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PHARMACOECONOMIC RESULTS OF HIGHLY INNOVATIVE DRUGS APPROVED FOR TEMPORARY REIMBURSEMENT IN THE CZECH REPUBLIC

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BACKGROUND

Highly innovative drugs (HIDs) can be granted two plus facultative one year extension of temporary reimbursement (TR) to provide timely access and to collect additional real-world evidence by creating a HID's registry.

To obtain a HID status, drug needs to meet legally prespecified criteria such as treatment of highly severe illness plus a) no alternative reimbursed treatment available (including drug resistance, inadequately controlling treatment) or b) substantially better effectiveness or safety compared to the alternative (in terms of survival, severe complications, side effects, hospitalization, drug interactions).[1]

TR applicant does not need to comply with strict cost-effectiveness (CE) requirements and willingness-to-pay threshold (WTP) when there are no sufficient data about the use in real clinical practice or about CE at a time. Pharmacoeconomic analysis must be submitted, but incremental cost-effectiveness ratio (ICER) is not confronted with standard threshold (currently equal to €45,000/QALY[2]) and does not play a role in decision process of TR.

TR is finally approved, provided that market authorisation holder (MAH) signed the commitment to initiate a local real-world evidence (RWE) registry in order to confirm efficacy and safety of HID. Secondly, MAH commits to cover the costs of patients on treatment if permanent reimbursement (PR) is not achieved after two or maximum three years of TR period.[1,3]

After two/three years of TR when switching to PR, drug must comply with strict CE and WTP requirements.

OBJECTIVES

The main objective was to analyse pharmacoeconomic results at the entry of drug into TR and compare them with results presented in consecutive PR procedure.

Additionally, we analysed the length of evaluation procedure with respect to temporary or permanent application and the utilization of temporary reimbursement by type of pharmacological treatment.

Finally we explored the success rate of costly HIDs in entering PR system after going through TR.

