The Czech Republic's Dedicated Orphan Drug Reimbursement Mechanism 2022–25: Improvements in Access

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Objectives

The Czech Republic (CR) introduced a new reimbursement pathway for orphan-designated medicines in 2022, contained in the new Section 39da of the Public Health Insurance Act (PHIA). It has given patients' groups a key role in the evaluation and decision-making procedures, and has introduced "soft" criteria, such as societal impact, for orphan drugs. One of the aims of the 2022 amendment was to bring therapies that had only been accessible to patients through laborious individual applications for exceptional reimbursement into the reimbursement system, making the process more predictable and therapies easier to access. The aim of this analysis is to assess the impact of this new pathway on the orphan drugs market in the CR, and its impact on access to orphan medicines in the country.

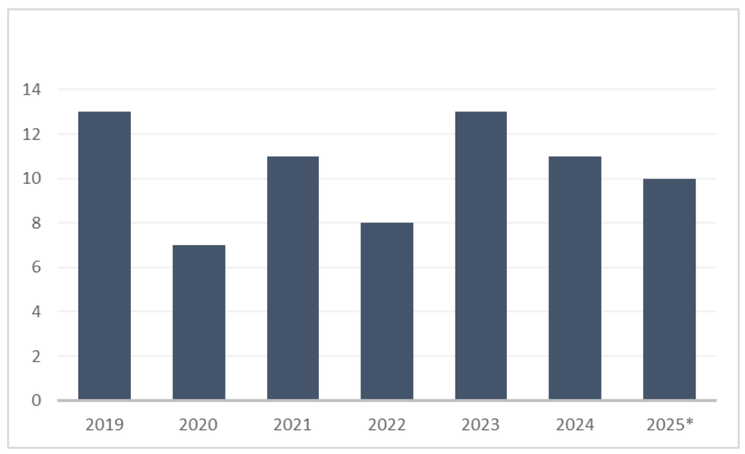
Methods

The number of orphan-designated therapies approved for reimbursement from 2019 until October 2025 was extracted from the State Institute for Drug Control (SÚKL) website, in order to compare the number prior to 2022 with the number after. The basis for reimbursement was noted in each case, to assess the market relevance and the uptake of the new orphan-drug pathway as compared with standard reimbursement and temporary reimbursement based on highly innovative medicinal product (VILP) classification. A review of publicly available information relating to the reimbursement of orphan-designated medicines in the CR was conducted, and information relevant to the research question extracted.

Results

The annual number of reimbursement approvals for orphan-designated therapies stabilized after the introduction of the new reimbursement pathway, although there has been no discernible surge. This is partly the result of the process of embedding the new pathway into the wider pricing and reimbursement system.

Number of new orphan-designated drugs/new indications approved for reimbursement in CR, 2019–25



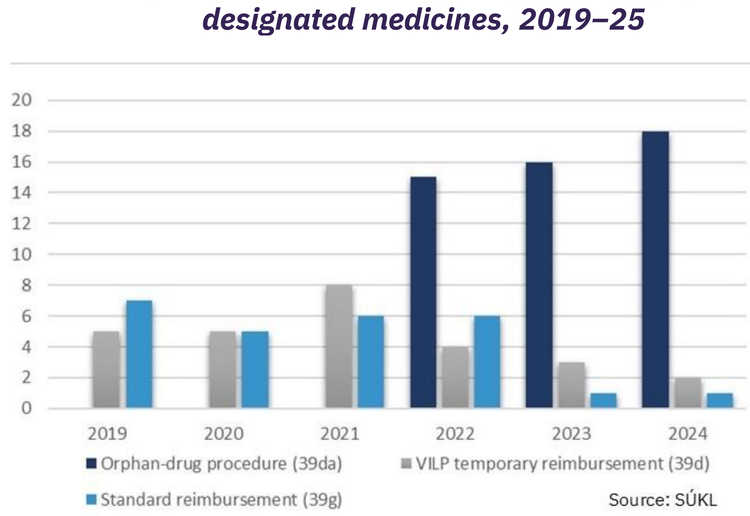
Includes all reimbursement decisions for centrally approved drugs with EU orphan designations including new indications and new basis for reimbursement (e.g. temporary to permanent reimbursement, "standard" to 39da reimbursement, second temporary reimbursement period).
*up to October inclusive
Source: SÚKL

