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Title: Access to treatment and the constitutional right to health in Germany: A triumph of hope over evidence?

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Abstract

Health technology assessment is frequently credited with making difficult resource allocation decisions in health care fairer, more rational and more transparent. In Germany, a constitutional 'right to health' allows patients to challenge decisions by sickness funds to withhold reimbursement of treatment excluded from public funding because of insufficient evidence of effectiveness. The ability to litigate was qualified by the Constitutional Court in its 2005 'Nikolaus decisions' that sets out criteria to be applied to these cases. Treatment must be made available if (1) the condition is life-threatening, (2) no alternative treatment is available, and (3) there is some indication that the treatment could cure the patient. This paper examines how courts struggled to apply these criteria based on an analysis of cases of patients who sought treatment for cancer between 2005 and 2015, and explores the implications of applying a constitutional 'right to health' to treatment decisions.

Access to treatment and the constitutional right to health in Germany: A triumph of hope over evidence?

Health technology assessment is frequently credited with making difficult resource allocation decisions in health care fairer, more rational and more transparent through the rigorous and systematic application of scientific evidence (Banta, 2003, Velasco Garrido et al., 2008). Yet contrary to expectations, this has not reduced the potential for conflict and controversy, especially when treatment for life-threatening illness is at stake (Syrett, 2003, Timmins et al., 2016). Politicians and administrations have been under sustained pressure to ensure access to novel treatment, often at high costs to society, as the rise and fall of the NHS Cancer Drug Fund in England demonstrates (Aggarwal et al., 2017). In Germany, patients who are denied reimbursement by their sickness funds are entitled by law to challenge these decisions in social courts. In 2005, the Federal Constitutional Court (*Bundesverfassungsgericht)* strengthened the position of sickness fund members suffering from life-threatening illness vis-à-vis their funds through the *Nikolaus-Beschluss* (so called after the day the decision was taken which was 6th of December, Saint Nicholas Day), which links access to treatment to the right to health enshrined in the Basic Law (*Grundgesetz*), the German constitution. This ruling has become a powerful lever for patients to force sickness funds to fund treatment that has been excluded from reimbursement (Bohmeier and Penner, 2009).

This paper demonstrates that these court cases are the result of tensions between two norms that apply to access to treatment: the 'right to health' implied in the 'right to life and physical integrity' enshrined in Article 2 of the Constitution, and the principles of evidence-based medicine(EBM) that underpin the regulation of access to treatment. The first norm represents a social right that pertains to the individual citizen vis-a-vis the state. The second norm - extended to health technology assessment used in support of decisions on access to treatment - draws on principles of scientific rationalism and distributive justice that benefit society

(here the society of sickness funds members) as a whole, if not necessarily all members equally or individually.

The paper examines how courts in Germany struggle to reconcile these two norms, using a sample of court cases involved in access to treatment decisions relating to three types of cancer treatment: Avastin, Hyperthermia, and Brachytherapy. Access to cancer drugs and other forms of treatment of cancer is considered generous in Germany compared to other high-income countries. Between 2011 and 2014 all new cancer drugs have been authorised for reimbursement in Germany, in contrast to most other European countries (Maynou-Pujolras and Cairns, 2015). In part, this lenient approach reflects the reluctance of legislators to allow regulators to use cost-effectiveness criteria as a decision-making tool. It also shows the influence of 'right to health' jurisprudence on regulatory practice, which has raised the threshold for exclusion. Treatment options that were taken to court were therefore either proven to be not effective (i.e. sufficient high quality studies exist that demonstrate that the treatment is not effective) or evidence was inconclusive or insufficient (i.e. there were not enough studies assessing effectiveness or existing studies were not sufficiently reliable or a combination of both).

The following sections review the international debate about the role of litigation in access to treatment decisions, introduce the legal framework relevant to making such decisions in Germany, and outline the regulatory approach to access to health care decisions. Then, the cases selected for this analysis are presented, followed by a discussion of the legal argumentation courts deployed when applying the criteria set out in the 'Nicolaus decision'. The paper concludes with a discussion of the implications of this approach for both evidence use as a regulatory approach and patients' right to health.

The role of courts in making decisions on access to health care

The involvement of courts in access to health care decisions is widely debated internationally (Flood and Gross, 2014, Kavanagh, 2016, Syrett, 2007, Wang, 2015, Moes et al., 2016). In low and middle income countries, a constitutional right to

health can provide a lever to improve access to health care and promote universal health coverage (Forman et al., 2016). However, given the more limited resources of these countries, the right to health is largely seen as an aspirational, political project rather than an opportunity for individual law enforcement only. A study of access to health care cases in Brazil highlights some undesirable consequences of having relatively unrestricted opportunities to invoke a constitutional 'right to health' to challenge decisions of regulatory bodies to exclude health services that are proven to be insufficiently effective or cost-effective (Wang, 2015). An excessive use of litigation can endanger the financial sustainability of public health care systems, especially those that already experience severe funding constraints (Wang, 2015, Kuchenbecker and Planczyk, 2012). In Colombia, excessive litigation based on 'right to health' legislation has threatened the financial sustainability of publicly funded health care, with almost 3 million cases documented in one decade (1999-2010) (Lamprea, 2014). Other concerns are that pharmaceutical manufacturers exploit the legal route to circumvent the regulator by encouraging patients to seek legal redress and that litigation disproportionately benefits wealthier patients (Afonso da Silva and Vargas Terrazas, 2011).

