

# EXPENDITURES AND AVAILABILITY OF ORPHAN DRUGS IN THE CZECH REPUBLIC: SEVEN YEAR EXPERIENCE (2004 – 2010)

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## OBJECTIVE

Across all developed countries it is very often discussed issue of drugs for treatment of rare diseases (Orphan Drugs; ODs). The most often discussed issues are: What is the best approach how to subject them to HTA process? Should they be treated like “normal” non-orphan drugs? Should they prove to be cost-effective or on the other hand, does it suffice them to prove efficacy/ effectiveness to be reimbursed? And what is the payers’ budget impact of these drugs?

Within our study, we did an analysis of availability (patients’ access) to ODs and determined expenditures in the horizon of 7 years (within years 2004 – 2010) in absolute figures and relative proportion related to expenditures of all medicinal products in the Czech Republic. We also tried to express the proportion/ percentages of ODs expenditures related to total drugs reimbursed by Health insurance funds. We also provided a small discussion about assessment of ODs within the reimbursement process.

## METHODS

We identified all ODs registered by EMA until September 2011. We also compared the year of registration with the year of patients’ access to particular OD. Patients’ access to ODs was defined as availability in official Czech distribution chain (wholesalers and/or pharmacies). This availability does not have to necessarily mean that the drug is officially reimbursed. This availability can be implemented by clinical trials, compassionate use or “unofficial” reimbursement per individual patient (under exceptional circumstances on request from physician).

Total expenditures for ODs in particular year were calculated from the databases of State Institute for Drug Control (SUKL). Based on these databases, we can either capture maximum ex-factory prices or the highest possible pharmacy prices (including all margins and VAT), which was our approach. However, within our analysis, we would like to express real expenditures of Health insurance funds on ODs.

We presented the relationship between our calculations of ODs expenditures and total (even OTC) drug expenditures in ex-factory prices. We also presented the relationship between our calculations of ODs expenditures and total drug expenditures by Health insurance funds. The real burden of ODs into Health insurance funds can be either underestimated or overestimated, based on those approaches named above. Since in the first case (underestimation), there are interferences of OTC drugs and also methodology discrepancy (all drugs in ex-factory prices vs. ODs in pharmacy prices). In the second case (overestimation), there must be noted the fact, that ODs are usually not reimbursed up to highest possible pharmacy price. To provide estimation of the real friction of expenditures of ODs related total drugs budget of Health insurance funds, we present a mean value of both approaches.

## RESULTS

Until September 2011, 49 out of 66 EMA registered ODs (74%) have been available in the Czech distribution chain, hence available to Czech patients. The mean duration of period between registration year and year of availability in the Czech distribution chain (among already available ODs) was 2.27 years and median 2 years. This period was longer within past years compared to recent years (Figure 1).

The expenditures for ODs revealed an extreme increase up to year 2007, nevertheless the increase in expenditures has been more stable and rather smaller in recent years. In particular, the expenditures (in million EUR) within years 2004 – 2010 were 0.3, 3.0, 14.5, 35.3, 50.9, 69.9 and 84.1, respectively (based on exchange rate 1 EUR = 25.1 CZK), see Figure 2.

These expenditures represented 0.8%, 1.8%, 2.3%, 3.0% and 3.6% of total medicine expenditures within years 2006 – 2010 in the Czech Republic (Figure 3, orange colour) and slightly higher, when these expenditures are attributed to all drug expenditures of Health insurance funds (Figure 3, blue colour). The real expenditures of Health insurance funds on ODs are then presented as mean values (Figure 3, yellow columns).

The highest burden among all ODs have been represented by medicines for treatment of Chronic Myeloid Leukemia and Renal Cell Carcinoma, those medicines represented 48.3% of all expenditures for ODs in 2010. Detailed distribution of percentage expenditures of particular OD is presented in Figure 4.

Figure 1. “A window” between registration by EMA and availability to Czech patients (just for already available ODs; 74% of them)

