

ДОКТРИНА МЕДИЧНОГО ПРАВА

УДК 340.6(492):33:366.54:61

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WHERE LAW AND ECONOMICS MEET EACH OTHER: COST-EFFECTIVENESS ANALYSIS IN HEALTH CARE

In health policy, cost-effectiveness analysis (CEA) can help rationalize coverage decision-making. Although some countries apply economic evaluation methods to new medical interventions (mostly pharmaceuticals), approaches are not standardized. One of the reasons is that CEA is inextricably linked with health care rationing, which may result in denial of a less cost-effective treatment method to an individual patient.

From a legal perspective, denial of treatment for financial reasons challenges basic human rights, including the right to life, human integrity and the right to health care. This paper therefore explores the meaning of cost-effectiveness in the courtroom, its relevance to health policy-making, and the link with EU health legislation.

Key words: cost-effectiveness, health law, prioritizing health care, justiciability.

INTRODUCTION

The economic crisis and the global need for cost-containment in health care have urged governments to consider whether specific expensive therapies or medicines covered by the national health care benefit package could be replaced by cheaper alternatives. Generally,

considerations include: medical necessity, clinical effectiveness, costs, and other aspects. What is missing so far is a more systematic approach based on cost-effectiveness analysis, i.e. evaluating alternative treatments in terms of the cost of an equivalent amount of benefit. In health policy, cost-effectiveness analysis (CEA) can help rationalize coverage decision-making. Although some countries apply economic evaluation methods to new medical interventions (mostly pharmaceuticals), approaches are not standardized. One of the reasons is that CEA is inextricably linked with health care rationing, which may result in denial of a less cost-effective treatment method to an individual patient. In the most extreme case, it could even result in death of the patient with a life-threatening disease. Denial of treatment for financial reasons is an extremely sensitive issue. From a legal perspective, it challenges basic human rights, including the right to life, human integrity and the right to health care.

Adjudicating the denial of life-saving treatment, courts have been reluctant to strike down coverage decisions, as this is generally considered as a policy affair not subject to judicial review. Reviewing the constitutionality of denial of necessary health care, national courts on rare occasions applied cost-effectiveness arguments, with major consequences for individual patients and health policy makers. This paper therefore explores the meaning of cost-effectiveness in the courtroom, its relevance to health policy-making, and the link with EU health legislation. But first things first: what is cost-effectiveness analysis?

1. CEA: A NEW KID ON THE BLOCK

Most national health care systems have defined (legal) standards and guidelines on what's in and what's out of the mandatory health (insurance) system. International social security law and human rights law have set some minimum standards for harmonizing the statutory benefit package, but generally, international law is silent about what kind of treatment methods or technologies should be covered. Defining the nature and scope of statutory health benefits (coverage decisions) is above all a national competence, primarily based on medical criteria. Leading academics such as Michael Drummond (UK) and Peter Neumann (USA) have made a plea to incorporate CEA, next to existing criteria, in coverage decision-making.* This analytical technique allows comparing the costs of two health interventions (i.e., diagnosis and surgical intervention, medication) with the expected health gains, or: effectiveness (adverse reactions avoided, prevented death, etc.).** Though opponents may criticize the idea of valuing lived time in money, this is what economists do by calculating the cost-effectiveness (C/E) ratio based on number of days saved by treatment*** costs per life-years gained or costs per '*quality-adjusted life year*' (QALY) gained. The C/E ratio serves as a standard measure of the benefit of medical interventions to a patient. Interventions with a relatively low C/E-ratio are

* P.J. Neumann, *Using cost-effectiveness analysis to improve health care. Opportunities and Barriers* (New York: OUP, 2005); M. Drummond, A. McGuire, *Economic evaluation in health care: merging theory with practice* (New York: OUP, 2001).

** See Neumann (note 2) 8

*** Standardizing effects in QALYs enable to compare interventions' effectiveness.

'good buys'.* The C/E ratio represents a measure of how efficiently the proposed intervention can produce an additional QALY. It may help health care payers decide what innovations they should adopt. The goal of the decision-maker is to adopt all interventions that represent efficient ways of producing QALYs, and to disapprove of interventions with ratios that are too high.**

For instance, assume there are two treatment options for breast cancer: option A (€50,000) and option B (€150,000). Since option B is the more effective (3 life years gained versus 1 year with option A) but at the same time the most costly, one must decide if the greater effectiveness justifies the cost of achieving it. This decision is guided by calculating an incremental cost-effectiveness ratio, or: the difference in costs divided by the difference in outcomes. The ratio, expressed in QALYs, varies between 0–1. The value of 1 stands for subjectively perfect health, while 0 corresponds with death.***

QALYs as a measure of medical benefit are therefore suitable to set a threshold for the costs of treatment. Above the threshold, treatment will be considered unaffordable and be excluded from coverage. The decisive criterion for coverage is therefore the maximization of QALYs gained. For instance, in the UK, the National Institute for Health and Care Excellence ('NICE') has formulated a relative threshold of £20,000–30,000.**** This means that *Bevacizumab*, a new colorectal cancer drug, calculated as £103,000 per QALY, was considered unaffordable.***** Other countries, such as the Netherlands, are only discussing whether they should introduce such a threshold.***** But apart from the political sensitivity of the willingness-to-pay threshold, the gospel of economic evaluation spread all over the world.