Others argue that courts are ill-equipped to adjudicate on access to care decisions, as they lack the expertise required to make such decisions "especially about the more technical matters involved in assessing efficacy and safety" (Daniels and Sabin, 2008: 59). Yet others argue that courts can have an important role in resolving health care related conflicts. Morales notes that despite their lack of scientific expertise, judges are trained to act as "intelligent, objective observers" and are typically committed to consider all evidence without prejudicing the outcome (Morales, 2015: 190). Syrett (2014) concludes that the argument that courts are not competent to deal with access to care cases is weak, but suggests that courts are not well placed to take allocative decisions. In part, this is the result of the fact that courts do not have responsibility for health care budgets and lack oversight of the resource impact of their decisions. More importantly, courts struggle to account for the 'polycentric' nature of allocative decisions that tend to affect members of populations (e.g. tax payers, members of sickness funds, all

current and future patients) unevenly; the impact of these decisions therefore differs structurally from cases that affect individuals only (Syrett, 2014). It is for this reason that decision-making requires democratic legitimation and should be taken by representatives of the affected body politic rather than by a small number of legal professionals.

However, political decisions reflecting voter interests can unduly eclipse the interests and rights of individuals that find themselves not represented by the majority. In Germany, there is sustained debate about the appropriate representation of patients in decision-making processes of the Federal Joint Committee (Gemeinsamer Bundesausschuss, GBA), the committee that determines health service coverage within the statutory health insurance system, which in the past only included representatives of doctors, hospitals and insurers (Gassner, 2016).

Another strand of the debate concerns the contested boundary between 'facts' and values that can be brought to bear on access to care decisions. It is now widely acknowledged that decisions on access to health care –discussed as prioritisation, rationing and rationalisation – speak to different concepts of justice and draw on different sets of social values (Rawlins, 2012), which cannot easily be reconciled, if at all. Should priority be given to the highest individual need or should we aim to maximise benefits more evenly across society (Cookson and Dolan, 2000)? As courts tend to focus on individual cases rather than collective problems of resource distribution, they are likely to prioritise individual need and in consequence are "liable to disrupt the inherently collective task of allocation of finite resources" (Syrett, 2010: 474).

Internationally, courts have handled access to care cases very differently. Syrett (2011) notes that courts in England tend to refrain from making substantive decisions on access to health care cases and focus on procedural justice only. This reflects a legal tradition in which legal interventions in political decisions are rare, as individuals have few opportunities to contest decisions taken by Government or its agencies through the legal system. In effect, courts have only questioned whether the National Institute for Health and Care Excellence (NICE) uses

appropriate procedures to arrive at its decisions and in the early years of the organisation demanded that NICE improved its appeals process to allow affected parties to challenge decisions.

In contrast, courts play a prominent role in German policy-making and citizens have a constitutional entitlement to take bodies of the public administration to court if they find that a decisions infringes upon their constitutional rights (Landfried, 1994). The idea of the 'Rechtsstaat' (the 'constitutional state', used as shorthand to signify the rule of law) is firmly rooted in legal and political systems, as well as the public psyche. Surveys have shown substantial support of Germans for the role of the courts within the state, with the judiciary being more trusted than Government and Parliament (Patzelt, 2005).

The legal framework regulating access to health care cases in Germany

Cases concerning access to health care are typically decided by social courts, of which there is a hierarchy of 68 district social courts, 14 state social courts and the federal social court at the apex. Access to social courts is free of charge for individuals in the first instance, meaning that the barriers to seeking legal redress are low. Cases in which patients take sickness funds to court are decided at district level initially. District court decisions can then be challenged before state social courts (*Landessozialgerichte*) and ultimately the federal social court (*Bundessozialgericht*).

Social courts adjudicate on access to health care based on a body of social law that is set out in Social Code Book V (SGB 5). SGB 5 spells out the responsibilities of sickness funds vis-à-vis their members (ca. 70 million, almost 80 percent of the population). In particular, it states that sickness funds have to fund treatment requested by a patient and authorised by a physician qualified to provide services within the statutory system, as long as these services are 'necessary', 'adequate', 'appropriate' and 'economical' (*notwendig, ausreichend, zweckmässig, wirtschaftlich*) (SGB 5, article 12). These legal terms are not well defined, but they broadly mean that the patient's medical need cannot be met in other ways

(necessary); that treatment has to be sufficient to meet this need (adequate); that is has to be effective (appropriate); and that it represents a good use of resources (economical or 'value for money', but not necessarily cost-effective).

