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Cooperation Policy of Rare Diseases in the European Union

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Abstract

4-5% of neonates are affected by rare diseases, 75% of which occur in children under ten years of age. This fact requires additional costs and special access. The aim of this paper is to analyze expenditures, policy and rare disease health care legislation (children comprise most of the patients). The survey focuses on the EU countries in comparison to the situation in the Czech Republic. The basic methods used are literature review, the analysis of available statistics data regarding the number of persons and related financial requirements for the treatment of rare diseases. The time series and histograms are generated.
Based on the analysis, it was discovered that there are no fundamental differences between the care of people with rare diseases in the EU and in the Czech Republic. Current legislation and activities at the EU level stimulate the member states (including the Czech Republic) to further support in this field. Suitable and appropriate conditions for the social integration of children suffering from rare diseases are created. Treatment of rare diseases involves significant financial costs associated not only with the drugs themselves, but also with the followed social integration. New methodologies and programs for this segment are formed in the EU and in the Czech Republic. These strategies set out a shared vision for improving the lives of all those with rare diseases. The focus throughout is patients and families.

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Keywords: Rare disease; costs; orphan drugs; European Union

1. Introduction

RD's are defined in the EU as affecting no more than 5 per 10,000 people. This low prevalence results into small or very small numbers of patients who therefore feel particularly isolated. The isolation felt by RD patients is not only geographical but also means marginalization within society at large and within healthcare systems designed for common diseases (EUROPEAN COMMISSION, 2012). Rare Diseases are a complex mix of heterogeneous diseases, currently numbering 5,000 to 7,000 in total. Up to 2009, one or more responsible genes were identified for only 2105

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of the over 6,000 rare diseases listed on the Orphanet website. For the vast majority of these diseases, no research is being conducted. There are only 395 patient registries across Europe and less than 150 rare diseases do have a marketed drug. Their heterogeneity means that research and therapeutic responses should be diverse and elaborated in each disease or group of RDs. In addition, for the same disease, symptoms can affect different organs or systems.

This is largely a hereditary or congenital disease, which has very low incidence (prevalence) in the population. A rare disease is defined in the event when it affects less than 5 people in every 10,000. In conversion it is less than 1 patient in 2,000. There are more than 8,000 rare diseases from which 80-90% are genetically determined. The main problem is that there are too many patients, but in the Czech Republic is a low number of specialised departments (in the future 10-20 departments are planned for development). The most common symptoms of these diseases are usually detected soon after birth, when 4-5% of newborns and infants are affected (e.g. some congenital defects, hereditary metabolic disorders, genetically determined diseases and rare cancers) (DOLEŽAL, T. & KLIMEŠ, 2013). 75% of rare diseases occur in children under ten years of age. A specific treatment is only possible for a small number of diseases. Because of this, approximately 1/3 of patients die before five years of age. Furthermore, they can also occur in childhood and adulthood. The neonatal screening is crucial for the diagnosis. In the Czech Republic there are 13 diseases examined and the introduction of screening significantly restricted the number of affected individuals.

"The strategy of the EU in the field of rare diseases aims to unite the limited resources that are currently across the member countries. Specific measures include (EUROPEAN COMMISSION, 2012):

- implementation of national plans for rare diseases in member states, whose aim is to ensure that patients with rare diseases have universal access to high quality care, including diagnostics, therapies and medicinal products for rare diseases (orphan drugs);
- ensuring research of the causes and treatments for rare diseases;
- gathering expertise on rare diseases at European level;
- strengthening co-operation with patient organisations for rare diseases;
- ensuring the sustainability of activities in the field of rare diseases.

All these areas are further developed at EU level within European legislation and incorporated into national health systems of different EU member states through national strategies. The National Strategy aims to initiate the process of creating functional and cost-effective systems for the treatment of rare diseases as well as the involvement of all stakeholders, including patients. This task has been implemented since 2011 also in the Czech Republic (EURORDIS , 2012).

The aim of this paper is to analyse the evolution of expenditure on rare diseases in recent years in EU countries, focusing on the Czech Republic, and answering the research question of whether a higher economic level of certain countries also has higher expenditure on orphan drugs. First, the basic economic characteristics such as GDP and the share of health expenditure to GDP in recent years will be compared. Subsequently, the attention will be focused on the actual cost of treatment of rare diseases. From the perspective of the needs of children with rare diseases, the requirements for integration of these children into the team will be specified.