* Garber et al, Theoretical Foundations of Cost-Effectiveness Analysis, (reference made in Neumann note 2) 27.

** Garber et al, Theoretical Foundations of Cost-Effectiveness Analysis, (reference made in Neumann note 2) 27.

*** Modified example based on M.R. Gold, et al, *Cost effectiveness in health care and medicine* (New York: OUP 1996)14-17.

**** NICE has always avoided the term 'threshold': 'There is no empirical basis for assigning a particular value to the cut-off between cost-effectiveness and cost ineffectiveness. The consensus amongst the Institute's economic advisors is that the Institute should, generally, accept as cost effective those interventions with an incremental cost-effectiveness ratio of less than £20,000 per QALY and that there should be increasingly strong reasons for accepting as cost effective interventions with an incremental cost-effectiveness ratio of over £30,000 per QALY. These reasons include the degree of uncertainty surrounding the estimate of the incremental C/E-ratio and, where appropriate, reference to previous appraisals. 'The Institute ... will also wish to consider social value judgments including consideration of the nature of the condition, the particular patient population, and the intervention itself.' NICE Guide to the methods of technology appraisals reference NO515, April 2004, available through <<http://www.nice.org.uk>> (last visited 10 December 2013).

***** NICE technology appraisal, 15 December 2010, TA212, Bevacizumab in combination with oxaliplatin and either fluorouracil plus folinic acid or capecitabine for the treatment of metastatic colorectal cancer, available through <<http://www.nice.org.uk>> (last visited 10 December 2013).

***** E.g., the Dutch Council for Public Health and Health Care (Raad voor de Volksgezondheid en Zorg, RVZ) recommended a threshold of €80,000 per QALY gained,

An alternative to CEA is the so-called cost-benefit analysis (CBA), in which costs and benefits are compared in monetary terms (e.g., valuing life-years saved at €100,000 each), leading to a simple decision rule: when the benefits exceed the costs, then the intervention should be adopted.* CBA, however, raises the problem of the monetary valuation of health benefits. For instance, how to quantify the case of cancer avoided or a life saved?** Another technique, cost-utility analysis (CUA), is a species of genus CEA, measuring benefits in QALYs or 'disability-adjusted life years' (DALYs). Despite its limitations,*** international health economists consider CEA as the 'gold standard' and it is most often used for economic evaluation studies.****

2. COST-EFFECTIVENESS ANALYSIS AND THE LAW

Cost-effectiveness analysis and economic evaluation in general raises several legal concerns. Hereafter, the focus is on two related issues: regulating CEA and the relationship with fundamental principles of law.

Regulating CEA: Country experiences

For lack of systematic assessment based on CEA, it has been suggested to regulate CEA by law.***** By defining cost-effectiveness as a legal condition for allocation decision-making, the outcomes may be expected to contribute to a more rational decision-making. In the Netherlands, defining the benefit package is the responsibility of the Minister of Health, advised by the Health Insurance Board, a public body with several tasks set by law.***** The Board uses standard methods and procedures for its assessments. Assessment criteria include: i) necessity: does the illness – seen in a societal context – justify claiming solidarity?; ii) medical

RVZ report 'Zinnige en duurzame zorg', 7 June 2006 <<http://www.rvz.net/publicaties/bekijk/zinnige-en-duurzame-zorg>> (last visited 11 December 2013).

* See Neumann (note 2) 14.

** See Neumann (note 2) 14.

*** First of all, CEA requires a robust database covering the overall effectiveness of a given clinical intervention, which is often not known. Secondly, due to the limited number of CEAs, there is a lack of knowledge, and thirdly, the reliability of individual CEA studies has been questioned. Finally, CEA can be used for evaluating the treatment of schizophrenia and interventions for treating heart disease but the outcomes are so different that a direct comparison of the QALYs created might be impossible. L.B. Russell et al, 'Cost-Effectiveness Analysis as a Guide to Resource Allocation in Health: Roles and Limitations in: M. Gold (note 3) 12.

**** L.B. Russell et al, 'Cost-Effectiveness Analysis as a Guide to Resource Allocation in Health: Roles and Limitations in: M. Gold (note 3) 15.

***** See, e.g., the Dutch Health Insurance Board ('College voor Zorgverzekeringen', CVZ). Advies 'Kosteneffectiviteit in de zorg: Op weg naar een genuanceerd en geaccepteerd gebruik van Kosteneffectiviteitsgegevens in de zorg'. Annex Kamerstuk 29689 no. 476, available through <<https://zoek.officielebekendmakingen.nl/>> (last visited 10 December 2013).

