PSY147

AVAILABILITY AND REIMBURSEMENT OF ORPHAN DRUGS IN THE CZECH REPUBLIC

Ornstova E[1*], Paris M[2*], Sebestianova M[1], Mlcoch T[1], Bartakova J[1], Chadimova K[1], Hajickova B[1], Dolezal T[1]

[1]VALUE OUTCOMES, Prague, Czech Republic; [2]COGVIO, Prague, Czech Republic
*These authors made equal contribution to this work.

INTRODUCTION

The European Orphan Medicinal Products (OMPs) Regulation[1] intended to incentivise research, development and marketing of new therapies for rare and chronically disabling or life-threatening diseases. Marketing authorisation is granted to OMPs centrally in the European Union, however, it is only the first step. Patients have access to medicines once reimbursement decisions are implemented by national health systems. OMPs are different compared to other medications because their accessibility is threatened by the fact that expected sales are insufficient to return the investment.

In Germany reimbursement of OMPs with budget impact less than €50mil per year is automatically granted after authorisation, in some other countries the OMPs are directly procured through special funds. The Czech Republic, on the contrary, has no established specific reimbursement policy for Orphan medicinal product. OMP has to prove that incremental cost-effectiveness ratio (ICER) is acceptable as well as every other new medicine in the cost-effectiveness evaluation. In order to gain permanent reimbursement from public health insurance, fixed willingness to pay threshold (WTP) has to be respected (currently 1.2 million CZK per QALY ~ €47,000) and this is applicable for all new treatments, including OMPs. Alternative option is temporary reimbursement for highly innovative drugs which allows to accept a higher ICER in the Czech Republic (described in detail in different publication[2]). It has to be mentioned that highly innovative drug programme is not focused on facilitating access to OMPs and is mostly used by costly breakthrough drugs with potential of "real world" evidence development, which is often problematic for low prevalence (rare) diseases in a smaller country.

Therefore many patients with rare diseases are reliant to access the OMP treatment through exceptional individual reimbursement (application of Section 16 Act No. 48/1997 Coll., on Public health insurance).[3] However, Section 16 in its sense is aimed at exceptional cases based on an individual assessment whether or not the other reimbursed treatment option exists. Obtaining reimbursement through Section 16 is not a "standard way" which guarantees continuous access to the OMP for patients. Section 16 is only allowing the reimbursed use in the individual patient. The access is approved on a case by case basis by health insurance fund, however, transparency and consistency as in regular reimbursement procedure are not claimed.

OBJECTIVES

The objective of this analysis was to analyse the Czech market from orphan drug perspective. How does current reimbursement policy influence access of patients with rare diseases to their medication was studied by analysing data about distribution and reimbursement decisions. The size of orphan market, as well as the distribution of orphans among different reimbursement schemes were analysed in order to evaluate their availability and patient access in the Czech Republic.

METHODS

- As a first step, we identified drugs with valid orphan status granted by European Medicines Agency (EMA). The examined time period was from 2009 to 2017. We searched in Community Register of orphan medicinal products for human use on European Commission website. Orphan drugs in this analysis were those with active market exclusivity.[4] Those after the period of market exclusivity and therefore loss of orphan status were not included in the analysis. Terms Orphans and OMPs are used interchangeably in this poster.
- **Availability** of OMPs is represented by the number of prescribed OMPs compared to the number of OMPs which received a market authorisation. **Access** is defined as a standard reimbursement based on reimbursement decision by the national regulatory authority State Institute for Drug Control (SUKL).
- The presence (availability) of orphans in the Czech Republic was extracted from monthly mandatory distributor's reports about medicinal products supplies.[5] Publicly available distribution reports provide information whether designated OMP was marketed, which means prescribed by a physician regardless of reimbursement decision.
- Consumption and the real price volume of prescribed OMPs recorded in the distributor's reports were analysed. The ex-manufacturer prices from distribution reports were adjusted to the amount of reimbursement by using the published list of reimbursed medicinal products[6] or by adding maximum profit margin as per the price regulation of the Ministry of Health of the Czech Republic[7] and VAT (in case of OMPs not included in the list of reimbursed medicinal products).
- Orphans with standard reimbursement were acquired from the list of reimbursed medicinal products published by the responsible authority (SUKL).[6]
- Orphans marketed but not detected in SUKL's list of reimbursed products were considered reimbursed through individual patient scheme (individually approved exceptional reimbursement) allowed by Section 16 of public health insurance law[3]. We do not reflect the fact, that some OMPs could have been financed by patient itself, because it is very unlikely due to the high costs of orphan treatment.