2. Methods

The basic methods used are literature review, the analysis of available statistics data regarding the number of persons and related financial requirements for the treatment of rare diseases. The time series and histograms are generated.

3. Healthcare spending in the European Union and the Czech Republic

This paper aims to answer the research question of whether higher economic level is related to greater expenditures on the treatment of rare diseases. For this purpose, the evolution of GDP, healthcare expenditure and subsequently expenditure on treatment of rare diseases will be observed. So far there were fears that the cost of treatment of rare diseases will have a negative impact on the structure of healthcare expenditure, given their high level. On the other hand, as already mentioned above, the economies' costs relate primarily to the treatment and

subsequent care of children up to ten years of age. Due to the general attitudes of the EU, in terms of efforts for improving living conditions and the promotion of mutual solidarity, the expenditure on research and treatment of rare diseases are supported.

In terms of the development of one of the main macro-economic indicators – GDP, it can be said that the overall EU economic level is higher than in the Czech Republic. Moderate growth in recent years is typical for both comparator subjects (Figure 1). Within the EU, 18 Eurozone countries were selected, because for this group of countries there are indicators of healthcare expenditure available on the treatment of patients with rare diseases and later comparison is possible.

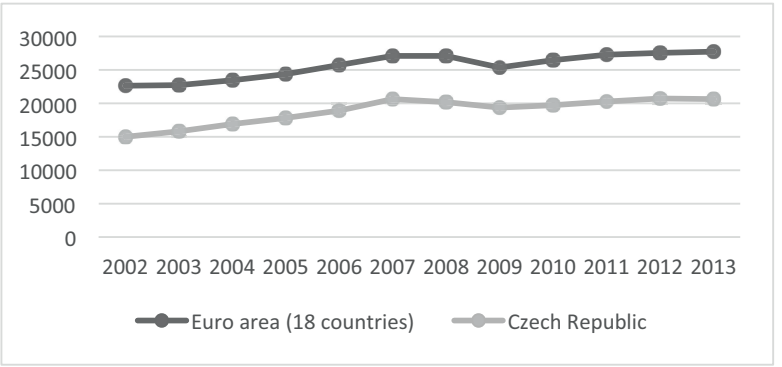

Fig. 1: Gross Domestic Product at market prices (Purchasing Power Standard per inhabitant)*, own processing according to (EUROSTAT, 2014)

Expenditure on healthcare make up 9-11% of GDP in the European Union, in the Czech Republic it is 6-8%. In both entities, there has been a slight increase since 1995 (Figure 2).

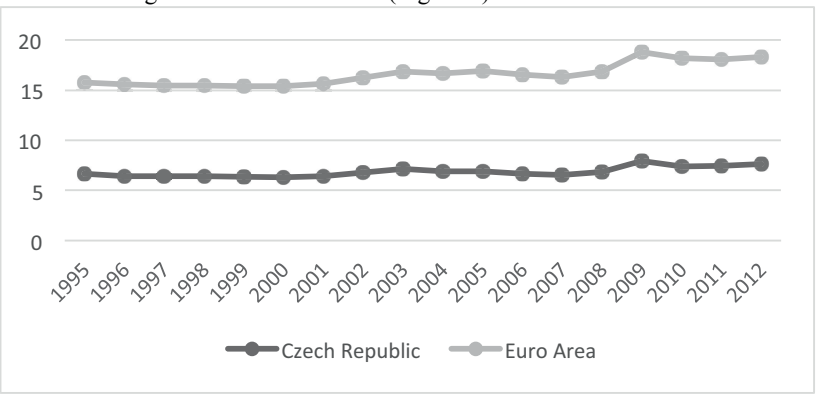

Fig. 2: Health expenditure, total (% of GDP), own processing according to (WHO, 2014)

From the point of both of these indicators, the Eurozone countries enjoy a better economic level.

* The Eurozone, officially called the euro area, is an economic and monetary union (EMU) of 18 European Union (EU) member states that have adopted the euro (€) as their common currency and sole legal tender.

3.1. Expenditure for the treatment of rare diseases

The annual cost of existing orphan drugs per patient ranged from €1,251 to €407,631 with a median per patient cost of €32,242. The budget impact of orphan medicines in Europe has grown steadily over the 10 years (European Commission, 2012). The share of these expenditures in total expenditures on drugs in healthcare is shown in figure no., which also shows the expected expenditure up to 2020 (Figure 3). Expenditure for orphan drugs should stagnate around 5%.

