C DS1 D	C DS1 D	C DS1 D

5.3.3. Simulations: What would be the impact of a change in the redistribution system on the comparison of the social welfare functions between strategies B, C and D?

Previous sections show that strategy B always dominates the two other strategies whatever the degree of inequality aversion is considered on a one year time horizon. Therefore, we decided to check the sensitivity of our results to changes in the redistribution system regarding contribution to national health insurance expenditures. The aim is to identify what evolutions of the French health care system would be needed in order to guaranty antihypertensive treatments to be efficient.

- Changes regarding the rate of coverage of antihypertensive treatments

Firstly, changes regarding the coverage of antihypertensive treatments and care induced by cardiovascular events related to high blood pressure were tested. The objective is to compare strategies A and B, as well as strategies B, C and D when individuals do not pay out-of-pocket for antihypertensive treatments and follow up anymore as well as for follow up of renal failure. In this scenario, total costs induced by the strategies are supported by tax payers: they are divided between all individuals within the sample according to their participation to the national health insurance expenditures. Figure 22 to Figure 29 show that such changes have no impact on the comparison of the social welfare functions between strategies B, C and D and between strategies A and B. Strategy B always dominate strategy A: whatever the degree of inequality aversion is considered, more than 98 % of the values taken by the social welfare function in B are higher than the value of the social welfare function in A (Figure 22 to Figure 25). Moreover, strategy B always first-order stochastically dominates strategy C and D (and strategy C still dominates strategy D) (Figure 26 Figure 29).

Comparison of strategies A and B

Figure 22

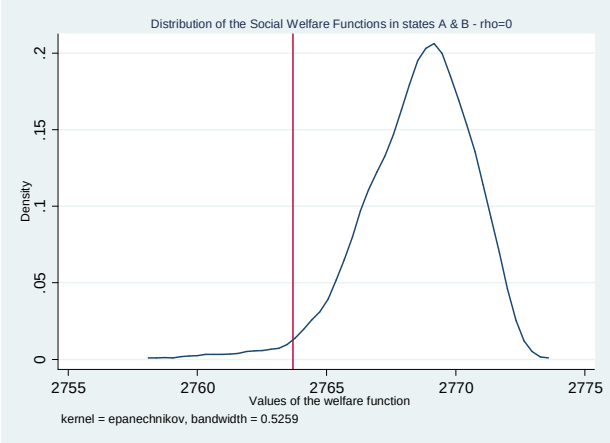


Figure 23

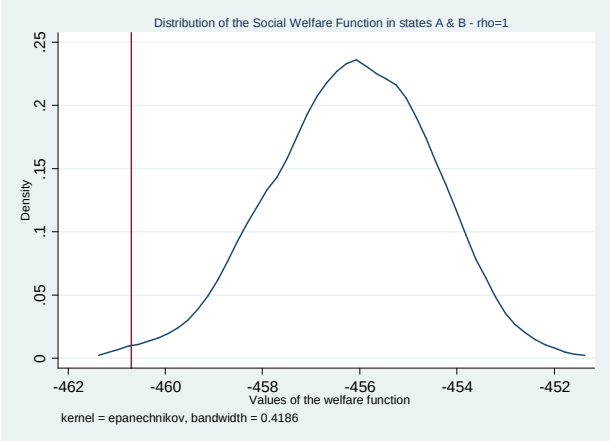


Figure 24

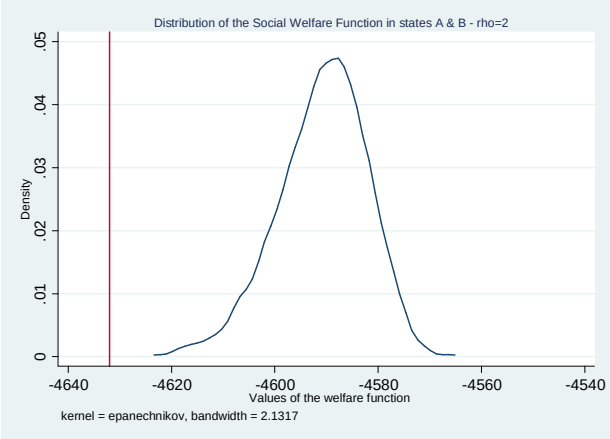
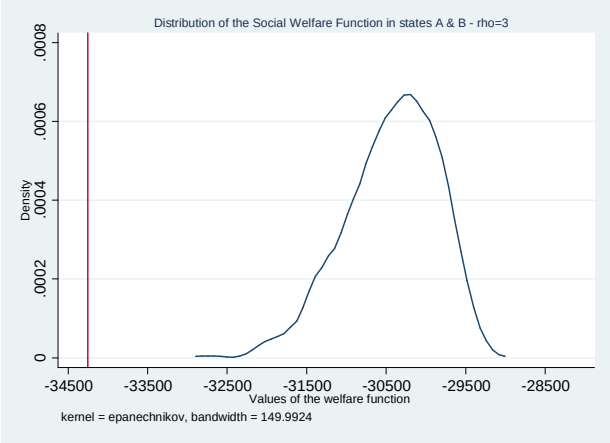


Figure 25



Comparison of strategies B, C and D

Figure 26

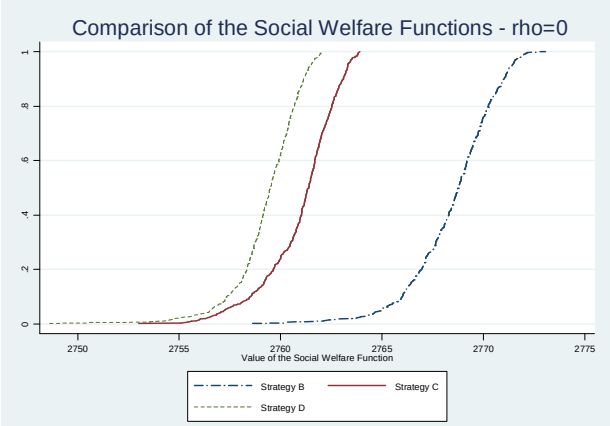


Figure 27

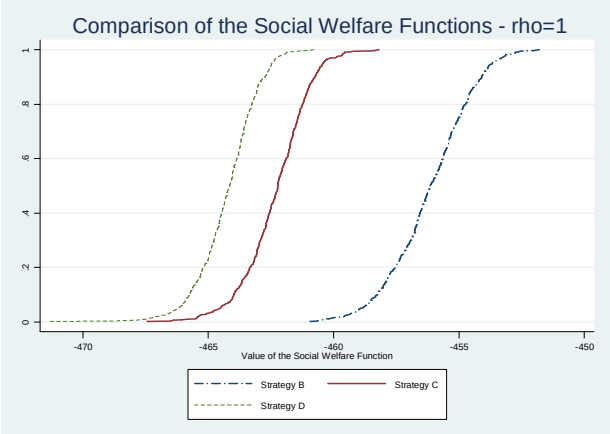


Figure 28

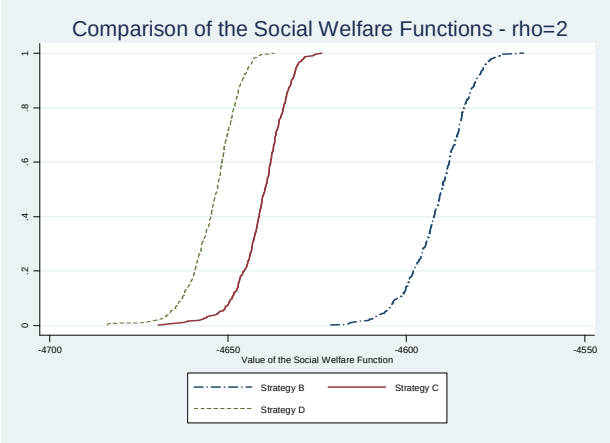
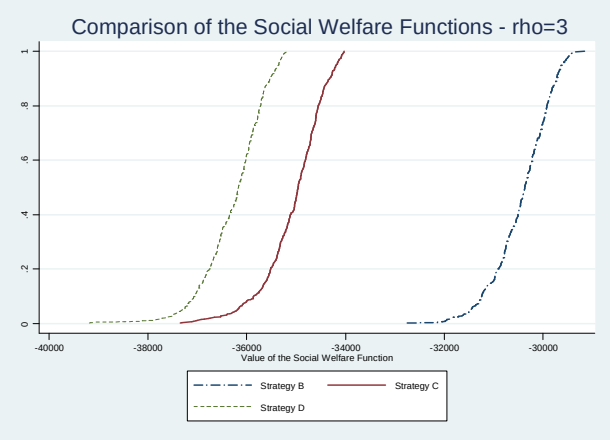


Figure 29



- <u>Changes regarding the rate of participation to expenses of the national health insurance</u>

Secondly, changes regarding the rate of participation to expenses of the national health insurance expenditure were tested. The objective is to compare strategies A and B, and strategies B, C and D if the redistributive system were stronger, i.e. if the poorest individuals of the sample did not support the increase in the cost of insurance due to antihypertensive treatments prescription in primary prevention. In the baseline scenario, as it has been explained in section 4.2.2, the total cost induced by antihypertensive treatments and cardiovascular events that are reimbursed by the national health insurance have been divided between all individuals within the sample according to their rate of participation to the national health insurance expenditures (Caussat *et al.*, 2005). In this simulation, we assess the consequences of strategies B, C and D if individuals who belong to the first quintile of household income were not contributing to national health insurance expenditures anymore (Cf. Table 14).

Table 14: Hypothesis about the participation to health expenditure: actual practice and simulated scenario

Quintile of household income	Current participation to health expenditures (Caussat *et al.*, 2005)	Simulated participation to health expenditures
1	5.05 %	0 %
2	10.7 %	10.6 %
3	16.6 %	17.1 %
4	21.6 %	23.2%
5	46.1 %	49.1%
Total	100 %	100 %

When removing the contribution of individuals who belong to the first quintile of income to national health insurance, strategy B dominates strategy A when the degree of inequality aversion is equal to 0 or 1: more than 98 % of the values taken by the social welfare function in B are higher than the value of the social welfare function in A (Figure 30 to Figure 31) to Figure 31). However, when the degree of inequality aversion is equal to 2, only 88% of the values taken by the social welfare function in B are higher than the value of the welfare function in A, and only 19% when the degree of inequality aversion if 3 (Figure 33).

Moreover, the comparison of strategies B, C and D shows that strategy B first-order stochastically dominates strategy C and D when the degree of inequality aversion is 0, 1 or 2, but only second-order stochastically dominates strategies C and D when the degree of inequality aversion is 3. The ranking of the different strategies is therefore less obvious when a stronger degree of inequality aversion is considered (Figure 37).

Comparison of strategies A and B

Figure 30

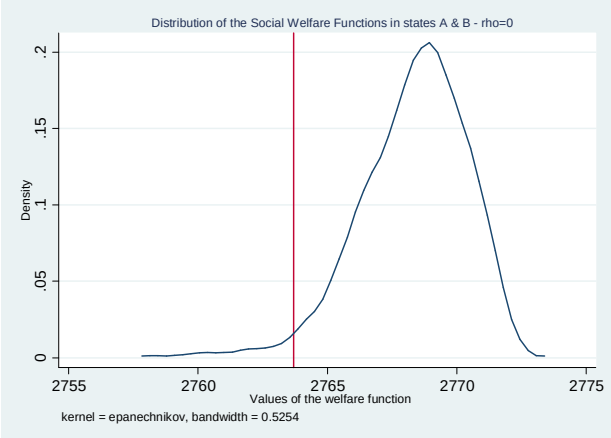


Figure 31

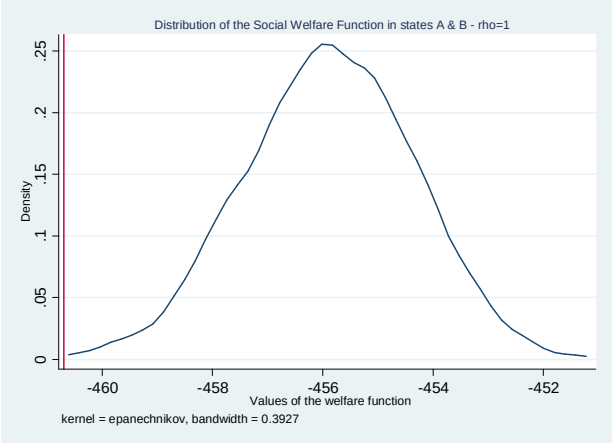


Figure 32

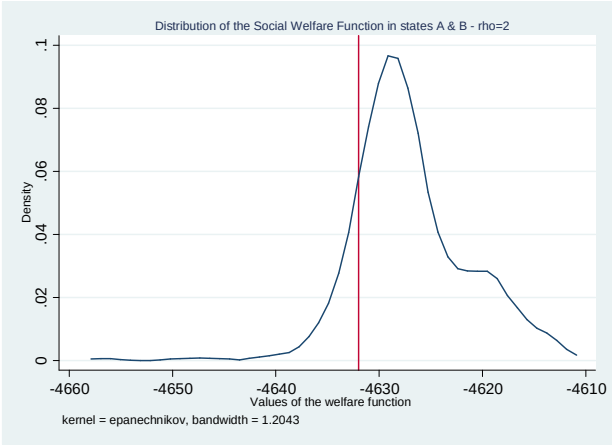
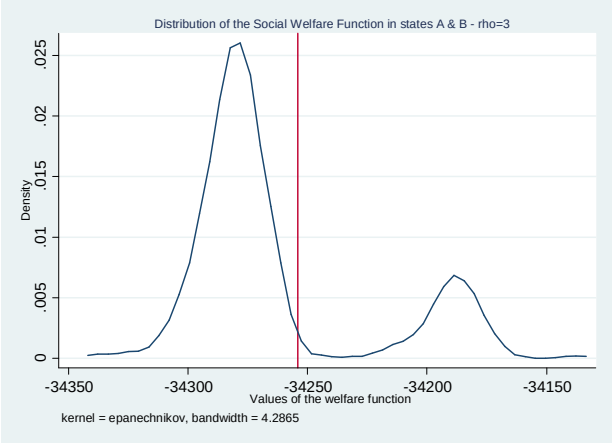