***** Article 58(3) Health Insurance Act 2006, available through <<https://zoek.officielebekendmakingen.nl/>> (last visited 11 December 2013).

effectiveness: according to international standards of science and practice?; iii) cost-effectiveness: does the intervention have an acceptable costs/benefits relationship?; and iv) feasibility: is inclusion in the package feasible, now and in the long term? The Board issued an authoritative advice for each newly developed and existing technology, which enabled the Minister to decide on the composition of the benefit package.* So far, the four above-mentioned criteria have no explicit legal basis, but they are proposed in the Health Insurance Act's explanatory memorandum.** Currently, the government is considering an explicit statutory basis of CEA in coverage decision-making.***

Comparative research shows that economic evaluation is a core-criterion incorporated in the German Social Code (*Sozial Gesetzbuch V*). Article 12 stipulates that social health insurance benefits should comply with the so-called 'wirtschaftlichkeit'-criterion, i.e. it should be sufficient, effective and economically efficient.**** Despite widely applied evaluation techniques, economic evaluation studies are generally measured in cost-benefit ratios rather than C/E ratios.***** Under the German health insurance regime, issuing guidelines on entitlements is delegated to the Federal Joint Committee (*Gemeinsamer Bundesausschuss*, G-BA), and these are binding to health insurance stakeholders.***** The G-BA is supported by the Institute of Quality and Efficiency in Health Care (IQWiG), which engages in economic evaluation studies and quality research.*****

A slightly different approach can be found in the Swiss Health Insurance Act (*Krankenversicherungsgesetz*, KVG),***** anchoring the responsibilities of a

* The outcomes of these assessments are available at: www.cvz.nl. Originally, the 4 criteria were based on the framework of criteria recommended by the Dunning Committee 'Choices in Health Care', The Hague, 1991. This framework basically functions as a series of sieves separating care that should be funded from that which should not be funded by public funds.

** 'Zorgverzekeringswet' (Health Insurance Act 2006), Tweede Kamer, vergaderjaar 2003–2004, 29 763, nr. 3, p. 40, available through <<https://zoek.officielebekendmakingen.nl/>> (last visited 12 December 2013).

*** Letter from the Minister of Health, Kamerstukken II 29 689, Nr. 476, Den Haag, 30 september 2013, available through <<https://zoek.officielebekendmakingen.nl/>> (last visited 12 December 2013).

**** Sozialgesetzbuch (SGB) Fünftes Buch (V) – Gesetzliche Krankenversicherung – (Artikel 1 des Gesetzes v. 20. Dezember 1988, BGBl. I S. 2477) § 12 Wirtschaftlichkeitsgebot (1) 'Die Leistungen müssen ausreichend, zweckmäßig und wirtschaftlich sein; sie dürfen das Maß des Notwendigen nicht überschreiten. Leistungen, die nicht notwendig oder unwirtschaftlich sind, können Versicherte nicht beanspruchen, dürfen die Leistungserbringer nicht bewirken und die Krankenkassen nicht bewilligen.' <Sozialgesetzbuch.de> (last visited 10 December 2013).

***** For a critical review, see S. Huster, 'Die Methodik der Kosten-Nutzen-Bewertung in der Gesetzlichen Krankenversicherung', *MedizinRecht* 28 (2010): 234-240.

***** Article 92 SGB V.

***** Article 139a SGB V; Article 35a-b SGB V.

***** Bundesgesetz über die Krankenversicherung, 18 March 1994, as amended (KVG), available through <<http://www.admin.ch/>> (last visited 10 December 2013).

dedicated Parliamentary expert committee recommending the Minister of Health on entitlement issues.* According to the KVG, health insurance entitlements have to comply with the so-called 'WZW' criteria, i.e. 'Wirksamkeit' (effectiveness), 'Zweckmässigkeit' (appropriateness), and Wirtschaftlichkeit (economic viability), and will be periodically reviewed.**

Lastly, the National Institute for Health and Care Excellence (NICE) in the United Kingdom is a well-known institution involved in economic evaluation. NICE was originally established 'to reduce variation in the availability and quality of NHS treatments and care' (1999) but in the new Health and Social Care Act 2012, its responsibilities were redefined as 'developing quality standards and giving advising or guidance on health care and social care issues'.*** Evidence-based guidance and standards are based on clinical and cost-effectiveness assessments of health technologies.****

Comparative experiences with economic evaluation show therefore a preference for a statutory obligation to include economic evaluation in health policy decision-making since it may contribute to more rational decision-making on content of the benefit package.***** Economic evaluation imposes policy makers to argue why a certain intervention is excluded from coverage, and thus provides the transparency needed to legitimize coverage decisions.

But more difficult, if not impossible, is defining the threshold level; what will be maximum limit a society is willing to pay? At international level, there seems no consensus on this issue. For instance, in the UK, NICE applies a cut off of around £30,000 per QALY, whereas the World Health Organization suggests a norm 'three times the national product per head of the population'.***** In the Netherlands, the Health Insurance Board does not apply a ceiling value. Here, the cost-effectiveness of many interventions lies within a bandwidth with a median value of €40,000/QALY.*****

Because of the variety, and given the current practice, a bandwidth or *relative* threshold is preferred above a fixed threshold, meaning that additional arguments

* The KVG established a so-called expert-committee, the 'Eidgenössische Kommission für allgemeine Leistungen und Grundsatzfragen' (EGK), Article 37 b of the KVG Regulation, (KVV) 27 June 1995.