Companies retain the right to choose between different reimbursement pathways. If choosing the orphan-drug-specific or the VILP temporary reimbursement options, there are mandatory cost-limitation contracts and product-specific clawbacks (based on budget-impact assessments) to consider. The orphan drug pathway offers "permanent" reimbursement, although positive decisions are potentially subject to reassessments. Since its introduction in 2022, the orphan drug pathway has become the predominant reimbursement option for orphan-designated therapies. In 2025, up to October, the basis of all 10 positive reimbursement decisions on orphan drugs by the SÚKL was the orphan-drug-specific reimbursement pathway.

Basis for new reimbursement approvals for orphan-designated therapies in CR, 2019–21 and 2022–25

While there has not been a significant increase in the number of reimbursement approvals for orphan medicines since the new pathway was introduced, SÚKL has reported a considerable increase in the number of initiated reimbursement proceedings for orphan drugs. Given the relatively slow evaluation procedures in the CR, it can be expected that 2025 and the following years will see an increase in orphan drug reimbursement approvals, and patients will be increasingly less reliant on individual applications for exceptional reimbursement.

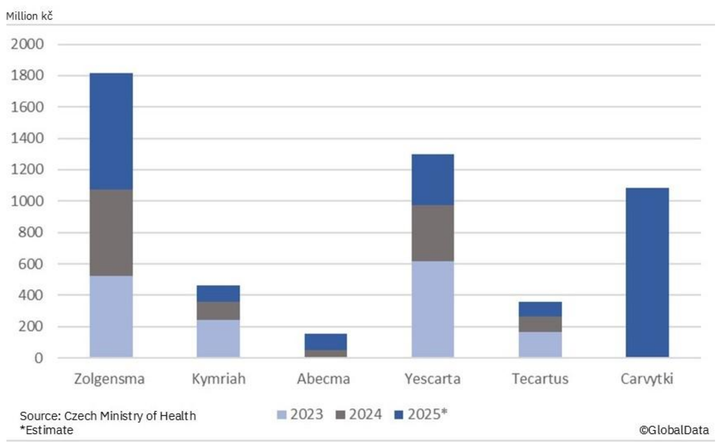
Number of initiated reimbursement proceedings for orphan designated medicines, 2019–25



Source: SÚKL

Many important orphan drugs, including CAR T-cell therapies and gene therapy drugs, are still not subject to standard, national reimbursement approvals, and remain available to patients only through exceptional reimbursement (based on Section 16 of the PHIA). The expected arrival of many new gene and cell therapies in the coming years means that lawmakers and regulators in the CR are increasingly occupied with finding suitable pricing and reimbursement mechanisms that will maintain the financial balance of the public health insurance system.

Expenditure on selected orphan-designated gene and cell therapies under Section 16 of the PHIA, 2023–25



Source: Czech Ministry of Health
*Estimate
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Conclusions

The introduction of the dedicated orphan drug reimbursement pathway in the CR has had a positive impact on patient access and has meant that some orphan drugs that would otherwise have been subject to individual, exceptional reimbursement, with all the additional bureaucracy and uncertainty that this entails, have become available under full, national reimbursement decisions. There has been strong acceptance and use of the new pathway on the part of the industry. While the number of therapies gaining reimbursement through the orphan drug pathway has not yet shown a significant increase, the annual number of reimbursement approvals has shown signs of stabilizing from 2023, and on the basis of the SÚKL's data on reimbursement applications, a larger number can be expected to be approved in 2025 and subsequent years. Still to be resolved, however, is the place of gene and cell therapies in the Czech pricing and reimbursement system. These continue to be subject to exceptional reimbursement, and as a result, are not subject to the same health technology assessments and pricing procedures as therapies going through the main reimbursement pathways, while patients face the challenge of individual applications and potential bureaucratic delays. Although they are subject to risk-sharing agreements and their impact on the budget is controlled, the current arrangements are unsustainable in the longer term, as the number of new gene and cell therapies coming on to the market increases.