Figure 33



Comparison of strategies B, C and D

Figure 34

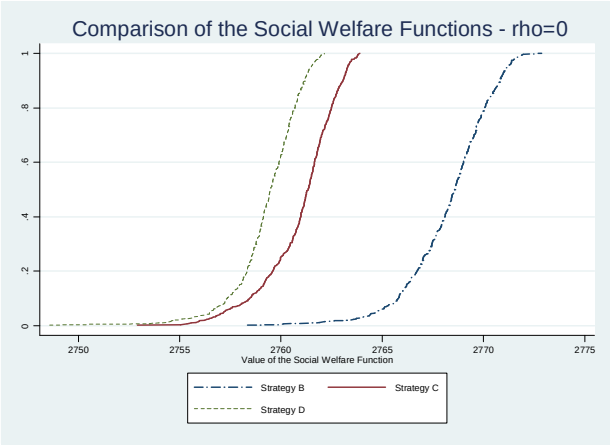


Figure 35

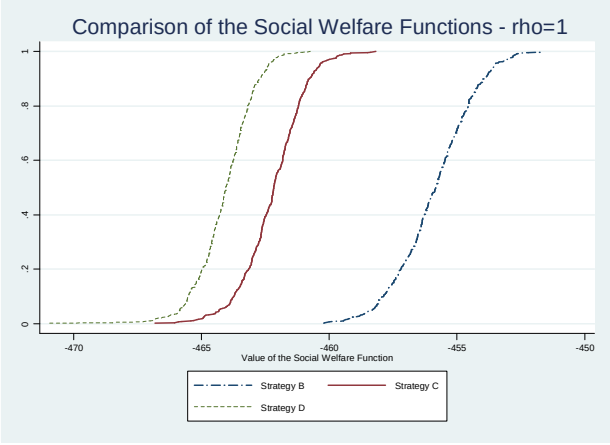


Figure 36

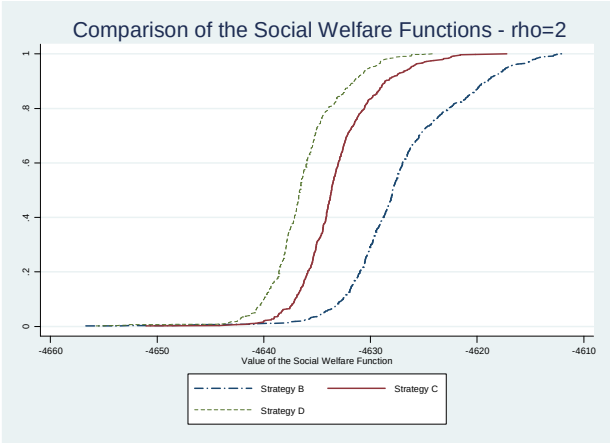
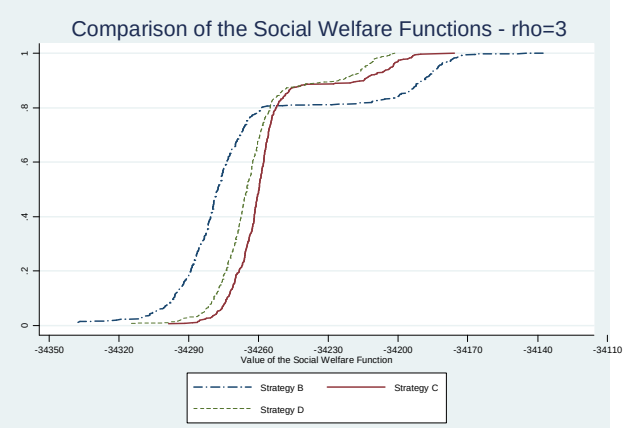


Figure 37



6. Conclusion and Discussion

The comparison of social welfare functions associated with the implementation of strategies B, C and D indicates that, with a time horizon of one year, the two antihypertensive treatments that have been assessed in the present study are not efficient. The comparison of social welfare function associated with strategies A and B also indicates that the current practices of prescribing antihypertensive treatment are not efficient. The inclusion of inequality aversion does not change this outcome. Three reasons could explain this result.

Firstly, the occurrence of cardiovascular events is relatively rare both with the placebo as well as with the two strategies of antihypertensive treatments. Even if they are more frequent with placebo, the differential between the frequencies of events with the placebo compared with the frequencies of events with the two combinations of antihypertensive treatments is not great. Secondly, given our estimations, cardiovascular events have only a small impact on the self-assessed health of individuals affected, according to the simulations, by one or several cardiovascular events in the last 12 months. They also have an even smaller impact on the level of individuals' equivalised income. Thirdly, the willingness-to-pay to be in perfect health is relatively low in this equivalent income survey: on the whole sample, the average is about 82 € and the median is only 1€, given the huge number of individuals who declare a willingness-to-pay equal to 0.

However this study shows that changes in the redistribution system in terms of individuals' contributions to the national health insurance expenditure have an impact on the efficiency of antihypertensive treatments. If poorer individuals (i.e. those who belong to the first quintile of the distribution of equivalised incomes) were not participating to the funding of strategies B,

C and D, through an exoneration of taxes, strategy B would no longer be efficient once an inequality aversion equal to 3 is taken into account. These findings lead us to conclude that the increase in equivalent incomes of individuals who benefit from antihypertensive treatments *via* the prevention of cardiovascular events is competing with the decrease in equivalent incomes of individuals who are tax payers, who do not declare hypertension and whose equivalised incomes are lower when antihypertensive treatments are prescribed in primary prevention. Given the current cost and the current effectiveness of antihypertensive treatments and given the current preferences for health expressed in the survey, those treatments are not worth the decrease in income of poorer individuals who participate to the funding of the national health insurance.

These results differ from the results obtained by HAS who found that antihypertensive treatments are always efficient because placebo (9,923€) is always more expensive than strategy C (9,005€) and strategy D (9,242€). In the present study, strategy B is cheaper than strategies C and D in the 500 draws. However this discrepancy is explained by the difference in the time horizon: it is only one year in this study whereas the time horizon chosen in the HAS model is a lifetime horizon. On a one year time horizon, the results of the HAS model also show that placebo is cheaper than every class of antihypertensive treatments. The differences between the results obtained on a one year time horizon and those obtained on a lifetime horizon are certainly due to the accumulation of secondary cardiovascular events over several years with the placebo. This accumulation of secondary cardiovascular events increases the total cost incurred by strategy B compared with the total cost incurred by strategies C and D. Using a one year time horizon in our assessment is clearly a limitation of our findings. It would have been possible to extent its time horizon, as cumulative risk expectations on 10 years are given by the HAS model. However, we would also need to measure the impact of cardiovascular events on self-assessed health and equivalised income over these 10 years. Only panel data could allow us to overcome this difficulty, as they would give information on the impact of diseases on individuals' careers and health on the long run. Given that the survey is only a cross section, we should put forward too many hypotheses to estimate the cumulative effects of cardiovascular events on health and income over such a long period..

The other divergence with the results of the HAS model is the ranking between strategies C and D. HAS finds that strategy D is the most effective strategy (12.384 mean life years gained) compared with strategy C (12.338 mean life years gained) (Cf. Appendix, Table 32).

In the present study, strategy C is more effective than strategy D in terms of prevention of occurrence of cardiovascular events. This divergence may be explained by the fact that the impact of diuretics in terms of excess risk of diabetes, and thus on renal failure and end-stage renal failure occurrence have not been taken into account in the present study as they were in the HAS model. As treatment C includes diuretics whereas treatment D does not, the efficiency of strategy C in our study may be overestimated.
The results of the HAS assessment and the result of NICE's which are based on cost/QALY approach are very similar. On a lifetime horizon, NICE finds too that treating individuals with high blood pressure with antihypertensive treatments in primary prevention is efficient. They conclude that Calcium antagonist (which are also a component of the treatments C and D in the HAS model) is the most efficient class of drugs in first line treatment (the NICE model does not assess efficiency of second and third line treatments). NICE's result seem consistent with that of the HAS.

Beyond these divergences about the ranking of the three strategies, it is interesting to emphasize the conceptual differences between the results obtained with the three approaches: the cost/life years gained study provided by HAS, the cost/QALY provided by NICE and the equivalent income that is provided in this study. Compared with cost/life years gained study, both cost/QALY study and equivalent approach study take into account not only the impact of cardiovascular events on mortality but also their impact on quality of life, or more broadly, on the individual's welfare. However the cost/QALY study takes into account the impact of cardiovascular event on quality of life on the ground of trade-off between length of life and quality of life. It does not allow assessing the relevance of implementing antihypertensive treatments compared with other competing resource allocation beyond the health care sector. Moreover, cost/QALY implies a threshold to be defined in order to determine if a strategy is efficient or not, when the equivalent income approach implies only to choose the degree of inequality aversion. Finally, the traditional cost/QALY approach cannot take into account the distribution of health gains among the population in order to include egalitarian principles in the health technology assessment aiming to weight differently the consequences of the different strategies depending on the former situation of individuals in terms of health and on other dimensions of their life.

The main objective of the present study was to prove the feasibility of the equivalent income approach? for public decision making, and more particularly to identify the data that would be

needed to process to this assessment compared with traditional approaches. Clinical data about effectiveness of the treatments and data about costs that have been used to process to the simulation of the social welfare functions were the same than those used in the Markov model that are produced within cost/life years gained and cost/QALY studies. Only information about the willingness-to-pay to be in perfect health, the self-assessed-health and the level of equivalised incomes, that are necessary to compute individuals' equivalent incomes, are specific to our approach and not needed by traditional ones. However, it seems that these information are not more difficult to obtain than information about utility weights in the EQ-5D scoring function used in cost/QALY studies. Moreover, like surveys that are used to define utility weights in the EQ-5D scoring function, surveys used to estimate the level of equivalent income do not need to be realised for each assessment. Once a survey is conducted on a representative sample of the population, it provides generic information that can be used for several years and that can be used to assess various treatments in many conditions. Indeed, the survey that has been used in this study to assess efficiency of antihypertensive treatments may also be used to assess cancer screening or another kind of medical treatment. The only constraint is to have a sufficient number of individuals in the sample who declare the disease in order to guaranty the representativeness of the data and of the conclusions of the assessment. Thus, we can conclude that data that are necessary to process to equivalent income assessment were not more difficult to obtain than data needed to process to traditional health technology assessments. Moreover, the modelling method that has been presented here is not more complex than the modelling method needed to produce a traditional Markov model in cost/life years gained and cost/QALY studies.

Finally, to test to feasibility of equivalent income approach in health technology assessment, a further development would be to use this approach in order to assess health care that does not prevent the disease but that helps to reduce its impact on life expectancies and quality of life. Data provided by the equivalent income survey were focused on the willingness-to-pay to be in perfect health: it was consistent to use them to assess the willingness-to-pay to prevent cardiovascular events. However the use of information about the willingness-to-pay to be in perfect health to assess the efficiency of an improvement of health rather than a prevention of the disease is still a methodological difficulty that should be raised without falling in a cardinal estimation of health gains.

Conclusion générale

L'objectif poursuivi dans le cadre de cette thèse était d'étudier les implications philosophiques des choix méthodologiques que sous-tendent les évaluations économiques des interventions de santé lorsqu'elles sont réalisées dans un objectif d'aide à la décision publique en matière de définition du périmètre des soins remboursables. Il s'agissait également d'examiner comment les réflexions récentes menées en économie du bien-être pouvaient être mobilisées de telle sorte que les résultats de ces évaluations soient plus adaptés aux principes de justice sociale que visent les décideurs au nom de la collectivité qu'ils représentent. La démarche qui a été adoptée ne permettait pas d'identifier de façon systématique l'ensemble des controverses philosophiques que soulève l'évaluation économique des interventions de santé en général dans la mesure où nous avons choisi de proposer trois « cas pratiques » de mise en œuvre d'un dialogue entre éthique et évaluation économique. Néanmoins, nous avons pu tirer de l'analyse de ces « cas pratiques » des enseignements dont la portée dépasse leur strict périmètre.

Le premier chapitre nous a permis d'identifier, au travers de la comparaison des guides méthodologiques des trois agences d'évaluation - le NICE, l'IQWiG et le KCE - l'existence de deux controverses majeures qui structurent l'ensemble des choix méthodologiques :

- la première controverse porte sur le choix de l'objet dont il convient d'assurer une juste répartition dans la population : elle renvoie, dans le cas particulier de l'évaluation des politiques de santé, au choix du critère de mesure des gains en santé (objectifs, subjectifs ou mixtes) ;
- la seconde controverse porte sur le choix du critère de répartition de cet objet dans la population : elle renvoie à la question de l'objectif du système de santé (maximisation de la somme totale des gains en santé ou égalisation de la répartition de ces gains en santé dans la population).

Nous avons vu que la première controverse mettait en concurrence les tenants d'une conception universaliste et rationaliste selon lesquels les conditions du « bien vivre » pourraient être définies objectivement et de façon consensuelle par opposition aux tenants du préférentialisme qui s'appuient sur une tradition philosophique utilitariste, et plus généralement empiriste, et accordent moins de confiance dans la capacité de s'accorder rationnellement sur ce qui serait bon pour tous. Par conséquent, ces derniers recommandent de laisser chaque individu juger des conditions de son propre bien être.

Par ailleurs, nous avons vu que la seconde controverse mettait en concurrence les tenants d'une approche déontologique, qui recommandent de juger la moralité d'une action en tant que telle, quelles qu'en soient les conséquences à long terme, par opposition aux tenants d'une approche conséquentialiste. C'est bien en effet la polémique classique entre déontologie et conséquentialisme que l'on retrouve lors des débats que suscitent, notamment, la prise en charge des traitements en fin de vie et le principe de la « règle de sauvetage ». Pour autant, l'analyse des méthodes d'évaluation des trois agences a permis de montrer qu'aucune d'entre elles n'adopte réellement de position déontologique, y compris lorsqu'elles s'écartent d'un objectif de maximisation des gains en santé. Elles semblent plutôt se tourner vers une position de type « prioritariste » qui consiste à donner davantage de valeur à l'amélioration de l'état de santé de certains individus en fonction de leur situation initiale. Les critères de cette priorisation ne sont cependant pas pris en compte dans le calcul économique. Ils apparaissent plutôt au travers des dispositifs de dérogation par rapport à la valeur seuil coût/efficacité, par exemple pour les traitements de fin de vie dans les recommandations du NICE ou dans le cadre du processus de délibération qui accompagne le refus de fixer une valeur seuil coût/efficacité qui soit valable à un niveau transpathologique (KCE et IQWiG)

Nous aurions pu aller plus loin et tenter d'établir un lien, au moyen d'une approche historique, entre la prévalence de certaines idéologies, ou systèmes de pensée (libéralisme français fondé sur le principe d'universalité, radicalisme anglais ou libéralisme nord-américain), et les positions qu'adoptent les trois agences à l'égard de ces deux controverses[102]. Mais il parait ici plus intéressant de souligner que les choix méthodologiques des agences publiques

[102] Sur les différentes formes de libéralisme Cf. Jaume L., 1997, 2000 ; Audard C., 2009 ; Halévy E., 1995 ; Burdeau G., 1979. *« La tradition du libéralisme de la liberté date au moins de la Réforme et assigne une priorité spécifique à certains droits fondamentaux : la liberté de conscience et de pensée, la liberté personnelle et le libre choix d'une vocation – le fait d'être libre de l'esclavage et de la servitude –, pour en mentionner quelques-uns des principaux. (…) Cela ne garantit pas pour autant leur bonheur, qui relève de la responsabilité de chacun d'entre eux. Le libéralisme des utilitaristes (classiques) – Bentham, James Mill et Sidgwick – est différent du libéralisme de la liberté. Son premier principe est celui du plus grand bonheur du plus grand nombre. »* (Rawls, 2002, p. 357).

d'évaluation tendent aujourd'hui de plus en plus à converger vers la recherche d'un équilibre entre ces controverses. Il est donc plus pertinent de chercher les moyens de la conciliation de ces différentes positions (universalisme *versus* préférentialisme, maximisation *versus* égalisation) plutôt que de rechercher à comprendre les racines de ces oppositions. Ainsi, il faut, comme l'exigeait Platon au sujet de la polémique entre Parménide et Héraclite, et conformément à ce que recommande Sen, *« être comme un enfant et vouloir les deux à la fois »*[103] : non pas en juxtaposant ces diverses exigences, mais en travaillant en vue de leur dépassement[104]. *«Un dilemme est un conflit entre plusieurs choses bonnes en elles-mêmes, dont chacune mérite notre considération, mais qui s'opposent mutuellement. Nous ne pouvons donc espérer résoudre un véritable dilemme en choisissant d'ignorer totalement un aspect du cas qui nous occupe en faveur de l'autre »* (Sen A., 1999). Il nous paraît certain que c'est grâce aux travaux qui sont actuellement menés en philosophie politique et en économie normative, en particulier au sein des théories libérales égalitaristes, que l'on peut trouver les moyens d'une telle conciliation (Gamel, C., 2005). Pour cette raison, nous avons fait le choix de nous concentrer sur la faisabilité de leur mise en œuvre dans les pratiques quotidiennes d'évaluation des interventions de santé dans le cadre des chapitres II et III.

