Journal of Applied Biomedicine xxx (2018) xxx–xxx

Contents lists available at ScienceDirect

Journal of Applied Biomedicine

journal homepage: www.elsevier.com/locate/jab

Review article

Legislation, regulation and policies issues of orphan drugs in developed countries from 2010 to 2016

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ARTICLE INFO

Article history:
Received 8 October 2017
Received in revised form 9 December 2017
Accepted 26 April 2018
Available online xxx

Keywords:
Legislation
Policy issues
Rare diseases
Orphan drugs
Europe

ABSTRACT

The purpose of this study is to evaluate current legislative policies and regulations with respect to the use of orphan drugs, to emphasize strengths and weaknesses of these policies in the period of 2010–2016 in European countries, especially in relation to the objectives set by the European Council in 2009. This was done by the method of literature search in the databases PubMed, Scopus, ScienceDirect, and Web of Science from 2010 to 2016. The findings show a lack of polices for the appraisal and reimbursement of orphan drugs, a lack of uniform European legislation and different orphan drugs expenditure.
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Introduction

The term orphan disease was firstly coined by Melnick (1954), who called new viruses, which occur among patients suspected of having non-paralytic poliomyelitis, orphan viruses. An orphan disease may be a rare disease (RD) or a common disease that has been ignored (such as cholera or typhoid) because it is far more prevalent in developing countries than in the developed world. Its prevalence in Europe is considered to be less than five people in 10,000 (European Commission, 2012). Per capita it is less than one patient out of 2000. The same number is true for Australia. In the USA, the incidence of orphan diseases per capita is slightly higher, 1 in 1500, while in Japan it is 1 in 2500. Thus, surprisingly, RDs are quite common. In fact, they are more frequent than diabetes and as common as asthma (Elliot and Zurynski, 2015). Unfortunately, RDs are complex and chronic and only a small number of these diseases can be treated. Therefore, in other cases there is a long-term palliative treatment.

Nowadays, there are more than 8000 RD. 80% of them are of genetic origin, while the rest are the results of infections, allergies and environmental causes, or are degenerative. Although RDs are typical of both adults and children, their incidence is much higher among children since 75% of RDs occur among children till the age of 10 (Maresova et al., 2015).

In comparison with common diseases, these RDs are connected with complications such as high costs of treatment and legislative conditions. Currently, the highest costs of orphan drugs (ODs) on the total drug expenditure are in France, Belgium, UK, Spain, and Italy (Hughes-Wilson et al., 2012). For example, in their study, Denis et al. (2010), focused on the situation in Belgium and state that in the course of five years 2008–2013, the costs on ODs would increase from 5% to 10%. This increase is based on the estimation that 10 new ODs would reach the market each year. This would represent almost 4% of the costs of all drug reimbursements to the budget. These values are among the higher ones in the EU countries. In addition, the study by Kanters et al. (2014) describes the budget impact of ODs in the Netherlands in the period of 2006 to 2012. The findings show that the number of ODs, patients and costs significantly increased over a period of six years. However, the growth rate of public expenditure slowly decreased at the end of the reference period. In addition, the research studies conducted in Sweden and France predicted the growth of costs on these drugs (Hutchings et al., 2014). The main reason of these increasing costs was the amount of new Orphan Medicinal Products (OMPs) obtaining the OMP designation. Nevertheless, these studies illustrate that ODs in comparison with other drugs do not represent any immediate threat for the system of public health insurance. Despite this fact, the issue of expenditure and approval of payment from the public

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https://doi.org/10.1016/j.jab.2018.04.002
1214-021X/© 2018 Published by Elsevier Sp. z o.o. on behalf of Faculty of Health and Social Sciences, University of South Bohemia in Ceske Budejovice.

Please cite this article in press as: P. Maresova, et al., Legislation, regulation and policies issues of orphan drugs in developed countries from 2010 to 2016, J. Appl. Biomed. (2018), https://doi.org/10.1016/j.jab.2018.04.002

sources is being discussed in each country in connection with the threat of high expenditure.

Orphan product designation was first introduced in the EU in 2000 under the Regulation (EC) No. 141/2000. The regulation established the procedure for orphan product designation. Detailed incentives for products granted orphan designation and formed the Committee for Orphan Medicinal Products (COMP) which is charged with reviewing applications for the designation (Mezher, 2015).