Figure 1. Availability EMA designated OMPs in the Czech Republic

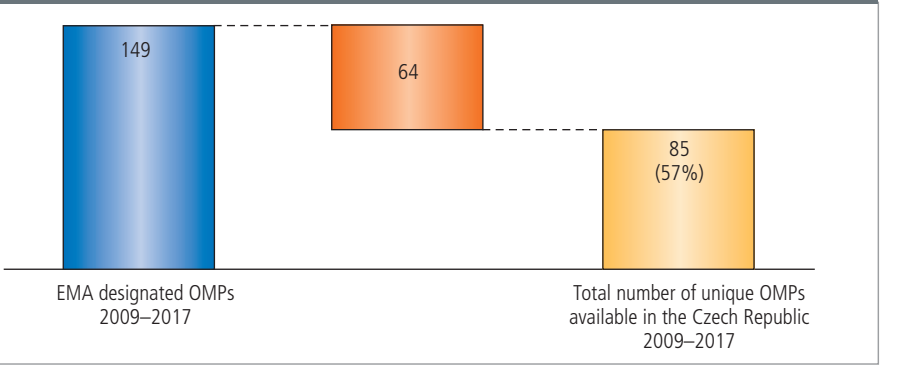
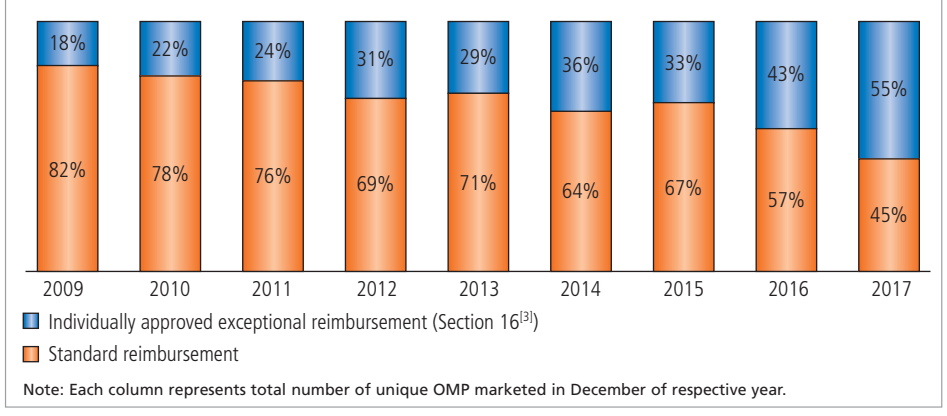


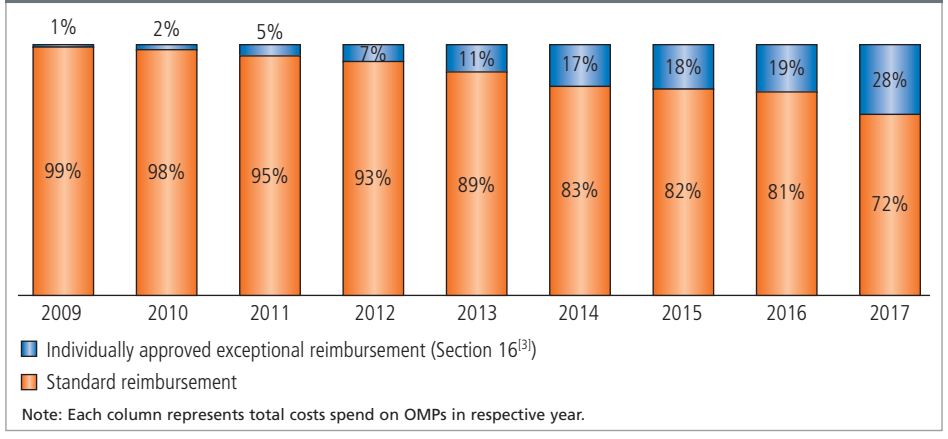
Figure 2. Total number of unique OMPs marketed in the Czech Republic

Figure 3. Proportion of unique OMPs by reimbursement scheme over time



Note: Each column represents total number of unique OMP marketed in December of respective year.