Figure 1. Temporary reimbursement utilization according to ATC class

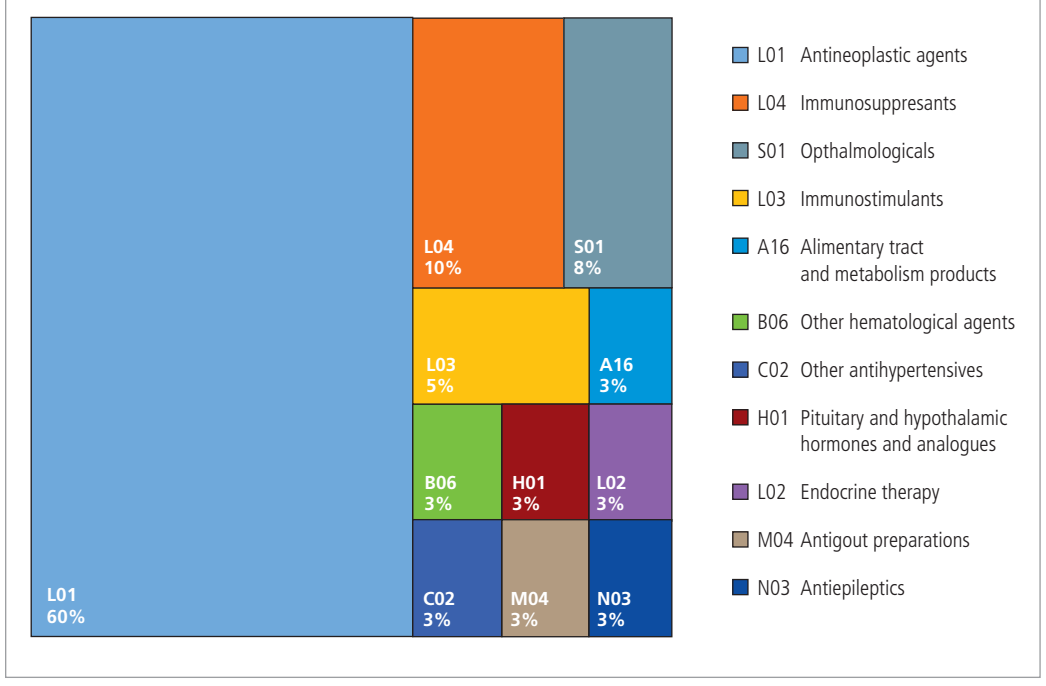
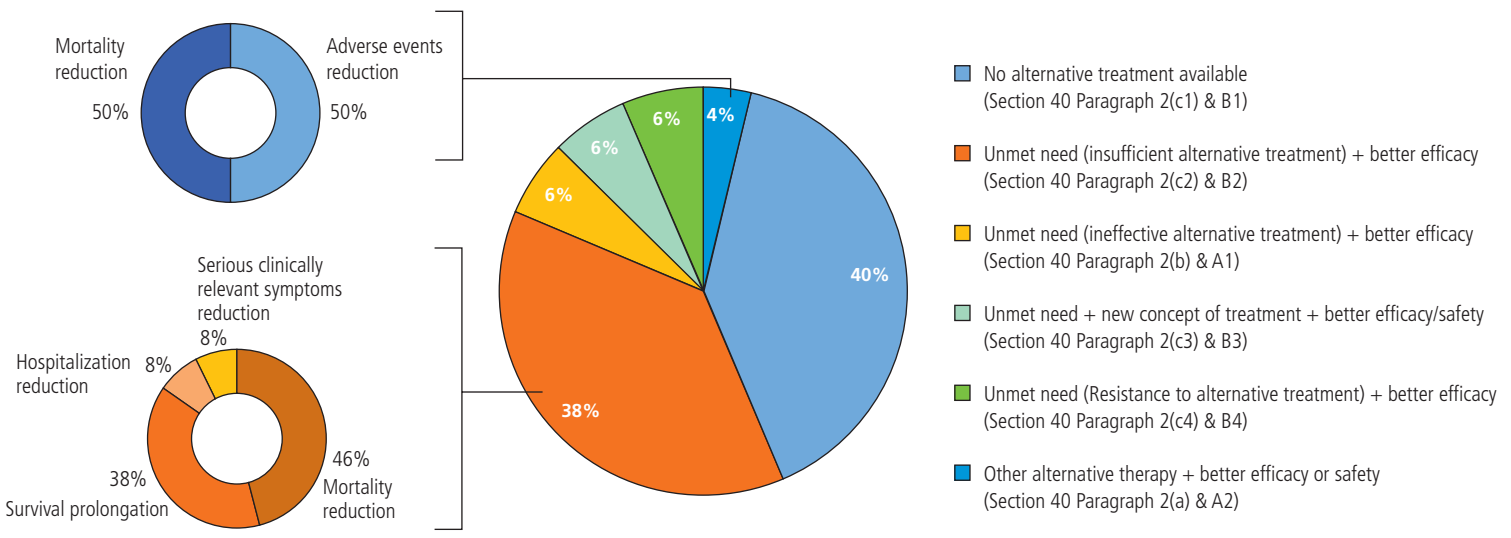


Figure 2. Classification of HID status based on legally prespecified criteria[1,4]



METHODS

All drugs along with their indications approved for TR until 5/2017 were identified (TR for HIDs has been legally valid since 2008). Pharmacoeconomic settings and results (i.e. type of analysis, ICERs, net budget impact (netBI)) at the time of TR and consecutive PR application were analysed. We also examined therapeutic class (ATC), indication of interest, orphan status, type of HID according to legal criteria and compared the length of approval procedure of TR versus PR. We used publicly available database of pricing and reimbursement procedures maintained by State Institute for Drug Control (SUKL) to access all relevant information.

We distinguished between first TR procedure and an extension of TR by one year, which is usually allowed when RWE data are not mature for PR and HID status is still valid.

RESULTS

Forty individual procedures of HIDs approved for TR were identified over 10-year period.

Figure 1 shows distribution of ATC classes among all drugs approved for TR. Majority (31; 78%) TR drugs belonged to ATC class L. Twenty eight (70%) of TR drugs were of oncologic indication.

Temporary reimbursement and HID classification does not rely on orphan status. Orphan status does not belong to criteria taken into account. Out of all identified TR products, only 13 (33%) were with orphan status.

The most common legally prespecified criteria used in classification of HID status were absence of alternative treatment (20/50; 40%) followed by unmet need (insufficient alternative treatment) + better efficacy (18/50; 38%) (**Figure 2**).

