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 foe 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

SHANKAR IAS PARLIAMENT
Information is Empowering