Figure 4. Total reimbursement costs of OMPs – market share by reimbursement scheme

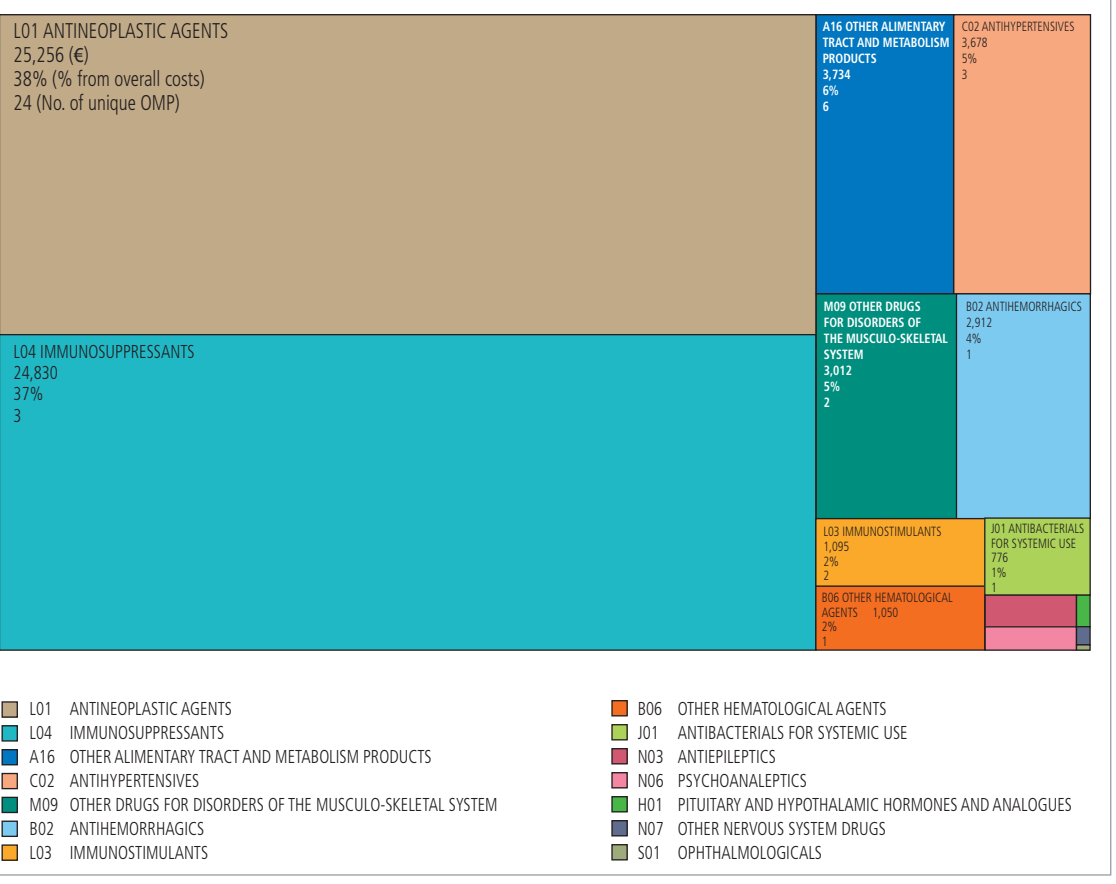


Note: Each column represents total costs spend on OMPs in respective year.

