



RARE DISEASE TREATMENT - POLITY

NEWS: The Delhi High Court issued directions aimed at improving the availability of orphan drugs, which are medications used to treat rare diseases.

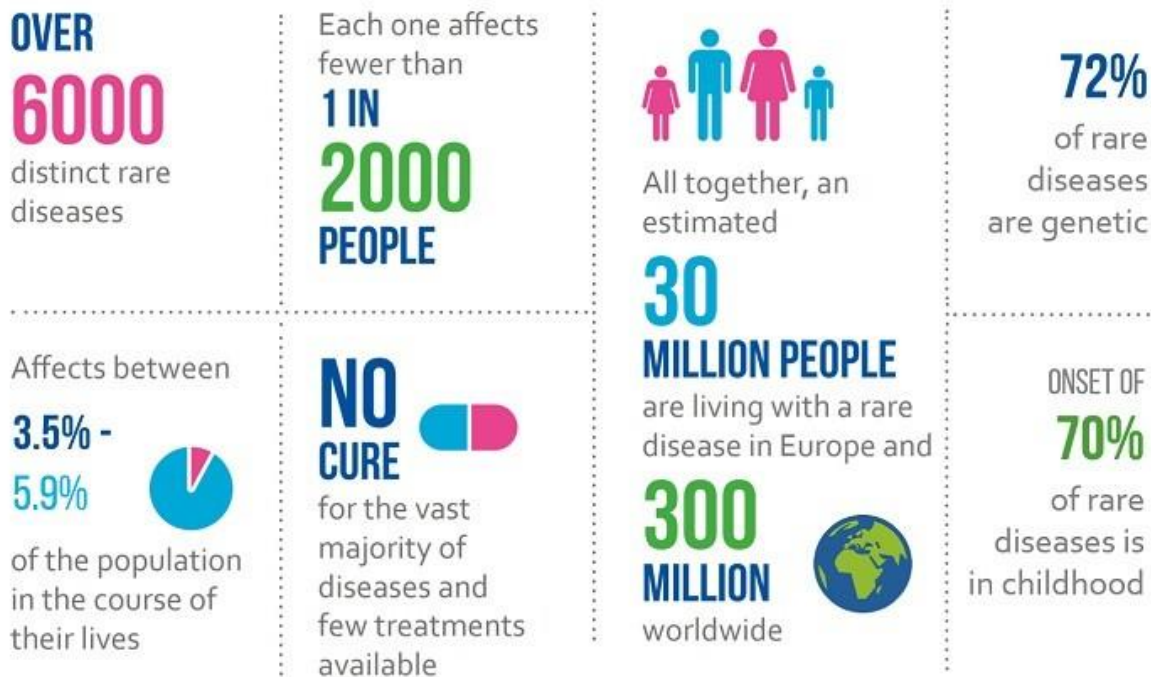
WHAT'S IN THE NEWS?

What are Rare Diseases?

- Rare diseases, also known as **orphan diseases**, are conditions that occur infrequently within a population.
 - **They are characterized by three key markers:** Total number of people with the disease, Prevalence and Availability /Non-availability of treatment options.
- **The World Health Organization (WHO)** defines a rare disease as a condition that affects a small percentage of the population, typically fewer than 1 in 1,000 to 2,000 people.

Status of rare diseases in India

- Around **55 medical conditions**, including Gaucher's disease, Lysosomal Storage Disorders (LSDs), and certain forms of muscular dystrophy are classified as rare diseases in India.
- **The National Registry for Rare and Other Inherited Disorders (NRROID)** started by the Indian Council of Medical Research (ICMR) has the records of 14,472 rare disease patients in the country.



Challenges in the Treatment of Rare Diseases

- **Limited Availability:** Less than 5% of rare diseases have available therapies, leaving fewer than 1 in 10 patients with access to disease-specific care.



- **High Cost:** Many rare disease treatments are patented, leading to high prices due to limited market size and high development costs.
 - Pharmaceutical companies find it unprofitable to produce these drugs, further driving up costs.
- **Delays in approval processes:** the National Rare Diseases Committee discussed delays in the Drug Controller General of India (DCGI) approving Sarepta Therapeutics' medicines, leaving patients without timely access.
- **Unequal Treatment Across Groups:** While limited assistance is available for Group 1 and Group 2 diseases, Group 3 patients face significant financial and healthcare barriers.

National Policy for Rare Diseases (NPRD), 2021

- It was launched in **2021**, under which financial assistance up to Rs 50 lakh is provided to patients receiving treatment at an identified Centre of Excellence (CoE).
- In India, rare diseases are categorized into three groups based on the nature and complexity of available treatment options.
 - **Group 1** includes diseases that can be treated with a one-time curative procedure.
 - **Group 2** diseases require long-term or lifelong treatment which are relatively less costly and have shown documented benefits, but patients need regular check-ups.
 - **Group 3** diseases are those for which effective treatments are available, but they are expensive and must often continue lifelong.

Other initiatives taken in India

- The Health Ministry has opened a **Digital Portal for Crowdfunding & Voluntary Donations** with information about patients and their rare diseases.
 - Donors can choose the CoE and patient treatments they wish to support.
- Each CoE also has its own **Rare Disease Fund**, which is used with approval from its governing authority.
- The Department of Pharmaceuticals has launched the **Production Linked Incentive (PLI) Scheme** for Pharmaceuticals, offering financial incentives to selected manufacturers for **domestic production of orphan drugs**.

Way Ahead

- **Domestic Manufacturing:** Developing and manufacturing orphan drugs within India can significantly reduce costs.
 - The government should offer incentives such as **tax breaks and subsidies** to encourage pharmaceutical companies to invest in research and production of rare disease treatments.



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- **Leveraging the Patents Act of 1970:** If treatments for rare diseases are unavailable or unaffordable, the government can use provisions under the Patents Act, 1970, to enable third-party manufacturing of patented drugs.
- **Faster approval** processes for life-saving therapies will ensure that patients get quicker access to essential treatments.
- **A sustainable, long-term funding mechanism** needs to be established, especially for Group 3 rare diseases, to cover both immediate and lifelong treatment costs.

Source: <https://indianexpress.com/article/explained/explained-health/issues-in-the-treatment-of-rare-diseases-and-what-the-govt-can-do-9618942/>

