

The political construction of orphan drugs market: between innovation and access to care

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Abstract

Health care systems have both a crucial role in the access to care and in insuring profitability of innovation, so they have a role in health care policy but also in industrial policy. At the same time, the so called blockbuster business model, shaped by the specificities of US health care systems have some troubles, constraining *Big Pharma* companies to find new markets and sources of profits. Orphan drugs, i.e drugs dedicated to treat rare diseases, are at the same time a solution and a problematic for *Big Pharma*: a problematic because of the size of the market, which normally is a problem for obtaining a return on investment and then does not offer a lot of incitation to innovate, and a solution if health care systems reimburse these drugs at a high price and guarantee their access for the population. 'Rare disorders' is the name given to those diseases, of very varied aetiology, whose common denominator is that they are low-prevalence diseases, and for the majority of which there is no treatment available. They are frequently life-threatening or chronically debilitating and the impact on the quality of life of affected patients and their family members is thus significant. About 5,000 identified diseases are classed as "orphan" because of the lack of any response in terms of diagnosis, prevention and treatment. However, drug development for these conditions has been limited by a lack of understanding of the underlying mechanisms of disease and the relative unavailability of subjects for clinical trials, as well as the prohibitive cost of investing in a novel pharmaceutical agent with poor market potential. To encourage the development of such drugs for rare diseases, orphan drug legislation came into effect in the European Union on 2000, and are widely inspired from the American model where a specific legislation on orphan drugs dates back to 1983. The introduction of this Orphan Drug status brings important incentives for the development of orphan by the pharmaceutical companies. The European Union understands a rare disorder to be one with a prevalence of 5 : 10 000 Europeans; the USA defines it as an ailment affecting fewer than 200 000 Americans. Designation as an orphan drug is clearly different from marketing authorization. Criteria for the designation as an orphan drug are the low prevalence of the disease, severity of the disease and the expected significant benefit for the patients. Incentives include fee reductions and up to ten years market exclusivity. With the assignment of more than 500 orphan drug designation by the EMA, the regulation on orphan drugs have contributed to the development of many innovative biotechnology products to patients with rare diseases. Indeed it is anticipated that pharmaco-genomics will result in the identification of more 'orphan diseases'. The increased availability of orphan drugs with their high cost, and potential for long-term administration raises important debate surrounding their affordability and cost-effectiveness, and so their public funding by national health services. If European Union (EU) regulations promote the development of orphan drugs; but to contain costs, some EU healthcare systems assess the cost-effectiveness of therapies when deciding if they should be funded. So there are numerous gaps in transition from science into orphan drug development as important bottlenecks that exist in several European countries. Therefore mechanisms for creating a policy that would reduce geographical inequalities in provision across Europe would be necessary in the future. While the European orphan drug act is encompassing its 10th anniversary in 2010, we might ask several questions: does the OD act

stimulate the production of truly non-profitable drugs? Does the orphan drug legislation is the real cause of the increase of new OD on the market? OD market is a new strategic opportunity for big pharmaceuticals companies? Then, we have to consider that markets do not exist *a priori* but are a social and political construction designed by a political work of companies, patients, experts and State to define the pre-existing conditions and the rules of the game of the markets (definition of property rights; quality...). As a political construction, it is a product of conflicts, where firms are trying to shape the rule to their own advantages. After presenting our theoretical grid based on institutionalist approaches for analysing construction of markets, we study the social construction of orphan drug market in Europe and the industrial strategies of some companies involved in this market, using at the same time analysis of political work of some stakeholders, and patents, marketing authorisation and financial databases to study the business strategies of the firms. So, to understand this specific market we undertook a study looking through different levels. In order to do so we are building a knowledge database crossing patents, drugs safety reference and epidemiology statistic as well market trends. The characteristics of OD designations, approvals, sponsors, and evaluated the effective patent and market exclusivity life of orphan new molecular entities since the EU Orphan drug act funding in 2000 will be characterized. In the same time, we are looking at legal and social policy regulations especially public programs, EU regulations, research design support, tax incentives, market exclusivity and innovation policies supporting orphan drugs R&D. The importance of cooperation between academic institutions, pharmaceutical companies, patient advocacy groups and society in the OD innovation for rare diseases, should be highlighted. Preliminary observations will be presented regarding the organization of this market with a comparison with the US. We also analyze case studies of firms, showing the diversity and the transformation of business models related to the development of orphan drugs: we are showing the potential emergence of a new conception of control based on niches and personalized medicine and the use of orphan drugs to sustain and restructure *Big Pharma* and blockbuster business model.