PHARMA

Pharma industry seeks financial assistance from government for rare diseases

National level pharma associations organised a virtual meeting to discuss challenges, opportunities in this segment

By **Usha Sharma** On **Aug 2, 2020**

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With an aim to drive research and development activities in the rare diseases segment and encourage orphan drug development in India, industry stakeholders have sought government support in terms of sustainable research funding, tax exemption in orphan drug development, unified definition of rare disease etc.

Recently, five national pharma associations i.e. BDMA, IPA, IDMA, FOPE and OPPI, along with a patient group called Indian Organization for Rare Diseases (IORD), conducted a webinar on the need for essential medicines for rare diseases. During the

virtual conference, regulatory experts, industry stakeholders and the patient group discussed steps which need to be taken to make orphan drugs accessible and affordable in the country.

This year in February, the Ministry of Health and Family Welfare had released the draft national policy for rare diseases for public comments.

Commenting the need to ensure that rare diseases get equal focus from all fronts and stakeholders, Dr VG Somani, Director Controller General of (India) stated, “The draft guidelines for compassionate use of medicines will be getting finalised soon. And to provide regulatory assistance to patients affected by rare diseases, the authority is in touch with different research groups and patient groups. It is also working in a direction to ensure Indian population also gets participation in global clinical trials so that the country will have its own data for research understanding.”

He also appreciated the fact that all the associations have jointly come forward to discuss the issue in detail with a single objective of creating treatment avenues for rare diseases.

Dr Ramaiah Muthyala, President & CEO, Indian Organization for Rare Diseases, gave an overview of rare disease scenarios, not only in India but across the globe.

He pointed out that the definition of rare disease differs between ICMR and CDSCO and stressed on the unification of ‘rare disease’ definitions to assist the policymakers in drafting the document describing incentives for the pharma industry.

Dr Ramaiah also informs that due to a small number of rare diseases patients, public health professionals do not include rare diseases into public health programs. However, he argues that because the burden of rare diseases far exceeds the combined burden of tuberculosis and malaria, and besides, they are lifelong diseases, rare diseases should be a public health priority.

Dr Ramaiah mentioned that the call for creating a model essential medicine list for rare diseases came from Rare Diseases International, a global alliance to ensure the achievement of Universal Health Care that includes rare diseases and leaves no one behind. During his presentation, he raised the challenges that would face the expert committee recommending orphan medicines in the WHO model list of essential medicines, considering their smaller population, lack of accessibility of inexpensive medications for low- and middle-income countries.

Dr Ramaiah states that rare disease patients have equal rights and opportunities in the area of the diagnosis, treatment, and social services, which are enjoyed by patients with common diseases. He urges the Government of India to acknowledge the challenges faced by people living with a rare disease, on the top of the misery caused by the COVID-19 pandemic and the unfairness within the current system towards rare diseases patients.

Sudarshan Jain, Secretary-General, IPA highlighted, “There is a need to create a corpus fund, which will support the research and development activities of rare disease. Along with this, there is also a need to create a patient registry, which will give the exact number as well as help in conducting the research.”

He also added, “We need to have a centre of excellence dedicated to rare diseases. Understanding the amount of research and development work required in this segment, to begin with, a minimum four such centres should be created. Also, considering several aspects which have a direct impact on patients affected by rare diseases like social stigma, economic burden, etc., there is also a need to create a rare disease cell in the health ministry which will dedicatedly work in this area. Besides, there is an immense requirement to create awareness programmes at the doctors’ level as well as including them in medical education.

BR Sikri, Chairman, FOPE, suggested, “Considering the amount of research work as well as capital cost which is required in carrying the research and development activities for developing orphan drugs, the industry looks for support from the government and proposes that it should consider exempting such drugs, R&D activities from different tax structures. The industry is of the opinion that it will encourage companies to carry out R&D under their CSR activities and help society.”

Venkat Jasti, Managing Director, Suven Life Sciences, emphasised, “To encourage the pharma industry to carry out research activities pertaining to rare diseases, the government needs to bring a policy assisting the industry financially. Along with this, the government also needs to come forward with some mechanism which will ensure free treatment as well as diagnosis of rare diseases.”

He also felt that there is a need to create robust awareness programmes as well as genetic testing to deal with rare diseases in the early stages itself.

Highlighting the challenges in this arena and recommending a few measures to increase access to orphan drugs in the country, Dr Viranchi Shah, National Vice President, IDMA, mentioned, “There is a need to create a fast track approval system for handling of orphan drugs applications. Along with this, if the office of CDSCO makes a provision in the clinical trial rule, especially for volunteers of orphan drug clinical trials to get access to those drugs from the innovator company, it will help the community in a much larger context.”

He continued, “Besides, if the drug approving authority can also frame guidelines and give the approval to the orphan drug manufacturing company to launch the product within a stipulated timeline or allow compulsory licensing to other companies, it will certainly reduce the burden and benefit the patient significantly.”

“Considering the data availability of rare disease patients’ in open source platforms, if the IT and pharma industries work together, with government support, in generating the data for research purposes, then the joint efforts will lead in the right direction with positive outcomes,” expressed Shah.

KG Ananthakrishnan, Director General, OPPI urged, “To understand the depth of requirements in each and every aspect related to rare diseases, there is a need for multi-stakeholder partnerships between the pharma industry, academia, medical professionals as well as diagnostic players. Along with this, there is also a need to create sustainable funding to increase R&D activities along with early diagnosis.”

He said that ambiguity related to the definition of rare diseases in India also should get cleared. He also suggested that an ICMR-initiated patient registry should be made live and updated regularly. Another recommendation was that drugs related to the treatment of rare diseases should be exempted from all kinds of duty and tax structure.

V V Krishna Reddy, National President, BDMA also opines if the pharma industry gets financial assistance from the government to carry out research for rare diseases segment, then many companies will come forward to bring new drugs for the treatment of these diseases.

Mahesh Doshi President IDMA said, “We have recommended that the amendment should be extended to include rare and tropical diseases to encourage R&D and manufacture of drugs for such essential requirements. In this regard, we must note that pharma R&D is very expensive and time-consuming as compared to other

industrial research, on account of regulatory requirements of safety, efficacy and quality. Sustained R&D efforts by Indian companies have enabled the Government to address India's needs on tropical/country-specific diseases like TB, Kala-azar, dengue and other life-threatening inflictions like cancer, asthma, diabetes etc."

Thereafter, he added, "It is time for India to notify the national policy for rare diseases to pave the way for greater funding and mechanisms to support R&D innovation. Creating an enabling environment for research and innovation will be crucial if India is to achieve the target set in Sustainable Development Goal 3.3 to end epidemics of neglected tropical diseases by 2030."

The draft national policy for rare diseases provides for lowering the incidence of rare diseases based on an integrated preventive strategy encompassing awareness generation and screening programmes and enables access to affordable healthcare to patients of rare diseases are amenable to a one-time treatment. The policy has also noted that the number of persons suffering from diseases considered rare globally, is lacking in India and accordingly provides that for the purpose of the policy the term 'rare diseases' shall be construed to three groups of disorders identified and categorised by experts based on their clinical experience.

Considering the limited data available on rare diseases and in the light of competing health priorities, the focus of the policy is on prevention of rare diseases as a priority for all the three groups of rare diseases identified by experts. The policy includes infectious tropical diseases and identifies a need to support research on treatments for rare diseases.

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