** Article 32 KVG.

*** Articles 234-237 Health and Social Care Act 2012 <<http://www.legislation.gov.uk/>> (last visited 10 December 2013).

**** NICE website <www.nice.org.uk> 'what we do'. (last visited 10 January 2014).

***** Most recently, the Dutch government is considering such an approach by amending the Dutch Health Insurance Act, see: Health Insurance Board (CvZ), 'Kosteneffectiviteit in de zorg. Op weg naar een genuanceerd en geaccepteerd gebruik van kosteneffectiviteitsgegevens in de zorg' (Cost-effectiveness in Health Care, in Dutch), 30 september 2013.

***** CVZ, A background study on the cost-effectiveness' package principle for the benefit of the appraisal phase in the package management, Number 291, 17 November 2010, available through www.cvz.nl (last visited 9 December 2013). CPB Document Number 152, at 10, available through www.cvz.nl (last visited 9 January 2014).

***** CPB Document Number 152, at 10, available through www.cvz.nl (last visited 9 January 2014).

become increasingly important above a certain threshold. As the cost-price per QALY rises, coverage decisions require a balancing of arguments, based on medical need and the availability of alternatives, proportionality, etc. Challenged in court, the judiciary may review whether the outcome of such a process in individual cases was 'manifest unjust' or irrational, taking into account fundamental human rights.*

But what if health policy makers cannot or are unwilling to formulate a threshold, either fixed or relative? Then, the risk that courts open a 'Pandora's box', challenging the use of cost-effectiveness, is high, as will be explained hereafter.

Relationship with fundamental principles of law

The statutory basis of economic evaluation does not exclude judicial review on fundamental principles of law underlying health insurance legislation. This is one of the lessons learned from the *Nikolaus* case, ruled by the German Federal Constitutional Court in 2005.** A young patient suffers from a Duchenne Muscle disease (DMD), a progressive and lethal illness. At present, there is no effective therapy for DMD available. Reimbursement of cost of a new treatment method, the so-called immune biological therapy, was rejected by the social insurance fund since it was not evidence-based ('*wirksamkeit*' criterion). The Constitutional Court ruled, however, that statutory criteria for limiting health benefits (i.e. '*ausreichend, zweckmäßig, wirtschaftlich*') should be interpreted in line with constitutional values such as the right to life, bodily integrity and the welfare (or social) state principle.*** More specifically, in case of life-threatening diseases for which medical treatment is lacking according to general medical standards, except availability of experimental treatment with curative or positive effect ('*spürbare positive Einwirkung*') on the disease course, this alternative cannot be excluded in the absence of scientific evidence. The alternative's effectiveness could be based on other evidence, for instance expert opinions and medical practice.****

With this ruling the Court has, although in exceptional cases, extended health care access to newly developed, and in most cases extremely expensive, diagnostic and treatment methods that are likely to have a positive effect on the disease course.***** It means that when scientific evidence is absent, the required probability standard of effectiveness is rather flexible: the more severe, the more hopeless the situation, the less stringent the likeliness standard. And although the Court recognized the

* In a way this is the UK judicial review practice, except for the human rights review. See K. Syrett, 'Health technology appraisal and the courts: accountability for reasonableness and the judicial model of procedures justice', *J Health Economics, Policy, and Law* 4 (2011): 469-488.

** Case BvR 347/98, 6 December 2005, also known as the 'Nikolausbeschluss', available through <<http://www.bverfg.de/>> (last visited 8 February 2014).

*** Case BvR 347/98, 6 December 2005, at 55.

**** Case BvR 347/98, 6 December 2005, at 66.

***** Examples accepted under this provision concern an experimental combined therapy for Ovarian cancer (€15,000 p.m) BvR 2045/12, 26 February 2013, available through <<http://www.bverfg.de/>>; experimental stem cell transplantation LSG Baden Würtemberg, 13 November 2012, L11 KR 2254/10 <<http://www.juraforum.de/>> (last visited 9 December 2013).

'Wirtschaftlichkeitsgebot', (Article 12 SGB V), and the need for cost (or cost-benefit) considerations,* these criteria were not decisive.

The *Nikolaus* ruling stirred feelings in German legal doctrine.** In essence, it shows that despite the legislature's (c.q. G-BA) discretionary powers to formulate binding guidelines on evidence-based medicine and applied selection criteria, standards should ultimately comply with constitutional values.