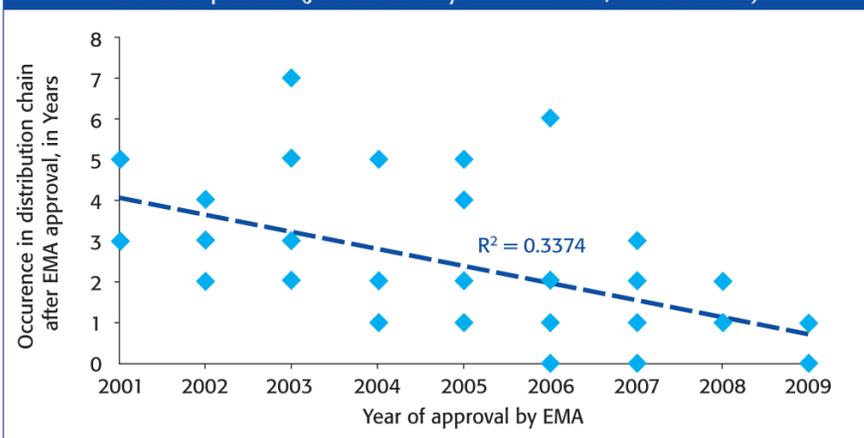


Figure 2. Expenditures of ODs in particular year

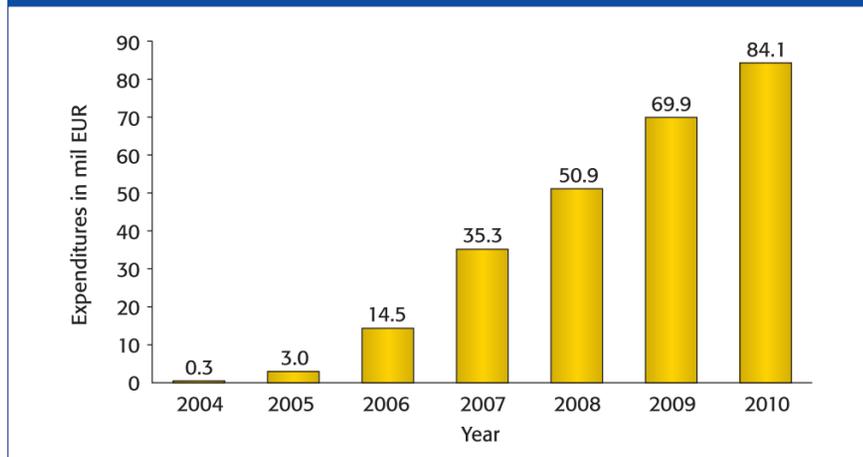
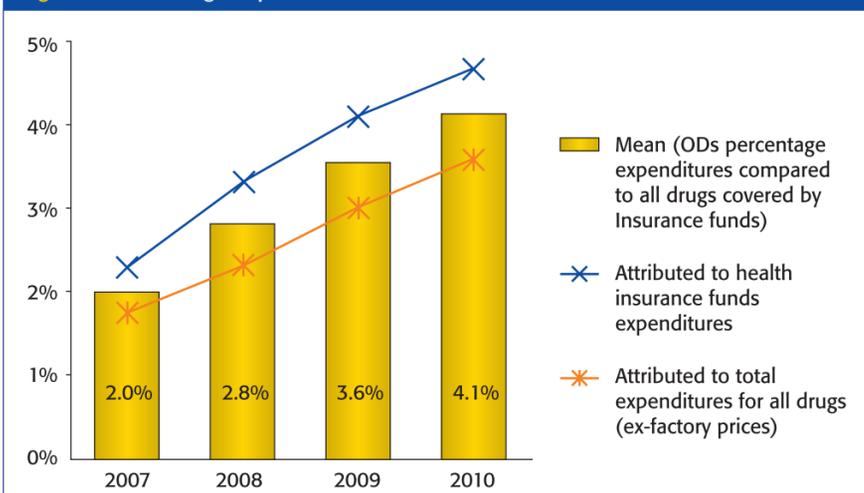


Figure 3. Percentage expenditures of ODs



## CONCLUSIONS

The real expenditures of Health insurance funds on ODs were approximately 2.0%, 2.8%, 3.6% and 4.1% in years 2007 – 2010.

It must be noted, not all of available ODs are reimbursed on a standard basis, since some ODs have conditional or exceptional (per individual patient) reimbursement. The current expenditures (year 2012) for ODs in the Czech Republic is approximately 4 - 5% of total drug expenditures by Health Insurance funds, which is comparable with other EU countries.

Despite the fact that current availability of ODs to patients (based on our analysis) is relatively good, there has not been a systemic approach how to assess ODs in respect of reimbursement procedure yet. Currently, most ODs are reimbursed on temporary basis in Czech (which can be possibly set up to 3 years); however there is a huge debate how to treat them after this period expiration. It must be noted, that all medicines reimbursed on a standard basis in the Czech Republic must fulfill criteria of being cost-effective under the acceptable threshold of 3 GDP/ capita per QALY gained.

Figure 4. Percentage in expenditures of particular OD of total expenditures on ODs in year 2010

