PHARMACY SECTION

1. LEGISLATIVE REGULATION OF ORPHAN DRUGS IN CERTAIN COUNTRIES

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Introduction: The rare diseases that are treated with orphan drugs is a global problem of XXI century. The limited health insurance budgets and economic inability of patients with rare diseases to pay for medications, improves to revise economic affordability and physical accessibility of orphan drugs through reimbursable drugs system of national compulsory health insurance company.

Materials and methods: A descriptive analysis of orphan drugs legislation in different countries in order to determine potential methods of adjusting the normative legal framework in the Republic of Moldova (RM) has been carried out.

Results: The low prevalence is the basic characteristic that describes rare disease. In the European Union (EU), rare diseases are defined as life-threatening or chronically debilitating diseases with prevalence of 5 out of 10,000 individuals or less. The EU defines an orphan drug as either a medicinal product intended for a life-threatening or chronically debilitating rare disease or a medicinal product that would not be developed without incentives because its sales are unlikely to generate sufficient return on investment. An additional requirement to qualify a drug as orphan as orphan drug is the absence of a satisfactory method for the diagnosis, prevention or treatment of rare disease or, if there is such a method, the drug will have a significant effect to the patients suffering from the disease. Availability of orphan drugs presents obstacles caused by the decision-making process of national health insurance companies on their pricing and reimbursement. There are about 8,000 known rare diseases in the world, 75% of those affecting children. While the diseases are rare, patients are numerous, especially children. As a response to this situation, states impose programs to stimulate the development of orphan drugs. In 2000, the EU introduced new legislation Regulation (EC) No 141/2000, which establishes a centralized procedure for orphan drugs designation and implement incentives for research, development and marketing for them. Pharmaceutical companies with a designation in this area, take a great advantage: tax exemption, market exclusivity for a period of 10 years for designated products, scientific support for marketing authorizations and the possibility of a Community marketing authorization. In the U.S., after confirmation of an orphan drug, Official Development Assistance offers three main incentives: federal funding of grants and contracts for clinical trials, tax credits for clinical trial costs; granting the exclusive right to market the orphan drug for seven years. In RM National Health Insurance Company, beginning with 2013, introduced in the list of reimbursed drugs 8 medications intended to ameliorate the suffering of patients with rare diseases: epidermolysis bullosa (methylprednisololum, clemastinum, desloratadinum), myasthenia gravis (pyridostigmidi bromidum) autoimmune system diseases (prednisolonum, methylprednisolonum, methotrxatum) and mucoviscidosis (pancreatinum).

Conclusions: Orphan drugs are the most accessible ways to ameliorate the health of patients with rare diseases. The results of this study emphasize the worsening of this problem, especially in middle country, such as RM. In this context the present study will continue with a complex analysis of the current situation in RM.

Keywords: Orphan drugs, rare diseases, reimbursement system, reimbursable drugs