

Resources, rarity, and rationing

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A patient with late-onset Pompe disease began taking enzyme replacement therapy, Alglucosidase alfa, which at the time had not yet been approved in Switzerland for sale/use. The patient's doctors sought and received approval for off-label use, and the patient's insurer paid for the first six months. The patient had improvements in symptoms and function. After six months, during which time the drug was approved for sale in Switzerland, the expense turned out to be greater than expected, and the insurer denied a request for continued coverage. Previously improved symptoms worsened after the drug was discontinued. The patient appealed the decision and the Swiss Supreme Court ruled that coverage could be denied.

Although the Swiss health care system has been characterized as one that does not ration care [1], this case illustrates the economic reality: even in a country with a generous mandatory health insurance package, «you can't have it all». Trade-offs must be made in contexts of limited resources. How to allocate (or prioritize) limited resources fairly and openly is perhaps the most pressing moral and practical concern in the health care arena today. The need for interpersonal trade-offs and balancing individual with social or group needs requires courts and other decision makers to consider justice, both distributive (i.e. fair distribution of benefits and burdens) and procedural (i.e. fair processes for decision making). Procedural justice demands that a fair process be used to make rationing decisions, about which we say more below. The core question in this case concerns distributive justice, that is, how to balance this patient's claim to limited resources with other patients' current or future claims.

When considering competing needs for limited resources, decision makers should take care to consider all of the morally relevant features of a case. Two categories of considerations stand out – the extent to which the patient might benefit from the resources, and the resources required. The benefit expected for the patient includes both the magnitude of a predicted change in health, and the duration of that change. Sometimes the benefit is uncertain, and while the certainty or uncertainty of benefit can be a morally relevant consideration, particularly at the extreme of uncertainty (e.g. «experimental» interventions), judgments about benefit to *individual* patients often rely on incomplete and imperfect evidence. In addition, of great relevance for this case, considering the severity

of the underlying illness when judging competing claims for resources can be justified as helping those who are worse off.

The resources required for an intervention, and how those resources could benefit others (in economic terms, the opportunity cost) are key moral considerations. Besides considering how much benefit might be achieved for others, the effect on the distribution of health services and health outcomes is a morally relevant consideration. That is, alleviating inequities in health and access to health care can sometimes justify using resources that might provide greater benefit overall, but contribute to or have no impact on inequity.

With these criteria in mind, we examine the court's judgment. The court based its decision, in part, on a distinction between the drug's impact on symptoms and function and its impact on life extension. The bulk of the court's reasoning, however, seems to have been based on legal equality – the idea that if this patient were to be granted coverage, individually insured persons in similar circumstances (albeit not having the same disease), facing similar benefits and costs, should also be granted coverage. That is, the court considered the potential benefit to the patient and the resources required in terms of foregone benefits to other patients. We first reject the criticism, doubtless heard by the court, that the cost of a recommended health care service should not matter. Trade-offs must be made among competing needs for limited resources available for health care. When the court cited the cost of providing similar benefits at similar costs to others, as a multiple of the current spending on health care and also as a proportion of GDP, it implicitly appealed to the reality of limited resources, resources constrained because there are other valued goods competing for individual, cantonal, and federal resources.

Symptom relief vs. life extension

Treatment of adults with Pompe disease seems to improve symptoms and function a modest amount for an uncertain duration, according to published evidence, including some case reports, observational studies and one randomized placebo-controlled trial [2]. The evidence that the drug benefits patients could be deemed «promising», but not conclusive. The court

correctly noted that the degree of benefit is modest – stabilization or slight improvement in ability to walk and/or breathe, for instance. It is unclear how sustained such improvements may be; the court noted that the best-documented benefits had been noted in the first six months of treatment, which had been covered. Since this research is in its early stages, however, one could reasonably expect evidence to accumulate, even if at a slow pace given the rarity of the condition.

The court drew a distinction between life-lengthening treatment and symptom relief, stating: «A life-lengthening effect of the therapy is documented neither in a general manner nor in the specific case. The defendant can continue her life with the help of other measures (ventilation, crutches) – even if this is in a limited manner.» It would be more accurate to say that the effect on life extension of treating adult Pompe patients with enzyme replacement therapy is not yet known, because it has not yet been properly examined. There is some evidence that mortality can be affected by the functions treated by the drug [3]. Evidence for interventions for rare conditions, or unusual patients with more common conditions, is often less plentiful and rarely as high quality as «gold standard» randomized clinical trials. The rarity of the clinical question unavoidably prevents large-scale research. This also affects the cost of care, as we will discuss below.

We would not dispute the value of extending lives; one can justifiably prioritize spending that saves lives over spending that relieves symptoms. However, much of the medical care covered by the mandatory insurance in Switzerland, as in most industrialized countries, provides symptom relief and/or improves function. Besides recognizing the difference in value between extending life and relieving symptoms, finer distinctions can and should be drawn between coverage for severe versus minor symptoms. Should insurance pay for antihistamines and decongestants for allergic rhinitis (stuffy noses), or alpha-blockers for urinary obstructive symptoms in men, or treating ringworm, or surgery for urinary stress incontinence? The total cost to the health system of paying for any one of these more modest symptoms and functional impairments might well out-spend treating all Swiss citizens with Pompe disease with enzyme replacement therapy. Critiques of Oregon's initial ranked list of diagnosis treatment pairs, which prioritized treatment for thumb-sucking over appendicitis, illustrates the considered moral judgment that severity of illness is a morally relevant criterion for rationing. Drawing a distinction only between life-extending and symptom-relieving is too blunt; some priority should be granted to treatments that benefit those with more serious conditions.

