Research of orphan medicines regulation in different countries

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Introduction. Orphan medicines are medicinal products intended for the diagnosis, prevention, or treatment of rare diseases, which are diseases affecting less than 1 in 2000 persons in the European Union (EU). An «orphan medicine» is a pharmaceutical agent that has been developed specifically to treat a rare medical condition, the condition itself being referred to as an orphan disease. Pharmaceutical companies are unwilling to create such medicines under normal market conditions, as the expected sales of drugs would not recover the cost of bringing them to the market without providing incentives.

There are 8 000 rare diseases, most of them with a genetic basis. A very rough estimate would be that one out of 15 persons worldwide could be affected by an orphan disease – 400 million people worldwide, of whom 30 million are in Europe and 25 million in the United States.

The primary **objective** of this study was to analyze the orphan medicines regulation in different countries.

Materials and methods of research. Regulations governing the provision of orphan medicines and the data of scientific publications (PubMed) were studied. Content analysis, method of scientific generalization, logical, and comparative methods were used during the study.

Research results. The USA was the first country to implement a policy for the development of medicines to treat rare diseases with the Orphan Drug Act of 1983 and has since approved the most drugs via this pathway. The success of the original Orphan Drug Act in the USA led to it being adopted in other key markets, most notably in Singapore in 1991, in Japan in 1993, in Australia in 1997, and by the European Parliament of the Regulation on Orphan Medicinal Products in the EU in 2000.

At the first stage of the study, we highlighted the main provisions of the Orphan Drug Act of USA, among which protocol assistance to design research protocols; tax credits for clinical research; 7 years of market exclusivity; funding grants for clinical research to support development (up to $30 million per year in R&D grants provided for phase I through III clinical trials); up to one half of research and development costs can be recouped through tax credits; penalty for intentionally false statement of orphan status; parallel track program and treatment investigational new medicines provide access to unapproved medicines; process patents granted for biotechnology products; accelerated approvals.

At the next stage, we highlighted the main components of regulation in the EU, among which development of a telematic network to facilitate clinical trials and research; legislation is already in place providing market exclusivity during ten years and provisions for fee exemptions under cost recovery; many member states already have incentives in place for research and development related to orphan diseases; individual member states control access to drugs through their programs (France, United Kingdom).

Further, we highlighted the main components of regulation in Japan,

among which grant program for research and development for manufacturers and importers of orphan medicines; guidance and advice available to industry on both research and development and new medicine application procedures; tax incentives granted to manufacturers doing research and development on orphan medicines; new medicine application for orphan drugs are given priority review; 10 years registration validity period (also known as re-examination period); if the medicine is marketed, a portion of profits over 100 million yen must be paid to the government.

Among the main components of regulation in Canada we highlighted scientific research and experimental development tax incentive program would support research and development in the area of orphan medicines; provision for the reduction of fees for small market drugs under cost recovery; process patents granted for biotechnology products; conditional approvals proposed under a new licensing framework.

Conclusion. Orphan medicines legislation in all countries we analyzed aims at providing incentives for pharmaceutical companies to develop and market medicinal products to treat rare diseases. Financial incentives by law include orphan drug exclusivity during the period of marketing exclusivity, and the regulatory bodies are barred from approving the same product for the same orphan indication. A product holding several separate orphan designations for different indications can have several separate market exclusivities, which can run concurrently. The financial incentives provided to pharmaceutical companies have increased rare disease research and drug repositioning opportunities.

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