Le deuxième chapitre nous a permis d'examiner les controverses philosophiques que soulève le choix de la perspective de l'évaluation des gains de santé que permettent obtenus par deux dispositifs de compensation du handicap : l'hormone de croissance chez l'enfant non déficitaire et les implants cochléaires bilatéraux chez l'enfant présentant une surdité à la naissance. Nous avons vu que le caractère congénital de ces deux incapacités et l'irréversibilité des conséquences des deux interventions favorise la survenue d'un phénomène d'adaptation des préférences qui génère un dilemme moral pour l'évaluateur : le

[103] Platon évoque cette double exigence au sujet de la controverse philosophique sur la substance de l'Etre : tandis que Parménide défendait l'idée d'une permanence absolue de l'Etre, Héraclite défendait au contraire l'idée d'une impermanence complète de l'Etre qui est le plus souvent résumé autour de la célèbre formule *« on ne se baigne jamais dans le même fleuve »*. Pour Platon, l'élaboration d'un discours rationnel nécessite que l'on mobilise l'une et l'autre de ces positions. On ne peut pas penser le monde qui nous entoure, et qui est en mouvement, sans accepter l'idée de devenir et de causalité. De même, on ne peut pas être rationnel si l'on refuse de considérer qu'il existe des concepts qui transcendent l'apparente fluctuation du monde sensible. En l'occurrence, si l'eau du fleuve dans laquelle je me baigne n'est jamais, littéralement, « la même », l'idée du fleuve, elle, existe bel et bien. (Platon, le Sophiste, ed. Garnier Flammarion)

[104] C'est également ce que Scanlon semble indiquer lorsqu'il explique que le rejet de l'utilitarisme ne peut pas passer par une mise à l'écart de principe, mais par la reconnaissance de sa puissance, qui est une étape obligée de son dépassement. *« L'utilitarisme occupe une place centrale dans la philosophie morale de notre temps. Ce n'est pas le point de vue que la plupart des individus partage ; certainement un très petit nombre se réclamerait de l'utilitarisme d'acte. Mais pour une beaucoup plus grande partie d'entre eux, c'est la position vers laquelle ils se trouvent eux-mêmes acculés quand ils tentent de donner une référence théorique à leurs convictions éthiques. A l'intérieur de la philosophie morale, l'utilitarisme représente une position contre laquelle il faut lutter si on veut la contourner »* (Scanlon, 1982, p.103).

choix des répondants proxys chargés d'estimer la valeur des gains obtenus aurait donc un impact déterminant sur les résultats de l'évaluation. Et, dans la mesure où les individus concernés pourraient s'adapter à leur incapacité, la valeur qu'ils accordent à l'amélioration de leurs capacités fonctionnelles serait moindre que celle que leur accorderaient des individus issus de la population générale qui n'y sont pas adaptés.

Le caractère extrême du phénomène d'adaptation des préférences dans le cas de la surdité, révèle que l'économiste ne peut effectuer un choix concernant la perspective de l'évaluation des gains en santé sans effectuer un jugement de valeur social. En définitive, nous avons vu que le choix de la perspective dépend de l'objectif du système de santé. Si l'objectif du système de santé est d'égaliser les capabilités, il convient alors de privilégier la perspective des individus qui bénéficient de meilleures capacités fonctionnelles et qui font l'expérience des opportunités que ces capacités fonctionnelles leur offrent au quotidien. Au contraire, si l'objectif du système de santé est d'égaliser les chances de bien-être, il peut être justifié de centrer l'évaluation sur la perspective d'individus qui souffrent de l'incapacité, sur laquelle porte l'intervention, pour identifier les conditions d'émergence du phénomène d'adaptation des préférences qui, *in fine*, favorise leur bien-être.

Nous nous sommes en effet interrogés sur la coïncidence entre certaines ressources fondamentales, telles que l'éducation, l'accès à des activités de loisir ou à des activités artistiques etc., et les facteurs d'adaptation des préférences, ce qui nous a conduit à proposer d'élargir le périmètre de l'évaluation de telle sorte que l'on puisse comparer des interventions non médicales permettant de favoriser l'acquisition de ces ressources fondamentales. Pour poursuivre dans cette voie, il était cependant nécessaire de distinguer : d'une part, les ressources fondamentales dont la juste répartition dans la population relève de la responsabilité de la collectivité ; d'autre part, celles qui sont du ressort de la sphère privée de l'individu. Nous avons alors proposé de nous appuyer sur les principes de justices qui sont avancés par Fleurbaey dans son article intitulé *Equal Opportunity or Equal Social Outcome* (Fleurbaey M., 1995). Nous avons ainsi considéré que la collectivité était justifiée de financer une intervention de santé dès lors qu'il était démontré qu'une incapacité avait un impact significatif sur les réalisations sociales fondamentales de l'individu et que l'intervention pouvait efficacement réduire ces inégalités.

Les conclusions que nous avons pu tirer dans le cadre de ce chapitre dépassent le périmètre de ces deux interventions de santé. Elles pourraient effectivement s'appliquer à l'évaluation de l'ensemble des dispositifs de compensation du handicap ; elles pourraient même être pertinentes dans le cadre de l'évaluation d'interventions médicales en général. Il serait en

effet possible d'évaluer l'ensemble des interventions médicales en termes d'égalisation des capabilités, d'égalisation des chances de bien-être ou en termes d'égalisation des réalisations fondamentales sociales. Et ce, dans la mesure où, comme nous l'avons rappelé dans l'introduction générale, un état de santé minimum est la condition de possibilité qui permet de jouir des autres biens, de même qu'elle est la condition de possibilité des opportunités de fonctionnements.

Enfin, le troisième chapitre nous a permis de montrer la faisabilité de l'évaluation des interventions de santé fondée sur une approche par le revenu équivalent-santé dans l'aide à la décision en matière de définition du périmètre des soins remboursables. L'objectif était d'examiner comment l'économiste pouvait concrètement adapter ses méthodes d'évaluation de telle sorte qu'il puisse proposer aux décideurs des conclusions qui soient conformes à d'autres modèles de justice sociale qu'utilitaristes.

Nous avons constaté que cette approche n'impliquait pas de disposer de données supplémentaires par rapport aux évaluations fondées sur des approches classiques de type coût/efficacité ou coût/QALY. La modélisation que nous avons utilisée repose, certes, sur une méthode de tirage relativement originale, justifiée par le choix d'une perspective *ex post*. Toutefois, ces tirages ont été effectués en utilisant les mêmes données d'efficacité que celles utilisées dans les modèles de Markov traditionnellement utilisés dans l'évaluation économique des interventions de santé. De même, les coûts permettant d'estimer l'impact de la mise en œuvre des différentes interventions sur les revenus équivalent-santé de l'ensemble des individus (patients et contribuables) sont similaires à ceux mobilisés dans les analyses coût/efficacité ou coût/utilité dès lors qu'elles sont réalisées dans une perspective dite « sociétale ». Les seules informations particulières que nécessite l'approche par le revenu équivalent-santé concernent les préférences des individus en matière d'arbitrage revenu *versus* parfaite santé, ainsi que des informations sur l'impact de la maladie sur les revenus. Nous avons cependant avancé l'hypothèse selon laquelle ces données ne sont pas plus difficiles à obtenir que les données qu'utilisent les économistes pour évaluer l'impact d'une intervention sur la qualité de vie au moyen de scores d'utilité mesurés en population générale par exemple dans le cadre de l'échelle EQ-5D.

Le principal résultat de cette étude est la démonstration que l'approche par le revenu équivalent-santé permet d'inclure la question du financement de l'intervention de santé dans l'évaluation économique. Nous avons pu en effet constater que la condition sous laquelle les traitements antihypertenseurs pouvaient être jugés efficients était la modification de la participation actuelle des contribuables aux dépenses de l'assurance maladie dès lors que les

décideurs choisissaient d'adopter un fort degré d'aversion aux inégalités. Il est vrai qu'une telle conclusion ne peut pas avoir de conséquences pratiques sur les modalités de financement, dans la mesure où celles-ci sont réparties entre tous les individus pour une enveloppe globale comprenant le financement de l'ensemble des dépenses de l'assurance maladie. Toutefois, il nous semble que cette information permet de mettre en exergue les arbitrages que doivent nécessairement effectuer les décideurs publics et qui mettent en concurrence les intérêts des uns (en l'occurrence les individus qui souffrent d'hypertension essentielle) et ceux des autres (les contribuables dont les revenus sont les plus faibles).

Les travaux qui sont présentés dans le cadre des trois chapitres pourraient chacun faire l'objet de développements ultérieurs. Il serait par exemple intéressant de mettre en œuvre les propositions méthodologiques qui sont avancées dans le deuxième chapitre au sujet de l'évaluation de l'hormone de croissance et/ou des implants cochléaires bilatéraux, ou au sujet d'autres interventions de santé présentant une problématique similaire.
De même il serait utile de mener d'autres évaluations fondées sur une approche par le revenu équivalent-santé pour tester sa faisabilité au sujet d'autres interventions de santé, en particulier celles qui ne permettent pas d'éviter la survenue d'une maladie mais qui permettent d'améliorer la qualité de vie et/ou l'espérance de vie des individus qui en sont atteints (en l'occurrence les traitements antihypertenseurs permettaient d'éviter la survenue d'événements cardiovasculaires). De telles évaluations soulèvent en effet des enjeux méthodologiques importants car l'utilisation du concept de revenu équivalent-santé, qui s'appuie sur les préférences des individus pour la parfaite santé, pose question lorsqu'il s'agit d'évaluer l'intérêt d'une amélioration partielle de l'état de santé.

L'objectif de ce travail était de donner davantage de visibilité aux diverses possibilités qu'offre l'évaluation économique en matière de prise en compte de principes de justice, afin que les acteurs de l'évaluation puissent fournir aux décideurs des résultats qui soient adaptés aux valeurs que la collectivité souhaite appliquer en matière d'allocation des ressources en santé.
Il semble toutefois nécessaire de souligner que la mise à disposition d'outils permettant de prendre en compte ces différents principes de justice sociale, comme le propose notamment l'approche par le revenu équivalent-santé au sujet du degré d'aversion aux inégalités, accroit la responsabilité du décideur lorsqu'il s'agira d'assumer de tels choix de valeurs. En ce sens il existe bien une dimension éthique dans le travail de l'économiste puisque celui-ci favorise

l’explicitation et la cohérence des critères qui sous-tendent la définition du périmètre des soins remboursables. Les interactions entre éthique et économie sont donc bien réciproques.

Annexes

Table 15 Impact of the diseases on the SAH – full estimation

	SAH (Model 1)	SAH (Model 2)	SAH (Model 3)
hypertension	-3.125***	-1.952**	-1.316
	(0.936)	(0.942)	(0.939)
angina	-6.088**	-5.378**	-5.055**
	(2.393)	(2.385)	(2.367)
myocardial infarction	-6.254***	-5.506**	-5.719***
	(2.244)	(2.231)	(2.207)
heart rythm disorder	-3.326***	-3.179**	-2.876**
	(1.248)	(1.237)	(1.225)
stroke	-7.200**	-6.556**	-6.837**
	(2.942)	(2.906)	(2.879)
arteritis	-5.025*	-4.590*	-4.303*
	(2.596)	(2.567)	(2.551)
varicose vein	0.773	1.021	0.892
	(1.352)	(1.346)	(1.332)
hemorrhoids	2.129	2.290	2.230
	(1.424)	(1.414)	(1.399)
bronchitis	-4.578***	-3.366***	-2.772**
	(1.187)	(1.187)	(1.191)
asthma	-3.294***	-3.517***	-3.177***
	(1.205)	(1.195)	(1.187)
angine	0.741	0.526	0.210
	(2.213)	(2.190)	(2.171)
sinusitis	-4.064***	-4.222***	-4.041***
	(1.076)	(1.065)	(1.055)
nasopharyngitis	2.042**	1.140	0.802
	(0.913)	(0.910)	(0.902)
allergic rhinitis	1.391	0.656	0.378
	(1.030)	(1.023)	(1.014)
otite	-0.416	-0.882	-0.200
	(1.694)	(1.688)	(1.677)
deafness	-2.565**	-1.416	-1.568
	(1.077)	(1.097)	(1.086)
glaucom	1.221	1.455	1.100
	(2.571)	(2.544)	(2.519)
cataract	-3.389*	-2.480	-2.652
	(1.766)	(1.776)	(1.757)
caries	0.222	-0.226	-0.178
	(0.747)	(0.751)	(0.744)
ulcer	-2.456	-2.343	-2.273
	(1.982)	(1.962)	(1.944)
acid reflux	-0.550	-0.170	-0.045
	(1.156)	(1.145)	(1.135)
gastralgia	-2.023*	-2.067**	-1.870*
	(1.045)	(1.033)	(1.023)