SGB 5 also lists a few exclusions (e.g. over-the-counter drugs) and explicitly includes alternative medicine (*besondere Therapierichtungen*), provided such services are 'adequate' and 'appropriate' (i.e. there is some evidence of effectiveness). SGB 5 also sets out the expectations for quality and effectiveness (*Wirksamkeit*) noting that services should reflect a 'generally accepted state of medical knowledge' (*allgemein anerkannter Stand medizinischen Wissens*) and take account of 'medical advances' (*medizinischer Fortschritt*) (SGB 5, article 2). In combination, these legal stipulations provide the framework that defines the scope of services funded by sickness funds, resulting in access to treatment being judged as generous compared to other (wealthy) countries (Busse and Blümel, 2014).

Within this framework, SGB 5 mandates the GBA to specify the catalogue of services available to sickness fund members. To this end, the GBA uses health technology appraisals to inform decision on service inclusions or exclusions, guided by a detailed set of rules in its rules of procedure (GBA, 2008) and involving a range of formats to review the evidence of effectiveness. These reviews are typically conducted by the Institute for Quality and Efficiency in Health Care (*Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen*, IQWiG) on behalf of the GBA (with the exception of orphan drugs for the treatment of rare diseases that are undertaken by the GBA directly).

However, different rules apply to different health care sectors. In the hospital sector, all services are reimbursed by sickness funds unless the GBA has excluded them explicitly (Perleth et al., 2009). SGB 5 provides that hospital services can be excluded only if there is evidence that the treatment is ineffective or harmful; a lack of evidence of effectiveness is not sufficient. In the ambulatory sector, in contrast, all services require approval by the GBA to be available to sickness fund members. In ambulatory care, it is also possible to exclude treatments for which there is insufficient evidence, so the barrier for exclusion is somewhat lower than for hospital services. A third set of rules applies to pharmaceuticals. In principle,

sickness fund members have access to all pharmaceuticals that have received market authorisation, which for cancer drugs now rests with the European Medicines Agency (EMA). After one year, the GBA, through IQWiG, appraises the evidence of effectiveness of the drug compared to other treatment options, usually based on a dossier provided by the manufacturer. If the new drug is equally or less effective than existing treatment it will be priced according to the reference price established for existing products; if the drug is more effective, sickness funds will collectively negotiate the price with the manufacturer. Guidelines for 'off-label use' are provided by an expert committee hosted by the Ministry of Health (GBA, 2016). In addition, a decision by the Federal Social Court stipulates that drugs that are used 'off-label' have to be funded by sickness funds if there is no alternative treatment, they are used to treat a condition that is life-threatening or permanently reducing the patient's quality of life and if 'the available data give rise to the prospect that the compound concerned will be successful in treating [the patient] (curatively or palliatively)' (B 1 KR 37/00 R). In all sectors, health technology assessment currently focuses on evidence of effectiveness, with preference given to evidence from randomised controlled trials (RCTs) and other controlled designs if RCTs are not available. While cost implications for sickness funds are considered, cost-effectiveness analysis is not routinely undertaken as the GBA has not been given the legal mandate to conduct such analysis (in fact this option was removed in 2010), with the exception of informing arbitration procedures in cases in which manufacturers and sickness funds cannot agree on a price.

In cases of cancer treatment, courts frequently draw on the constitutional 'right to life and physical integrity', which in legal practice is interpreted to encompass a right to health and health care. In 2005, a year after the GBA was established, the Constitutional Court reaffirmed the relevance of Article 2 in a landmark decision on the access to treatment in cases of life-threatening illness. The 'Nikolaus decision' stipulates that sickness funds cannot withhold funding for treatment if a patient suffers from a life-threatening illness (*lebensbedrohliche oder regelmäßig tödliche Erkrankung*). Invoking this right requires that no alternative treatment is available that conforms to the 'current state of medical knowledge' and that the treatment

has a 'not entirely remote prospect of curing or noticeably improving the condition' (*eine nicht ganz entfernt liegende Aussicht auf Heilung oder eine spürbare Einwirkung auf den Krankheitsverlauf*) (1 BvR 347/98). This was later specified by the Federal Social Court to mean that 'the more serious an illness and the 'more hopeless' a situation is, the lower are the requirements of 'serious indications' of a not entirely remote prospect of improvement' (BSG B1 KR 7/05 R).

This application of the 'right to health' significantly lowers the threshold of evidence of effectiveness, resulting in tensions with the definition used in health technology assessments whose use is also anchored in legislation. However, the Constitutional Court explains that the use of the constitutional right to health is also linked to the principle of the welfare state (*Sozialstaatsprinzip*), also embedded in the Basic Law (Article 20). Here the Court argues that if the state forces its citizens to take out mandatory health insurance, it has a duty to protect them in cases of severe illness (1 BvR 347/98).

The German legal literature emphasises the inconsistencies within the legal framework resulting from the decision of the Constitutional Courts and the practical challenge of operationalising the decision both for the GBA and in court (Welti, 2007, Francke and Hart, 2006). This specifically applies to the difficulty of interpreting the phrase used by the court to describe the level of evidence necessary in support of a case (a 'not entirely remote prospect'). Nevertheless, this ruling has since informed a large number of social court decisions, and has led to amendments to SGB 5 and the GBA's rules of procedure to include new rules relating to the treatment of 'life threatening illness'.

