Summer School

Health benefit package according to a human rights-based approach. Standards of care at European case-law

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Outline

- Background and characteristics SHI & Rationing
- Coverage disputes, rationing and the judiciary: national and European experiences
- Rationing, healthcare access and recent developments
- Conclusions
- Case studies

1. Background and key characteristics

Social health insurance (Bismarckian model): a ‘way of life’

Health care access as a legal right: (inter)national law

Catalogue of entitlements: HILaw, health professions and health insurance plans

Institutionalised coverage decision-making

Individual choice

Provided by contracted providers

Members only: nationals and legal status decisive

Controlling health care costs

Health care rationing in SHI systems

Largely implicit

Human Rights: health care access and informed consent

Notion of progressive realization; non-retrogressive measures

Accountability: ad hoc public debate and role of judiciary

2. Coverage disputes, rationing and the courts

- Health care as a human right
- Judicial review: procedure & substance
- Criteria:
  - Statutory entitlements
  - Constitutional rights, international HRL
  - Cost-effectiveness, and
  - Transparency & rationality
- Outcomes: diverse picture

2 (i). Challenging Rationing Decisions: Germany

- New technologies and limited cost-effectiveness:

  - Nikolausbeschluss (BVG 6 Dec 2005) German CC: "*statutory criteria for limiting health benefits should be interpreted in line with constitutional values*.."
    - lifesaving (experimental) medicine
    - "*spürbare positive Einwerkung*" (alternative effectiveness)

  - Elaborated by Federal Social Crt (BSG) 2006

  - Narrowed in IVIG therapy: life-threatening, critical situation Off-label use BVG 11 April 2017

2 (ii). Challenging Rationing Decisions: Dutch Supreme Crt

ECLI:NL:HR:2014:3679): Claim for reimbursement non-listed medicine Bosentan

- HIA: “necessary health care”
- Bosentan: not evidence-based (Off-label use Children)
- Exceptional circumstances:
  - price
  - alternative treatment absent
  - life threatening/serious disease;
  - included ‘positive list’ *seems likely*
- Reimbursement under “reasonableness criterion”
- Fear of Judicial Activism unfounded

2(iii). Challenging Rationing Decisions: Switzerland

- *Myozyme* case, Sw. Supreme Crt. 23 Nov 2010:
  - Cost-effectiveness threshold 100.000 CHF QALY
  - “limited cost-effectiveness”
- Court acting as substitute legislator?

2 (iv). ECtHR and Healthcare Rationing

- *Sentges v. the Netherlands,* July 8, 2003, (27677/02): “fair balance” test (Art.8)
- *Nitecki v. Poland*, March 21, 2002, (65653/01), para 1,….

  “it cannot be excluded that the acts of omissions of the authorities in the field of healthcare policy may in certain circumstances engage their responsibility under Article 2.”
- *Hristozov v Bulgaria,* (*47039/11): Does the Convention include a ‘Right to Try medicines’? (Art. 2)*
- *Panaitescu v Romania,* (*30909/06): violation comply court order (Art. 2)*
- *Wiater v Poland*, (42290/08) par 39 “The allocation of public funds in the area of health care, …, is not a matter on which the Court should take a stand. It is for the Member States to consider and decide how their limited resources should be allocated.”

2 (v). CJEU ‘rationing’ cases and Cross-border care

The responsibilities of the Member States shall include the management of health services and medical care and the allocation of the resources assigned to them (Art 168(7)TFEU)

PA principle: Barrier to free movement?

- *From: Decker & Kohll* (C-120/95, 158/96) - *Smits/Peerbooms* (C-157/99, C-385/99); *Watts* (C-372/04) (‘rationing by delay’)
- *To Elchinov (C-173/09) (lack of effective treatment) - Petru* (Case C-268/13): lack of medicines and medical supplies)
- And to Directive on cross border care (Dir 2011/24/EU) (with new inequalities)

3. Rationing, healthcare access & recent developments

- Rationalise decision-making: Health Technology Assessment (HTA)
- EUnetHTA: Cooperation on HTA (art. 15 Directive 2011/24/EU):
  - voluntary HTA network facilitating exchange info
  - standardised/ joint HTAs
  - no harmonisation of HTA conclusions
- Proposal Regulation HTA (COM(2018) 51 final)
- But do not forget human rights
- Statutory HTA requirement (cost-effectiveness) in SHI Act?