colite	-1.683 (1.294)	-1.879 (1.284)	-1.965 (1.271)
hepatitis	-7.560** (3.435)	-6.913** (3.421)	-5.987* (3.389)
lumbago	-4.748*** (0.731)	-4.127*** (0.729)	-3.970*** (0.723)
arthrosis of the knee	-4.068*** (1.042)	-2.821*** (1.054)	-2.333** (1.049)
arthrosis of the hip	-7.325*** (1.396)	-6.530*** (1.385)	-6.402*** (1.371)
urinary infection	-0.843 (1.475)	-1.479 (1.473)	-1.266 (1.458)
menstrual disorders	-4.273** (1.781)	-5.280*** (1.786)	-4.853*** (1.772)
menopause troubles	-4.987*** (1.931)	-3.765* (1.938)	-3.798** (1.919)
overgrowth of the prostate	0.650 (2.755)	1.119 (2.745)	0.616 (2.716)
diabetes	-5.573*** (1.251)	-4.672*** (1.242)	-3.589*** (1.244)
malfunction of thyroid	-6.467*** (1.575)	-6.064*** (1.573)	-5.704*** (1.559)
cholesterol	-3.231*** (0.934)	-2.414** (0.941)	-2.146** (0.933)
depression	-9.972*** (1.140)	-9.433*** (1.136)	-9.086*** (1.125)
anxiety	-3.710*** (0.776)	-3.424*** (0.770)	-3.429*** (0.765)
Parkinson’s	-1.515 (5.769)	-0.464 (5.698)	-0.035 (5.641)
migraine	-1.001 (0.943)	-1.626* (0.952)	-1.621* (0.943)
Alzheimer’s	-10.416 (8.502)	-10.650 (8.407)	-10.682 (8.318)
epilepsy	-4.173 (3.285)	-3.747 (3.263)	-3.553 (3.231)
eczema	-2.393* (1.342)	-2.779** (1.333)	-2.520* (1.320)
psoriasis	-1.111 (1.616)	-1.559 (1.598)	-1.606 (1.583)
handicap	-11.307*** (2.028)	-10.588*** (2.012)	-10.077*** (1.994)
infirmity	-6.203** (2.745)	-5.680** (2.717)	-4.928* (2.706)
cancer	-11.071*** (2.044)	-10.469*** (2.025)	-11.208*** (2.008)
age_cont		-0.362*** (0.101)	-0.266*** (0.102)
age_cont2		0.002** (0.001)	0.001 (0.001)

male		0.167 (0.667)	0.366 (0.676)
equiv.Inc ≤ 875€		-2.982*** (0.918)	-2.350** (0.913)
equiv.Inc є [875-1290]		-1.737** (0.876)	-1.545* (0.867)
equiv.Inc ≥ 1800		0.662 (0.885)	0.698 (0.876)
marital life		-0.207 (0.685)	-0.382 (0.684)
children		0.484 (0.731)	0.519 (0.726)
no diploma		-3.119** (1.283)	-2.427* (1.272)
primary school certificate		-1.445 (1.302)	-1.251 (1.289)
“brevet” (=GCSE)		-2.112** (0.930)	-1.992** (0.921)
university (≤ 3 years)		-0.265 (1.156)	-0.245 (1.144)
university (≥ 4 years)		-1.136 (1.108)	-1.803 (1.100)
other diploma +		-18.214* (9.594)	-19.165** (9.496)
national Health Ins. only		-1.869 (1.274)	-1.396 (1.265)
CMU only		-0.791 (1.403)	-0.401 (1.394)
smoker			-2.417*** (0.697)
alcohol – no risk +			2.673*** (0.700)
alcohol–risky behaviour +			-0.972 (1.477)
underweight			-0.203 (1.673)
overweight			-1.693** (0.731)
obese			-5.083*** (0.949)
severely_obese			-6.510*** (1.932)
_cons	83.026*** (0.467)	96.018*** (2.452)	93.778*** (2.467)
N	2513	2513	2513
adj. R2	0.29	0.31	0.33
F	24.128	19.718	18.996
rmse	15.153	14.942	14.778

Standard errors in parentheses
* p<.1, ** p<.05, *** p<.01

Table 16 : Number of events that occur with strategy B at least once during the 500 draws

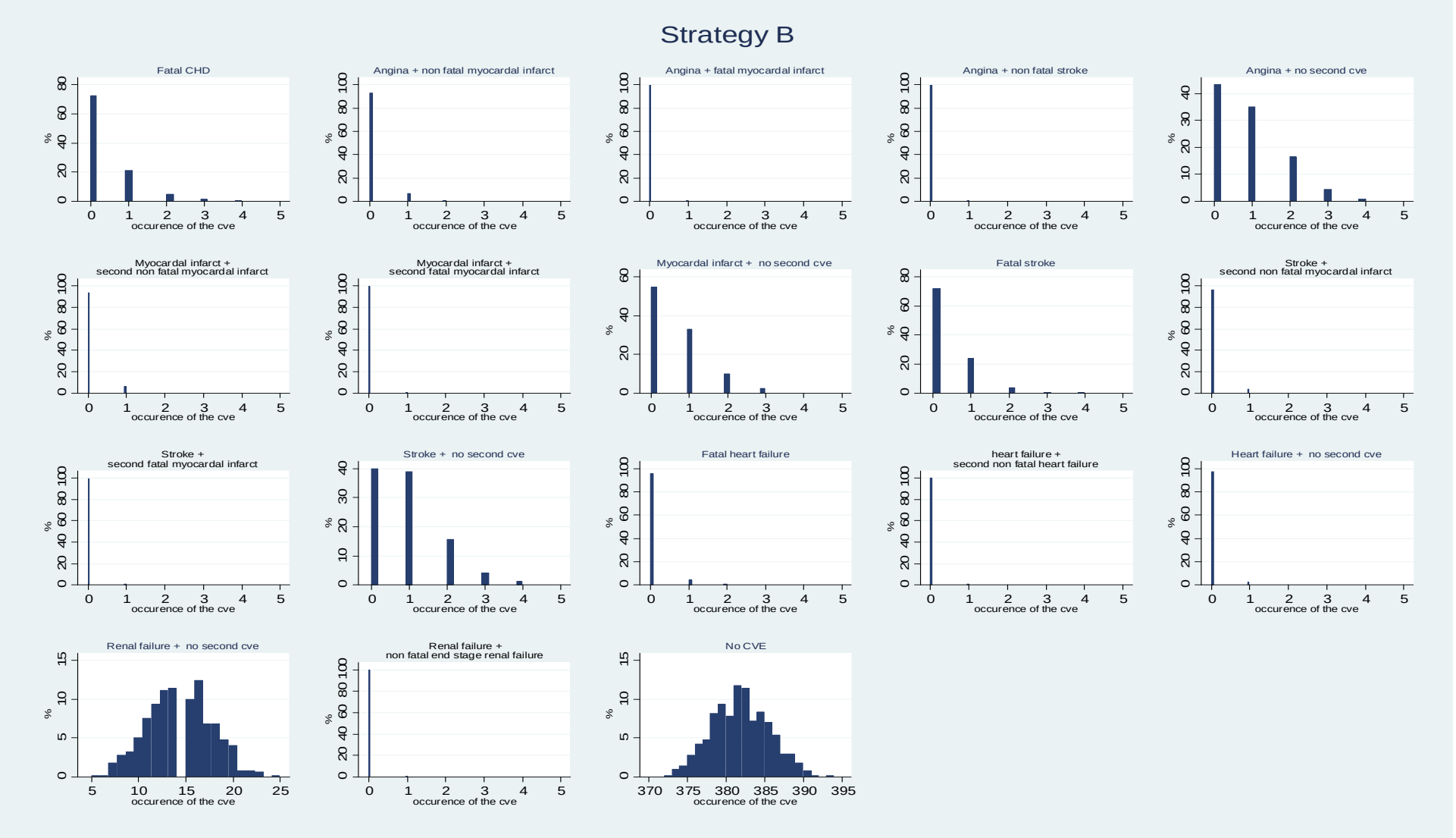


Table 17: Number of events that occur with strategy C in the 1st-line treatment (monotherapy) at least once during the 500 draws

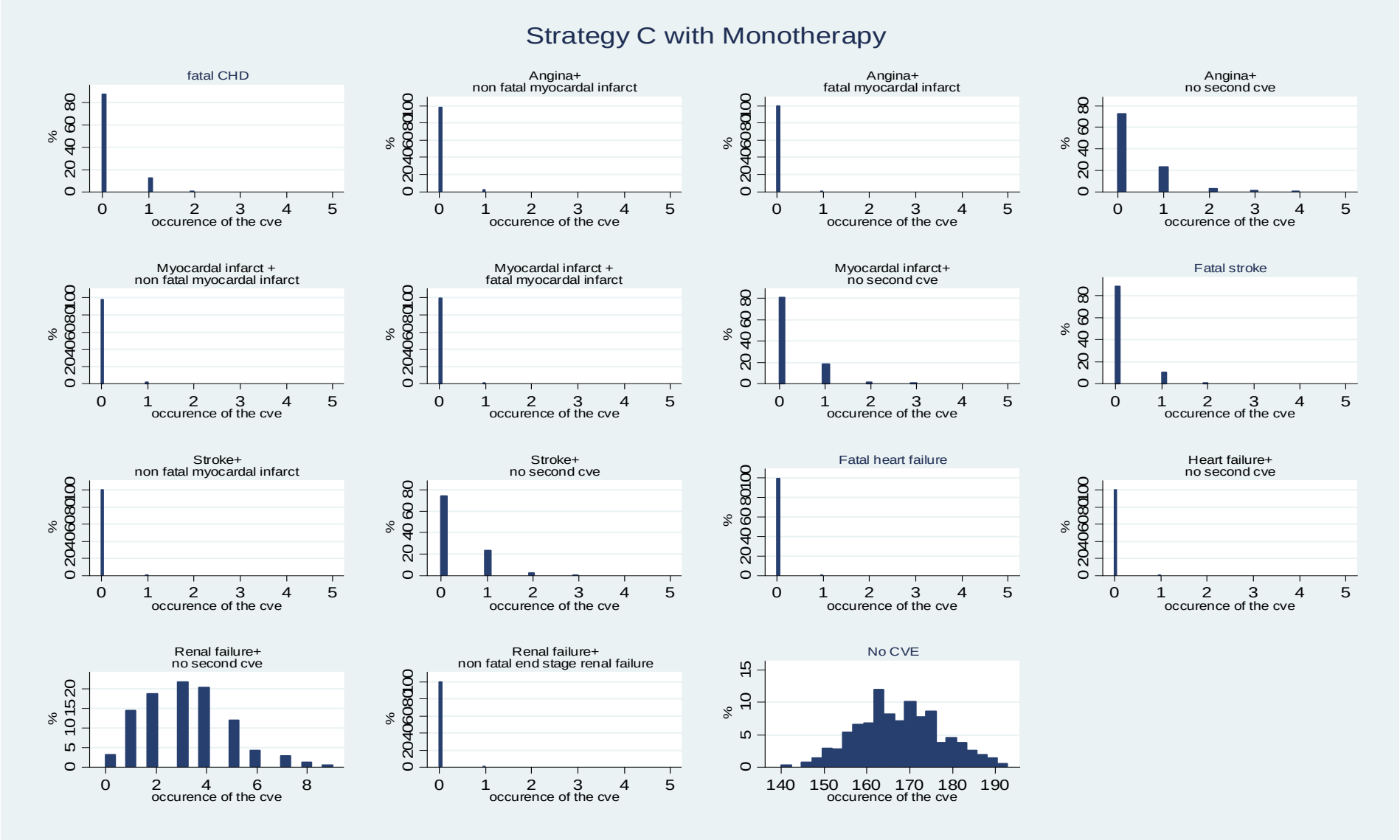


Table 18: Number of events that occur with strategy C in the 2nd-line treatment (bitherapy) at least once during the 500 draws

Table 19: Number of events that occur with strategy C in the 3rd-line treatment tritherapy) at least once during the 500 draws

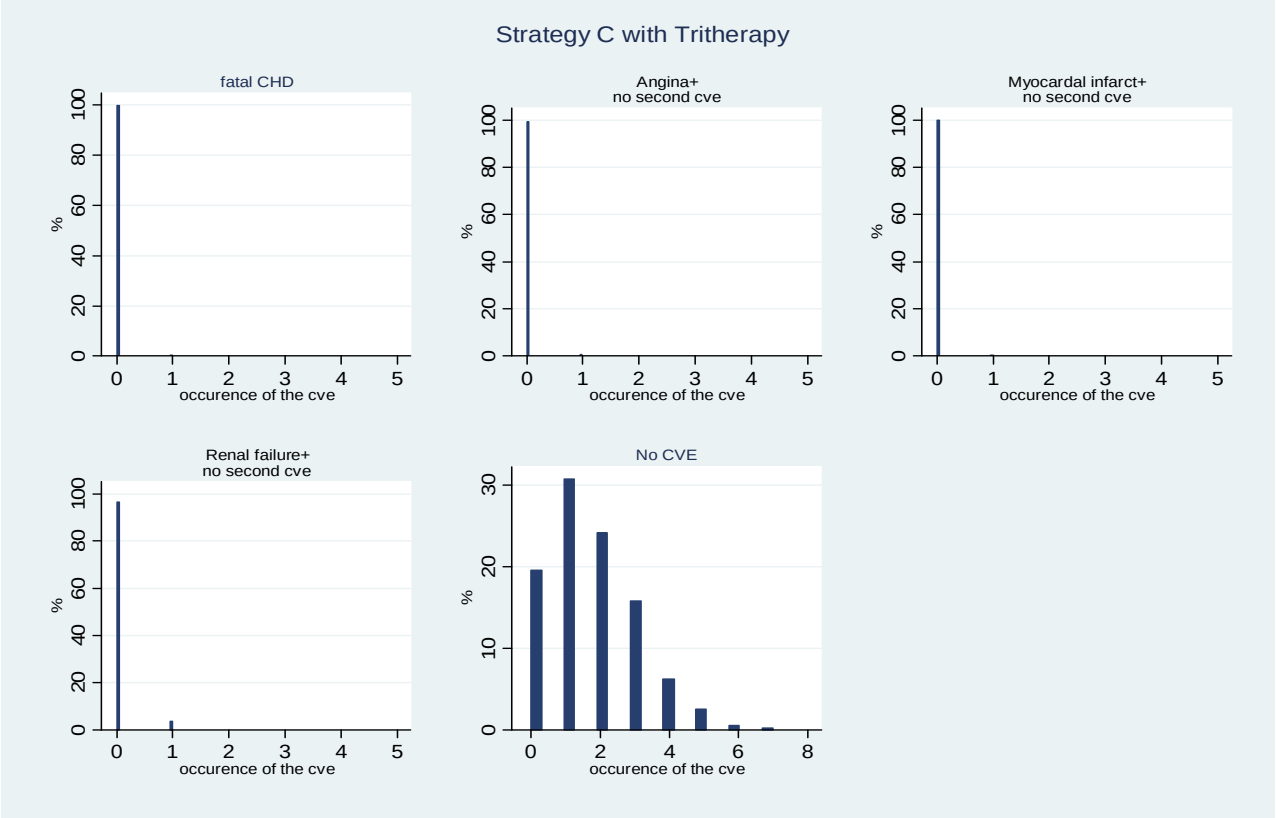


Table 20: Number of events that occur with strategy D in the 1st -line treatment (monotherapy) at least once during the 500 draws

