ADDRESSING LEGISLATIVE AND FINANCIAL HURDLES IN RARE DISEASE MEDICINES ACCESSIBILITY:

Insight from Czechia with a focus on the pediatric population

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BACKGROUND

Advanced therapy medicinal products (ATMPs) and orphan drugs provide crucial treatment opportunities for rare diseases, especially **pediatric patients**. However, the current system for financing and reimbursing these medicines in the Czech Republic is often unsustainable and lacks sufficient transparency. Legislative reforms are needed to ensure **fair access and fair medicine practices** for rare disease treatments.

While a budget impact analysis (BIA) is necessary to secure reimbursement, there is an exemption from the cost-effectiveness analysis (CEA) requirement for some products, though not universally applied.[1] ATMPs are frequently denied reimbursement within the standard system and are instead financed through alternative pathways, such as **exceptional reimbursement schemes** or external funding sources like foundations or public collections.

OBJECTIVES

This study aims to map the current legislative framework for reimbursement for rare disease medicines in Czechia, highlighting legislative ‚grey areas' that create barriers to equitable access. The goal is to identify gaps in the legislation and point to necessary reforms. Additionally, the study emphasizes the increasing tendency for these medicines to be financed through unpredictable or alternative methods within the healthcare system.

METHODS

Legislative documents review

- ✓ Conducted a comprehensive review of **Czech legislative documents** related to the reimbursement of orphan drugs + ATMPs
- ✓ Focused on **§16** (Act No. 48/1997 Coll.: On Public Health Insurance)[1], which addresses **REIMBURSEMENT IN EXCEPTIONAL CASES**

Data collection

- ✓ Collected data from **University Hospital Brno**, including:
  - ✓ **number of approved applications** for exceptional reimbursement under §16 for orphan drugs + ATMPs
  - ✓ **financial expenditures** on treatments approved under §16 for orphan drugs + ATMPs

Legislative frameworks comparison

- ✓ Analyzed differences in reimbursement conditions for **mass-produced** vs **individually prepared advanced therapy medicinal products**

