# Management of Rare Diseases in India

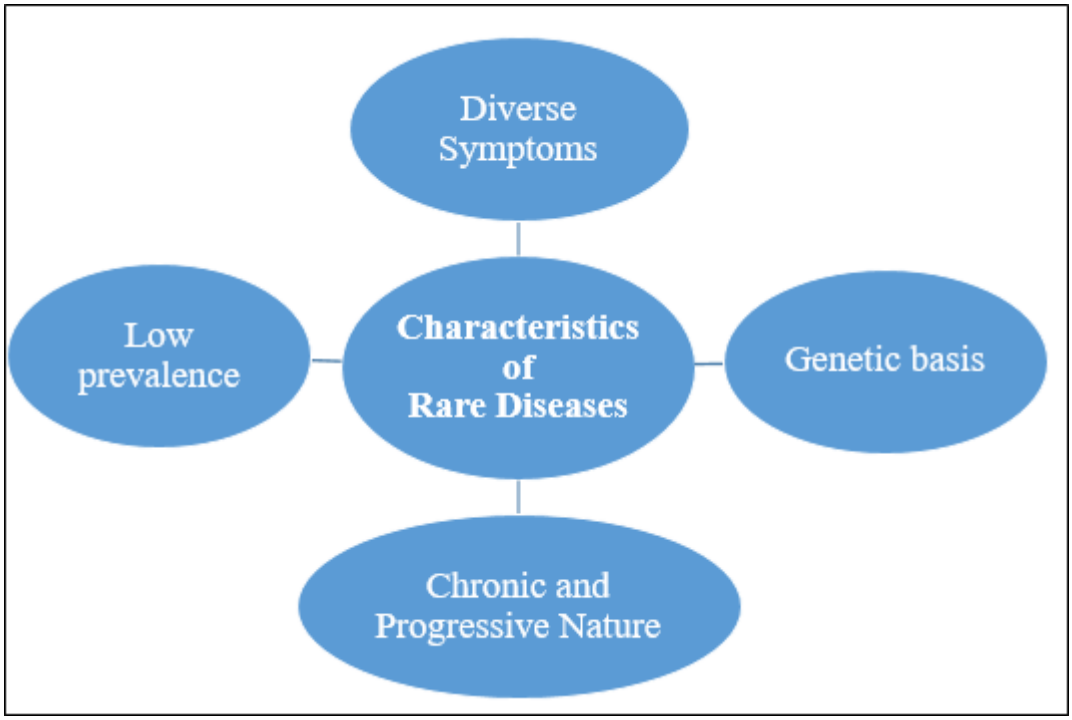
***Mains (GS II):*** *Issues relating to development and management of Social Sector/Services relating to Health, Education, Human Resources.*

## 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