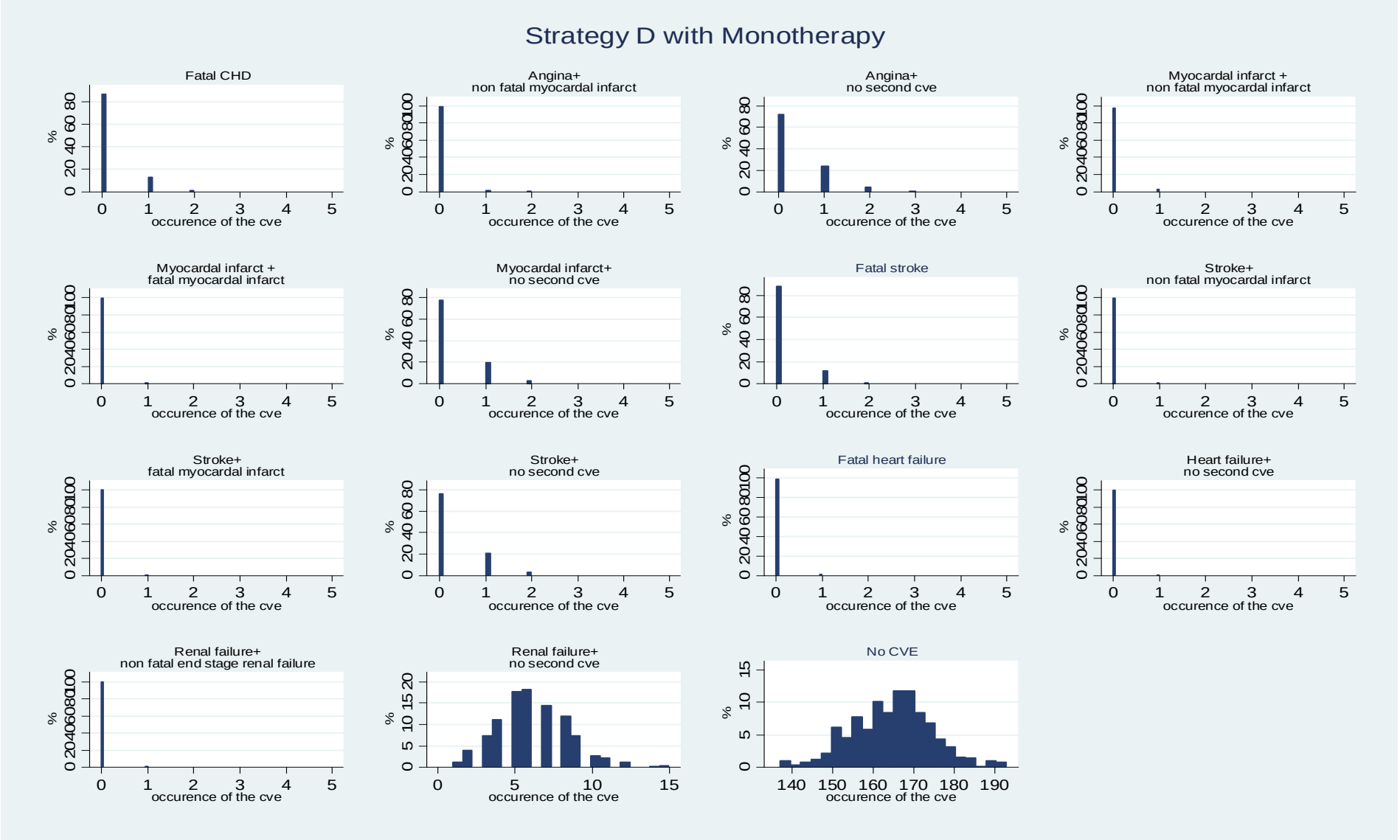


Table 21: Number of events that occur with strategy D in the 2nd-line treatment (bitherapy) at least once during the 500 draws

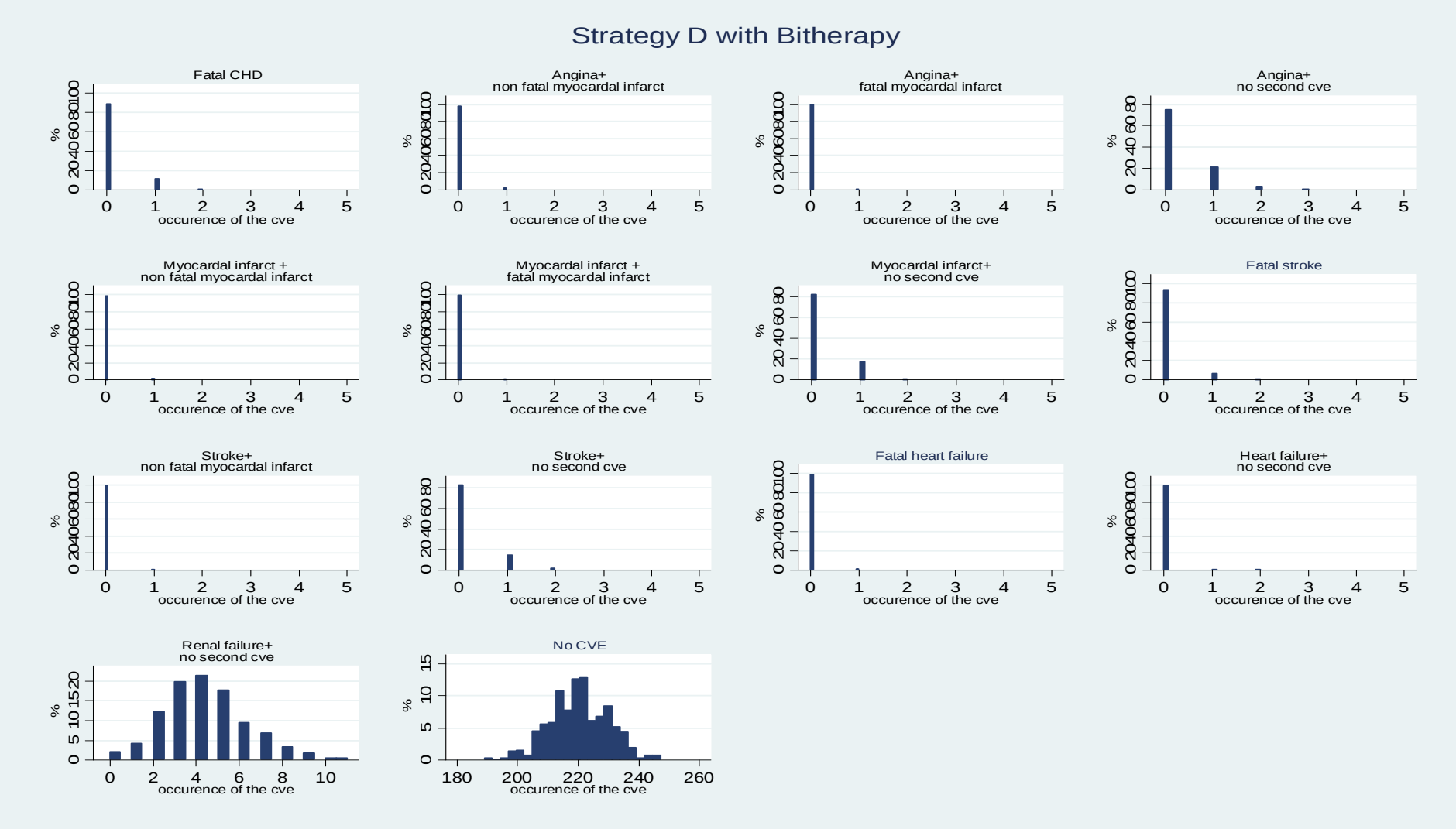


Table 22 : Number of events that occur with strategy D in the 3rd-line treatment (tritherapy) at least once during the 500 draws

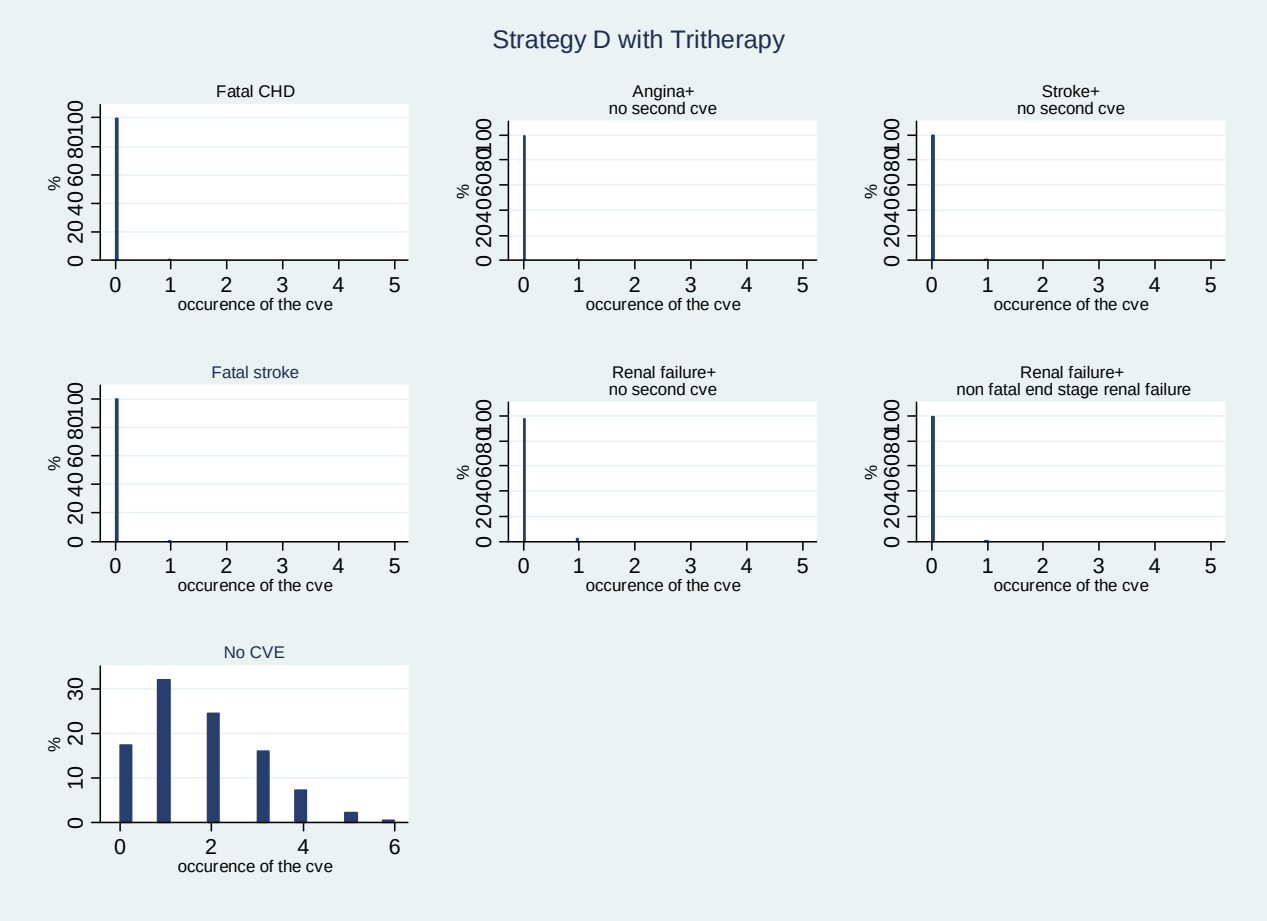


Table 23: Data source of cardiovascular events with strategies B, C and D

		Strategy B	Strategy C[105]	Strategy D
Coronary heart disease (CHD)	*Risk expectations*	Laurier and al., 1994* Occurrence of myocardial infarction for patients with history of CHD (PREVENIR, Bouhanick, 2006)*. *Nota bene:* CHD include myocardial infract and angina. For the distribution between the two events (Marques-Vidal, 2000, Ducimetière 2001; Cf. Table 24)	Law *et al.*, 2009	Law *et al.*, 2009
	Hospitalisation	100%	Idem strategy B	Idem strategy B
	Death	Marques-Vidal, 2000; Ducimetière, 2001 Cf. Table 24		
Stroke	*Risk expectations*	Wolf and al., 1991 (Framingham model)	Law *et al.*, 2009; Reboldi *et al.* 2008	Law *et al.*, 2009; Reboldi *et al.* 2008
	Hospitalisation	100%	Idem strategy B	
	Death	PREVENIR, 2006 The probability of dying from a stroke is 0,28.		
Heart failure	*Risk expectations*	Kannel and al., 1999*	Sciarretta *et al.* 2011	Sciarretta *et al.* 2011
	Hospitalisation	CHU Reseau , 2010 PMSI GHM data (HAS, 2009)	Idem strategy B	Idem strategy B
	Death	Ho and al. (1993)* Nota bene: This probabilities increase with time: cumulative risks (monthly and annual rate) have been computed with Miller risk equation depending on the gender and the age (30, 60, 70 or 80 years old).		
Renal failure	*Risk expectations*	UKPDS38 (1998)* Nota bene: Renal failure can only occur for patients with type 2 diabetes. Probabilities were estimated on a three year horizon time, the Miller formula was used to estimate the probabilities on 1 year horizon time.	Strippoli *et al.* 2005	Strippoli *et al.* 2005
End-stage-renal failure	*Risk expectations*	UKPDS38 (1998) Nota bene: End-stage-renal-failure can only occur for patients with renal failure. Probabilities were estimated on a three year horizon time, the Miller formula was used to estimate the probabilities on 1 year horizon time.	Strippoli *et al.* 2006	Strippoli *et al.* 2006
	Death	UKPDS 38, 1998. The probability of dying death from end-stage-renal-failure is about 0.13% for men and women.	Idem strategy B	Idem strategy B

Source: HAS, 2012

[105] The effectiveness of bitherapy and tritherapy was assessed in the HAS's model in multiply the relative risk associated with each class of drug in monotherapy. This methodological choice is justified with the hypotheses of a correlation between the decrease of arterial blood pressure and the decrease of cardiovascular morbid-mortality (Law *et al.*, 2009; Prospective Studies Collaboration, 2002). Effectiveness of tritherapy was computed for an average of every tritherapy available in the French market share accordingly to their distribution in the prescribing practices.

Nota bene: The symbol * show that risk expectations are estimated in the HAS's model on the base on risk equations, which means that probabilities are adjusted on individual characteristics. When risk equations were not available, the data are coming from literature and are not adjusted on individual's characteristics.

Table 24 Repartition of myocardial infarction, angor and fatal CHD (Marques-Vidal, 2000, Ducimetière 2001)

	Fatal coronary heart disease	**Myocardial infract**	**Angor**
Male	29%	24%	47%
Female	16%	37%	47%

Source: HAS, 2012

Table 25 Relative risk of cardiovascular event for each class of antihypertensive treatment versus placebo

Strategy	Treatment	Myocardial infarction	Stroke	Heart failure	Renal Failure	End-stage-renal-failure
		Law 2009	Law 2009	Sciarretta 2010	Strippoli 2005	Strippoli 2006
B	Placebo (ref)	1	1	1	1	1
C	ACE	0.83 [0.78;0.89]	0.78 [0.66;0.92]	0.71 [0.58;0.84]	0.52 [0.31;0.88]	0.6 [0.39;0.93]
	ACE_ DIU	0.71 [0.59;0.87]	0.48 [0.35;0.66]	0.42 [0.27;0.6]	0.52 [0.31;0.88]	0.6 [0.39;0.93]
D	IC	0.85 [0.78;0.92]	0.66 [0.58;0.75]	0.83 [0.67;0.99]	1 [1;1]	1 [1;1]
	IC_ACE	0.57 [0.37;0.88]	0.41 [0.29;0.56]	0.59 [0.39;0.83]	0.52 [0.31;0.88]	0.6 [0.39;0.93]
C and D, after a CHD or HF	BB_ACE	0.74 [0.61;0.91]	0.65 [0.46;0.91]	0.62 [0.37;0.94]	0.52 [0.31;0.88]	0.6 [0.39;0.93]
C and D	Tritherapy	0,62 [0,35-1,14]	0,34 [0,20-0,57]	0,39 [0,19-0,70]	0,56 [0,36-0,89]	0,74 [0,61-0,92]

Source: (HAS, 2013)

The table as to be read as following: Example for column « myocardial infarction » : Individuals who are controlled with ACE inhibitors (strategy C) are 17 % less likely than individuals who are controlled with the placebo to experience myocardial infarction.