Figure 3 presents the pathway of drugs from the time they entered temporary reimbursement. In cases where collected RWE has not yet provided robust and mature data (to be used in CE analysis), the first period of temporary reimbursement may be extended. This was the case for 25 (76 %) drugs which did not have such data after two years.

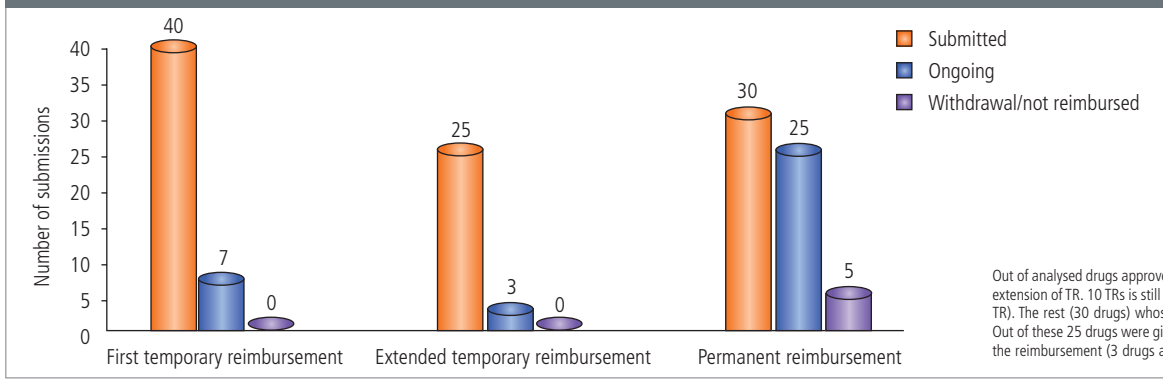
Ten (25 %) drugs had an ongoing TR (first or extended one). Thirty (75 %) drugs applied for PR after the expiration of TR. Out of these, 83% had positive subsequent decision on PR and 17% did not manage to receive PR (due to withdrawal or not reaching positive decision yet). Price negotiation with payers was present in 50% of cases.

Cost-utility analysis was used to present results of CE in 44% of TR procedures. This allowed us to compare CE results of different treatments. The mean ICER (cost/QALY) of TR products was €96,668 (SD €67,033, median €72,360). In the subsequent PR procedure, the mean ICER was lower by 56% (€42,512; SD €36,413; median €44,200) (**Figure 4**).

The mean NetBI has decreased by 32% and 50% in 1st and 5th year respectively when applying for PR after going through TR (**Figure 5** and **Figure 6**).

Mean decision time about TR was longer (404 days; SD 194) than consecutive decision about PR (259 days; SD 251) (**Figure 7**).

Figure 3. Pathway of HIDs to Permanent reimbursement



Out of analysed drugs approved for TR (40). 25 drugs were granted extension of TR. 10 TRs is still ongoing, (7 in first and 3 in extended TR). The rest (30 drugs) whose TR already expired applied for PR. Out of these 25 drugs were given positive decision about PR, 5 lost the reimbursement (3 drugs are still under evaluation).

Figure 4. ICER in temporary vs. permanent reimbursement

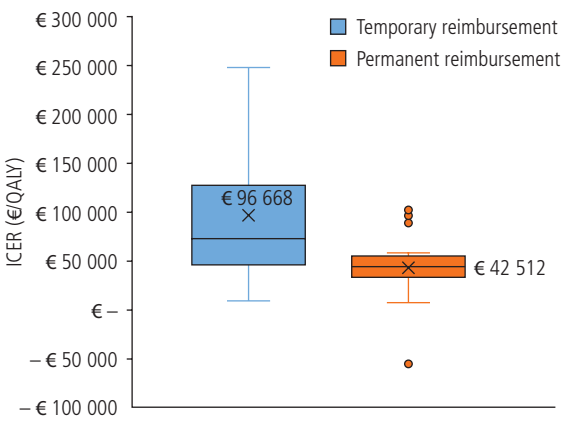


Chart shows distribution of data into quartiles, highlighting the mean, median and outliers.

