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Case Report

ORPHAN DRUG POLICY

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ABSTRACT

Pharmaceutical industry is crucial for mankind and contributes in some way in socio economic development of the society through jobs, community welfare, and supply chains. Indian pharmaceutical companies are one of the largest markets in the world, estimated 10% of global production and 2% of world market. The industry is has shown significant growth in infrastructure development, producing wide range of pharmaceutical products with new technical advancements. The country is famous for producing and providing pharmaceutical products at much cheaper prices than the US and EU. However, the country lacks in investment in research on rare disease or orphan drugs. This paper tries to highlight the ways in which the market in orphan drugs can grow in India with the help of international partners.

Objective: The objective of the study was to look at existing orphan drug policies and how we can aim at making it more equitable in India.

Methods: Research is completely based on secondary data from online journals and government data. The study is analytical in its approach and is descriptive in nature.

Results: Pharmaceutical companies invest less in Orphan drugs as they do not mark an assured profit with present investment.

Conclusions: To maintain the interest of Indian pharmaceutical companies it is suggested that, Indian companies should work in collaboration with countries which lead in orphan drug markets.

Keywords: Orphan Drug, rare disease, pharmaceutical sales, government policies.

INTRODUCTION

Millions of people in developing countries do not have access to health-care services and medications which are available in developed countries. Most of the rare diseases are genetic in nature. They disproportionately impact children: 50% of new cases are in children and are responsible for 35% of deaths before the age of 1 year, 10% between the age of 1 and 5 years, and 12% between 5 and 15 years [1]. However, the most neglected diseases by pharma industries are tropical diseases that are caused by parasites and spread by insects or contaminated water or soil, for example [2]. Development of orphan drug policies is least concern of pharmaceutical companies. They work on the calculation of number of vaccines manufactured on cost of per vaccination. Market is a precious venture for pharmaceutical companies. They are hesitant on the sale of the product. Development of such drugs relies heavily on intellectual property rights to ensure the return of money invested on it. However, in India, Trade-Related Aspects of Intellectual Property Rights (TRIPS) has been introduced but has not been enforced as big companies provide medicines at cheap prices [3]. Therefore, the paper tries looking at policies that can make pharma companies to invest more on the research of orphan drugs.

DISCUSSION

Rare diseases are chronic and life-threatening illness that requires proper long-term medical treatment. As these diseases are rare, so the development of medication requires considerable amount of fund which affect the family of a patient suffering from rare disease. Thus, Government of India asked Ministry of Health and Family Welfare to draft a policy on treatment of rare disease because of the above-mentioned reasons. Hence, the Ministries looked at multisectoral approach to prepare India to tackle the problem of rare disease in areas like estimating the number that can affect or had affected the person, research on medication and diagnostic of disease, awareness, training of health caregivers, and affordability of treatment. Government of India recommends policies which mark a balance between the affordability of treatment and sustaining of medical research centers. This paper also marks a shift from health economics to managerial perspective by looking at societal values of orphan drugs in a realm of sustainable development from two categories: (1) Social equality and (2) inclusiveness perspective through the example of COVID-19.

If we apply international estimation of 6–8% people getting affected by rare disease, then in India, it has affected 72–96 million people which is a significant number [1]. The economic burden in such scenarios for rare diseases left unknown which puts a challenge in estimating the funds required for research.

However, Hughes professor at Bangor University stated in his study that companies with orphan drug authorization have more investment and profitable opportunities that non orphan drugs; as incentives related to research reduce marketing cost and premium pricing. In support to the argument, orphan drug market grows at a compound annual growth rate [4]. The market is expected to reach $178 billion by 2020 and Defrag account for 19% of total branded prescription drug sales [5].

The major pharmaceutical sales originate from the US, EU, and Japan. Together, they account for 80% of global market. The U.S itself accounts for 45% of pharmaceutical sales, then Europe 24% and Japan 11%. Further, the cost of treatment and medication is much expensive in the U.S; and orphan drug cost per patient in the U.S is 4.5 times higher than non-orphan drug cost. In recent times, the median between the price of orphan and non- orphan is reduced by 50% in the U.S. This shows market exclusivity. Companies working on coronavirus treatment are registered as rare disease treatment with the US regulatory, a status which is worth million in tax breaks. Celgene, Abbvie, and Johnsons & Johnsons are the leading companies of orphan drug market [6]. Gilead Sciences comes on 13th position accounting for $0.4 billion worldwide orphan drug sale in 2018 with 0.7% market share. They have been successful in making an experimental drug remdesivir for treating coronavirus and have also taken permission from Food and Drug Administration to use it on patients suffering from COVID-19. In addition, more than 50 drugs have

been made to cure coronavirus patients worldwide. The emergency for inventing a vaccine for COVID-19 is because it has spread across borders, including countries with poor health system.

