

Practical measures for strengthening access to medicines for rare diseases

Summary of report from government assignment 2024¹

TLV suggests how to strengthen access to medicines for rare diseases

TLV has been tasked by the government to continue to analyse and develop measures to strengthen access to medicines for rare diseases within the current system, while ensuring that the government's costs for medicines do not increase. The assignment is a continuation of the one TLV completed in September 2023, where we proposed at a more general level how TLV can achieve the purpose of the commission².

Research advances in areas such as precision medicine have resulted in the development of medicines that can offer new and important treatment opportunities for patients with severe and rare diseases. In our previous government assignment, we showed that many medicines for rare diseases are available to patients under today's conditions, but that there is room for improvement. To address this, we proposed several measures, including that TLV should take patient numbers and sales volumes into account to a greater extent than previously in its decisions on pricing and reimbursement.

In this government assignment, we have deepened our analyses and continued developing our methods in order to be able to apply them in TLV's health economic evaluations and pricing and reimbursement decisions. It has been important that the methods and procedures we develop are applicable in practice and that the Swedish regions and pharmaceutical companies can contribute to implementing them. We have focused on answering the following four questions:

¹ TLV 2025, *Praktiska förutsättningar för stärkt tillgång till läkemedel för sällsynta hälsotillstånd* https://www.tlv.se/download/18.6eef4347193defb9de261bd1/1735568391077/slutrapport%20-praktiska_forutsattningar_for_starkt_tillgang_till_lakemedel_vid_sallsynta_halsotillstand_00663-2024.pdf

² TLV 2023, *Stärkt tillgång till läkemedel för sällsynta hälsotillstånd, till långsiktigt hållbara kostnader* <https://www.tlv.se/publikationer/publikationer/2023-10-02-starkt-tillgang-till-lakemedel-vid-sallsynta-halsotillstand---till-langsiktigt-hallbara-lakemedelskostnader.html?query=s%C3%A4llsynta> English summary of the report: https://www.tlv.se/download/18.56bb14ac18b37f17d083e082/1697793328049/strengthened_access_to_medicines_for_rare_diseases_2023-10-19.pdf

1. Under which conditions can TLV accept a higher cost in relation to benefit?

We have clarified the conditions that must apply for TLV to be able to accept a higher cost in relation to benefit for medicines for rare diseases. We have developed methods to assess whether the conditions are met.

- The health condition treated by the medicine must be very severe and result in a long-term loss of quality of life or many years of life lost.
- The medicine must lead to a clinically relevant benefit for the patient, compared to the treatment the patient would otherwise receive
- The medicine must be intended for a very rare health condition. TLV will use the number of patients in Sweden with the health condition to determine how much higher cost per health benefit TLV should accept. But TLV will also take into account how many patients with the condition are added each year.
- If there is great uncertainty in the health economic assessment, for instance due to uncertainty about the size of the health benefit, TLV will not accept an equally high cost-benefit ratio, as when uncertainty is lower.

2. How can TLV ensure that the cost of a medicine continues to be reasonable after TLV has accepted a higher cost in relation to benefit?

TLV needs to be able to handle situations when more patients may need a medicine for a rare disease than was expected when TLV made the initial price and reimbursement decision and accepted a higher cost in relation to benefit than TLV normally does. One way to manage this is to state in the pricing and reimbursement decision how the price will be reduced if the sales value exceeds a certain level. We have developed methods to evaluate whether the number of patients treated exceeds the expected number. Another way to reduce the cost in such a situation is for regions and companies to sign an agreement whereby the cost per package will depend on to what extent the medicine is used.

3. How can we achieve sustainable costs for medicines?

Our proposals for medicines for rare diseases will increase costs for the state as well as the regions. Therefore, we also make proposals on how to reduce spending on medicines. TLV should be able to reject an application for reimbursement in the event of very high expected sales values, even if it is deemed to be cost-effective, if TLV judges that the costs are not economically reasonable and there are no means of reducing the costs. TLV has also developed methods to mitigate the costs of medicines with high sales volume, which means that TLV can set a price, but also alternative, lower prices, that will apply if the actual sales volume exceeds certain levels. We will continue to analyse how to implement these methods and their consequences.

4. Negotiations on agreements is an important piece of the puzzle to strengthen access at reasonable costs - how can the conditions be improved?

Medicines for serious and rare health conditions often have high prices. We note that managed entry agreements (MEA) between regions and companies with confidential

discounts are often required for the company to be able to reduce prices to a level that allows TLV to determine that the cost in relation to benefit is reasonable.

TLV has investigated the interest and possibilities of regions and companies to negotiate MEA in different situations, both for medicines for rare diseases and medicines with high sales value. We have also explored how TLV can provide support and space for parties to conduct negotiations on agreements.

What are the impacts of our measures?

We estimate that our proposals for medicines for rare diseases will increase patients' access to treatment but will also increase costs for the state and regions. We also believe that the methods we are developing to reduce the costs of medicines with high sales volume will provide a better basis for sustainable pharmaceutical costs than the current system. How society's spending on medicines develops in the future will largely depend on which medicines reach the market.

What happens now?

TLV will gradually begin to work according to the principles and procedures we describe in our report. We believe that this will improve access to treatment for patients with serious and rare health conditions and keep the pharmaceutical costs at a sustainable level. But we also recognise that what TLV does is part of a bigger picture, where many different stakeholders need to take action and responsibility to ensure that patients have equal access to treatment according to their needs at a reasonable cost to society.