Table 26 Probability of controlling high blood pressure: data

Initial arterial blood pressure (mmHg)	**Arterial blood pressure target**	**Proba N(moy,SD)**
Monotherapy :		
150	**140**	**0.4316**
160	140	0.0000
180	150	0.0000
Bitherapy :		
150	140	**0.9921**
160	140	0.6409
180	150	0.2209
Tritherapy (resistance)	-	Not available in the HAS model→ p=1 (hypothesis)

Source: HAS, 2012 (Law *et al.*, 2003 and Wald *et al.*, 2009)

Note: This table must be read as following. The probability of controlling high blood pressure for an initial arterial blood pressure of 150mmHg is 0.43 with a monotherapy. Individuals who cannot be controlled with this monotherapy try a bitherapy, and in that case, the probability of controlling high blood pressure is about 0.99. Those who cannot be controlled with biotherapy are then controlled by tritherapy and the probability to be controlled in that case is 1.

Table 27 Comparison of utility level associated with cardiovascular events in technology assessment reports about antihypertensive treatment and renal disease management produced by NICE

Cardiovascular events that could be prevented with HTA treatment		Utilities data used to compute QALY by NICE	Proxy to estimate WTP
Heart failure	not observed in the survey	0,71 (Harvard CE Registry, ref 264)	Angina
Unstable angina (first 6 mounth)	observed in the survey	0,77	Angina
Post unstable angina	-	0,80	Angina
Stroke	-	0,63 (Statins model)	Stroke
Myocardial infarction	-	0,76 (first 6 mounth) (Statins model]	Myocardial infarction
	-	0,88 (post) (Harvard C 6 mounth E Registry)	Myocardial infarction
Renal failure	not observed in the survey	0,734 (australian study, Cf. Mowatt 2003 ; Gonzalez-Perez 2005)	Angina
End-stage-renal-failure	-	0,603 (de Wit, Cf. Mowatt 2003 ; Gonzalez-Perez 2005]	Stroke

Source: NICE, 2011

Table 28 Distribution of antihypertensive treatment prescriptions in France

	Individuals with hypertension (prevalence)			Individuals with hypertension (incidence)		
	Number	**2010%**	**2009%**	**Number**	**2010%**	**2009%**
Total of prescriptions	**59 602 070**	**100,0%**		**933 117**	**100,0%**	
BB	11 066 953	18,6%	18,7%	146 434	15,7%	15,8%
CA	9 741 133	16,3%	16,7%	150 996	16,2%	16,0%
ARAII	8 146 557	13,7%	13,8%	224 156	24,0%	24,5%
DIU	7 472 633	12,5%	12,8%	86 332	9,3%	10,2%
ACE	5 480 876	9,2%	9,2%	130 125	13,9%	13,3%
Central	2 739 518	4,6%	4,9%	24 406	2,6%	2,9%
Aliskiren	384 795	0,6%	0,1%	15 966	1,7%	0,7%
Bitherapy with small dose	1 601 092	2,7%	3,0%	30 906	3,3%	
ARAII+DIU	7 719 564	13,0%	13,3%	66 488	7,1%	7,4%
ACE+DIU	2 265 989	3,8%	3,9%	18 019	1,9%	
BB+DIU	329 412	0,6%	0,3%	4 904	0,5%	
ARAII+CA	1 199 857	2,0%	1,4%	14 484	1,6%	1,3%
ACE+CA	862 565	1,4%	0,8%	14 769	1,6%	0,8%
BB+CA	468 945	0,8%	0,8%	3 649	0,4%	0,4%
Alisken+DIU	116 009	0,2%	-	1 426	0,2%	-

Source: HAS, 2012 (data are coming from Thalès database)

Table 29 Assessment of daily costs of antihypertensive treatment[106]

Class of drugs	Daily cost (EUR)	Minimum cost	Maximum costs
Monotherapy			
DIU	0.26	0.07	0.50
BB	0.32	0.12	0.64
CA	0.35	0.14	0.86
ACE	0.30	0.11	0.67
ARAII	0.50	0.27	1.36
IR	0.82	0.82	0.82
Bithérapie			
BB+DIU	0.31	0.19	0.57
ACE+DIU	0.33	0.17	1.36
ARAII+DIU	0.48	0.28	0.75
IR+DIU	0.82	0.82	0.82
CA+DIU	0.62	0.21	1.36
BB+ARAII	0.82	0.39	2.00
BB+CA	0.74	0.12	1.08
BB+ACE	0.63	0.23	1.31
CA+ACE	0.60	0.37	0.75
CA+ARAII	0.53	0.40	0.56
Trithérapies			
ARA II+ CA+DIU	1.62	1.61	1.62
ARAII_DIU+BB	0.80	0.40	1.40
ARAII_CA+BB	0.85	0.52	1.21
DIU_CA+BB	0.94	0.34	2.00
DIU_ACE+BB	0.65	0.29	2.00
DIU_IR+BB	1.15	0.95	1.46
CA_ACE+BB	0.93	0.49	1.39
BB_CA+IR	1.56	0.94	1.90
DIU_ACE_CA	0.68	0.31	2.23
DIU_CA_ACE	0.92	0.32	2.03
DIU_IR_CA	1.18	0.96	1.69
DIU_CA_IR	1.44	1.04	2.18

Source: HAS, 2012

[106] The daily costs of each class of drugs were assessed according to prices of each pharmaceutical products in 2010 (http://www.codage.ext.cnamts.fr/codif/bdm_it/index.php?p_site=AMELI) adjusted on the distribution of each pharmaceutical product in the market share in France (bases LMPSO, IMS Health) and adjusted to the defined daily dose (http://www.whocc.no/atc_ddd_index/).

Table 30 Costs of medical care induced by cardiovascular events: hospitalizations and follow up[107]

Events	**Cost for the national health insurance in €**	**Out-of-pocket in €**
Hospitalization cost for non fatal Stroke	5366,6	-
Hospitalization cost for fatal Stroke	6253,6	-
Hospitalization cost for non fatal Heart Failure	4607,3	-
Hospitalization cost for fatal Heart Failure	5446,9	-
Hospitalization cost for non fatal MI	5114,7	-
Hospitalization cost for non fatal Angor	3640,5	-
Hospitalization cost for fatal CHD	6241,7	-
Hospitalization cost for recurrent MI (non fatal)	5256,0	-
Hospitalization cost for recurrent MI (fatal)	5901,4	-
Follow-up cost per cycle (excl drugs), no history patients	112,4	121,4
Follow-up cost per cycle (excl drugs), diabetes patients	591,4	121,4
Follow-up cost per cycle (excl drugs), renal disease patients	591,4	121,4
Follow-up cost per cycle (excl drugs), ESRF patients	61266,0	-
Follow-up cost per cycle (excl drugs), post-HF	4406,0	-
Follow-up cost per cycle (excl drugs), post-stroke	6506,1	-
Follow-up cost per cycle, post-CHD	3893,9	-
Follow-up cost per cycle, post-CVD	6506,1	-

Source: HAS, 2012

Note: There is no out of pocket expenses except for three kinds of patients (no history patients, diabetes patients, renal disease patients - and among them, only for those who have no complementary insurance that covers out of pocket expenses). Indeed, the other patients belong, after their cardio vascular event, to the "long time disease" category (ALD) and all their expenses are covered by the national health insurance.

Table 31 Participation to health insurances expenditures in France depending on individual's income index quintile

	Levies for the funding of the national health insurance		CSG (taxes)		**Compulsory impositions paid by household**		Premiums paid to private insurance		Total household contributions	
	Raw data	Adjusted by structure of age	Raw data	Adjusted by structure of age	Raw data	**Adjusted by structure of age**	Raw data	Adjusted by structure of age	Raw data	Adjusted by structure of age
Total	12	12	59	59	72	**72**	28	28	100	100
Income quintile before taxes										
1	4	4	14	14	18	**18**	19	19	37	38
2	5	6	30	32	36	**38**	26	26	62	64
3	7	8	51	51	58	**59**	29	29	87	88
4	10	9	69	68	79	**77**	32	32	111	109
5	35	34	132	130	167	**164**	36	35	203	199

Source: DREES, 2005

Note: We use the column "compulsory impositions paid by household" and data "adjusted by the structure of age". The participation of individuals to health expenditures is estimated as follows: the index associated to all impositions is 356 (18+38+59+77+164). For individuals who belong to the first quintile of the distribution of income (before taxes), their contribution is then measured by (18*100)/356=5.05%.

[107] Costs of hopsitalization related to cardiovascular events were assessed with French data of PMSI (2009) adjusted on the number of hospitalizations visits (ENCC 2008). Costs of follow up related to cardiovascular events were assessed on the ground of data provided by the National Health Insurance computed by Vallier (2006).

Table 32 : Cost and effectiveness of each strategy for a 65 years old man with 150 mmHg arterial blood pressure

Strategy	Mean total costs [95% CI]	Mean life years gained [95% CI]
IEC-IEC+DIU-tri	9005 [8990 ; 9021]	12,338 [12,327 ; 12,349]
DIU-DIU+IEC-tri	9041 [9026 ; 9057]	12,341 [12,330 ; 12,353]
IEC-IEC+ICA-tri	9178 [9164 ; 9191]	12,380 [12,369 ; 12,392]
ICA-ICA+IEC-tri	9242 [9228 ; 9257]	12,384 [12,372 ; 12,395]
DIU-DIU+ARAII-tri	9252 [9227 ; 9276]	12,344 [12,332 ; 12,356]
ARAII-ARAII+DIU-tri	9321 [9296 ; 9345]	12,344 [12,332 ; 12,355]
ICA-ICA+ARAII-tri	9369 [9346 ; 9393]	12,314 [11,98 ; 12,683]
DIU-DIU+BB-tri	9384 [9366 ; 9402]	12,308 [12,297 ; 12,319]
ARAII-ARAII+ICA-tri	9388 [9365 ; 9412]	12,331 [12,319 ; 12,343]
BB-BB+DIU-tri	9521 [9503 ; 9540]	12,284 [12,273 ; 12,295]
DIU-DIU+ICA-tri	9634 [9619 ; 9650]	12,349 [12,337 ; 12,360]
ICA-ICA+DIU-tri	9675 [9659 ; 9690]	12,345 [12,338 ; 12,356]
IEC-IEC+BB-tri	9811 [9794 ; 9828]	12,272 [12,261 ; 12,283]
Placebo	9923 [9906 ; 9 940]	12,02 [12,01 ; 12,03]
BB-BB+IEC-tri	10 003 [9 985 ; 10 020]	12,250 [12,239 ;12,261]
ICA-ICA+BB-tri	10 175 [10158 ; 10192]	12,281 [12,270 ; 12,292]
ARAII-ARAII+BB-tri	10 180 [10154 ; 10206]	12,283 [12,272 ; 12,295]
BB-BB+ICA-tri	10 270 [10 252 ; 10 287]	12,262 [12,251 ; 12,273]
BB-BB+ARAII-tri	10 298 [10 272 ; 10 325]	12,254 [12,242 ; 12,265]

Source: HAS, 2012

Bibliographie

Audard C. Qu’est-ce que le libéralisme ?, Paris, Gallimard, 2009.

Aristote, Tricot J. Ethique à Nicomaque. Paris: Vrin; 2007.

Atkinson A. On the measurement of inequality. Journal of Economic Theory 1970;2(3):244-63.

Benamouzig B. La santé au miroir de l'économie. Paris: PUF; 2005.

Benamouzig, D. Besançon, J. Administrer un monde incertain : les nouvelles bureaucraties techniques, le cas des agences sanitaires en France, Sociologie du Travail, 2005, vol. 47, n°3, pp. 301-322

Benamouzig D. L'institutionnalisation de l'économie de la santé en France, 1950-1990. J écon méd 2006;24(1):55-63.

Berheim D, Rangel A. Behavioral public economics: welfare and policy analysis with non-standard decision-makers. In: Behavioral economics and its applications. Princeton: Princeton University Press; 2007.

Birch S, Gafni A. Cost effectiveness/utility analyses. Do current decision rules lead us to where we want to be ? J Health Econ 1992;11(3):279-96.

Birch S, Donaldson C. Valuing the benefits and costs of health care programmes: where's the 'extra' in extra-welfarism? Soc Sci Med 2003;56(5):1121-33.

Birch S, Gafni A. The biggest bang for the buck or bigger bucks for the bang: the fallacy of the cost-effectiveness threshold. J Health Serv Res Policy 2006;11(1):46-51.

Birch S, Gafni A. Economists' dream or nightmare? Maximizing health gains from available resources using the NICE guidelines. Health Econ Policy Law 2007;2(Pt 2):193-202.

Boorse C. Health as a theoretical concept. Philosophy of Science 1977;44(4):542-73.

Bouhanick B, Cambou JP, Ferrieres J, Amelineau E, Guize L. Characteristics and six-month outcomes in a cohort of 8288 diabetic and non-diabetic patients with previous history of acute coronary syndrome or stroke: the French PREVENIR 3 survey. Diabetes Metab 2006;32(5 Pt 1):460-6.

Brazier J, Ratcliffe J, Salomon JA, Tsuchiya A. Measuring and valuing health benefits for economic evaluation. Oxford: Oxford University Press; 2007.

Brickman P, Coates D, Janoff-Bulman R. Lottery winners and accident victims: is happiness relative ? J Pers Soc Psychol 1978;36(8):917-27.

Burdeau G., Le Libéralisme, Edition du Seuil, Points. Inédit Politique. 1979

Canguilhem G. Le normal et le pathologique. Paris: PUF; 1966.

Canto-Sperber. M. Dictionnaire d'éthique et de philosophie morale. Paris: PUF; 2004.

Caussat L, Le Minez S, Raynaud D. L'assurance maladie contribue-t-elle à redistribuer les revenus ? Paris: DREES; 2005.