In India to stimulate research regarding rare diseases and development of its medication, authorities and ministries have incentivized biotechnology and health industries. To accelerate the research for orphan drugs countries like India have provided incentivization to pharmaceutical companies that is, including tax benefits and have increased the period of market exclusivity to maintain the interest of the companies. About 70% of domestic demand for bulk drugs is delivered by the Indian pharmaceutical industries. With 40% of total pharmaceutical products are exported, out of which 55% of total export constitute formulation and 45% consists of bulk drugs. "The Indian Pharmaceutical Industry is one of fastest emerging international center for contract research and manufacturing services or CRAMS." The reason for its emergence is international high quality with low cost. India has biggest number of the US Food and Drug Administration. The manufacturing cost of drugs is half of the price than in the U.S., and the cost incurring for research is 8 times more in the U.S. This indicates India's service deliverance globally at lower cost [7].

It is crucial for Indian pharmaceutical companies to work in collaboration with the U.S registered rare treatment pharma companies as their market investment will help Indian pharma companies to conduct research and develop vaccines at lower price. However, policy is always made with an understanding that people will look for good health care and services. Pricing of medicines is crucial for consideration while developing a policy. Lower cost will directly affect the cost of the treatment. Furthermore, product patent has been introduced in the country. Furthermore, a "Pharmaceutical Research and Development Support Fund with a corpus of Rs.150 crores has been set up under the administrative control of the Department of Science and Technology" [8]. To encourage pharma companies to work on orphan drugs, it is important to increase time for renewal of orphan drug, for example, person who is eligible for grants, for example, in Japan orphan drug applicant receives consultation from Ministry of Health, Labour, and Welfare for free, increase in time period of product renewal for orphan drug, and government support doctors to use the product [4].

Government has always allowed smooth functioning of pharma companies. These pharma companies have enjoyed trade surplus since 1987, because of which it was expected that Indian pharma companies will increase the manufacturing of generic drugs worldwide. Their effort to increase export some of the pharma companies has established their marketing and trade supporting centers in developed countries and launched generic segments as cheaper alternatives to branded products. At national level, as policy-making ought to involve level of ministries, it is highly recommended to look on the smooth implementation mechanism. For example, corpus funds should not only be made available by center and the state government but also by local authorities for easy access by local health centers [9]. Furthermore, government needs to set up its own diagnostic laboratories and research departments to create epidemiological data. Better infrastructure leads to a development of the department. More focus needs to have on biogenetics as they account for 50% of orphan drug market globally. Furthermore, revision on drugs and their usage need to be done1. All these recommendations can help in striking a balance between pharmaceutical companies to work for orphan drug.

CONCLUSION

The scenario of orphan drugs stands differently in developing countries like India than in developed countries. The issue is approached differently as nature of this practice is linked with high costs with fewer returns. Government approach to it through incentivization has led a paradigm shift in pharmaceutical companies toward orphan drugs. Nevertheless, the lack of interest of Indian medical institutions toward orphan drugs has led poor awareness among public. To tackle the problem of interest of pharmaceutical companies in investing in orphan drugs, government established legislation as well as provided corpus fund to reduce premium pricing of drugs. This will make the vaccine available for people who cannot afford it. However, working with companies worldwide will help in the development of medicines at lower cost.

1. *Products withdrawn from the market for economic or therapeutic reasons.* Few drugs which are withdrawn from the market for some reasons, e.g., thalidomide widely used as a hypnotic drug some years ago for its high teratogenic (triggering fetal malformations) risk may show a very interesting therapeutic application, i.e., analgesic proprieties in rare diseases such as leprosy and lupus erythematosus. These are diseases for which no satisfactory treatment is available (Sharma, Jacob, Tandon, and Kumar, 2010).

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