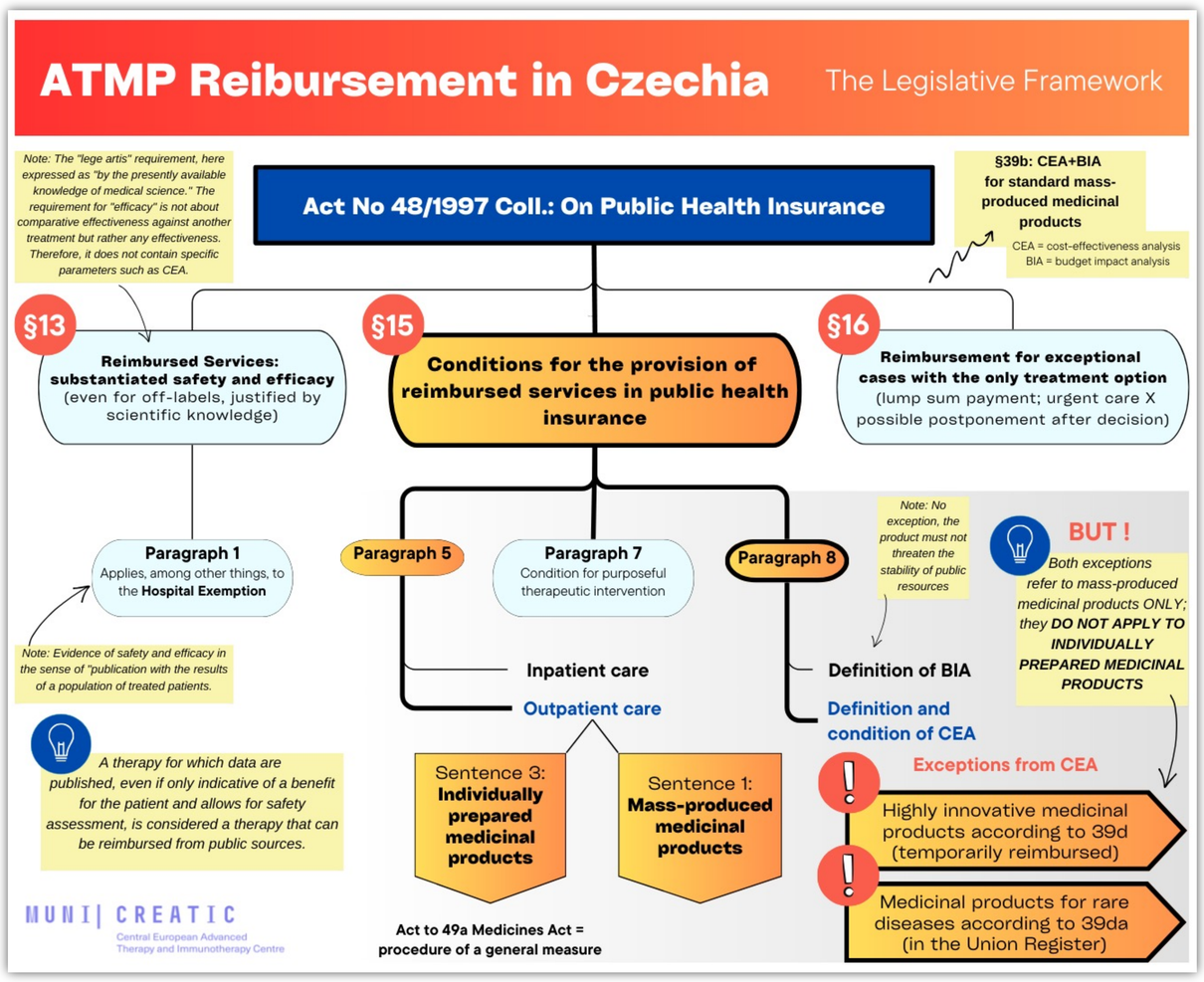
RESULTS

Czech legislation distinguishes between financing for inpatient and outpatient care.[1] For inpatient care, all medicines are covered without exception. In contrast, **outpatient care** is subject to specific reimbursement conditions. While a budget impact analysis (BIA) is required for all medicines, there is an exception to the **cost-effectiveness analysis (CEA)** requirement for highly innovative mass-produced orphan drugs. However, this exemption does not extend to **individually prepared medicines** frequently used in the pediatric population.[1] **As a result, demonstrating cost-effectiveness for these medicines under the standard requirements is nearly impossible.**

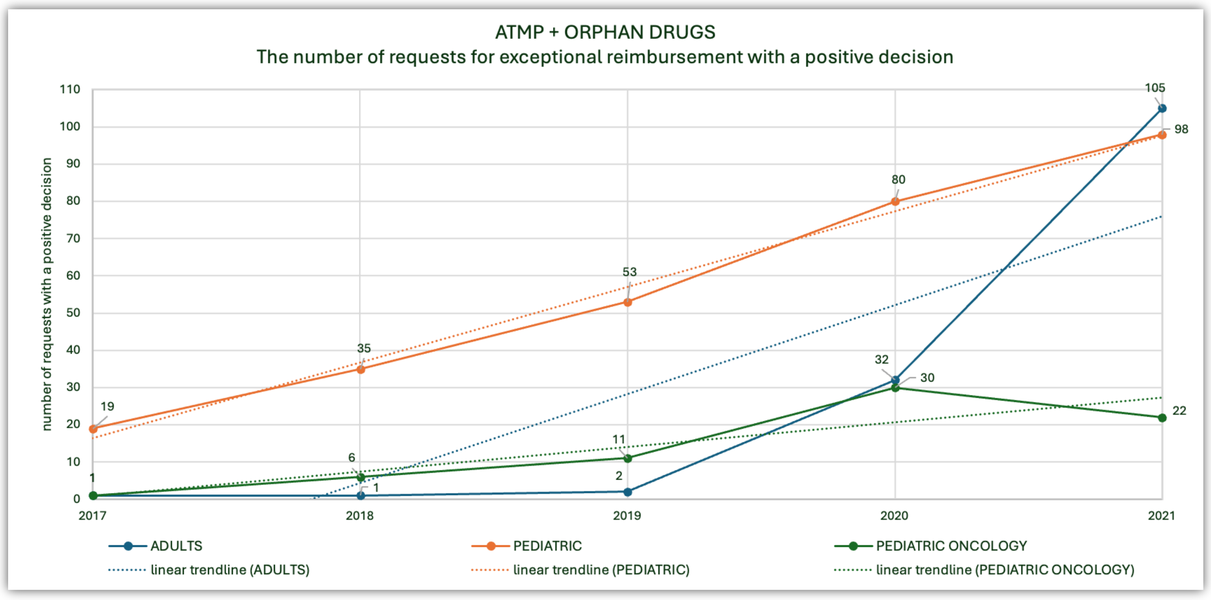
An alternative funding route for orphan drugs is through applications **for exceptional reimbursement (§16)**. Data on the number of such requests indicate a clear upward trend, particularly for pediatric patients (and notably in oncology). The annual expenditure on these medicines amounts to **millions of euros per hospital**, with costs for pediatric treatments representing a significant share of the total.