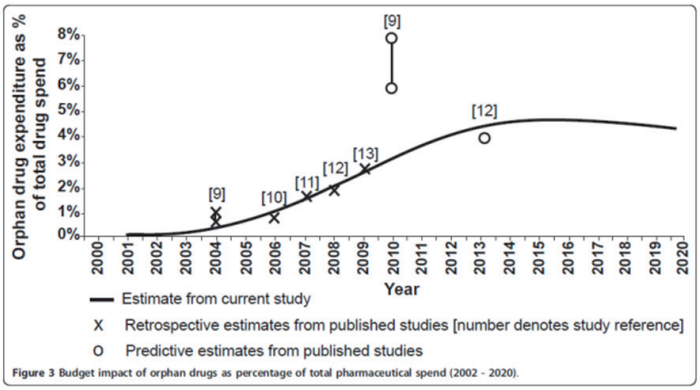

Fig. 3: The percentage of the cost of total expenditure on drugs in Orphan Drugs of the Eurozone, (Doležal, 2014)

The situation in the Czech Republic for the period available for comparison, i.e. the years between 2007 - 2012, corresponds almost identically to expenditures in the EU. In relation to the economic situation of the Czech Republic, it is a higher burden than in Eurozone countries (Figure 4)

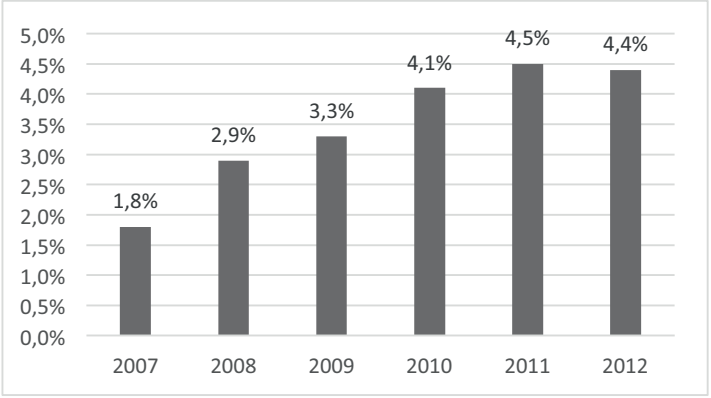

Fig. 4: The percentage of the cost of Orphan Drugs in relation to total expenditure on drugs in the Czech Republic, own processing according to (Doležal, 2014)

Over a thousand drugs in the research covers only 1% of illnesses and more than 75% of drugs are available in the Czech Republic. Currently all of the orphan drugs require additional payment (IHETA, 2012). In the Czech Republic it is approximately 600-800,000 patients. If we divide them according to the individual diseases, there would be hundreds of them, often dozens of patients, or even individuals within each diagnosis (LHOTÁKOVÁ & KUBÁČKOVÁ, 2013). In comparison with the European Union, the cost in the Czech Republic is slightly lower. Costs in the EU in 2010 accounted for 3.3% and the estimate for 2016 is 4.6%. Furthermore, the cost should not rise before 2020 but should actually decrease slightly. Sweden and France are around 3%, more precisely Sweden 2.7% and France 3.2%. Compared to the study for the EU and the Czech Republic, these countries are predicted to have a steady increase in the budget.

3.2. The potential of the market for orphan drugs

The development of orphan drugs may among other things mean a burden on the economic system. The present studies suggest that the development of these drugs brings higher profits than the development of other drugs (Figure 5). (DRUMMOND, 2008), (HUGHES-WILSON, 2012), (MEEKINGS, 2012),