RDs and ODs are a highly important issue. This fact can be illustrated by the current focus of the Maltese Presidency of the EU Council. They aim "to focus on enhancing cross-border cooperation through the promotion of centres of excellence, for example in the area of rare diseases" (European Commission, 2017). One of the major goals in the programme of Maltese presidency concerns health. Specifically, the programme states that "the Health Systems of Member States face common challenges which can be mitigated when Member States work together in synergy. The Maltese Presidency will work to identify mechanisms of voluntary structured cooperation between health systems driven by Member States, to further support Member States and provide tangible benefits for health professionals and patients. The two areas which have been identified are: structured cooperation to improve access to innovative health technologies for RDs in the EU and cooperation to address the EU health workforce challenges, with a focus on that associated with the provision of highly specialised health services" (European Commission, 2016). The main motivation stems, on the one hand, from the efforts to increase the quality of life and the related impact of treatment and, on the other hand, the cost of illness in the context of limited financial resources of governments in developed countries, including the expected impact of new treatments on public budget.

The purpose of this review study is, on the basis of available studies, to evaluate current legislative policies and regulations with respect to the use of ODs, to emphasize strengths and weaknesses of these policies in the period of 2010–2016 in European countries, especially in relation to the objectives set by the European Council in 2009.

Materials and methods

The methodology of this review study is based on Moher et al. (2009). The main methods included a literature review of the full-length research studies examining the issue of orphan drug legislation. This was done by searching databases PubMed (www.ncbi.nlm.nih.gov/pubmed), Scopus (https://www.scopus.com), ScienceDirect (https://www.sciencedirect.com/) and Web of Science (https://pcs.webofknowledge.com) from 2010 to 2016 for the keywords: *ODs* AND *European legislative*, or *ODs* AND *legislative or rare diseases AND regulations*. The search was limited to the years of 2010–2016 only because in June 2009 the European Council adopted the Recommendation supporting adoption of national plans and strategies for responding to RDs. The study was included if it explored the research issue, i.e. the OD legislation, if it was a full-length study written in English, and if it covered the designated period from 2010 till 2016.

Articles that met the inclusion criteria of the quality of research papers were evaluated according to adequate description of the theoretical framework, background, and methodology (Hutchings et al., 2014; Mays and Pope, 2000). For those papers that fulfilled the criteria for quality, data was extracted according to the following content: date published, study funding source, possible conflicts of interest, county, study objectives, application of tool, description of tool or approach, all stakeholders involved, literature search incorporated and results of implementation.

Altogether 622 were identified: in the database PubMed it was 282 studies, ScienceDirect 279 studies, Scopus 41 studies, and in Web of Science 20 studies.

The selection procedure of the final number of studies was done in the following four steps:

- Identification (identification of the key words and consequently; available relevant sources);
- Duplication check (114 were excluded);
- Assessment of relevancy (verification on the basis of the title – 211 excluded) and abstracts (203 excluded), only the studies which evaluate the present legislation, availability of drugs in individual countries, and political framework remained;
- Full text analysis.

The criteria for the content analysis were as follows:

- Studies for the last six years;
- Studies dealing with legislative changes of the development of distribution and sale of ODs;
- Studies focused on Europe and/or the USA as the main leaders in this field.

The selection process of the research studies is illustrated in Fig. 1 below.

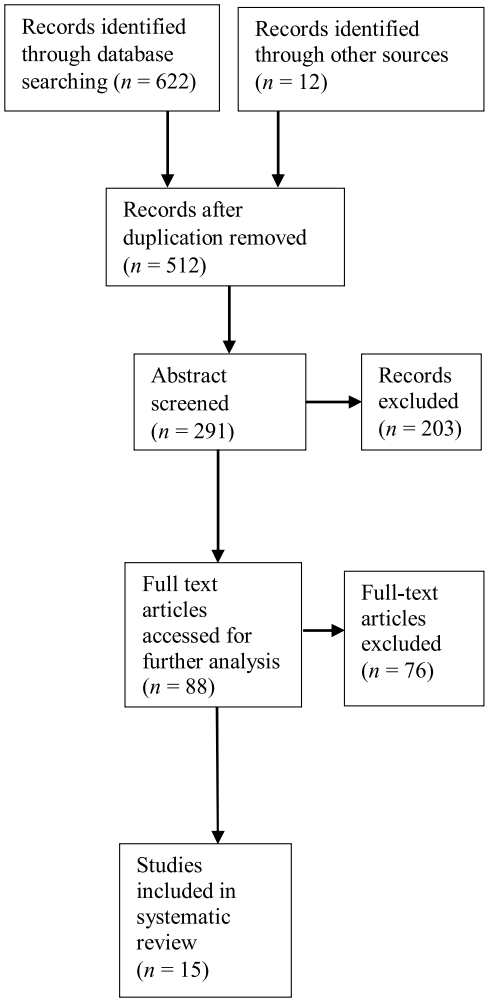

Fig. 1. Results of the selection procedure.

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