Legal equality

The intervention in question is very expensive, between 500 000 and 600 000 francs per year. The court relied on arguments about legal equality, that is, that individually insured persons in similar circumstances should also be granted coverage for interventions that could provide some relief and improvement at great cost, because «there are many persons who do not suffer from Pompe disease, but from other diseases which cause similar limitations to quality of life (for example chronic obstructive pulmonary disease [COPD])». The court noted that paying for interventions for that large population to provide comparable benefit as that experienced by the patient would cost 1.6 times the entire cost of the health system.

The court's analogy has some flaws. First, because the population of those with COPD is large, the options for treatment are varied, have been studied extensively, and, since many of the treatments have been available for a long period of time, are often cheap. Patients with rare conditions, or with unusual examples of common conditions (e.g. men with breast cancer), or whose disease is difficult or expensive to study for other reasons, or with conditions for which treatment has only recently become possible, will tend to have more expensive treatment options. New drugs are more expensive in part because they are more complicated to discover and produce, and new drugs for diseases without alternative treatments can be more expensive because the market will bear it. Some allowance can and should be made for how disease rarity and a newly discovered treatment, particularly for a condition without existing treatment options, influences the «economicity» of a treatment. We do not suggest that the judgment would necessarily be different for this case if allowances were made for these features. Given the extreme expense of enzyme replacement therapy for adult onset Pompe disease, paying for treatment even for this rare condition could well result in loss of substantial health benefits to others who rely on pooled, shared resources.

As this analysis illustrates, setting priorities is messy, trading off not just apples and oranges, but also cheese, chocolate and wine. Given the complex, contentious and value-laden nature of the judgments, engaging the public in deliberations about spending priorities can contribute to better decisions and policy, and to legitimacy and justice [4, 5]. In a democratic society, decisions about how to use limited resources, about trade-offs between competing needs, should reflect the values and preferences of a population or community. Involving those affected by decisions in the decision-making process can incorporate those preferences and values.

Numerous challenges surround ensuring fair representation and inclusion of a community's disadvantaged sectors, but those challenges can and must be met. Through participation and deliberation, rationing can be self-imposed.

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Standpunkt

Rechtsgleichheit hat zwei Gesichter

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Für die an der seltenen Krankheit Morbus Pompe leidende Frau bedeutet der Bundesgerichtsentscheid, dass die sehr hohen Kosten für Myozyme[®], ein Medikament mit fraglicher Wirkung, von der Versicherung nicht vergütet werden, wahrscheinlich einen schweren Schlag.

Ihren Entscheid unterlegen die Bundesrichter mit drei Grundsätzen: 1. mit der Forderung nach wissenschaftlicher Evidenz (die Wirksamkeit des Medikaments ist nicht dokumentiert), 2. mit ökonomischer Verhältnismässigkeit (Therapiekosten von 750 000 bis 900 000 Franken pro 18 Monate übersteigen versicherungspflichtige Zahlungen für nicht fremdverursachte Krankheiten) und 3. mit Rechtsgleichheit (ähnliche Behinderungen und Einschränkungen der Lebensqualität wie bei Morbus Pompe kommen auch bei anderen, häufigeren Krankheiten vor).

Im Kontext hoher nationaler Krankheitskosten und endlicher Ressourcen ist der Bundesgerichtsentscheid aus politischer und ökonomischer Sicht zu begrüssen. Erstmals wird in der Schweiz ein wegweisendes Argumentarium für künftige Diskussionen über die soziale bzw. private Bezahlung sehr hoher Therapiekosten zur Verfügung gestellt. Wichtig scheint, dass Rechtsgleichheit auch im Fall von vergleichbaren Einschränkungen durch Krankheit gilt und dass limitierte Ressourcen –

soziale Versicherungsleistungen bzw. Zahlungen durch die öffentliche Hand – nach Grundsätzen der Verhältnismässigkeit zu verteilen sind.

Für Menschen, die an Orphan Diseases leiden, mag dieser Bundesgerichtsentscheid eine weitere Benachteiligung bedeuten gegenüber Menschen, deren Krankheiten häufig sind. Ihnen fehlen oft einflussreiche Interessenvertreter in der Öffentlichkeit und in der Wissenschaft (analog z.B. der Schweizerischen Krebsliga). Entsprechend sind die wissenschaftlichen Erkenntnisse zu Ätiologie, Krankheitsverlauf und Therapie von seltenen Krankheiten häufig begrenzt. Um diesem Missstand zu begegnen, kann im Gegenzug das bundesrichterliche Argument der Rechtsgleichheit zur Förderung der wissenschaftlichen Gleichberechtigung von Menschen, die an Orphan Diseases leiden, herangezogen werden. Zwingend müssten dann auch entsprechende Finanzen für die Erforschung dieser Krankheiten zur Verfügung gestellt werden.

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