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â??About 10-12 orphan drugs are available for patients in Indiaâ?•

31 May 2025 | Interviews | By Narayan Kulkarni

The Organisation for Rare Diseases India (ORDI), a national umbrella organisation representing the collective voice of all people with rare diseases in India held the 10th edition of its flagship annual awareness run, RaceFor7. Over 8300 participants, including patients and caregivers, took part in this 7-kilometer marathon across 21 cities. RaceFor7, an annual awareness run/walk held to commemorate World Rare Diseases Day, observed on February 28 aims to raise awareness among the public and policymakers about the challenges faced by individuals living with rare diseases. In an interaction with BioSpectrum India, Prasanna Kumar Shirol, Co-founder & Executive Director, ORDI shared his views about the efforts of ORDI in reducing inequities and ensuring that persons with rare diseases have access to the same resources as the general community.



There are 443 rare diseases which ORDI has listed. How many of these rare diseases are being treated in India? Off these 443 which diseases are more predominantly visible in the country?

These are the conditions reported by patients who have registered with ORDI through helpline, online and referrals. As per National Policy for Treatment of Rare Diseases (NPTRD), 2017, the Government of India mentions about 450 conditions have been identified in India, but the list of diseases being treated is not available.

Lysosomal Storage Disorders (LSDs), a group of genetic metabolic disorders where enzymes responsible for breaking down complex molecules within lysosomes are deficient or absent such as Gaucher, Pompe, MPS and Neuro Muscular Diseases like DMD, SMA and IEMs like PKU, MSUD, Organic Acidemias, Cystic Fibrosis, Osteogenesis Imperfecta, Primary Immuno Deficiencies, are predominantly visible across India.

The National Policy for Rare Diseases (NPRD) has listed only 67 diseases under group 3 (group 1a (8), group 1 (5), group 1bRT (11), group 2a (14), group 2b (11), group 3b (8), group 3a (10)). How is ORDI pushing the government to include other rare diseases under NPRD?

Currently, the disease inclusion process is going at a very slow pace. We have requested the government to list all the rare disease / genetic conditions and treat which are treatable and provide supportive care for the non-treatable.

We have been advocating for inclusion of all conditions regularly. Recently we have also met Prof. Vinod K Paul – Member NITI Aayog about the same and submitted a request letter for the same endorsed by all ORDI member Patient Advocacy Groups.

Of ~500 approved orphan drugs exist in the market; how many drugs are available for patients in India? Which are the companies that offer orphan drugs to Indian patients under charitable access programmes?

About 10-12 drugs are available for patients in the country. Currently Sanofi, Takeda, Novartis etc. have been offering orphan drugs to Indian patients under charitable access initiative.

Currently ORDI is associated with 8250 patients and only 372 patients have the treatment access. Besides the RaceFor7 mass awareness campaign, what other programmes ORDI has in place to reach out to more patients?

The number of patients associated is dynamic. We get on average 2-3 calls on our helpline and registration. And several patients succumbed to death also.

We also closely work with more than 25+ Registered Disease specific Patient Advocacy Groups (PAG). They have their own patients' members for a particular condition and their patients need not register with us.

The 372 patients referred here are the patients supported by ORDI to receive treatment before the announcement of the National Rare Disease Policy through various sources such as ESI, Public Sector, CSR, Crowd Funding etc.

Currently more than 1000 patients are taking treatment under NPRD across the country. But sustainability is a challenge specially after their treatment cost crosses Rs 50 lakh.

Regularly we conduct patient outreach programmes through medical camps. Recently we have initiated a questionnaire-based patient screening model for identifying patients suffering from Rare Diseases Conditions in collaboration with Kolkata City Municipal Corporation (KCMC).

We are conducting a questionnaire-based screening of all the walk-ins of 149 Primary Healthcare Centres of KCMC.

ORDI noted that there are 3300 doctors supporting the organisation. Of these, how many are actually involved in providing treatment to rare disease patients in India?

We conduct various awareness programmes and initiatives among Medical Fraternity. These are the doctors who have participated, attended, referred, doctors etc. in the last 11+ years. These are Paediatrician, General Practitioner, Specialist etc. These may not be treating physicians, they are the ones who refer patients to the ORDI, Rare Disease Experts or to the Centre of Excellence (CoE) now.

Has any company approached ORDI seeking help in identification of patients who can participate as volunteers in clinical trials?

Yes, we regularly receive requests from Clinical Research Organisations (CROs)/Consultants; we inform the patients to discuss with their treating physician and take a decision. As you know this is at the discretion of patients.

How many companies are showing interest to do clinical trials related to rare diseases in India and for what kind of diseases?

We regularly get enquiries from many companies who are in different stages like Pre clinical, Approved Trials etc.

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