Cleemput I, Crott R, Vrijens F, Huybrechts M, van Wilder P, Ramaekers D. Recommandations provisoires pour les évaluations pharmacoéconomiques en Belgique. KCE reports 28B. Bruxelles: Centre fédéral d'expertise des soins de santé; 2006. https://kce.fgov.be/sites/default/files/page_documents/d20061027311.pdf

Cleemput I, van Wilder P, Vrijens F, Huybrechts M, Ramaekers D. Recommandations pour les évaluations pharmacoéconomiques en Belgique. KCE reports 78B. Bruxelles: Centre fédéral d'expertise des soins de santé; 2008. https://kce.fgov.be/sites/default/files/page_documents/d20081027324.pdf

Cleemput I, Neyt N, de Laet C, Leys M. Valeurs seuils pour le rapport coût-efficacité en soins de santé. KCE reports 100B. Bruxelles: Centre fédéral d'expertise des soins de santé; 2008. https://kce.fgov.be/sites/default/files/page_documents/d20081027395.pdf

Clément V., Le Clainche C. et Serra D., Économie de la justice et de l'équité, Paris, Economica, 2008

Cohen GA. On the currency of egalitarian justice. Ethics 1989;99(4):906-44.

Collège des économistes de la santé, Synthèse du congrès de l'ISPOR, dans « Lettre du Collège des économistes de la santé », Décembre 2008 http://www.ces-asso.org/docs/Lettre_ISPOR_2008.pdf

Com-Ruelle L., Dourgnon P., Jusot F. Lengagne F. Prévalence et facteurs socio-économiques associés aux problèmes d'alcool en population générale en France. *Document de travail de l'IRDES* 2008 ; 1703

Contandriopoulos AP, Champagne F, Denis JL, Avargues MC. L'évaluation dans le domaine de la santé: concepts et méthodes. Rev Epidemiol Sante Publique 2000;48(6):517-39.

Cookson R, Dolan P. Principles of justice in health care rationing. J Med Ethics 2000;26(5):323-9.

Cookson R, McCabe C, Tsuchiya A. Public healthcare resource allocation and the Rule of Rescue. J Med Ethics 2008;34(7):540-4.

Cookson R, Drummond M, Weatherly H. Explicit incorporation of equity considerations into economic evaluation of public health interventions. Health Econ Policy Law 2009;4(Pt

2):231-45.

Cornilleau G, Debrand T. Crise et déficit de l'assurance maladie. Faut-il changer le paradigme ? Observations et disgnostics économiques : Revue de l'OFCE 2011;(116):315-32.

Culyer AJ. The normative economics of health car finance and provision. Oxf Rev Econ Policy 1989;5(1):34-58.

Culyer AJ. NICE's use of cost effectiveness as an exemplar of a deliberative process. Health Econ Policy Law 2006;1(Pt 3):299-318.

Daniels N. Just health care. Cambridge: Cambridge University Press; 1985.

Daniels N, Sabin JE. Last chance therapies and managed care. Pluralism, fair procedures, and legitimacy. Hastings Cent Rep 1998;28(2):27-41.

Daniels N. Just health : meeting health needs fairly. Cambridge: Cambridge University Press; 2008.

Daniels N. L'extension de la justice comme équité à la santé et aux soins de santé. Raisons Politiques 2009;34:9-29.

Dauphinot V., Naudin F., Guéguen R., Perronnin M., Sermet C. Ecarts entre morbidité déclarée et morbidité diagnostiquée. L'exemple de l'obésité, de l'hypertension artérielle et de l'hypercholesterolémie. *Questions d'Economie de la santé* 2006 ; 114 :1-8

Davidson R., Duclos JY. Statistical Inference for Stochastic Dominance and for the Measurement of Poverty and Inequality. *Econometrica* 2000; 68:1435-1464

Davey-Smith G., J. Schipley M., Rose G. Magnitude and causes of Socioéconomic Differentials in Morality: Further Evidence from Whitehall Study, Journal of Epidemiology an Community Helath, vol.44, 1990

De Boyer J. La pensée monétaire. Histoire et analyse. Paris: Editions Les Solos; 2003.

De Wit GA, Busschbach JJ, De Charro FT. Sensitivity and perspective in the valuation of health status: whose values count ? Health Econ 2000;9(2):109-26.

De Wit GA, Merkus MP, Krediet RT, De Charro FT. Health profiles and health preferences of dialysis patients. Nephrol Dial Transplant 2002;17(1):86-92.

Demeulenaere P. La complexité de la notion d'utilitarisme dans les sciences sociales. Cités 2002;2(10):37-48.

Espin J, Rovira J. Analysis of differences and commonalities in pricing and reimbursement systems in Europe. DG Enterprise and Industry of the European Commission, Escuela Andaluza de Salud Pública. Brussels: European Commission; 2007. http://ec.europa.eu/enterprise/sectors/healthcare/files/docs/study_pricing_2007/andalusian_school_public_health_report_pricing_2007_en.pdf

Dickson M, Hurst J, Jacobzone S. Survey of pharmacoeconomic assessment activity in eleven countries. Paris: OECD Health Working Papers; 2003.
http://www.oecd.org/els/healthpoliciesanddata/2955828.pdf

Diekema DS. Is taller really better ? Growth hormone therapy in short children. Perspect Biol Med 1990;34(1):109-23.

Dolan P, Edlin R, Tsuchiya A. The relative societal value of health gains to different beneficiaries: a summary. Discussion paper.

Dolan P, Gudex C, Kind P, Williams A. The time trade-off method: results from a general population study. Health Econ 1996;5(2):141-54.

Dolan P. Whose preferences count? Med Decis Making 1999;19(4):482-6.

Dolan P. The measurement of health-related quality of life. In: Culyer A, Newhouse JP, ed. Handbook of health economics. Amsterdam: Elsevier; 2000.

Dolan P, Shaw R, Tsuchiya A, Williams A. QALY maximisation and people's preferences: a methodological review of the literature. Health Econ 2005;14(2):197-208.

Dolan P, White M. Dynamic well-being : Connecting indicators of what people anticipate with indicators of what they experience. Social Indicators Research 2006;75(2):303-33.

Dolan P, Kahneman D. Interpretations of utility and their implications for the valuation of health. The Economic Journal 2008;525:215-34.

Dolan P, Peasgood TWM. Do we really know what makes us happy A review of the economic literature on the factors associated with subjective well-being. Journal of Economic Psychology 2008;29(1):94-122.

Dolan P, Lee H, King D, Metcalfe R. Valuing health directly. BMJ 2009;339:b2577.

Dolan P. Thinking about it: thoughts about health and valuing QALYs. Health Econ 2011;20(12):1407-16.

Dormont B, Huber H. Causes of health expenditures growth : the predominance of changes in medical practices over population ageing. Annales d'Economie et de Statistique 2006;(83-84):188-217.

Dormont B. Les dépenses de santé, une augmentation salutaire. Rue d'Ulm / Presse de l'Ecole normale supérieure ed. Paris: 2009.
http://www.brigitte-dormont.fr/pdf/OPUS15.pdf

Drummond M. NICE: a nightmare worth having ? Health Econ Policy Law 2007;2(Pt 2):203-8.

Drummond MF, Sculpher M, Stoddart GL, Torrance G, O'Brien B. Methods for the economic evaluation of health care. Third edition. London: Oxford University press; 2005.

Ducimetiere P, Ruidavets JB, Montaye M, Haas B, Yarnell J, PRIME Study Group. Five-year incidence of angina pectoris and other forms of coronary heart disease in healthy men aged 50-59 in France and Northern Ireland: the Prospective Epidemiological Study of Myocardial Infarction (PRIME) Study. Int J Epidemiol 2001;30(5):1057-62.

Dworkin R. "What is equality ? Part 2: equality of resources". Philosophy and public affairs 1981;10(4):283-345.

Dworkin R. Sovereign virtue : the theory and practice of equality. Cambridge (MA): Harvard University Press; 2000.

Elster J. Sour grapes – utilitarianism and the genesis of wants. In: Sen A, Williams B, ed. Utilitarianism and beyond. Cambridge: Cambridge University Press; 1982.

Fassin D, Bourdelais P. Les constructions de l'intolérable. Études d'anthropologie et d'histoire sur les frontières de l'espace moral. Paris: La Découverte; 2005.

Feeny D, Furlong W, Torrance GW, Goldsmith CH, Zhu Z, DePauw S, *et al.* Multiattribute and single-attribute utility functions for the health utilities index mark 3 system. Med Care 2002;40(2):113-28.

Fleurbaey M. Equal opportunity or equal social outcome ? Economics and Philosophy 1995;(11):25-55.

Fleurbaey M. Théories économiques de la justice. Paris: Economica; 1996.

Fleurbaey M. Choix social : une difficulté et de multiples possibilités. Rev écon 2000;51(5):1215-32.

Fleurbaey M. Equality of resources revisited. Ethics 2005;113:82-105.

Fleurbaey M. Health, wealth and fairness. Journal of Public Economic Theory 2005;7(2):253-84.

Fleurbaey M. Health, equity and social welfare. Ann Econ Stat 2007;(82):24-62.

Fleurbaey M. Le revenu équivalent-santé, un outil pour l'analyse des inégalités sociales de santé. Rev Epidemiol Sante Publique 2007;55(1):39-46.

Fleurbaey M, Luchini S, Muller C, Schokkaert E. Economic evaluation of health care: distribution matters, and what about preferences ? [working paper]. 2007.

Fleurbaey M, Luchini S, Schokkaert E. Evaluation économique en santé: qui a peur de l'étalon monétaire ? Revue de philosophie économique 2009;10(1).

Fleurbaey M. Assessing risky social situations. Journal of Political Economy 2010;118(4):649-80.

Fleurbaey M, Luchini S, Muller C, Schokkaert E. Equivalent income and fair evaluation of health care. Health Econ 2012.

Frederick S, Loewenstein G. Hedonic adaptation. In: Kahneman D, Diener E, Schwartz N, ed. Scientific perspectives on enjoyment, suffering, and well-being. New York: Russell Sage Foundation; 1999.

Gamel C. Leçons de philosophie économique, vol. 2 Economie normative et philosophie morale, Leroux A. et Livet P. (eds), Paris, Editions Economica, 2005.

Gafni A, Birch S. Incremental cost-effectiveness ratios (ICERs): the silence of the lambda. Soc Sci Med 2006;62(9):2091-100.

Gonzalez-Perez JG, Vale L, Stearns SC, Wordsworth S. Hemodialysis for end-stage renal disease: a cost-effectiveness analysis of treatment-options. Int J Technol Assess Health Care 2005;21(1):32-9.

Goodin RE. Laundering preferences. In: Elster J, Hylland A, ed. Foundations of social choice theory. Cambridge: Cambridge University Press; 1986.

Goold SD. Allocating health care: cost-utility analysis, informed democratic decision making, or the veil of ignorance ? J Health Polit Policy Law 1996;21(1):69-98.

Gray AM, Clarke PM, Wolstenholme JL, Wordsworth S. Applied methods of cost-effectivness analysis in health care. Oxford: Oxford University Press; 2011.

Gress TW, Nieto FJ, Shahar E, Wofford MR, Brancati FL. Hypertension and antihypertensive therapy as risk factors for type 2 diabetes mellitus. Atherosclerosis Risk in Communities Study. N Engl J Med 2000;342(13):905-12.

Habermas J. Morale et communication, trad. C. Bouchindhomme, Les Editions du Cerf. 1986.

Halévy E. La Formation du radicalisme philosophique. Tome I, II et III. Première édition 1901. Paris: Les Presses universitaires de France, 1995.

Hausman DM. Valuing health: a new proposal. Health Econ 2010;19(3):280-96.

Haut comité de santé publique. Le panier de biens et services de santé. Première approche. Rapport adressé à la Conférence nationale de santé et au Parlement. Paris: HCSP; 2000.

Haut comité de santé publique. Le panier de biens et services de santé : du concept aux modalités de gestion. Paris: HSCP; 2001.

Haut conseil pour l'avenir de l'assurance maladie. L'assurance maladie face à la crise : éléments d'analyse. Rapport annuel 2010. Paris: HCCAM; 2010.

Haute Autorité de Santé. Prise en charge des patients adultes atteints d'hypertension artérielle essentielle - Actualisation 2005. Recommandations pour la pratique clinique. Saint-Denis La Plaine: HAS; 2005.

Haute Autorité de Santé. Evaluation du dépistage néonatal systématique de la surdité permanente bilatérale. Evaluation médico-économique et santé publique. Saint-Denis La Plaine: HAS; 2007.

Haute Autorité de Santé. Traitement de la surdité par pose d'implants cochléaires ou d'implants du tronc cérébral. Saint-Denis la plaine: HAS; 2007.

Haute Autorité de Santé. Définir, ensemble, les nouveaux horizons de la qualité en santé. HAS ; 2007

Haute Autorité de Santé. Surdité de l'enfant : accompagnement des familles et suivi de l'enfant de 0 à 6 ans, hors accompagnement scolaire. Saint-Denis La Plaine: HAS; 2009.

Haute Autorité de Santé. Evaluation de la stimulation phrénique implantée. Evaluation des dispositifs médicaux et des actes. Saint-Denis La Plaine: HAS; 2009.

Haute Autorité de Santé. Situation actuelle et perspectives d'évolution de la prise en charge médicale du transsexualisme en France. Saint-Denis La Plaine: HAS; 2009.

Haute Autorité de Santé. L'évaluation économique à la HAS : principes et méthodes, Saint-Denis La Plaine: HAS; 2011

Haute Autorité de Santé. Évaluation des médicaments antihypertenseurs et place dans la stratégie thérapeutique [à paraître]. Saint-Denis La Plaine: HAS; 2012.

Haute Autorité de Santé. L'hormone de croissance chez l'enfant non déficitaire, évaluation du service rendu à la collectivité. Saint-Denis La Plaine: HAS; 2012.

Herpin N. La taille des hommes : son incidence sur la vie en couple et la carrière professionnelle. Econ Stat 2003;361(1):71-90.

Ho KK, Anderson KM, Kannel WB, Grossman W, Levy D. Survival after the onset of congestive heart failure in Framingham Heart Study subjects. Circulation 1993;88(1):107-15.

Hume D. Traité de la nature humaine, Edition Garnier Flammarion, traduction de P. Saltel. 1993

Institute for Quality and Efficiency in Health Care. Development of a prognosis model to identify effects of threshold values on health care. Cologne: IQWiG; 2006. https://www.iqwig.de/download/G05-01A_Executive_summary_Development_of_a_prognosis_model_to_identify_effects_of_threshold_values_on_health_care.pdf

Institute for Quality and Efficiency in Health Care. Methods for assessment of the relation of benefits to costs in the german statutory health care system. Cologne: IQWiG; 2008. https://www.iqwig.de/download/08-10-14_Draft_Methods_of_the_Relation_of_Benefits_to_Costs_v_1_1.pdf

Jaume L. L'individu effacé ou le paradoxe du libéralisme français. Paris: Fayard; 1997.

Jaume L. Les origines philosophiques du libéralisme, Champs essais, Editions Flammarion, Paris, 2010

Johannesson M, Meltzer D. Some reflections on cost-effectiveness analysis. Health Econ 1998;7(1):1-7.

Kahneman D, Wakker P, Sarin R. Back to Bentham ? Explorations of experienced utility. The Quarterly Journal of Economics 1997;11(2):375-405.

Kahneman D. Evaluation by moments: Past and future. In: Kahneman D, Tversky A, ed. Choices, values and frames. New York: Cambridge University Press; 2000.