How different is the outcome in the *Myozyme* case from the Swiss Supreme Court.*** In appeal, a Swiss Health Insurance Fund challenged the court order of the Insurance Tribunal to continue reimbursement of an experimental treatment for Pompe disease, a rare and life-threatening disease. The Supreme Court annulled the Tribunal's ruling by reasons based on both lacking clinical effectiveness ('Wirksamkeit') and cost-effectiveness (i.e., a limited cost-benefit ratio). The costs of treatment were calculated at CHF 700,000 per year (€565,000). With this ruling the Court set a maximum threshold of costs covered by public health insurance to preserve an individual's state of health at 100,000 CHF (€81,000) per life-year gained.**** This maximum is based on consensus among health economists and a previous ruling challenging the 'wirtschaftlichkeit' principle, although it concerned an expensive nursery care treatment method.*****

Because general criteria to assess cost-effectiveness were absent, in the *Myozyme* case the Court applied a – controversial – cost-benefit analysis, concluding that the excessive costs of treatment would be disproportionate to the benefit (i.e. only relieving the symptoms of the disease, not postponing or preventing its fatal outcome).***** Moreover, approval would violate the equality principle when a disproportionate amount of scarce resources would be allocated to a certain individual but not to others who are in the same position.*****

* Case BvR 347/98, 6 December 2005, at 57–59.

** J. Huster, 'Anmerkung', *JuristenZeitung* 9 (2006): 466–468; G. Dannecker, A.F. Streng, 'Die Bedeutung des Nikolaus-Beschlusses für die Priorisierungsdebatte', in *Priorisierung in der Medizin. Kriterien im Dialog*, ed. B. Schmitz-Luhn & A. Bohmeier (Berlin: Springer, 2013): 135–146.

*** Judgment of the Federal Supreme Court of Switzerland of 23 November 2010 (BGE 136 V 395), available through <<http://www.bger.ch/>> (last visited 14 March 2014).

**** BGE 136 V 395 paragraph 7.6.2, although such an interpretation has been denied by some authors, in personal correspondence with prof. S. Dagron, university of Zurich.

***** *BGE 126 V 334 E. 3b S. 342 ... 'Im Bereich der Pflegefinanzierung für Spitex-Leistungen wird als obere Grenze der Verhältnismässigkeit ein Aufwand bezeichnet, der ca. 3,5 mal höher liegt als der Aufwand in einem Pflegeheim und in absoluten Zahlen gegen Fr. 100'000. – pro Jahr beträgt. Unverhältnismässig bzw. unwirtschaftlich sind Kosten, die vier- bis fünfmal höher sind als diejenigen im Pflegeheim und absolut über Fr. 100'000. – pro Jahr betragen (Urteil des Eidg. Versicherungsgerichts K 95/03 vom 11. Mai 2004 E. 3.2)'.*

***** BGE 136 V 395, paragraph 7.4.

***** BGE 136 V 395, paragraph 7.7–7.8.

This line of reasoning has been criticized by legal scholars.* Although cost-benefit/effectiveness analysis is relevant at macro level (benefit package decision-making), it seems less appropriate at the individual doctor-patient level since it will ultimately force the judiciary to decide about society's willingness to pay for rare diseases.

Different from the German *Nikolaus* case, the Swiss Supreme Court declined to review the constitutionality of denial under the right to life, personal freedom and the right to assistance when in need.** Unfortunately, as these rights were not challenged at the Supreme Court, it could abstain from such a human rights assessment.*** Ultimately, this case triggered public deliberation which resulted in a Federal by-law providing a legal basis and guiding principles of cost considerations in coverage decision-making, but *without* setting a threshold.**** Instead, health insurance funds are supposed to review (partial) reimbursement of expensive interventions on a case-by-case basis, applying CEA evidence.

Apart from these two landmark cases, economic evaluations arguments are rarely raised or challenged in courts. Certainly, in numerous cases cost considerations were like 'the elephant lurking in the room that is not discussed'.***** The explanation

* E.g., F. Kesselring, 'First Fundamental Decision of the Federal Supreme Court of Switzerland on Cost-Effectiveness in the Area of Human Healthcare', *European J. Risk Regulation* 3 (2011): 442-446; S. Huster & A. Bohmaier, 'Die Myozyme-Entscheidung des Schweizerischen Bundesgerichts aus der Perspektive des deutschen Verfassungs- und Krankenversicherungsrecht', *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 106 (2012): 443-448. Also interesting are the comments made by: H. Raspe, 'Das «Morbus-Pompe» Urteil des Schweizerischen Bundesgerichts – eine Diskussion aus rechtlicher, socialmedizinischer und medizinischer Sicht', *MedizinRecht* 30 (2012) 291-294.

** Articles 10 and 12 of the Swiss Federal Constitution, available through <<http://www.admin.ch/>, Bundesverfassung> (last visited 9 March 2014).

*** See Kesselring note 44, p. 446.

**** Federal By-law on Health Insurance AS 2011 654 (explanatory note), Article 71a (3) KVV, reading: 'Die zu übernehmenden Kosten müssen in einem angemessenen Verhältnis zum therapeutischen Nutzen stehen (...)', which can be interpreted as an implicit cost-benefit assessment, also Article 71 b (3) KVV; confirmed by the government's reply on Parliamentary question number 11.3154 (6 June 2011), in particular question number 4.

