AVAILABILITY AND REIMBURSEMENT OF ORPHAN DRUGS IN THE CZECH REPUBLIC

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INTRODUCTION

The European Orphan Medicinal Products (OFPs) Regulation1 intended to incentivize research, development and marketing of new treatments for rare conditions or chronically disabling or life-threatening diseases. Marketing authorization is granted to OFPs centrally in the European Union; however, it is only the first step. Patients have to access to medicines on the local market. The OFPs REIMBursement (OPR) is entitled to incentivize national health systems. OFPs 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 OFPs with budget impact less than €400 per year is automatically granted after authorities assess the OFPs directly proposed by applicants. The Czech Republic, on the contrary, has no established specific reimbursement policy for Orphan medicinal product. OMP has to prove the 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 ~ 447,000€). This is applicable for all new treatments, including OFPs. 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 publication2). It has to be mentioned that highly innovative drug programme is not focused on facilitating access to OFPs and is mostly used by cost-beneficial drugs with potential of "real world" evidence development, which is often problematic for low prevalence (rare) diseases in a smaller country. Moreover, the time period was from 2009 to 2017. We searched in Community Register of orphan medicinal products for human use on the Czech market. As a first step, we identified drugs with valid orphan status granted by European Medicines Agency (EMA). The examined period was from 2009 to 2017. We searched in Community Register of orphan medicinal products for human use on the Czech market. Figure 3 shows the number of unique OFPs per year. The availability of OFPs is represented by the number of prescribed OFPs compared to the number of OFPs which received reimbursement. As a first step, we identified drugs with valid orphan status granted by EMA. We used the Community Register of orphan medicinal products for human use on the Czech market.

OBJECTIVES

The objective of this analysis was to analyze the Czech market from orphan drug perspective. The study reflects the current OFPs reimbursement policy influence access of patients with rare diseases to their medication was studied by analyzing data about distribution and reimbursement decisions. We do not reflect the fact, that some OFPs could have been financed by patient itself, because it is very unlikely due to the high costs of orphan treatment.

METHODS

As a first step, we identified drugs with valid orphan status granted by European Medicines Agency (EMA). The examined period was from 2009 to 2017. We searched in Community Register of orphan medicinal products for human use on the Czech market. Availability of OFPs is represented by the number of prescribed OFPs compared to the number of OFPs which received reimbursement. Access is defined as a standard reimbursement based on reimbursement decision by the regulatory authority State Institute for Drug Control (SUKL). The proportion of available OFPs in the Czech market was estimated from monthly mandatory distributor's reports about medicinal products supplied3. Publicly available distribution reports provide information whether designated OFP was reimbursed, which means prescribing a physician regardless of reimbursement decision.

RESULTS

• In the examined 2-year period, 149 OFPs received a market authorisation in the European Union. 57% (88) of all EMA designated OFPs were available on the Czech market, measured by occurrence in distributor's report. Figure 4 shows the proportion of OFPs distributed only on the Czech market. The majority of OFPs distributed only and therefore loss of orphan status were not included in the analysis. Terms Orphans and OFPs are used interchangeably in this poster.

• The OFPs availability in the Czech market was estimated from monthly mandatory distributor's reports about medicinal products supplied. Publicly available distribution reports provided information whether designated OFPs were reimbursed, which means prescribing a physician regardless of reimbursement decision.

• Consumption and the real private volume of prescribed OFPs in the distributor's reports were analysed. The manufacturer prices from distribution reports were adjusted to the amount of reimbursement by using the published list of reimbursed medicinal products4 on the base of their reimbursement profit margin as per the prescription regulation of the Ministry of Health of the Czech Republic5 and VAT (if OFPs not included in the list of reimbursed medicinal products). Of the OFPs reimbursed, reimbursement was financed as of 2011 by a public health insurance fund, however, transparency and consistency of the reimbursement procedure are not claimed.

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• Therefore many patients with rare diseases are reliant to access the OFPs treatment through exceptional individual reimbursement (application of Section 16 Act No. 48/1997 Coll., on Public health insurance). However, Section 16 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 OFPs for patients. Section 16 is only allowing the reimbursed use in the individual patient. This access is approved on a case by case basis by health insurance fund, however, transparency and consistency of the reimbursement procedure are not claimed.

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

CONCLUSIONS

This analysis showed that availability of OFPs on the Czech market is maintained. Newborn designated OFPs are available for Czech patients in the first period on market distribution. On the other hand, the guaranteed access to OFPs for all relevant patients has been decreasing, because programs OFPs with standard reimbursement was decreasing over the last 5 years and represent now just 45% OFPs available on the Czech market. The value spent on different reimbursement scheme of OFPs suggests that it is harder for patients to obtain orphan treatment via exceptional individual reimbursement (Section 16). The proportion of OFPs reimbursed based on reimbursement decision by the national regulatory authority State Institute for Drug Control (SUKL) was 35% in 2017. This is turnover observed in the past (December 2009), when only 18% of OFPs were not listed among reimbursed medicinal products. The remaining 55% were financed by an exceptional individual reimbursement scheme (Section 16). This is turnover observed in the past (December 2009), when only 18% of OFPs were not listed among reimbursed medicinal products. Therefore many patients with rare diseases are reliant to access the OFPs treatment through exceptional individual reimbursement (application of Section 16 Act No. 48/1997 Coll., on Public health insurance).

All costs were spent on OFPs from ATC class L (immunosuppressants) and L (immunosuppressants) with 38% and 37%, respectively, out of total costs. Split of costs by ATC is shown in Figure 5.}

REFERENCES


2 OFPs designated OMPs

3 Figure 3

4 Figure 4

5 Availability of OFPs in the Czech Republic 2009–2017

6 Total number of unique OFPs marketed in the Czech Republic

7 Total reimbursement costs of OFPs – market share by reimbursement scheme

8 Figure 5

9 Proportion of unique OFPs by reimbursement scheme over time

10 Individual reimbursement (Section 16)

11 Standard reimbursement

12 Note: Each column represents total number of unique OMP prescribed in December of reference year.

13 Individual approved reimbursement (Section 16)

14 Standard reimbursement

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


17 Proportion of unique OFPs by reimbursement scheme over time


19 Total reimbursement costs of OFPs – market share by reimbursement scheme

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