



## National Rare Disease Policy 2021

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### Why in News

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Recently, the Ministry of Health and Family Welfare has approved the **National Rare Disease Policy 2021**.

Earlier, the **Delhi High Court had directed the Centre** to set up a Rare Diseases Committee, a Rare Diseases Fund and to finalise and notify the National Health Policy for Rare Diseases on or before 31st March, 2021.

### Key Points

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- **Aim:**
  - To increase **focus on indigenous research and local production of medicines**.
  - To **lower the cost of treatment** of rare diseases.
  - To **screen and detect rare diseases early at early stages**, which will in turn help in their prevention.

- **Major Provisions of the Policy:**

- **Categorization:**

The policy has categorised rare diseases in **three groups**:

- **Group 1:** Disorders amenable to **one-time curative** treatment.
- **Group 2:** Those requiring **long term** or **lifelong treatment**.
- **Group 3:** Diseases for which **definitive treatment is available** but challenges are to make optimal patient selection for benefit, very high cost and lifelong therapy.

- **Financial Support:**

- Those who are suffering from rare diseases listed under Group 1 will have the financial support of up to Rs. 20 lakh under the umbrella scheme of

**Rashtriya Arogya Nidhi.**

**Rashtriya Arogya Nidhi:** The Scheme provides for **financial assistance to patients, living below poverty line (BPL)** and who are suffering from major life threatening diseases, **to receive medical treatment at any of the super specialty Government hospitals / institutes.**

- Beneficiaries for such financial assistance would not be limited to BPL families, but extended to about 40% of the population, who are eligible as per norms of **Pradhan Mantri Jan Arogya Yojana**, for their treatment in Government tertiary hospitals only.

- **Alternate Funding:**

This includes **voluntary crowdfunding treatment** by setting up a **digital platform** for voluntary individual contribution and corporate donors to voluntarily contribute to the treatment cost of patients of rare diseases.

- **Centres of Excellence:**

The policy aims to strengthen tertiary health care facilities for prevention and treatment of rare diseases through designating eight health facilities as '**Centres of Excellence**' and these will also be **provided one-time financial support of up to Rs. 5 crore** for upgradation of diagnostics facilities.

- **National Registry:**

A national **hospital-based registry of rare diseases will be created** to ensure adequate data and comprehensive definitions of such diseases are **available for those interested in research and development.**

- **Concerns Raised:**

- **Lack of Sustainable Funding:**

- Unlike conditions under Group 1 and Group 2, patients with Group 3 disorders require sustainable treatment support.
    - In the **absence of a sustainable funding support for Group 3 patients**, the precious lives of all patients, mostly children, are now at risk and at the mercy of crowdfunding.

- **Lack of Drug Manufacturing:**

- Where drugs are available, they are **prohibitively expensive**, placing **immense strain on resources**.
    - Currently **few pharmaceutical companies are manufacturing drugs for rare diseases globally** and there are no domestic manufacturers in India except for those who make medical-grade food for those with metabolic disorders.

## Rare Diseases

### What is a rare disease?

- Rare Diseases are life-threatening or chronically debilitating diseases with a low prevalence and a high level of complexity
- 6000-8000 rare diseases have been identified
- 80% are of genetic origin & 50% affect children
- No cure exists for the vast majority



- There are **6,000-8,000 classified rare diseases**, but less than 5% have therapies available to treat them.
- **Example: Lysosomal Storage Disorders (LSD)**, Pompe disease, **cystic fibrosis**, muscular dystrophy, spina bifida, **haemophilia** etc.
- About **95% rare diseases have no approved treatment** and less than 1 in 10 patients receive disease-specific treatment.
- These **diseases have differing definitions in various countries** and range from those that are prevalent in 1 in 10,000 of the population to 6 per 10,000.
- However broadly, a 'rare disease' is **defined as a health condition of low prevalence that affects a small number of people** when compared with other prevalent diseases in the general population. Many cases of **rare diseases may be serious, chronic and life-threatening**.
- India has close to **50-100 million people affected by rare diseases** or disorders, the policy report said almost 80% of these rare condition patients are children and a leading cause for **most of them not reaching adulthood is due to the high morbidity and mortality rates** of these life-threatening diseases.

Source:TH