***** E.g., UK: *R (Rogers) v. Swindon NHS Primary Care Trust* (UK) [2006] EWCA Civ 392 (refusal Herceptin treatment in case of breast cancer) <<http://www.bailii.org/>> (last visited 10 December 2013); *Eldridge v. British Columbia* (Att'y Gen.), (CA) [1997] 3 S.C.R. 624 (failure to provide language interpretation services in case of deafness), available through <<http://scc-csc.lexum.com/>> (last visited 10 December 2013); *J F Shortland v. Northland Health Ltd.* (Rau Williams case) [1998] 1 NZLR. 433 (CA) (N.Z.), available through <https://forms.justice.govt.nz/> (last visited 9 December 2013). (denial of dialysis of patient suffering from dementia). These *denials* of treatment should be differentiated from pharmaceutical companies' claims ordering admittance to a so-called pharmaceutical 'positive list' (conditional for reimbursement), based on cost-effectiveness arguments, e.g. District Court Amsterdam 6 February 2008, ECLI:NL:RBAMS:2008:BC5569, available through <www.rechtspraak.nl> (last visited 7 December 2013); *Eisai Ltd. v Nice* [2007] EWHC 1941 (Admin) (10 August 2007) (UK), available through <URL: <http://www.bailii.org/>> last visited 9 March 2014)

is obvious: courts are generally not competent to value life, because that is what it is all about. The Swiss example revealed the need for a fundamental debate on society's willingness to pay for health care, but then once conducted in Parliament. Ideally, the outcome of such a debate should recognize the value of economic evaluation as a piece of evidence, setting the standard of care maximized by a flexible threshold. Such a relative threshold should be understood in context, as an authoritative tool taking into account other considerations ([socio]-medical, ethics, law, etc). The legislature's upper limit of society's willingness to pay permits medical professionals and medical scientific associations (meso level) to formulate more specific guidelines and standards on clinical and cost-effective treatment methods. On micro level, these instruments enable physicians to decide whether or not they will apply these guidelines, or not take into account all relevant circumstances. In case of denial, the judiciary is bound by these normative standards, including fundamental legal principles. In the end, it appears that formulating such standards requires a multidisciplinary approach. Such an approach is suggested by health technology assessment.

3. THE LINK WITH EU DIRECTIVE 2011/24 AND HEALTH TECHNOLOGY ASSESSMENT

At European level, it is generally acknowledged that health care coverage decisions fall within the exclusive competence of national governments (Article 168 [7] TFEU).^{*} Consequently, economic evaluation as a decision-making criterion has not been harmonized. The Patients' Rights Directive (Directive 2011/24/EU) does not change this situation but still is of relevance by introducing the concept of Health Technology Assessment (HTA).^{**} HTA is defined as:

'a multidisciplinary process that summarizes information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe effective, health policies that are patient focused and seek to achieve best value.'^{***}

This definition emphasizes that evaluating health technologies is not restricted to *economic* science only but requires a multidisciplinary approach, including legal and organizational aspects (as understood from 'social issues'). HTA takes therefore an *integral* approach of relevant disciplines, providing input to decision making in policy and practice. But so far, most HTAs focus on *economic* evaluation, ignoring the input from other disciplines, thereby putting the comprehensiveness and

* As confirmed by the CJEU , e.g., Case C-385/99 *Müller-Fauré & Van Riet* [2003] ECR I-4509; Case C-372/04, *Watts v Bedford Primary Care Trust* [2006] ECR I-4325; Case C-173/09 *Elchinov* [2010] ECR I-08889; Case C-512/08 *Commission v French Republic* [2010] ECR I-08833, available through <curia.europa.eu> (last visited 9 December 2013).

** Officially, Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare. Article 15 introduces the European network of HTA, available through <<http://eur-lex.europa.eu>> (last visited 9 December 2013).

*** European Network for Health Technology Assessment. Definition of HTA. <<http://www.eunethta.net/HTA/>> (last visited 9 December 2013).

usefulness of the analysis at risk.* Moreover, the available HTA studies focus on newly developed medicines, excluding other medical technologies such as medical devices, diagnostic procedures and complex treatment interventions.**

But apart from these omissions, most Member States have recognized the value of HTA in health policy-making by establishing (independent) HTA entities, which carry out evaluation studies. In addition, an EU-wide HTA network (EUnetHTA) has been established.*** EU Directive 2011/24 supports these HTA initiatives by 'facilitating cooperation and the exchange of scientific information among Member States'.**** This information exchange (applied criteria, methodology, research outcomes, etc.) will prevent duplication in HTA research of new technologies. Although the Directive excludes that the exchange will harmonize the Member States' basic benefit schemes,***** still, the proliferating use of soft law mechanisms (non-binding guidelines, open method of coordination, mutual learning and peer review) may enable an incremental 'Europeanisation', i.e. convergence in HTA decision-making processes (and outcomes?).*****

Wishful thinking or not, still, including HTA in coverage and reimbursement decision-making, similarly to economic evaluation, would strengthen transparency in health policy decision-making. Therefore, a statutory HTA requirement should emphasize the multidisciplinary approach, as suggested by EUnetHTA's HTA Core Model identifying nine research domains: health problem and current use of technology; description and technical characteristics of technology; safety; clinical

