

## India's Fight against Rare Diseases

### Why in news?

February 29 is observed as Rare disease day, it aims to raise awareness and support for individuals with rare medical conditions.

### What is rare disease?

*The theme of rare disease day in 2024 is "Share Your Colours" underscores the importance of collaboration and support for individuals with rare diseases.*

- According to the *World Health Organization (WHO)*, a rare disease is a debilitating lifelong disease or disorder with a prevalence of 10 or less per 10,000 population.
- Rare diseases are broadly defined as diseases that infrequently occur in a population.
- **Indicators-**
  - The total number of people with the disease
  - Disease prevalence
  - Availability/non-availability of treatment options.
- **Treatment-** They can be very expensive going up to Rs 1 crore per year.
- **National Organization for Rare Disorders (NPRD)-** It estimates that for a child weighing 10 kg, the annual cost of treatment for some rare diseases may vary from Rs 10 lakh to more than Rs 1 crore per year.

### Status of rare disease in India

- Rare diseases affect 1 or less per 1,000 population globally, with India accounting for one-third of cases.
- Over 450 identified rare diseases exist in India, affecting approximately 8 crore-10 crore Indians.
- As per an estimate, there are 7,000 known rare diseases with an estimated 300 million patients in the world. Of this, 70 million are in India.
- They include inherited cancers, autoimmune disorders, congenital malformations, Hirschsprung's disease, Gaucher disease, cystic fibrosis, muscular dystrophies and Lysosomal Storage Disorders (LSDs).

### What are the challenges present in rare disease?

- **Lack of definition-** India has a significant incidence of rare diseases, with over 450 identified there is no standard definition for rare disease in the country.
- **Late diagnosis-** It takes an average of 7 years for rare disease condition to be diagnosed which increases the financial burden for patients and their families.
- **Skill deficit-** Doctors often lack training on interpreting signs and symptoms.
- **Limited treatment options-** Only less than 50% of identified rare diseases in India

are treatable and it is done only in few Centres of Excellence (CoEs).

- **Fund deficit**- The funds allocated for rare disease are insufficient and there is a lack of parity in fund utilization among CoEs.
- **Policy paralysis**- National Policy for Rare Disease 2021 has limited funds per patient, creating challenges for lifelong management and therapy.
- **High cost**- The drug prices are too costly which is not affordable for poor people.

### **What can be done to overcome the challenges in rare disease?**

- **Standard definition**- India should establish a standard definition of rare disease.
- **Data collection**- There is a need to enhance data collection and epidemiological assessments for better policymaking.
- **Increase fund allocation**- The government should increase budgetary outlays for rare disease considering the chronic nature of rare disease.
- **Drug discovery**- India should dedicate funds for drug development and therapy.
- **Encourage PPP**- The public-private partnerships should be encouraged for funding, utilizing CSR initiatives.
- **Expand CoEs**- The number of Centres of Excellence should be increased to ensure better coordination among themselves.
- **Social assistance program**- The State governments should introduce social assistance programs.
- **Wider accessibility**- The satellite centres should be developed under CoEs for larger reach of treatment and therapy of rare diseases.
- **Address drug price**- The government should provide incentives for domestic manufacturers, reduce clinical trial requirements, and explore repurposed drugs.

*GST can be withdrawn on life saving drugs.*

- **Effective policy**- The government should establish policies to ensure sustained and effective management of rare diseases.
- **Multifaceted approach**- Battling rare diseases requires a holistic approach that addresses 4A's namely *affordability, availability, awareness, and accessibility*.

### **Steps taken by India to combat rare disease**

- [National Policy for Rare Diseases \(NPRD\), 2021](#)- It outlines a comprehensive approach to address rare diseases in India.
- **Fund mechanism**- NPRD policy will make use of a crowdfunding mechanism to cover the cost of treatment of rare diseases.
- **Registry**-NPRD outlines a national hospital-based registry of rare diseases to ensure adequate data and comprehensive definitions of rare diseases are compiled properly for reaserch purposes.
- **Exemption from Custom duty** -Medicines and foods needed for the management of 51 rare diseases have been exempt from [custom duty](#).
- **Centres of Excellence (CoEs)** – 8 CoEs have been identified for diagnosis, prevention and treatment under National Policy for Rare Diseases 2021.
- **Financial support** - Up to Rs. 50 lakhs to the patients suffering from any category of the Rare Diseases and for treatment in any of the CoEs.
- **Nidan kendras** -They have been set up for genetic testing and counselling services.
- **Kerala Against Rare Diseases (KARE)**- It is Kerala’s initiative to devise a comprehensive care strategy to prevent and manage rare diseases, it will go beyond mere care to look at rare diseases in a holistic manner.

## Reference

1. [The Hindu- India’s fight against rare disease](#)
2. [The Hindu- KARE Initiative](#)

