RARE DISEASES AND INDIA - GS III MAINS

Q. Apart from resource constraints, India languishes near the bottom on awareness, diagnosis, and drug development for rare diseases. Examine (10 marks, 150 words)

News: *India's fight against rare diseases*

What's in the news?

- Recently, the tragic death of 19-year-old child actress Suhani Bhatnagar, due to a rare disease called dermatomyositis which causes muscular inflammation, has put the spotlight on the severity of rare diseases.

Key takeaways:

- Despite, rare diseases in India accounting for one-third of the global rare disease incidence, there has been apathy of the government and society in general towards these diseases.
- Resource constraints apart, India languishes near the bottom on awareness, diagnosis and drug development for rare diseases.

Rare Disease:

- A rare disease is a health condition which has a low prevalence and affects a small number of people.
- It includes genetic diseases, rare cancers, infectious tropical diseases and degenerative diseases.
- Only, 5% of the over 7,000 known diseases worldwide are treatable.
- However, there is no single, agreed-upon definition of Rare Disease. Different countries have different definitions of rare disease.

WHO's Definition:

- Rare diseases are the diseases which have a prevalence of 1 or less in every 1,000 people or less.

Rare Disease Definition in India:

- India, like many other developing countries, currently has no standard definition of rare diseases.

Rare Disease Burden in India:

- India accounts for one-third of the global rare disease incidence.
- According to the National Policy for Rare Diseases document, India has close to 50-100 million people who are affected by rare diseases or disorders.

- There are over 450 identified diseases in India, ranging from widely known ones such as Spinal Muscular Atrophy and Gaucher's disease to lesser-known ones such as Mucopolysaccharidosis type 1 and Whipple's disease.
- There are about 8 crore-10 crore Indians suffering from rare diseases in India, with over 75% of them being children.
- High morbidity and mortality rates of these life-threatening diseases are a leading cause for the majority of these children not reaching adulthood.

Challenges Posed by Rare Diseases in India:

1. Unavailability of Treatment:

- Less than 50% of the 450-odd rare diseases identified in India are treatable.
- Most patients typically receive only basic treatment that alleviates symptoms.

2. Unaffordable Treatment Costs:

- Some rare disease's treatment, requires exorbitantly priced antidotes and supportive medication, which poses the challenge of affordability.

3. Low Focus on R&D for Drug Development:

- The rare disease is not considered as a significant market by the drug manufacturers, as the number of persons suffering from individual rare diseases is small.
- Hence, these diseases are treated as "orphan diseases" and the drugs are treated as "orphan drugs" by the pharma giants.

4. Late Diagnosis:

- Rare disease takes on an average of seven years for their diagnosis in India.
- Delay in diagnosis or a wrong diagnosis increases the suffering of the patients exponentially.

5. Lack of Trained Health-care Professionals:

- Lack of trained healthcare professionals to interpret the signs and symptoms of rare diseases in the initial stages, has compounded the challenge posed by rare diseases in India.

Government Initiatives in Treating Rare Diseases:

1. National Policy for Rare Diseases 2021:

- It is an umbrella policy for treatment of rare diseases in India.
- Some of the major provisions and initiatives under the policy are mentioned below.
- Categorization of Rare Diseases into 3 Groups such as
    - **Group 1 -** Disorders amenable to one-time curative treatment.
    - **Group 2 -** Diseases requiring long term/lifelong treatment having relatively lower cost of treatment.
    - **Group 3 -** Diseases for which definitive treatment is available, but very high cost and lifelong therapy
- **Centres of Excellence (CoEs) and Nidan Kendras -** 12 Centres of Excellence (CoEs) have been opened for diagnosis, prevention and treatment of rare diseases, while Nidan Kendras have been set up for genetic testing and counselling services.

- **Financial Support -** Provision for financial support of up to Rs. 50 lakhs to the patients suffering from any category of the Rare Diseases and for treatment in any of the Centre of Excellence (CoE) mentioned in NPRD-2021.

2. PLI Scheme for Rare Drugs Manufacturing:

- Department of Pharmaceuticals has been provides for financial incentives to manufacturers of Orphan drugs under the Production Linked Incentive Scheme.

3. Tax Waiver for Rare Disease Drug imported for Personal Use:

- Department of revenue has provided full waiver of Basic Customs Duty (BCD) and Integrated Goods and Services Tax (IGST) to imported drugs for personal use to treat Spinal Muscular Atrophy (SMA).

Challenges in Government Initiatives:

1. Less Number of Diseases being Treated under the Policy:

- The treatment process of only 20 rare diseases have been approved by the Drugs Controller General of India.
- These treatments can be availed only from Centres of Excellence (CoEs).

2. Less Number of Centres of Excellence (CoEs):

- There are only 12 CoEs which are unevenly distributed considering the vast expanse of the country.
- Further, lack of coordination, late diagnosis, inadequate therapies and lack of timely availability of medicines at the CoEs, create further challenges.

3. Lack of Adequate Budgetary Support:

- Although, the budgetary support for rare diseases has increased over the years, to about Rs. 93crore for 2023-24.
- However, there have been reductions to the tune of 75% from Budget Estimate stage to the Revised Estimates to 90% actual expenditure.

4. CoE's Inability to Utilise the Budget Provided:

- More than ₹47 crore of the ₹71 crore financial assistance allocated to the 11 CoEs for the current year remains unused.
- CoEs are wary of beginning any treatment that they may need to suspend later, as they feel vulnerable to judicial action from patients and their kin.

5. Inadequate Financial Assistance Per Person:

- The limit of Rs. 50 lakh per person for treatment of chronic disease is woefully inadequate, as chronic rare diseases usually require lifelong management and therapy.

WAY FORWARD:

1. Training of Health Professionals:

- The healthcare professionals must be trained to improve their diagnostic accuracy.

2. Pre-natal and Post-natal Screening of Expectant Mothers:

- Expectant mothers with a history of rare diseases in their family must undergo mandatory pre-natal screening and post-natal diagnosis and care.

3. Improvement in the Implementation of NPRD 2021:

- The government must frame a standard definition of rare diseases, increase budgetary outlays, dedicate funding for drug development and therapy, and increase the number of CoEs.
- These will improve the effectiveness of the National Policy of Rare Disease 2021.

4. Social Assistance Programmes through PPP Partnership:

- Public and private companies must be co-opted for funding the social assistance programmes for rare diseases, through CSR initiatives and funding partnerships.

5. Withdrawal of GST on Life-saving Drugs:

- GST must be withdrawn from all life-saving drugs for rare diseases, which will make the drugs a bit affordable.