* A brief literature search on 'HTA and Law' yielded a limited number of citations (2005-2013), e.g., R. Franke & D. Hart, 'HTA in den Entscheidungsprozessen des IQWiG und G-BA', *Bundesgesundheitsblatt, Gesundheitsforsch-Gesundheitsschutz* 3 (2006): 241-250; G. Soskut, 'Technologiebewertung aus Sicht der Medizintechnologie: richtig und wichtig!', *MedizinRecht* 9 (2006): 524-527; B.K. Potter, et al, 'Ethical, legal, and social issues in health technology assessment for prenatal/preconceptional and newborn screening: a workshop report', *Public Health Genomics* 1 (2009): 4-10; K. Syrett, see note 31. Still there are numerous subjects that justify a legal assessment (organ transplantation, new medical technologies, such as genetic technologies, nanomedicine, etc).

** A search on 'cost-effectiveness' in the *International J of Technology Assessment in Health Care* yielded more than 1,200 citations, mostly focusing on new medicines.

*** EU-wide network on Health Technology Assessment <www.eunethta.net> (last visited 9 December 2013).

**** According to Article 15.1 of the Directive 'shall the Union support and facilitate cooperation and the exchange of scientific information among Member States within a voluntary network connecting national authorities or bodies responsible for health technology assessment designated by the Member States. The Member States shall communicate their names and contact details to the Commission. The members of such a health technology assessment network shall participate in, and contribute to, the network's activities in accordance with the legislation of the Member State where they are established. That network shall be based on the principle of good governance including transparency, objectivity, independence of expertise, fairness of procedure and appropriate stakeholder consultations.'

***** Article 15(7) of the Directive excludes any harmonizing effect.

***** See for an interesting analysis: E. Brooks, Europeanisation through soft law: the future of EU health policy, (2012) 6 (1) *Political Perspectives*: 86-104.

effectiveness; costs and economic evaluation; ethical analysis; organizational aspects; social aspects; and legal aspects.* Mandatory for evaluating several technologies, such comprehensive HTA reports would increase the political and legal accountability of HTA policy decisions.**

Concerning the legal assessment, the HTA Core Model identifies relevant domains (patients' rights, planning and regulating health services, health insurance and pharmaceuticals, liability issues, etc.) and sources of law (national, international and European law). What is missing however, are principles of law, professional standards, and relevant case law, which are of equal importance in the human rights assessment. Carrying out a human rights 'due diligence' should provide clarity how governments meet their responsibility to respect human rights, and addressing potential effects of a policy decision. More specific exploring the effects of introducing a new medical technology in terms of individual rights: right to life, private life; the equality principle; and social rights: health care access, the Sozialstaat-principle and applicable state obligations, as well as the concept of progressive realization and access to new technologies,*** and the role of the judiciary in adjudicating health care access. For instance, in case of a newly developed life-saving medicine, the human rights assessment requires a further analysis and discussion on applicable human rights, the balancing of human rights, and whether denial, or to what extent (conditional) reimbursement complies with the state obligation on guaranteeing equal access to essential medicines, progressively. Ultimately, such a human rights assessment, combined with economic and policy arguments may justify the need for making hard choices in health care, ie. denial of treatment as a result of scarce resources. But at least, such a painful decision is then based on the principles of legal reasoning, transparency, good governance, and public accountability.

4. TOWARDS A SEPARATE BRANCH OF LAW: HTA-LAW

The variety and complexity of legal disciplines involved raises the question whether we should consider HTA-law as a separate branch of law? The nature and scope of research seem to justify an affirmative answer. Such a new branch of health law examines health technologies from various perspectives: penal, civil and public law, international (human rights) law, and health law, but may also involve less likely aspects, such as public procurement rules (medicines), patent law issues,**** and medical malpractice issues (e.g., in case of e-health technologies). Such an assessment is not needed for every (new) technology, but for interventions that may pose a significant burden on the health and rights of the individual and community involved.

* EUnetHTA, The HTA Core Model Application for Medical and Surgical Interventions (2.0) www.eunethhta.net (last visited 9 December 2013).

** For an interesting analysis evaluating the policy and practice impact of HTA, see C. Sorenson & K. Chalkidou, 'Reflections on the evaluation of health technology assessment in Europe', *Health Economics, Policy and Law* 7 (2012): 25-45.

*** As explained in General Comment Number 14, United Nations, The right to the highest attainable standard of health (2000), E/C.12/2000/4.

**** A. den Exter, Personalized Medicine and Access to Genetic Technologies, (2010) 33 *Rev Der Gen H (Law and the Human Genome Review)* 49-64.