Kannel WB, d'Agostino RB, Silbershatz H, Belanger AJ, Wilson PW, Levy D. Profile for estimating risk of heart failure. Arch Intern Med 1999;159(11):1197-204.

Keynes JN. The scope and method of political economy. London: Mc Millan and Co; 1897.

Laurier D, Nguyen PC, Cazelles B, Segond P. Estimation of CHD risk in a French working population using a modified Framingham model. The PCV-METRA Group. J Clin Epidemiol 1994;47(12):1353-64.

Law MR, Morris JK, Wald NJ. Use of blood pressure lowering drugs in the prevention of cardiovascular disease: meta-analysis of 147 randomised trials in the context of expectations from prospective epidemiological studies. BMJ 2009;338:b1665.

Lalande A. Vocabulaire technique et critique de la Philosophie. viii-1005 pp., Presse Universitaires de France, 1926

Layard R. Happiness: Lessons from a new science. New York: The Penguin Press; 2012.

Lefranc A, Pistolesi N., Trannoy A. Le revenu selon l'origine sociale. *Economie et Statistiques* 2004 ; 371 :49-82.

Le Pen C. Théorie de l'utilité et mesure des états de santé. Le débat QALYs - HYEs. Cahiers du CERESA 1996;(1).

Leroux A, Marciano A. La philosophie économique. Paris: PUF; 1998.

Lewington S, Clarke R, Qizilbash N, Peto R, Collins R, Prospective SC. Age-specific relevance of usual blood pressure to vascular mortality: a meta-analysis of individual data for one million adults in 61 prospective studies. Lancet 2002;360(9349):1903-13.

Loewenstein G, Frederick S. Predicting reactions to environmental change. In: Bazerman M, Messick D, Tenbrunsel A, Wade-Benzoni K, ed. Psychological perspectives on ethics and the environment. San Francisco: New Lexington Press; 1997.

Frederick, S., and Loewenstein, G. Hedonic adaptation. in D. Kahneman, E. Diener, and N. Schwartz [eds]. Scientific Perspectives on Enjoyment, Suffering, and Well-Being. New York. Russell Sage Foundation. 1999

Loewenstein G, Ubel PA. Hedonic adaptation and the role of decision and experience utility in public policy. J Public Econ 2008;92(8):1195-205.

Marques-Vidal P, Ruidavets JB, Cambou JP, Ferrieres J. Incidence, recurrence, and case fatality rates for myocardial infarction in southwestern France, 1985 to 1993. Heart 2000;84(2):171-5.

Marmot M. The status Syndrome: How Social Standing Affects Our Health Longevity, New York, Time Books, 2004

Matillon Y, Durieux P. L'évaluation médicale. Du concept à la pratique. Paris: Flammarion Médecine Sciences; 1994.

Mehrez A, Gafni A. Evaluating health related quality of life: an indifference curve interpretation for the time trade-off technique. Soc Sci Med 1990;31(11):1281-3.

Menzel P, Dolan P, Richardson J, Olsen JA. The role of adaptation to disability and disease in health state valuation: a preliminary normative analysis. Soc Sci Med 2002;55(12):2149-58.

Moatti JP, Auquier P, Le Coroller AG, Macquart-Moulin G. QALYS or not QALYS: that is the question? Rev Epidemiol Sante Publique 1995;43(6):573-83.

Moatti JP. Ethical issues in the economic assessment of health care technologies. Health Care Anal 1999;7(2):153-65.

Mongin P. Positif ou normatif. In: Jessua C, Labrousse C, Vitry D, ed. Dictionnaire des sciences économiques. Presses Universitaires de France; 2001. p. 701-708.

Mooney G. Economics, communitarianism, and health care. In: Barer ML, Getzen TE, Stoddart GL, ed. Health, health care and health economics: perspectives on distribution. Chichester: Wiley; 1998. p. 397-413.

Mowatt G, Vale L, Perez J, Wyness L, Fraser C, MacLeod A, *et al.* Systematic review of the effectiveness and cost-effectiveness, and economic evaluation, of home versus hospital or satellite unit haemodialysis for people with end-stage renal failure. Health Technol Assess 2003;7(2):1-174.

National Institute for Health and Clinical Excellence. Quality Adjusted Life Years (QALYs) and the severity of illness. Report on NICE Citizens Council meeting. 31 January – 2 February 2008.

National Institute for Health and Clinical Excellence. Rule of rescue. NICE citizens council report 2006.

National Institute for Health and Clinical Excellence. Briefing paper for the methods working party on the cost effectiveness threshold. London: NICE; 2007.

National Institute for Health and Clinical Excellence. Guide to the methods of technology appraisal. London: NICE; 2008.

National Institute for Health and Clinical Excellence. Social value judgements. Principles for the development of NICE guidance. 2nd edition . London: NICE; 2008.

National Institute for Health and Clinical Excellence. Appraising life-extending, end of life treatments 2009.

National Institute for Health and Clinical Excellence. Appendix I. Cost-effectiveness analysis - pharmacological treatment [updated 2011]. In: Hypertension: The Clinical Management of Primary Hypertension in Adults: Update of Clinical Guidelines 18 and 34. London: The Royal College of Physicians; 2011.

Nord E, Richardson J, Street A, Kuhse H, Singer P. Maximizing health benefits vs egalitarianism: an Australian survey of health issues. Soc Sci Med 1995;41(10):1429-37.

Nord E, Pinto JL, Richardson J, Menzel P, Ubel P. Incorporating societal concerns for fairness in numerical valuations of health programmes. Health Econ 1999;8(1):25-39.

Nussbaum M. Aristotelian social democracy. In: Douglass RB, Mara GM, Richardson HS, ed. Liberalism and the good. London: Routledge;Chapman and Hall; 1990. p. 203-252.

Nussbaum M. Femmes et développement humain, trad. C. Chaplain, Paris, Editions Des femmes, 2008

Oliver A, Healey A, Donaldson C. Choosing the method to match the perspective: economic assessment and its implications for health-services efficiency. Lancet 2002;359(9319):1771-4.

Organisation for Economic Co-operation and Development. Pharmaceutical pricing policies in a global market. OECD health policy studies. Paris: OECD Publishing; 2008.

Organisation mondiale de la santé, Action on the social determinants of health: Learning from previous experiences », dans *Report of Commission on Social Determinants of Health*. 2005

Organisation mondiale de la santé, Bureau régional de l'Europe. La santé en période de crise économique mondiale : incidences pour la Région européenne de l'OMS. Copenhague: OMS; 2009.
http://www.euro.who.int/__data/assets/pdf_file/0007/66958/RC59_fdoc07.pdf

Poincarré H. Dernières pensées (1913). Edition Garnier Flammarion, 1994

Phelps CE, Mushlin AI. On the (near) equivalence of cost-effectiveness and cost-benefit analyses. Int J Technol Assess Health Care 1991;7(1):12-21.

Platon. Apologie de Socrate, Criton, Phédon, trad. fr. E. Chambry, Paris, Garnier-Flammarion, 1965

Platon. Gorgias, trad. Canto, Garnier-Flammarion, 1987

Pliskin J, Shepard D, Weinstein MC. Utility functions for life years and health status. Operations Research 1980;28(1):206-24.

Powers M, Faden R. Inequalities in health, inequalities in health care: four generations of discussion about justice and cost-effectiveness analysis. Kennedy Inst Ethics J 2000;10(2):109-27.

Qizilbash M. Capability, happiness and adaptation in Sen and J S Mill. Utilitas 2006;18(1):20-32.

Ravoux V. Le panier de biens et de services de santé. Paris: Editions de santé; 2003.

Rawls J. A theory of justice. Cambridge: Harvard University Press; 1971.

Rawls R. Leçons sur l'histoire de la philosophie morale. Edition La découverte / Poche, Paris. 2002

Rawls J. La justice comme équité. Une reformulation de "théorie de la justice". Paris: La Découverte; 2003.

Rochaix L, Tubeuf S. Equité en santé : apports de l'analyse économique et perspectives de recherche. Communication présentée lors des 29ème JESF du 6 et 7 décembre 2007 2007.

Roemer JE. Equality of talent. Economics and Philosophy 1985;1:151-87.

Sackett DL, Torrance GW. The utility of different health states as perceived by the general public. J Chronic Dis 1978;31(11):697-704.

Sandberg DE, Voss LD. The psychosocial consequences of short stature: a review of the evidence. Best Pract Res Clin Endocrinol Metab 2002;16(3):449-63.

Scanlon TM. Preference and urgency. J Philos 1975;72(19):655-69.

Scanlon T.M., Contractualism and utilitarianism. In: Sen A, Williams B, ed. Utilitarianism and beyond. Cambridge: Cambridge University Press; 1982

Schlander M. The use of cost-effectiveness by the National Institute for Health and Clinical Excellence (NICE): no(t yet an) exemplar of a deliberative process. J Med Ethics 2008;34(7):534-9.

Schneider-Bunner C. Equity in managed competition. In: Barer ML, Getzen TE, Stoddart GL, ed. Health, health care and health economics: perspectives on distribution. Chichester: Wiley; 1998. p. 295-318.

Schramme T. The significance of the concept of disease for justice in health care. Theor Med Bioeth 2007;28(2):121-35.

Sen A. On ethics and economics. Oxford: Basil Blackwell; 1987.

Sen A. Ethique et économie. 4ème éd. Paris: PUF; 1993.

Sen A. Development as freedom. New York: Alfred A. Knopf; 1999.

Sen A. Commodities and capabilities. Oxford: Oxford India Paperbacks; 1999.

Sen A. Why health equity ? Health Econ 2002;11(8):659-66.

Sen A. Un nouveau modèle économique. Développement, justice, liberté. Paris: Odile Jacob; 2003.

Singh J, Cuttler L, Shin M, Silvers JB, Neuhauser D. Medical decision-making and the patient: understanding preference patterns for growth hormone therapy using conjoint analysis. Med Care 1998;36(8 Suppl):AS31-AS45.

Slovic P. The construction of preference. American Psychologist 1995;50(5):364-71.

Stabler B, Clopper RR, Siegel PT, Stoppani C, Compton PG, Underwood LE. Academic achievement and psychological adjustment in short children. The national cooperative growth study. J Dev Behav Pediatr 1994;15(1):1-6.

Tsuchiya A, Dolan P. The QALY model and individual preferences for health states and health profiles over time: a systematic review of the literature. Med Decis Making 2005;25(4):460-7.

Tversky A, Kahneman D. Advances in prospect theory: cumulative representation of uncertainty. Journal of Risk and Uncertainty 1992;(5):297-323.

Tversky A, Kahneman D. Loss aversion in riskless choice: a referent dependant model. The Quarterly Journal of Economics 1997;106(4):1039-61.

Valmadrid CT, Klein R, Moss SE, Klein BE. The risk of cardiovascular disease mortality associated with microalbuminuria and gross proteinuria in persons with older-onset diabetes mellitus. Arch Intern Med 2000;160(8):1093-100.

van Parijs P. Réapprendre à mourir. In: Refonder la solidarité. Paris: Editions du Cerf; 1996. p. 45-65.

van Parijs P. Qu'est-ce qu'une société juste ? Introduction à la pratique de la philosophie politique. Paris: Seuil; 1991.

Veenhoven R. The four qualities of life: ordering concepts and measures of the good life. Journal of Happiness Studies 2000;(1):1-39.

Visser van Balen H, Geenen R, Looij J, Huisman J, Wit JM, Sinnema G. The views of young adults and their parents on hormone treatment for short stature in adolescence. Horm Res 2008;69(3):172-9.

Voss LD. Is short stature a problem ? The psychological view. Eur J Endocrinol Suppl 2006;155(1):S39-S45.

Wagstaff A. QALYs and the equity-efficiency trade-off. J Health Econ 1991;10(1):21-41.

Walliser B. L'intelligence de l'économie. Paris: Odile Jacob; 1994.

Weil-Dubuc,P. Penser les politiques de santé à l'âge de l'individualisation des risques. Paris: Ecole doctorale de Paris IV; 2012.

Weinstein MC, Stason WB. Foundations of cost-effectiveness analysis for health and medical practices. N Engl J Med 1977;296(13):716-21.

Whithead M., Townsend P. et Davidson N., Inequalities in Hleath : The black Report ; The health Divide, Londres, Penguin Group, 1988
Williams A. Cost-effectiveness analysis: is it ethical ? J Med Ethics 1992;18(1):7-11.

Weber, N. Le savant et le politique, préface de R. Arr., Paris, Plon, 1958

Wolfelsperger A. Comment peut-on être parétien ? L'économie du bien-être de l'utilitarisme au libéralisme. Rev Philos Econ 2010;(3):5-33.

Table des matières

Ethique et évaluation économique des interventions de santé en vue d'une définition du périmètre des soins remboursables

Les développements récents en économie du bien-être ouvrent la voie à des méthodes d'évaluations reposant sur d'autres modèles de justice sociale que l'utilitarisme. Leur faisabilité dans les pratiques quotidiennes d'évaluation des interventions de santé pose question : l'objectif de cette thèse est de contribuer à y répondre. Pour cela nous nous sommes intéressés à trois cas pratiques.
L'objectif du Chapitre I est de comparer les méthodologies d'évaluation de trois agences publiques d'évaluation, le NICE (Grande-Bretagne), l'IQWiG (Allemagne) et le KCE (Belgique), pour identifier les positions en matière de justice sociale qui en découlent. Le Chapitre II propose d'étudier le dilemme moral que suscite le phénomène d'adaptation des préférences dans l'évaluation de deux dispositifs de compensation du handicap. Trois options sont avancées résoudre ce dilemme, elles s'appuient sur les théories égalitaristes de la justice sociale. Enfin, le Chapitre III démontre la faisabilité de l'approche revenu équivalent-santé, développée par Fleurabey, dans l'aide à la décision publique, au sujet des traitements antihypertenseurs en prévention primaire.

Mots clés: évaluation des interventions de santé, justice sociale, aide à la décision publique, éthique, évaluation coût/bénéfice, égalitarisme, adaptation des préférences

---

Ethics and health economics evaluation in the context of decision-making about reimbursement of health technologies by the national health insurance

Welfare economics encompasses other public choice theories besides utilitarianism, like egalitarian social justice theory. Whether these economic frameworks provide practical tools that could be used in health technology assessment is an ongoing debate, and this study aims to contribute to answering this question.
The goal of the first chapter is to review health economics evaluation guidelines and compare methods from three national health technology assessment agencies - NICE (England and Wales), IQWiG (Germany) and KCE (Belgium) - and to assess what social justice principles are implied by their respective methodological choices. The second chapter studies the moral dilemma raised by adaptive preferences in growth hormone treatment and bilateral cochlear implants. Three options, grounded on egalitarian social justice theories, are put forward to solve this dilemma. The objective of the third chapter is to provide evidence about the feasibility of assessment based on the equivalent income approach, developed by M. Fleurbaey, in the context of public decision-making. For this application, we focused on antihypertensive treatments in primary prevention.

Key words: Health technology assessment, Social choice theory, Public decision-making, Ethics, Cost/benefit analysis, Egalitarism, Adaptive preferences