Examples of legal challenges relating to access to treatment

Case studies for this analysis were selected to represent a spectrum of legal decisions on access to health care. Cancer was selected as a condition that is typically life-threatening and cases are therefore likely to reference the 'Nicolaus decision'. As statutory insurance provides coverage for all new cancer drugs that have received market authorisation, court cases selected cover treatments that had

either not been licensed for a condition (Avastin, used for treating glioblastoma, a type of brain cancer) or had not been approved by the GBA (induced hyperthermia, brachytherapy).

Court decisions were identified through an online database that brings together all court decisions that refer to the 'Nicolaus decision', developed by a research team at the Institute for Social and Health Law at the Ruhr-University-Bochum (www.nikolaus-beschluss.de). Search terms used were 'Avastin', 'Hyperthermie' and 'Brachytherapie' and decisions identified were taken between December 2005 and December 2015. Full texts of court decisions were available on the internet for all federal court decisions and almost all state social court decisions (except three). District court decisions tended not to be available on the internet, with only 7 out of 23 decisions available online. In total, full texts of 42 decisions were retrieved, of which two refer to decisions taken by the Federal Social Court, 27 by state social courts, and 7 by district social courts. As social courts operate hierarchically, all decisions at state or federal level had previously been considered by district courts. Four full-text decisions relate to Avastin, 23 to induced hyperthermia and 6 to brachytherapy.

[Table 1 about here]

Avastin

Avastin is the trade name of a cancer drug produced by the pharmaceutical manufacturer Roche. Its active ingredient is Bavicizumab, an angiogenesis inhibitor that slows the growth of new blood vessels. Avastin first received European market authorisation in February 2004 for use in metastatic colorectal cancer in combination with 5-fluorouracil-based therapy as second-line treatment. It has since received market authorisation for treatment in several types of cancer including renal cell cancer (2007), non-small cell lung cancer (2007), untreated central nervous system (brain) metastases (2009), metastatic breast cancer (2009),

advanced epithelial ovarian, fallopian tube, or primary peritoneal cancer (2011), adjuvant colon cell carcinoma (2012) and metastatic carcinoma of the cervix (2015). In addition, the EMA considered Avastin for glioblastoma in combination with two chemotherapies in 2010 and 2014, and both times did not grant market authorisation as studies suggest that the drug is not effective to treat this particular type of cancer (EMA, 2014).

Five court decision were identified in which a decision by a sickness fund not to fund Avastin for the treatment of cancer was challenged by patients, leading to four positive decisions (i.e. in favour of the patient) and one negative decision.

Induced hyperthermia

Induced hyperthermia is based on exposing parts of the body or the entire body to temperatures above the normal body temperature with the aim to accelerate, and improve the changes of, healing. Induced hyperthermia is used as a stand-alone treatment but is typically applied in combination with radiotherapy or chemotherapy. Two Cochraine Reviews (from the same team) concluded that the use of hyperthermia in combination with radiotherapy for the treatment of advanced rectal cancer and locally advanced cervix carcinoma had a measurable additional effect, but that the evidence is insufficient to draw firm conclusions (De Haas-Koch et al., 2009, Lutgens et al., 2010). The GBA decided in 2005 to excluded induced hyperthermia as a service in ambulatory settings noting that there is no valid evidence of effectiveness (GBA, 2005). However, the treatment is offered in some hospitals, including some university hospitals (e.g. University Hospital Munich (2017)). In addition, methods of application, temperatures and technologies vary which makes it difficult to compare outcomes as there is no set standard.

Forty-five court decisions were identified relating to induced hyperthermia, of which 11 led to positive and 23 to negative decision outcomes. One court decision was neither positive nor negative, but commented on a matter of procedure (1 BvR 2496/07). Most decisions related to advanced cancers (pancreas, breast, prostate,

colon, soft tissue of the pelvis, liver, ovaries, brain, urinary tract cancer, and cancer of unknown origin).

A large majority of decisions (n=24) dealt with induced hyperthermia for the treatment of cancers that courts considered life threatening. In 14 cases, courts ruled that alternative treatment was available under statutory insurance, leading to courts deciding against the wish of the patient for reimbursement.

Brachytherapy

Brachytherapy is a type of radiotherapy in which a source of radiation is placed inside or next to the area requiring treatment. For prostate cancer, small radioactive rods ('seeds') are implanted directly into the tumour and stay there permanently without the need for removal. The treatment requires approval by the GBA if provided in ambulatory settings. The GBA commissioned an evidence review from IQWiG in 2004 which concluded that there was insufficient evidence of effectiveness and the absence of harm to support reimbursement (IQWiG, 2007). In an unusual step at the time, the GBA decided in 2009 to commission a longitudinal study that compares different treatments for prostate cancer including Brachytherapy with permanent seeds (PREFERE) (GBA, 2009). The GBA has therefore postponed its decision as to whether to include brachytherapy in ambulatory care to 2030 (GBA, 2015).