Orphan drugs repeatedly reimbursed through this non-standard pathway (at least until 2021) include Adcetris, Spinraza, Translarna, Soliris, Blincyto, Crysvita, Qarziba, and Zolgensma among ATMPs.

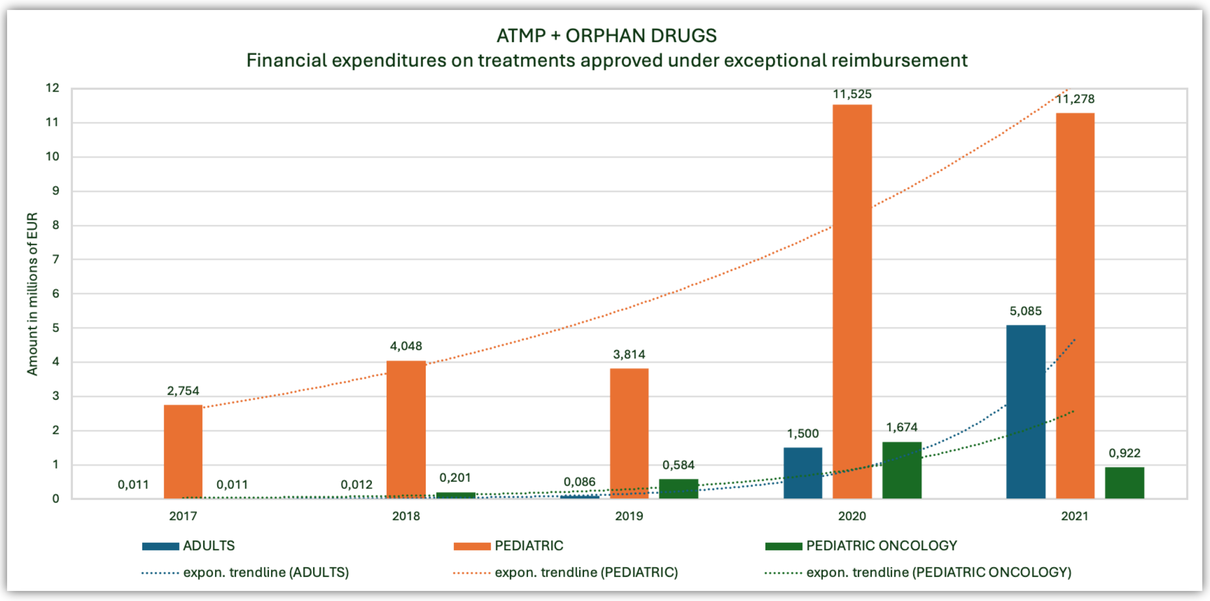
Figure 1: Legislative framework for reimbursement of ATMPs in Czechia – highlighting the possible route of exceptional reimbursement and the difference between mass-produced vs individually prepared advanced therapy medicinal products within outpatient care.



Graph 1: Number of requests for exceptional reimbursement under §16 (reimbursement has not yet been determined by standard legislation) within one faculty hospital in Czechia.



Graph 2: Overview of financial expenditures on treatments approved for exceptional reimbursement under §16 within one faculty hospital in Czechia.



CONCLUSIONS

Although Czech public health insurance legislation supports the arrival of ATMPs and orphan drugs, gaps and ambiguities remain. Exceptional reimbursement has been a temporary solution, providing treatment for many (mainly pediatric) patients. However, its frequent use suggests it is becoming the norm rather than the exception. The rising expenditures bypassing the standard reimbursement system for orphan drugs are increasingly uncontrolled. The overuse of §16 reduces pressure on manufacturers to advocate for legislative changes to ensure predictability in reimbursement decisions.

REFERENCES

1) Act No 48/1997 Coll.: On Public Health Insurance [as amended]. In Zákony pro lidi. Published 1997, updated 2024. Accessed October 22, 2024. Available at https://www.zakonyprolidi.cz/translation/cs/1997-48?langid=1033

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