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The Economy of Rare Diseases: Theory, Evidence and Public Policies

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Objectivos (Objectives): The main objective of this paper is to review theoretical and empirical research on the economics of rare diseases and orphan drugs trying to analyze their economic implications. The secondary objective is aswer: How are defined the rare diseases, what are the implications of this for the formulation of public policies and why they are considered a public health problem? What are orphan drugs that produce them and what economic incentives for their production? What are the effects and implications of current legislation, especially the Orphan Drug Act (ODA) of 1983 in the United States and the EC Resolution No. 141/2000 the European Union? They have been effective?

Metodologia (Methodology): Through evidence presented in the literature, the paper will go identify the magnitude of the problem, its current importance and describe the government incentives and tools used for developing a treatment for rare diseases.

Resultados (Results): Rare disease is a illness that presents a low prevalence in a given population. They are a public health problem that affects millions of people around the world. They are usually degenerative, chronically debilitating and require long-term treatment, affecting the physical, mental, sensory and behavioral patient. Orphan drugs are medicines used for diagnosis, prevention and treatment of rare diseases. The rarity of cases implies difficulties for proof of clinical efficacy of these medicines. Are presented data on rare diseases and orphan drugs in Brazil and around the world, as well as the key economic considerations related. Are presented the regulatory systems for rare diseases in the United States and the European Union and how are the influence of these mechanisms on the development of orphan drugs.

Conclusões (Conclusions): It was concluded that the regulatory mechanisms are able to stimulate the development of new kind of drugs to treat of rare diseases. In general, the incentives provided for development policy of orphan drugs represent a kind of pro-market regulation. Such mechanisms are able to encourage companies to develop drugs that would not be produced under normal conditions, which can be proved by the increasing availability of treatments based on the implementation of these regulations. From the analysis of the evolution of the number of orphan drugs available on the European and U.S. after the adoption of legal frameworks, it was concluded that the policy is effective. However, It is necessary to intensify the debate on rare diseases in Brazil, since there isnt a public policy to facing this problem in this country.