All cases identified in this study resulted in negative decisions (n=13) with courts concluding that prostate cancer, if non-metastatic and at an early stage, was not life threatening and several treatment alternatives were available to sickness fund patients (e.g. surgery, chemotherapy). Courts did therefore not consider the question of effectiveness, as the first two criteria were not fulfilled.

Analysis of court decisions

Life threatening illness

To establish whether a condition was 'life threatening', courts typically relied on the diagnoses provided by treating physicians and the assessment of the Medical Service of the Sickness Funds (*Medizinischer Dienst der Krankenkassen*, MDK). The MDK is a regionally organised service that advises sickness funds on the appropriateness of prescribed treatment, by drawing on relevant scientific studies.

Courts did not classify all cases as 'life-threatening' despite the fact that they all constituted diagnoses of cancer. More specifically, courts distinguished between stages of cancer progression, with advanced stages of cancer, e.g. recurrent or metastatic tumours or tumours that were classified as highly malignant, typically seen as life-threatening. Earlier stages of cancer, in contrast, were often not classified as 'life threatening, although this depended on the type of cancer (e.g. L 5 KR 343/13; S 13 KR 383/13; L 2 KR 189/14).

Cases in which Avastin or hyperthermia were requested included a high number of diagnoses of advanced cancer, leading to some of these cases being decided positively (provided the two other criteria were met), while cases in which funding for brachytherapy was requested mostly involved early stage, non-metastatic prostate cancer that were not regarded as life-threatening. One case was rejected because the claimant had not provided the information required to establish her state of illness (L 5 KR 2013/15 B ER).

[Table 2 about here]

Absence of alternatives

Courts also considered the existence of alternatives available to patients to the treatments requested and they mostly relied on MDK assessments to underpin their decisions. Court decisions show substantial variation in this respect, with courts applying different definitions of 'alternative'. In one case, a patient who requested Avastin had already received other types of treatment which had not been effective and was therefore not classified as an available alternative (L 5 KR

343/13). In another case, the court followed the argument of a claimant that a potential alternative treatment was not suitable because of a high risk of severe side effects (S 13 KR 383/13). Other courts reasoned in similar ways that the risk of severe side effects precluded possible alternatives (e.g. S 9 KR 29/15 ER), while others rejected this argument (e.g. L 5 KR 4/11).

In most cases in which hyperthermia was requested, courts considered alternatives as available (n=14). In a few cases in which courts came to a positive decision, alternatives were considered absent either because other treatment options had been exhausted (e.g. L 1 KR 21/13; S 8 7849/09) or because standard treatment was seen as inappropriate due to potential side-effects (S 9 KR 29/15 ER; S 1 KR 410/13; L 4 KR 206/11 B ER). In one case, a court argued that a potential additional benefit of hyperthermia compared to standard treatment (i.e. chemotherapy) only 'cannot be ruled out'. This was sufficient for the court to justify an injunction in favour of the patient (L 11 KR 473/12 B ER).

All brachytherapy cases were rejected by courts. In all cases, courts argued that suitable alternative treatment was available as standard and funded by sickness funds (e.g. surgery, chemotherapy). Courts reasoned that claimants desired funding for brachytherapy primarily because they considered the treatment as less invasive (e.g. as prostatectomy). In two cases, courts decided that established alternatives were acceptable in spite of their risk of side effects (L 6 KR 456/06, B 1KR 12/05 R). One court referred to the existence of clinical guidelines as evidence of alternative treatment options (L 1 KR 132/12).

Prospect of improvement

Courts considered various types of evidence to decide whether the treatments requested had a sufficiently strong prospect of improving claimants' conditions to qualify for reimbursement. In line with the Constitutional Court's 'Nicolaus decision', courts often accepted 'indications' of 'potential' effectiveness that were considerably weaker than those based on EBM established in regulatory practice (e.g. GBA, EMA).

The courts approved requests for Avastin for individuals with advanced cancer, even though the drug had not received market authorisation for these types of cancer. In a twist of legal reasoning, courts decided that some cases did not qualify for 'off-label use', as they lacked sufficient evidence to support the evidence of effectiveness criterion, but still resulted in positive decisions when the criteria of the 'Nicolaus decision' were taken into account (L 5 KR 343/13).

Courts argued that results from 'exploratory trials' (i.e. phase II trials instead of phase III trials) were sufficient so demonstrate that treatment was 'promising'. A court considering Avastin for the treatment of anaplastic astrocytoma also came to a positive verdict arguing that 'promising results' of a phase II trial in combination with the recommendation from the treating clinical team provided a sufficiently convincing prospect of improvement (S 13 KR 383/13).

In a similar vein, courts that came to a positive verdict on funding hyperthermia typically applied a low threshold of evidentiary support. In two cases, courts argued that a previously positive experience of the claimant proved that the treatment was effective in the patient (S 9 KR 29/15 ER; L 1 KR 21/13). Some courts also regarded the existence of phase II trials as sufficient to conclude that an additional benefit 'cannot be ruled out' (L 11 KR 473/12 B ER). This line of reasoning was taken by several courts (L 1 B 506/08 KR ER; S 12 KR 413/07 ER). In these cases, courts argued that the more severe the condition the fewer 'indications' of treatment effectiveness were required, following an earlier decision by the Federal Social Court (B 1 KR 4/13 R).

