HEALTHCARE & LIFE SCIENCES - SWEDEN

New pricing principles applied in decision on orphan drugs

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Introduction

In a 2014 judgment the Administrative Court of Appeal opened up a system whereby the Swedish reimbursement authority, the Dental and Pharmaceutical Benefits Agency (TLV), can set the accepted price for a medicinal product (for further details please see "Pricing uncertainty for orphan drugs and innovative new drugs"). On December 19 2016 the TLV used this new procedure for the first time and in a reassessment of the reimbursement status decided to decrease the price for Cerezyme and VPRIV – two products indicated against Gaucher's disease – by 66% and 67%, respectively. This caused the marketing authorisation holders behind the products to withdraw them from the reimbursement system. Consequently, since February 1 2017 there is no longer a product against Gaucher's disease that is nationally reimbursed in Sweden. It is unclear to what extent county councils are still treating existing (and possibly new) patients with these products and at what cost to the individual councils.

Facts

Cerezyme and VPRIV are orphan drugs indicated against Gaucher's disease, both of which have been covered by the Swedish reimbursement system. Gaucher's disease is considered to be an ultra-orphan disease, meaning that it is prevalent in only one case per 50,000 to 100,000 people. Sweden has around 50 Gaucher's disease patients at any given time. Untreated patients are expected to have a reduced lifespan of nine years (Type 1) or risk dying at a relatively young age (the expected median age is 12 years (Type 3)).

From 2011 to 2012 the TLV conducted a reassessment of the reimbursement of Cerezyme and decided to revoke the reimbursement status due to the cost per quality adjusted life year (QALY) for the product being more than Skr10 million(1) and thus well above the TLV's usual limit for retail pharmacy products. The decision was repealed by the Stockholm Administrative Court of Appeal in 2014. The court reasoned that since patients had been treated with Cerezyme in Sweden for a long time and that the revocation would mean that no product against Gaucher's disease would be reimbursed in Sweden, the effects of the decision were disproportional. Further, the court stated that if the TLV found Cerezyme not to be cost effective at the current price, it should instead, under the Pharmaceutical Benefits Act, decide on a price level at which it considers Cerezyme to be cost effective. However, this judgment has been criticised for going against established administrative procedure and the rule of law. The procedure has traditionally always required the applicant to prove cost effectiveness at its requested price of the medicinal product, making the decision for the TLV binary: proven or not proven cost effectiveness. Unfortunately, the judgment did not address whether the product was cost effective at its current price or if a cap existed, but instead opened up a new procedure within the TLV.

VPRIV

The TLV finalised another reassessment of Cerezyme (this time also including VPRIV) in December 2016. Supported by the reasoning of the Court of Appeal in 2014, on December 19 2016 the TLV decided to considerably decrease the price for both products. The marketing authorisation holders consequently decided to withdraw Cerezyme and VPRIV from the Swedish reimbursement system.

Decision

In its 2016 decision, the TLV stated that patients under treatment with Cerezyme would need resource-intensive healthcare measures if access to the product was denied and that the necessity of Cerezyme was therefore considerable. As the number of patients diagnosed with Gaucher's disease is limited and not expected to increase, the TLV argued that there is only insignificant uncertainty of the total cost for the treatment, estimated to be at most Skr50 million to Skr60 million for all patients per year, and thus would not supersede other more important healthcare. According to the TLV, the importance of the product and the foreseeability support the fact that the product should remain in the reimbursement system despite the high cost.

In the initial Cerezyme case of 2014, the TLV's interpretation at the time of the needs-solidarity principle of the ethical platform gave rise to an absolute cap for cost per QALY of around Skr1 million to Skr1.2 million for a product to be granted reimbursement, regardless of severity or chronic nature of the condition. However, in order to give patients that suffer from rare and severe diseases equal opportunity of achieving good health as patients that suffer from severe, common diseases, in its 2016 decision the TLV introduced an additional new consideration when deciding the maximum acceptable cost per QALY: how common or rare the medical condition is. This entails that higher costs for products against rare diseases can be accepted, even if the cost efficiency is lower when compared to more common diseases. In other words, the fewer patients under treatment, the higher the cost per QALY that can be accepted. According to the TLV, to apply this concept:

- patients must be few in number;
- the medical condition must be very severe;
- the treatment effect of the product must be very good; and
- there must be a lack of other clinically relevant treatment alternatives.

Cerezyme was deemed to fulfil these criteria.

The TLV's estimated cost per QALY after adjustments was Skr7.1 million compared to the cost per QALY according to the economic model of the company, which was Skr2 million for Type 3 patients and Skr2.9 million for Type 1 patients. The TLV stated that considering the merits of the case, it accepts a cost per QALY of up to Skr2 million. Compared to the previous cap, the cap has gone up significantly. However, the consequence for Cerezyme pricing was still a decrease of the Cerezyme list price by 67%. The TLV decision on VPRIV is identical, except for the costs per QALY generally being slightly lower.

On March 17 2017 the TLV decided not to grant reimbursement to the new product Cerdelga (another product indicated against Gaucher's disease (Type 1)), with reference to cost in relation to the healthcare benefits.(2) It used Cerezyme as a comparator to Cerdelga, and used the TLV economic model and thus the cost per QALY for Cerezyme calculated by the TLV itself and not the applicant company.

Comment

The TLV's decision may appear generous, accepting higher costs for the products in question and taking into account the rarity of Gaucher's disease. However, the decision actually means lowering the market authorisation holder's price by 66% and 67%, respectively. The court ruling from 2014, wherein the court suggested that the TLV could decide on list prices at its discretion (ie, not limited to a decision on whether the applicant's price was correct), was unsatisfactory due to the impact such prices would have on international reference pricing. It appeared that the result would be lengthy appeal procedures or active withdrawals from the reimbursement system. The latter outcome is now a fact, and the uncertainty that this causes to both patients and other innovative pharmaceutical companies is unfortunate. It remains to be seen what other consequences this new procedure will bring to the Swedish pharmaceutical and healthcare market if, for example, the county councils

continue to cover costs for treatment with Cerezyme and VPRIV within their regional budgets instead.

If a similar decision by the TLV is appealed to the Administrative Court by a marketing authorisation holder, it remains to be seen whether the court would object to the TLV's cost efficiency calculation or provide material guidance on what price levels should be accepted.

Sweden has initiated a government review on pricing and reimbursement of medicinal products and the public financing of such products (for further details please see "Sweden reviews financing and pricing").

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Endnotes

(1) Skr10 is approximately €1.

(2) See www.tlv.se/beslut/beslut-lakemedel/avslag-uteslutningar/Cerdelga-ingar-inte-i-hogkostnadsskyddet/.