Figure 7. Length of approval procedure

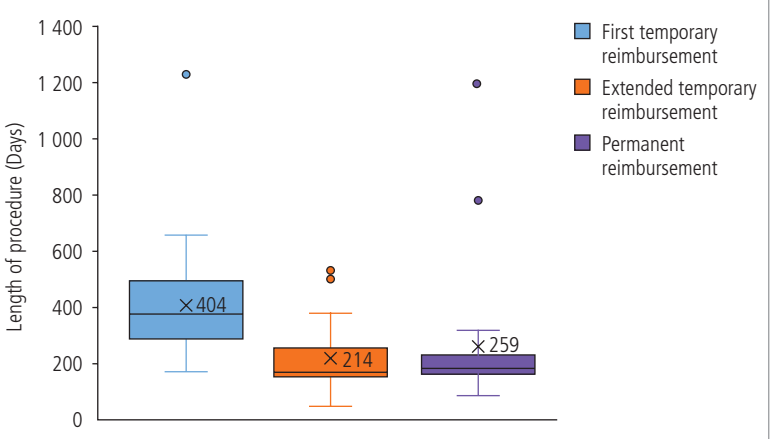


Chart shows distribution of data into quartiles, highlighting the mean, median and outliers.

Figure 5. NetBI in 1st year of temporary vs permanent reimbursement

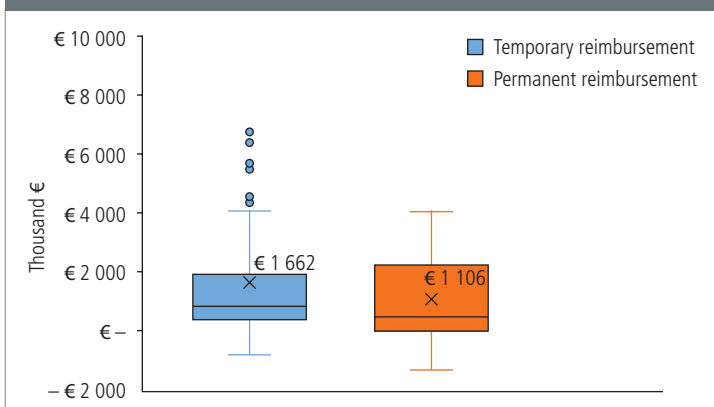


Chart shows distribution of data into quartiles, highlighting the mean, median and outliers. The highest outlier (16 346 EUR) in temporary reimbursement is not shown.

Figure 6. NetBI in 5th year of temporary vs permanent reimbursement

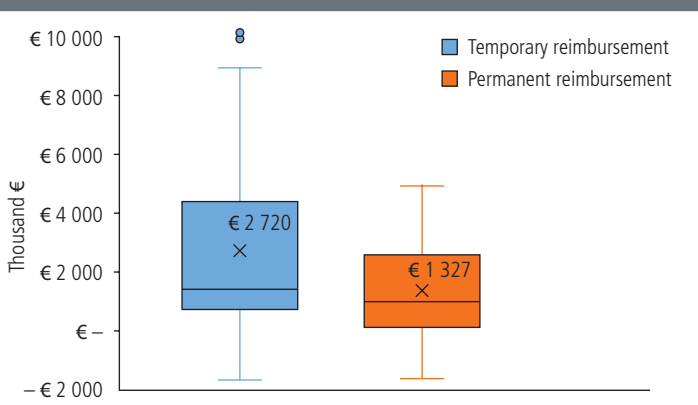


Chart shows distribution of data into quartiles, highlighting the mean, median and outliers. The highest outlier (15 577 EUR) in temporary reimbursement is not shown.

CONCLUSIONS

Costly HIDs accepted for a TR entered the system with on average twice higher ICERs and higher netBI than usually accepted in PR procedure (WTP threshold is equal to €45,000/QALY vs. €96,688/QALY in TR). Ninety-two percent of TR drugs were successful in consecutive PR procedure and continue to be available to patients and their treatment. Length of approval process was shorter in PR compared to TR, which can be due to earlier assessment (thus knowledge of the CE model and clinical data) and RWE data availability.

REFERENCES

1 Decree No. 376/2011 Coll., implementing some of provisions of the Act on Public Health Insurance. • 2 State Institute of Drug Control. Evaluation of cost-effetiveness Guidelines. 17/05/2017. • 3 Act No. 48/1997 Coll., onPpublic Health Insurance and amendments to some related acts, as amended. • 4 Decree No 92/2008 Coll., on the list of the reference basket, method of evaluation of the amounts, conditions and method of reimbursement of medicinal products and foods for special medical purposes and particulars of an application, as amended.

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