In contrast, in other cases, courts argued that there was insufficient evidence to suggest a positive effect (L 16 KR 677/15 B ER; L 5 KR 2013/15 B ER). In some of these cases, courts noted that the treatment could only be used as a palliative option (e.g. hyperthermia) as there was no evidence to suggest that the treatment would improve the patient's condition, but there were other alternatives available for this purpose (L 5 KR 4/11). Some courts noted that recommendations from the treating physician or other experts were sufficient to establish evidence of effectiveness (L 4 KR 206/11). Yet another court argued that effectiveness could

not be based on expert opinion alone if there were no study demonstrating effectiveness (L 5 KR 2013/15 B ER).

For brachytherapy, treatment effectiveness was typically not discussed in detail as courts determined that the condition was not life-threatening and alternative treatment options were available. However, several courts acknowledged that the GBA had not approved brachytherapy for reimbursement because of a lack of evidence of effectiveness.

Discussion and conclusion

This analysis shows that there are substantial tensions between the two sets of norms derived from EBM that underpins health technology assessment and the constitutional right to health as it is applied in Germany. While the right to health does not allow for a blanket approach that grants access to all treatments requested, as was the case in Colombia and Brazil (Lamprea, 2014, Wang, 2013), the 'Nikolaus decision' of the Constitutional Court has significantly reduced the threshold of evidence required for the reimbursement of treatment in cases of life-threatening illness.

The first criterion explicitly applies the right to health, which is derived from a constitutional right to life and physical integrity, by giving priority to individuals with life-threatening illness over considerations of HTA-based treatment decisions. This decision mirrors discussions about end of life treatment in other countries. It also resonates with the argument made in the literature that courts consider impacts on individuals rather than impacts on society. In the extreme, it implies that any costs are acceptable to extend a single life, although the same money could be used to benefit other groups, for example by investing in prevention or the treatment of chronic illness that helps avoid life-threatening illness in the first place (e.g. diabetes) (Syrett, 2014). However, given the relatively small number of cases (compared to those in some Latin-American countries) one could argue that the

accumulated cost implications do not threaten the sustainability of the German system of statutory health insurance in the same way (especially as it currently runs a substantial surplus). Still, this finding is a reminder of the unsolved problem of defining and operationalising '*Wirtschaftlichkeit*' as a criterion for treatment decisions. As has been discussed elsewhere (Cookson and Dolan, 2000), giving priority to groups of patients always comes at a cost as it puts those at a disadvantage who are not considered a priority and who in the case of cancer diagnosis may only be a step away from having a life-threatening illness (e.g. not having a diagnosis confirming metastasis).

The second criterion, the absence of alternatives, requires courts to define what is considered an acceptable alternative. This analysis suggests that situations in which patients have received a range of treatments that did not have the desired effect and in which alternatives were available but were considered inacceptable because of the risk of side effects were classified as 'without alternative'. This points to the problem of having to determine when curative efforts should be stopped and replaced by palliative care and whether these decisions should be taken by the patient, the medical professionals involved, the regulator or by courts. In many cases analysed here the patient had died before the court took its decision, despite having received the treatment (as most cases dealt with reimbursement not access per se) serving as a potent reminder that having additional options does not necessarily improve the odds of survival.

The third criterion, arguably, is the most problematic. The Constitutional Court stipulated that there should be a 'not entirely remote prospect of improvement', which significantly softens the requirement of evidence of effectiveness applied in regulatory practice. In some cases, the application of this interpretation is in direct conflict with EBM. For example, some courts argue that an observation suggesting that a patient has responded well to treatment proves that the treatment was effective; however, scientifically, causality cannot be derived from a single observation (i.e. the observed effect could be caused by something else, it could be a measurement error or resulting from placebo effect).

In other cases, courts saw the existence of 'promising' phase II trial results as sufficient to indicate that the treatment could be beneficial, despite the fact that the drug had not received market authorisation for the condition in question. This is problematic as market authorisation considers evidence of effectiveness as well as of harm, with the latter not being considered by the courts. In other cases, courts relied on medical professionals to assess whether the treatment was appropriate, ignoring the possibility of supplier-induced demand that only seemingly aligns the interests of providers with the interests of patients. Importantly, courts seem to be unable to distinguish between treatment for which there is no evidence of effectiveness and treatment that is proven to be ineffective (e.g. Avastin for glioblastoma; induced hyperthermia for the treatment of tumours). While these are difficult decisions also for regulatory agencies, both those involved in market authorisation and in making decisions about public reimbursement, courts have to rely on a rather blunt set of tools to inform these decisions.

