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Will rare diseases remain orphan?

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Despite medical advancements, rare diseases are still a common occurence in India. Rare Diseases India, an organization that focuses on providing scientific data concerning rare diseases and disorders, estimates that around 72,611,605 people in India are suffering from a rare disease. About 6,000 to 8,000 rare diseases, mostly genetic in nature have been identified across the world and only around 500 of them have known therapies. India is the second highly populated nation in the world and hence even the rarest of diseases will have many patients seeking treatment.

Considering the fact that the present government's priority is to address demanding health issues, its attention to the nebulous rare diseases area, barring a handful of rare diseases, has been non-existent. The need for orphan treatments in India is huge and with little in place so far, India represents a lucrative market. Most of these disorders are genetic in nature and timely treatment can assure the victims of a healthy life. The disorders, once rare, are now affecting larger populations, with one in 5,000 suffering from a rare genetic disorder. Currently, drug developers in India are receiving no formal incentives from the government. Hence, orphan drugs need to be imported, which in turn makes them highly unaffordable. Lack of a policy or a legal framework in these countries is deterring the development of these drugs and thus many rare disease patients die. The increasing number of people falling prey to these diseases has reiterated the need for a sound legal policy and framework to support drug development for diseases.

In an exclusive interview with BioSpectrum, Mr Sandeep Sahney, head-intercontinental, Genzyme said, "A major challenge in bringing new orphan drugs to the market is the ability to garner a share of voice at a societal level for the 'few' patients. The other challenge is creating support from governments and lack of an appropriate policy to create a platform to address these diseases."

For long, the Orphan Drug Act (ODA) has been considered a key game changer that can alter the dynamics of orphan drugs and rare diseases in any country. The ODA, formulated by the US in 1983 can be rightly termed as the catalyst that fostered the development of these drugs. Under this Act, orphan drugs enjoy many regulatory and commercial incentives such as tax credits, grants, waived FDA fees, reduced timelines for clinical development and higher probability of regulatory approval, coupled with commercial drivers such as premium pricing, faster uptake, lower marketing costs, and longer market exclusivity. Only five Asian countries have implemented this Act -Australia, Japan, Korea, Singapore, and Korea. However, countries with a huge population like China and India do not have a policy that can attract global players to show corporate

interest in this area. If the governments in these countries do not formulate a policy to address these rare disorders, orphan diseases will unfortunately remain orphan.

"In the case of Orphan Drug Development," explained Dr AKS Bhujanga Rao, president, technical/R&D and director, Natco Pharmaceuticals, "more than the medical advancements, it is the government's initiative, the concerted effort by the medical and pharmaceutical companies, government, academia, and research organizations that can create a difference. The government act can trigger tremendous response for orphan drugs given their high demand and market value."

Need for legal framework

Since the introduction of ODA in a few countries across the globe, nearly 1,100 drugs and biological products have been designated as orphan products and many have received approvals. According to statistics from the FDA, from the time of authorization of the Orphan Drug Act in 1983 in the US, until end 2013, Orphan Drug designation has been awarded to 2,843 compounds, and of these, there have been 442 approvals for Orphan indications.

Speaking to BioSpectrum, Dr Meenakshi Bhat, consultant in clinical genetics at the Center for Human Genetics in India accentuated, "India never had enough awareness and organized approach towards rare diseases. We have the talent and the money, however, it is not coordinated into actual deliverables. We have the largest biotech companies in India. Many are interested in investing in rare disorders R&D. But the moment they think in terms of marketing and business, which is where the hesitancy is."

Stating that the Orphan Drug Act will help in easing logistics for orphan drug imports as well as help in bringing new drugs into the market, Mr HarshaKarur Rajasimha, co-founder, Organization for Rare Diseases India (ORDI) stated, "Target pools are very high in India as compared to the US where the number of patients is almost three
times smaller. Due to the lack of any legal framework or policy that encourages the manufacturing of these drugs in India, we import them."

Orphan drug development provides ample opportunities for research and development and reducing the disease burden of the society, thus improving healthcare of the country at large. However, without a proper system and guidance from regulatory bodies or pharmacological associations, it is challenging for a pharmaceutical company to invest in the development of orphan molecules from scratch. It is important to integrate orphan drugs into the healthcare system of India and allocate adequate finances to aid reimbursement, as most of the drugs are very costly and many patients cannot afford them.

Mr Ketan Patel, chairman and managing director, Troikaa pharmaceuticals, highlighted, "Orphan drugs have to be kept out of the ambit of price control for the simple reason that economics of manufacturing of such products demands so. Besides, no company will be motivated to spend on developing an Orphan Drug, which, after a tedious development effort, will be brought under price control subsequent to its launch."

Challenges in Orphan Drug Space

There are many challenges faced in the orphan drug space. There is little known about the natural history of rare diseases and hence diagnosis and development of a drug is a huge challenge. There are over 7000 rare diseases which occur over a broad spectrum of illnesses that have little or nothing in common with each other. The disease affects a very small population of the society and mostly young children and hence structuring clinical trials is very difficult. "It is extremely challenging to find patients in required numbers to participate in clinical trials," observed Mr Sahney, "This makes recruitment of volunteers really tough. While the number of patients required for CT may be small, the costs of running the trials are high since these patients are spread far and wide. Additionally, there are challenges on reimbursement at 'right' pricing that surface across potential markets. Hence, without an ACT that addresses these challenges, it is impossible to spur the innovation of orphan drugs in India."

Many a times the disease is localized to one country and is not prevalent in other countries. Hence, without adequate financial assistance by the government, pharma companies will reap very little or no profits through their orphan business. In such circumstances, scientists, clinical practitioners, pharmaceutical companies, and most importantly non-governmental organizations including disease support groups should contribute and pool re
BioSpecial sources, however small, to highlight the general issues of rare diseases.

Mr Sahney said, "At present, the provisions to encourage Research & Development in rare diseases range from 'no provisions' to very 'limited provisions'. These are essential for incentivizing both R&D and reimbursement pathways for drugs for orphan diseases."

Looming patent cliff- An advantage?

As reported by Total Biopharma, it is estimated that biological patents worth $67 billion is due to expire by 2020. With many blockbuster drugs losing protection, big pharma companies have now gravitated towards specialty drugs, orphan drugs, and the biologics market.

If proper legislation and framework is enacted, orphan businesses may swell in this patent pool and thus can eventually treat many potential rare disorders.