Conceptualizing HTA-law contributes to better legal understanding of (newly developed) medical technologies and more comprehensive research on health care innovations. Instead of an – incomplete – ad hoc approach, HTA legal analysis examines the interaction between all relevant legal aspects, which will increase the quality of research.*

5. CONCLUSIONS

Generally, health lawyers feel uncomfortable when confronted with economic aspects, such as cost-effectiveness analysis in health care. Measuring and valuing health-related quality of health based on statistical simulation models may seem absurd from a normative perspective. Although the 'technicalities' of this type of economic evaluation may be unknown, we cannot ignore its role in current health policy making when defining the basic benefit package. The tendency to codify cost-effectiveness in statutory law confirms that practice, making it more explicit. But it is just a single tool, in a complex of domains relevant in coverage decision-making. Ignoring the value of cost-effectiveness, as appeared in the *Nikolaus* ruling, does not acknowledge the political reality. Otherwise, disregarding inherent human rights, as shown in the *Myozyme* case, undermines the separation of powers.

The mixed outcomes call for clarity. First of all, clarity on criteria that are considered relevant in coverage decision-making. Secondly, clarity on whether society is willing to define a threshold of health care costs. That requires a public debate in which health technology assessment emerges as a promising tool offering a multi-faceted approach, including legal science. The outcomes of HTA initiatives will enable health policy makers to balance the various interests and thus to make more explicit choices.

* As pleaded in: R. Francke & D. Hart, 'Einführung in die rechtlichen Aspekte bei HTA', *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 2 (2008): 63-68; more explicit, but focused on the social health context, see R. Francke & D. Hart, 'HTA in den Entscheidungsprozessen des IQWiG und G-BA. Bestandaufnahme und aktuelle Fragen der gesundheitsrechtlichen Regulierung', *Bundesgesundheitsbl. – Gesundheitsforsch-Gesundheitsschutz* 3 (2006): 241-250. Although most HTA initiatives are initiated from a social insurance coverage and reimbursement-perspective, one may argue that technologies excluded from public funding might also justify HTA (e.g., complementary medicine), therefore covered by HTA law.

Екстер А.

Точки перетину економіки та права: аналіз економічної ефективності у сфері охорони здоров'я

Охарактеризовано особливості врахування судами аргументів економічної ефективності при розгляді справ про відшкодування вартості лікування. З'ясовано роль економічної ефективності при формуванні політики. Висвітлено зв'язок між економічною ефективністю і законодавством Європейського Союзу. Відзначено, що розглядаючи справи щодо законності відмови в наданні необхідної медичної допомоги, суди Нідерландів у небагатьох випадках покликались на аргументи економічної ефективності, що мали б істотні наслідки для пацієнтів чи суб'єктів, які формують політику у сфері охорони здоров'я.

Висвітлено досвід Нідерландів з формування страхового пакета з урахуванням вимог економічної ефективності. У цій країні обсяг страхового пакета формує міністерство охорони здоров'я спільно з дорадчою радою з питань медичного страхування, спираючись на такі критерії: необхідність, медична ефективність, економічна ефективність, обґрунтованість. Доведено необхідність визначення фіксованого і кореляючого порога витрат, які мали б охоплюватись пакетом медичного страхування. Охарактеризовано зв'язок Директиви ЄС 2011/24 «Про права пацієнтів під час транскордонного надання медичних послуг» з оцінкою технологій у сфері медицини. З'ясовано питання можливості виокремлення підгалузі оцінки технологій у сфері медицини в межах медичного права. Наголошено на необхідності вироблення чітких критеріїв ухвалення рішень про компенсацію, а також з'ясування того, чи готове суспільство до встановлення порога витрат на охорону здоров'я, які підлягатимуть компенсації.

Ключові слова: економічна ефективність, медичне право, окреслення пріоритетів сфері охорони здоров'я, можливість розгляду в судовому порядку.

Экстер А.

Точки пересечения экономики и права: анализ экономической эффективности в сфере здравоохранения

Охарактеризованы особенности учета судами аргументов экономической эффективности при рассмотрении дел о возмещении стоимости лечения. Выяснена роль экономической эффективности при формировании политики. Освещены связи между экономической эффективностью и законодательством Европейского Союза. Отмечено, что рассматривая дела о законности отказа в предоставлении необходимой медицинской помощи, суды Нидерландов в некоторых случаях ссылались на аргументы экономической эффективности, которые имели существенные последствия для пациентов или субъектов, формирующих политику в сфере здравоохранения.

Освещен опыт Нидерландов по формированию страхового пакета с учетом требований экономической эффективности. В этой стране объем страхового пакета формирует министерство здравоохранения совместно с советательным советом по вопросам медицинского страхования, руково-

водствуясь такими критериями: необходимость, медицинская эффективность, экономическая эффективность, обоснованность. Доказана необходимость определения фиксированного и корелирующего порогов расходов, которые должны охватываться пакетом медицинского страхования. Охарактеризована связь Директивы ЕС 2011/24 «О правах пациентов во время трансграничного предоставления медицинских услуг» с оценкой технологий в области медицины. Выяснен вопрос возможности выделения подотрасли оценки технологий в сфере медицины в пределах медицинского права. Отмечена необходимость выработки четких критериев принятия решений о компенсации, а также выяснение того, готово ли общество к установлению порога расходов на здравоохранение, подлежащих компенсации.

Ключевые слова: экономическая эффективность, медицинское право, определение приоритетов здравоохранения, возможность рассмотрения в судебном порядке.