In addition, courts rely heavily on the MDK, to underpin their decisions as to whether a condition is life-threatening and whether treatment alternatives are available. However, they are less likely to follow MDK recommendations with regard to treatment effectiveness. This dependence on medical expertise raises the question of competence of courts in making these decisions and while it can be argued that the MDK represents the interests of sickness funds it is not clear why in some cases priority is given to providers (i.e. treating physicians). This is not to argue that courts are not competent to apply the law to these cases - they are - but there is a question as to whether they are best placed to make decisions about the benefits and harms of treatment given the difficulty of applying the criteria that qualify the application of the 'right to health' in German constitutional law. While this interpretation of the 'right to health' may result in fresh hope to patients and their relatives who are understandably desperate in the face of terminal illness, this hope will be ill founded if the treatment is not effective.

It can also be argued that, if society can afford it, it is only humane to allow for additional treatment, even if the odds for improvement are slim. However, at societal level, this comes with a side effect as this rationale, unwittingly or not,

plays into an existing narrative that suggests that exclusions from statutory health insurance are unjust and against the interest of patients. It can also strengthen the perception that privately insured patients have an advantage over sickness funds members with regard to access to treatment. The trade-off to having a strong constitutional right to health is here that it contributes nothing to helping patients and the public understand the importance of the concept of evidence of effectiveness, and indeed fosters a belief that treatment effectiveness is a random occurrence entirely specific to the individual. Of course it is not the responsibility of the courts to educate the public in scientific methods, but there is arguably a cost to judicial intervention in treatment decisions that lies beyond the individual cost of false hope or the monetary costs to society.

References

AFONSO DA SILVA, V. & VARGAS TERRAZAS, F. 2011. Claiming the right to health in Brazilian courts: The exclusin of the already excluded? *Law and Social Inquiry,* 36**,** 825-853.

AGGARWAL, A., FOJO, T., CHAMBERLAIN, C., DAVIES, C. & SULLIVAN, R. 2017. Do patient access schemes for high-cost cancer drugs deliver value to society? Lessons from the NHS Cancer Drug Fund. *Annales of Oncology,* https://doi.org/10.1093/annonc/mdx110.

BANTA, D. 2003. The development of health technology assessment. *Health Policy,* 63**,** 121-132.

BOHMEIER, A. & PENNER, A. 2009. Die Umsetzung des Nikolaus-Beschlusses durch die Sozialgerichtsbarkeit: Fortentwicklung und Widersprueche zu den Vorgaben des BVerfG. *Wege zur Sozialversicherung,* 3**,** 65-77.

BUSSE, R. & BLÜMEL, M. 2014. Germany: health system review. *Health Systems in Transition,* 10**,** 1-296.

COOKSON, R. & DOLAN, P. 2000. Principles of justice in health care rationing. *Journal of Medical Ethics,* 26**,** 323-329.

DE HAAS-KOCH, D. F. M., BUIJSEN, J., PIJLS-JOHANNESMA, M., LUTGENS, L., LAMMERING, G., MASTRIGT, G. A. P., D.K.M., D. R., LAMBIN, P. & VAN DER ZEE, J. 2009. Concomitant hyperthermia and radiation therapy for treating locally advanced rectal cancer. *Cochrane Database of Systematic Reviews,* 3.

EMA 2014. Refusal of a change to the marketing authorisation for Avastin (bevaczumab). 26 September 2014. London: European Medicines Agency.

FLOOD, C. M. & GROSS, A. 2014. Litigating the right to health: What can we learn from a comparative law and health care systems approach. *Health and Human Rights Journal,* 16**,** 62-72.

FORMAN, L., BEIERSMANN, C., BROLAND, C. E., MCKEE, M., HAMMONDS, R. & OOMS, G. 2016. What do core obligations under the right to health bring to universal health coverage? *Health and Human Rights Journal,* 18**,** 23-34.

FRANCKE, R. & HART, D. 2006. Die Leistungspflicht der gesetzlichen Krankenversicherung für Heilversuche. *Medizinrecht,* 3.

GASSNER, U. 2016. Götterdämmerung des Gemeinsamen Bundesausschusses. *Neue Zeitschrift fuer Sozialrecht,* 4**,** 121-127.

GBA 2005. Beschlussbegründung zur Änderung der Anlage B „Nicht anerkannte Untersuchungs- und Behandlungsmethoden“ der BUB-Richtlinie vom 18. Januar

2005 Bewertung der Hyperthermie (u.a. Ganzkörperhyperthermie, Regionale Tiefenhyperthermie, Oberflächenhyperthermie, Hyperthermie in Kombination mit Radiatio und/oder Chemotherapie) Siegburg: Gemeinsamer Bundesausschuss.

GBA 2008. Verfahrensordnung des Gemeinsamen Bundesausschusses (in its version of January 2014). Berlin: Gemeinsamer Bundesausschuss.

GBA 2009. Beratungen zur Brachytherapie ausgesetzt - Studie soll Klarheit über Evidenz der Methode bringen, press release, 17 December 2009. Siegburg/Bonn: Gemeinsamer Bundesausschuss.

GBA 2015. Gemeinsamer Bundesausschuss unterstreicht Bedeutung der PREFERE-Studie. Press release, 16 July 2015. Berlin: Gemeinsamer Bundesausschuss.

GBA 2016. Off-Label-Use. Hintergrundinformationen. https://www.g-ba.de/institution/themenschwerpunkte/arzneimittel/off-label-use/hintergrund, accessed 22 July 2016.

