

The Swiss Supreme Court's thoughtful decision regarding insurance of expensive medications for rare diseases

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The Swiss Supreme Court has rendered a decision that insurers should not be required to insure payment for a medication that would cost 900 000 francs per year for a patient with a rare and debilitating disease.

One might worry that the court is being very hard-hearted and calculating in making this decision. Perhaps they are being insensitive to the concerns of patients with rare diseases. One might worry that legal experts cannot appreciate the meaning of this decision for severely disabled persons with such diseases.

Are these worries well founded? The court's argument does not seem to ignore the plight of persons with disabling, rare diseases but rather points out that persons with more common diseases can be equally disabled and that spending the amount in question on a frequent basis, be it for persons with rare or common expensive and disabling diseases, would bankrupt insurers.

One might counter that the cost of the orphan drugs that patients with rare diseases require are always going to be extremely expensive because of the small market they command. Some have argued that as such, patients with rare diseases should be given some extra priority in rationing schemes that are based on cost effectiveness. Here I will review two opposing published views about the use of cost effectiveness as a criterion for coverage decisions and conclude with some additional points.

The view that costs of orphan drugs for rare diseases should be covered at the discretion of a clinician's judgement, is endorsed by Schlander and Beck in a review of the literature offered in defence of reimbursement for enzyme replacement therapy for one of the mucopolysaccharidoses, a rare and disabling disease in people with enzyme deficiency [1]. Without extra incentives, development of treatments for such rare diseases is prohibitively costly. Of course, as the authors acknowledge, this concern has been partially addressed through public legislation to stimulate the development and marketing of medication for rare diseases both in the United States and the European Union [2, 3]. The legislation provides incentives to research, reduces fees for approval applications, and gives market exclusivity to drug developers. In a five year period following passage of orphan drug legislation, from 2000 to 2005, 268 medications received orphan drug status in the EU and at the time of publica-

tion of their review, 44 had received marketing authorization. While recognizing the successful drug development that followed the EU legislation, Schlander and Beck go on to point out and criticize the variability of therapeutic evaluation, pricing, and reimbursement for medications for treatment of rare diseases in different countries. They argue that the low prevalence of rare diseases makes the clinical data for assessment of effectiveness very sparse, documentation of effectiveness difficult, and uncertainty in clinical decisions high. They go on to argue that despite the extra incentives for development, the cost of drugs for rare diseases remains high thus posing a disadvantage for orphan drugs when they are judged by cost-effectiveness criteria. Additional problems with reimbursement based on cost-effectiveness, they suggest, are the tendency to focus on demonstrable short term effects which may bias against treatments aimed at preventing disability before clinical symptoms have developed and against treatments of slowly progressive diseases like the mucopolysaccharidoses and other storage diseases. They then critique the utilitarian logic of cost effectiveness analysis as counter to the attitudes of much of the public, which prefers to give priority to the worst off and to those identifiable individuals who face avoidable death. They argue that these empirical observations correspond to a rights-based view, which aims to put the non-abandonment of those who are unfortunate enough to have a high cost illness at the center of attention. Instead of the utilitarian approach of cost-effectiveness analysis, they recommend attention to vertical equity – the unequal but equitable treatment of unequals – thus weighing health gains for different recipients differently [4, 5]. Apart from normative considerations, they believe that from an economic perspective, given the low prevalence, the budget impact of rare diseases is small despite the high cost per patient.

In contrast, health economists in the UK, McCabe, Claxton, and Tsuchiya, have offered arguments for adhering to cost effectiveness criteria in making coverage decisions for patients with rare diseases [6]. In their article, they provide arguments that address many of the concerns of Schlander and Beck. They do acknowledge that despite mechanisms for promoting the development of orphan drugs, few of these drugs go on to meet

the cost-effectiveness criteria required for funding by health care providers. However the appraisal process of orphan drugs does allow lower evidential standards for orphan drugs [7]. Moreover, the arguments regarding problems with measurement and evaluation of orphan drugs (outcomes with long time horizons, variable clinical manifestations of disease) are not unique to rare diseases. Claxton and colleagues ultimately argue that any of the features of rare diseases that may be brought to bear in arguing for their special treatment, are features that are not unique to rare diseases. They conclude that, the «idea that decisions should be made based on valuing health outcomes for no other reason than rarity of the condition seems unsustainable and incompatible with other equity principles and theories of justice».

I would argue that ultimately, the factor that undermines easy coverage of orphan drugs for rare diseases is high cost. As such, patients with rare diseases face the same difficulty or disadvantage as other patients with illnesses that are the most costly to other life extension and palliation when cost-effectiveness analysis is used as the basis for a priority setting scheme. But should this lead us to abandon cost-effectiveness as the basis for coverage decisions? I would argue not. Certainly, cost-effectiveness should not be used exclusively without any concern for equity. However, as long as persons with expensive diseases are included in the insurance pool, and they are provided with alternative care rather than being abandoned, they have the opportunity to be treated fairly.

In fact, medical insurance is a scheme that offers the redistribution of funds from those with the most affordable health care to those with the most expensive health care. If one examines the ratio of insurance paid to benefits received, patients with more costly illnesses (be they costly for whatever reason including rarity) are likely to be getting more benefit per unit currency than they have paid into the insurance fund.

If the Supreme Court had permitted reimbursement of orphan drugs without regard to a cost-effectiveness threshold, what would this have meant for Switzerland? Such a decision would have left little legal backing for Swiss insurers to manage the pool of financial resources for the populations they cover in a fiscally feasible manner, unless they had endorsed some other priority setting strategy. Given that there are approximately 6000 rare diseases [6] and a growing number of orphan drugs on the horizon [8], the lack of legal sanc-

tion for priority setting, would have posed the prospect that the Swiss public would pay higher insurance premiums to cover costs. At the same time, the Swiss pharmaceutical industry would have had greater financial incentives to develop and manufacture medications for rare diseases. With such an incentive to produce high cost drugs and a requirement to pay for them, the Swiss public would likely be subsidizing the availability of orphan drugs outside Switzerland (since they would be unaffordable at such prices elsewhere in the world and likely to be marketed at lower prices in other countries). The trade-off would have been at the expense of more affordable health care for Swiss citizens. Perhaps some observers would welcome such a scenario but it should not happen without the Swiss public's awareness of it.

Conflict of interest: The views expressed here are those of the author and not necessarily a reflection of the policies of the National Institutes of Health or the US Department of Health and Human Services.

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