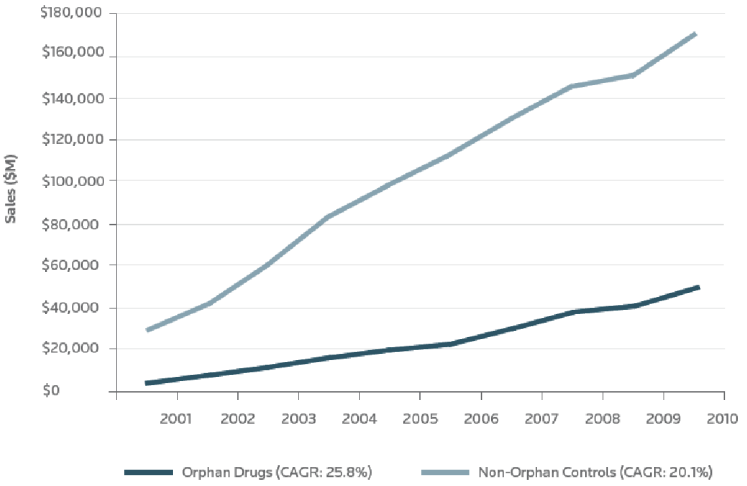

Fig. 5: Growth rate of orphan versus non-orphan drugs 2001 – 2010, (THOMSON REUTERS, 2012)

The compound annual growth rate (CAG R) of the orphan drug market between 2001 and 2010 was an impressive 25.8 percent, compared to only 20.1 percent for a matched control group of non-orphan drugs. This data, combined with the increasing number of orphan drug approvals, suggests that the CAG R of launched orphan drugs will outshine that of the non-orphan control drugs over the next 30 years (THOMSON REUTERS, 2012).

4. Needs of pupils with rare diseases

As has already been discovered, 75% of patients with rare diseases are children under 10 years of age. This fact is closely related to the issue of needs for students in the learning process. According to (MŠMT, 2002) the school that accepts a child with a rare disease, should meet certain minimum requirements. It's not just about the particulars of the administrative proceedings, architectural barriers and the possibility of providing special aids. It also concerns other aspects that affect the successful integration of a child with disabilities, such as the overall atmosphere of the school and the level (technical and human) of teaching staff members. The incorporation of these pupils is related to the creation of individual education plans and communication with their legal representatives.

4.1. Factors accompanying the education of a child with rare diseases at school

The adoption of a child with a rare disease into a normal class in a kindergarten, primary or secondary school, is still a more natural way of solving their educational needs.

For teaching staff the knowledge (and interpretation) of the following findings have great importance, which we can observe in many cases in families caring for a child with a rare disease (MICHALÍK, J. et al., 2012):

1. Concerns about the fate and health of a child, leading to greater caution.
2. Distrust and increased sensitivity to institutions and their representatives.
3. Strongly felt economic and social uncertainty.
4. Greater dependence on counselling and assessment service institutions and their staff.

5. Increased mental and physical fatigue.
6. Domestic support of the educational process.

Good and work with the above mentioned factors ultimately determines the success of this method of education. Education of pupils with SEP compared to the main educational stream significantly economically challenging because it requires an individual approach to a pupil, which is also regarded by reduced number of pupils in a classroom, higher demands on teachers or special education requirements and often also costly special aids to teaching. The competent authority of the county or territorial unit will provide funding for a school for additional costs associated with teaching this pupil and security their training needs. Even so, the care cannot manage without increased financial demands on the legal representatives.

5. Conclusion

The aim of this paper was to analyse the relationship between economic level of the EU and the Czech Republic (characterised by the development of GDP and healthcare expenditures) and expenditure on orphan drugs. The comparison of the development of basic macro-economic variables shows that the EU as a body has almost identical expenditure on orphan drugs as the Czech Republic, and despite the fact that the EU has a consistently higher level of GDP as well as higher spending on healthcare. The assumption of higher spending on orphan drugs in the case of higher economic level of countries was therefore not confirmed. In the Czech Republic in recent years there has been a concern regarding rapid cost increases for these drugs. In terms of predictions for EU countries and the Czech Republic, we expect a stop in the increase of the costs. Conversely, in countries where these expenses are lower than the Eurozone's average (Sweden 2.7% and France 3.2%) there is an expected increase. This also means that in the Czech Republic we do not need to worry about the rapid increase in costs because the limited number of existing orphan drugs in combination with small numbers of patients will not occur in the health system in the future by the way of a steep rise in the cost of treatment for rare diseases.

Currently, there are National plans for rare diseases, which include aid for research and drug development, strengthening co-operation with patient organisations for rare diseases, plans for the inclusion of students in the educational process. The limiting factor in the National Plan of the Czech Republic is the fact that there has not been any budget allocated for its implementation by the government nor the Ministry of Health. To what extent performance will be ensured depends largely on the initiative of professional institutes and physicians, patient organisations mainly working on a voluntary basis and the willingness of officials to dedicate to this issue.

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