RESULTS

- In the examined 9-year time period, 149 OMPs received a market authorisation in the European Union. 57% (85) of all EMA designated orphans were available on the Czech market, measured by occurrence in distributor's report. (**Figure 1**)
- The number of OMPs distributed yearly on the Czech market had a rising tendency. 35 orphans occurred in 2009 compared with 50 orphans prescribed in the Czech Republic in 2017. (**Figure 2**)
- However, out of all OMPs distributed in the Czech Republic only a proportion was widely and permanently accessible to all eligible patients thanks to reimbursement decision. This proportion was continuously decreasing and in December 2017 the proportion of OMPs listed as reimbursed drugs came down to 45% of all marketed orphans. The remaining 55% were financed by an exceptional individual reimbursement scheme (Section 16). This is turnover compared to proportions of OMPs reimbursed by authority's decision vs. OMPs reimbursed individually by Section 16 observed in the past (December 2009), when only 18% of orphans were not listed among reimbursed medicinal products. (**Figure 3**)
- The proportion of OMPs entering the standard reimbursement system was steadily decreasing over the past seven years while the use of exceptional individual reimbursement scheme via Section 16 was growing. Despite the fact that large portion of orphans stays out of standard reimbursement system, individual reimbursement represents smaller market share (28% of total costs) compared to orphans listed as generally reimbursed products (72% of total costs), showing on data observed for 2017. (**Figure 4**)
- Total costs of all OMPs marketed in 2017 were €66,916,000. The major costs were spent on OMPs from ATC class L01(antineoplatstics) and L04 (immunosuppressants) with 38% and 37%, respectively, out of total costs. Split of costs by ATC is shown in **Figure 5**.

Figure 5. Total reimbursement costs of OMPs in 2017 by ATC class



CONCLUSIONS

This analysis showed that availability of orphans on the Czech market is maintained. Newly designated OMPs are available for Czech patients in terms of presence on market distribution. On the other hand, the guaranteed access to OMPs for all relevant patient has been seen decreasing, because proportion of OMPs with standard reimbursement was decreasing over the last 9 years and represent now just 45% of OMPs available on the Czech market.

The price volume spent on different reimbursement scheme of OMPs suggests that it is harder for patients to obtain orphan treatment via exceptional individual reimbursement scheme (Section 16). The particular reasons for this are that reimbursement decision based on Section 16 is given upon individual application and is valid for limited time of 3 months only. Application has to be repeatedly submitted by individual patient/physician to health insurance funds every three months, which places significant administrative burden onto patients and officials involved in the individual approval process. Predictability of decision is low because individual cases are considered by assigned review physician which naturally cannot guarantee a consistency in the decision.

Moreover, orphans with low patient potential are not incentivised adequately to access the permanent reimbursement, because reimbursement procedure with cost--effectiveness evaluation is demanding and cost-effectiveness criteria are strict.

REFERENCES

1 Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, O.J. L 18, 22.1.2000. • 2 Ornstova E, Sebestianova M, Mlcoch T, Lamblova K, Dolezal T. Highly Innovative Drug Program in the Czech Republic: Description and Pharmacoeconomic Results–Cost-Effectiveness and Budget Impact Analyses. Value in Health Regional Issues. 2018 Sep;16:92–8. • 3 Act No. 48/1997 Coll., on public health insurance and on changes to certain related laws, as last amended. Available from: https:// www.zakonyprolidi.cz/cs/1997-48. • 4 Register of designated and non-active Orphan medicinal products. European commission-The Register of orphan medicinal products for human use designated and published by the EU in accordance with Article 5 of Regulation (EC) N141/2000. https://ec.europa.eu/health/documents/community-register/html/index_en.htm • 5 Supplies of medicinal products in the Czech republic. Available from: http://www.sukl.eu/dodavky-a-jina-hodnoceni • 6 List of reimbursed medicinal products. Available at: http://www.sukl.cz/sukl/seznam-leciv-a-pzlu-hrazenych-ze-zdrav-pojisteni • 7 Price Regulation of the Ministry of Health of the Czech republic (1/2013/FAR). Available from: http://www.mzcr.cz/obsah/cenova-regulace_1953_3.html

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Corresponding author: Eva.Ornstova@valueoutcomes.cz

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