4. Conclusions

- Rationing unavoidable and necessary
- Rationing litigation: Need for public debate on fair rationing: democratic deliberation (L. Fleck) (plea for explicit rationing)
- Incorporating HTA, incl. HR impact analysis, in rationing debate
- Role of the courts: triggering that debate and holding health rights justiciable

Conference annoucement

Health care rationing in Europe: the past, present and future. A multidisciplinary approach

- Venue: National School of Public Health (ENSP), Universidade Nova de Lisboa, Salao Nobre, Lisbon, Portugal
- Date: 26 October 2018, 9.00 – 17.00
- Language: English
- Contact: denexter@law.eur.nl

- Info: https://www.eur.nl/eshpm/onderwijs/postacademisch/cursussen/erasmus-observatory-health-law/conference-health-care-rationing

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Case studies

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Case 1. Orkambi: a new medicine for cystic fibrosis

Vertex has struck a market access deal in Italy for its cystic fibrosis treatment Orkambi

- Orkambi treats cystic fibrosis and is the first medicine to treat the underlying cause of the genetic disease.
- The firm did not provide further details of the deal, but said it already concluded several pricing and reimbursement agreements in Germany, Austria, Denmark, Ireland and Luxembourg. However, there are a number of countries with which Vertex has not been able to come to an agreement, among them France and England, whose cost-effectiveness watchdog rejected the drug last year.
- Publishing its final appraisal determination NICE said the £104,000 per patient price tag was "too high" for the drug's "modest" short term benefit, but Vertex is still working on coming to an agreement.

Q.: what would you recommend Dutch CT patients in need of Orkambi?

Case 2. Jehovah's Witness with PNH – request for treatment with Eculizumab

Case Summary

- 40 year old male patient with severe PNH, a rare, potentially life-threatening haematological disease
- Conventional treatment is blood transfusion
- Very expensive drug (Eculizumab) available, not normally covered (2011)
- Patient requests treatment with Eculizumab as an exceptional case because he is a Jehovah's Witness and refuses blood transfusions.

Source: Heale 2018

Background info PNH

- Prevalence: 5 per million. 200 people affected in X, 100 severely affected.
- Average age of diagnosis: 35-40
- Median survival after diagnosis: without Eculizumab 10-15 years (1/3 do not survive more than 5 years); with Eculizumab normal life expectancy
- Blood clots in veins are leading cause of death. Blood clots can potentially cause life-threatening complications by cutting off blood flow to vital organs: liver and brain most commonly affected.

Treatment Options (patients severely affected)

- When there is an acute paroxysm, the clinical situation may be life-threatening (similar to acute haemorrhage): in such cases there is an absolute indication for blood transfusion. Blood transfusions may also be given at other times to alleviate symptoms.
- The only curative treatment is stem cell transplant, but this is associated with high rates of mortality for patients with PNH.
- Eculizumab is the only approved medicine for the treatment of PNH. Paroxysms of haemolysis are rare in people taking eculizumab. It is highly effective in reducing, or in many cases eliminating, the need for blood transfusions. Eculizumab also greatly reduces the risk of blood clots and in most cases greatly improves quality of life.
- Generally safe and well-tolerated.

Request for coverage

- Eculizumab is very expensive (€240,000 per year) and has to be taken year-on year to maintain its effect
- At the time of this case, Eculizumab was not covered by SHI
- The patient is a JW and refuses blood transfusions. He cannot accept a transfusion even in a case of ‘dire necessity’. This affects his quality of life and severity of symptoms and his risks of serious and life-threatening complications
- The patient requested reimbursement because…(i) he was so severely affected, and (ii) as a JW it would be particularly advantageous for him as he could not accept blood transfusions and so his symptoms were significantly worse and risks were significantly higher than a comparable PNH patient who could accept blood transfusions.

Questions

- Should a medicine with a cost/QALY far outside the normal limits ever be approved?
- Should we accept that some conditions should not be treated where treatment is available only at very high cost?
- What would be the likely outcome of such a case when submitted at national court or at the ECtHR (Strasbourg)?