IQWIG 2007. Interstitielle Brachytherapie beim lokal begrenzten Prostatakarzinom. Abschlussbericht. Cologne: Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen.

KAVANAGH, M. M. 2016. The right to health: institutional effects of constitutional provisions on health outcomes. *Studies Comparative International Development,* 51**,** 328-364.

KUCHENBECKER, R. & PLANCZYK, C. A. 2012. Institutionalizing health technology assessment in Brazil: challenges ahead. *Value in Health Regional Issues,* 1**,** 257-261.

LAMPREA, E. 2014. Colombia's right-to-health litigation in a context of. *In:* FLOOD, C. M. & GROSS, A. (eds.) *The right to health at the public/private divide: A global comparative study.* Cambridge: Cambridge University Press.

LANDFRIED, C. 1994. The judicialization of politics in Germany. *International Political Science Review,* 15**,** 113-124.

LUTGENS, L., VAN DER ZEE, J., PIJLS-JOHANNESMA, M., DE HAAS-KOCH, D. F. M., BUIJSEN, J., VAN MASTRIGT, G. A. P. G., LAMMERING, G., DE RUYSSCHER, D. K. M. & P, L. 2010. Combined use of hyperthermia and radiation therapy for treating locally advanced cervix carcinoma. *Cochrane Database of Systematic Reviews,* 3.

MAYNOU-PUJOLRAS, L. & CAIRNS, J. 2015. Why do some countries approve a cancer drug and others don't? *Journal of Cancer Policy,* 4**,** 21-25.

MOES, F., HOUWAART, E., DELNOIJ, D. & HORSTMAN, K. 2016. Contested evidence: a Dutch reimbursement decision taken to court. *Health Economics, Policy and Law,* doi:10.1017/S1744133116000281.

MUNICH, U. H. 2017. Klinische Anwendungen der Hyperthermie. http://www.klinikum.uni-muenchen.de/Medizinische-Klinik-und-Poliklinik-III/de/klinik/hyperthermie-start/2_klinische_anwendungen/index.html, accessed 23 May 2017.

PERLETH, M., GIBIS, B. & GOEHLEN, B. 2009. A short history of health technology assessment in Germany. *International Journal of Technology Assessment in Health Care,* 25 (Suppl 1)**,** 112-119.

RAWLINS, M. D. 2012. Crossing the fourth hurdle. *British Journal of Clinical Pharmacology,* 73**,** 855-860.

SYRETT, K. 2003. A technocratic fix to the 'legitimacy problem'? The Blair Government and health care rationing in the United Kingdom. *Journal of Health Politics, Policy and Law,* 28**,** 715-746.

SYRETT, K. 2007. *Law, legitimacy and the rationing of health care. A contextual and comparative perspective,* Cambridge, Cambridge University Press.

SYRETT, K. 2011. Health technology appraisal and the courts: accountability for reasonableness and the judicial model of procedural justice. *Health Economics, Policy and Law,* 6**,** 469-488.

SYRETT, K. 2014. Courts, expertise and resource allocation: Is there a judicial 'legitimacy problem'? *Public Health Ethics,* 7**,** 112-122.

TIMMINS, N., RAWLINS, M. D. & APPLEBY, J. 2016. A terrible beauty. A short history of NICE. The National Institute for Health and Care Excellence. Nonthaburi (Thailand): Health Intervention and Technology Assessment.

VELASCO GARRIDO, M., KRISTENSEN, F. B., NIELSEN, C. P. & BUSSE, R. 2008. *Health technology assessment and health policy-making in Europe. Current status, challenges and potential,* Berlin, World Health Organization, on behalf of the European Observatory on Health Systems and Policies.

WANG, D. W. 2015. Right to health litigation in Brazil: The problem and the institutional responses. *Human Rights Law Review,* 15**,** 617-641.

WANG, D. W. L. 2013. Courts and health care rationing: the case of the Brazilien Federal Supreme Court. *Health Economics, Policy and Law,* 8**,** 75-93.

WELTI, F. 2007. Sozialrecht und evidenzbasierte Gesundheitsversorgung in Deutschland. *Zeitschrift für ärztliche Fortbildung und Qualität im Gesundheitswesen,* 101**,** 447-454.

Table 1

Outcomes of legal decisions per type of treatment for cancer

	Positive decision	Negative decision	Total number of cases
Avastin	4	1	5
Hyperthermia	11	23	35*
Brachytherapy	0	12	12

* One case involved a decision on a matter of principle and did not result in a positive or negative decision

Table 2

Number of legal decisions that saw 'Nicolaus decision' criteria fulfilled, by type of treatment for cancer*

	Life threatening condition	Absence of alternative treatment	Sufficient prospect of improvement	Total number of positive decisions
Avastin	5	4	4	4
Hyperthermia	24	12	11	11
Brachytherapy	1	0	0 (or not considered)	0

* Numbers only include decisions for whom the full text could be retrieved, excluding a number of decisions from district social courts that were not available for analysis of the exact reasoning.