

Management of Rare Diseases in India

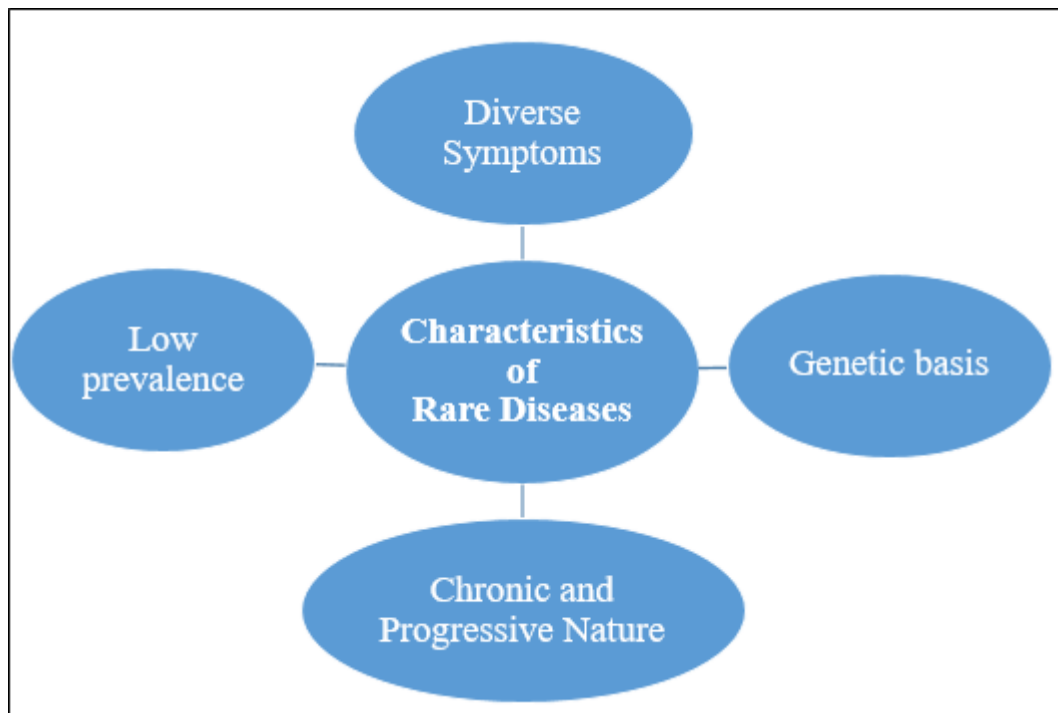
***Mains (GS II):** Issues relating to development and management of Social Sector/Services relating to Health, Education, Human Resources.*

Why in news?

Recently, Karnataka government seeks corporate support for treating children battling rare and ultra rare diseases.

What are rare diseases?

- **Definition** - According to World Health Organisation (WHO), Rare disease is a lifelong disease with a prevalence of 10 or fewer per 10,000 population.
- The Organization of Rare Diseases India (ORDI) defined rare disease as a disease when it affects 1 in 5,000 people.
- A disease is considered rare if it affects fewer than 200,000 people in the U.S. and fewer than 1 in 10,000 people in the European Union.
- **Spread** - It affect approximately 3.5% to 5.9% of the population.
- **Causes** - 72% of rare diseases are genetic, with over 7000 characterized by diverse disorders and symptoms.
- **Characteristics** - There are wide range of conditions that differ significantly in their symptoms and manifestations, even among individuals diagnosed with the same disorder.



- **No treatments** - The *absence of definitive treatments* further intensifies the physical and emotional burden on both patients and their families, severely impacting their overall quality of life.

Status of Rare Disease in India

- India lacks a standard definition for rare diseases.
- **Incidences** - India accounts for *one-third of the global rare disease* incidence, with over 450 identified diseases.
- **Population** - India has *over 13,000 registered patients* under the National Registry for Rare and Other Inherited Disorders.
- Estimate suggests that about 8 crore-10 crore Indians suffer from one rare disease or another and over 75% of them are children.

Most Common Rare Disease in India

- Lysosomal Storage Disorders
- Pompe disease (Glycogen storage disease type II)
- Anderson–Fabry disease
- Mucopolysaccharidosis
- Severe Combined Immunodeficiency (SCID)
- Phenylketonuria
- Cystic Fibrosis
- Duchenne Muscular Dystrophy

What are the measures taken by India?

- **National Policy for Rare Diseases (NPRD)** – It was launched by Ministry of Health & Family Welfare in **2021**.
 - Currently, 63 rare diseases are included under NPRD on recommendation of Central Technical Committee for Rare Diseases (CTCRD).
- **Centre of Excellence for Rare Disease** – They are institutions identified by the central government under NPRD, 2021.
- **Aim** – To treat patients suffering from rare diseases actively.
- **Numbers** – There are 12 such centres.
- **Coverage** – These centres cover with a quota of 2,420 rare disease patients from 6 categories across 3 groups.
- These 3 groups are based on the type of treatment they require
 - **Group 1** – Diseases that can be cured with a one-time treatment.
 - **Group 2** – Diseases that require long-term or lifelong treatment, but are relatively inexpensive.
 - **Group 3** – Diseases that have a definitive treatment, but are expensive and require lifelong therapy.
- **Tax exemption** – GST and Basic Customs Duty on drugs imported for Rare Diseases for individual use and through CoE.

Rare Disease Day was observed on the last day of February i.e., 28th February (or 29 in leap years).

What are the key challenges in India?

- **High cost of treatment** – Treatments for diseases like Spinal Muscular Atrophy (SMA) cost upwards of ₹72 lakh annually.
- **Lower financial support** – The NPRD's financial cap of ₹50 lakh per patient is inadequate and gets exhausted early.
- **Inadequate policy implementation** – Despite court directions (Delhi and Kerala High Court), MoHFW has been slow to implement the National Policy for Rare Diseases.
- **Patent monopolies** – Pharma companies exploit monopolies by delaying market entry of cost-effective alternatives.
- Drug patents block the manufacturing of affordable generics.
- **Lack of Institutional Coordination** – Inter-ministerial coordination (Health, Pharmaceuticals, Industry) is weak, also a direction to coordinate through a five-member committee was issued only recently.

- **Lack of institutional care at CoEs** - It fails to support sustained treatment for chronic conditions like Lysosomal storage disorders.

What lies ahead?

- **Enhancing financial support** - The current financial ceiling of the NPRD can be increased to ensure sufficient support for patients requiring high-cost treatments.
- **Time-bound disbursement of funds** - A clear timelines and can be defined for disbursing allocated funds to patients and institutions to avoid delays in treatment initiation.
- **Manufacturing generic drugs** - *Indian Patents Act can be invoked to issue compulsory licenses* for patented medicines, particularly when monopoly pricing restricts access.
- Indian pharmaceutical companies can be encouraged to manufacture generic versions of high-cost patented rare disease drugs through *public-private partnerships* and funding support.
- **Quicker regulatory approvals** - Clinical trials, regulatory clearances, and marketing approvals for indigenously developed rare disease medicines can be given in timely manner.
- **Activating oversight committee** - The court proposed *five-member oversight committee under the NPRD* can be made operational to monitor implementation & address emerging challenges.
- **Enhancing inter-departmental coordination** - Institutional collaboration between the ministries of Health, Pharmaceuticals, and Industry can be ensured for better policy alignment and seamless execution of rare disease strategies.

Reference

[The Hindu| Management of Rare Diseases in India](#)