



National Rare Diseases Committee

For Prelims: [National Policy of Rare Diseases](#), Rare Diseases, National Rare Diseases Committee

For Mains: Impact of rare diseases on the healthcare system, Initiatives Related to Universal Health Coverage in India.

Why in News?

Recently, the Delhi High Court has taken a proactive step to **address the challenges faced by patients with rare diseases** by establishing a **five-member panel to implement the Centre's rare diseases policy effectively**.

- The panel, known as the **National Rare Diseases Committee**, aims to ensure that patients enrolled with the All-India Institute of Medical Sciences (AIIMS), Delhi, receive timely treatment and benefit from the policy.
- The mandate of the Committee would broadly be to take all steps needed for implementation of the [National Rare Disease Policy, 2021](#).

What are Rare Diseases?

- 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.
- 80% of these diseases have **genetic origins**.
- 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.

What is the National Rare Diseases Committee?

- **About:**
 - The National Rare Diseases Committee is a **five-member panel** who will work together to

address the challenges faced by patients with rare diseases established by the Delhi High Court to implement the rare diseases policy and ensure efficient treatment for patients.

- The committee consists of experts from relevant fields, including **medical professionals, policymakers, and representatives from healthcare institutions.**

▪ **Responsibilities and Objectives:**

- Assessing Cases:
 - Focus on **patients enrolled with AIIMS in Delhi.**
 - Evaluate individual cases to understand medical needs and determine treatment.
- **Implementation of the Policy:**
 - Devise **strategies and guidelines for translating policy provisions into action.**
- **Coordination and Collaboration:**
 - Facilitate close coordination between **medical community, therapy providers, and governmental agencies.**
 - Create a collaborative environment for addressing challenges related to rare diseases.
- **Treatment Accessibility:**
 - Ensure **timely treatment for patients with rare diseases.**
 - Explore **avenues for procuring necessary therapies and drugs.**
 - Establish a **logistical framework for seamless administration of treatment.**

What is the National Rare Disease Policy 2021?

▪ **Aim:**

- Increase **focus on indigenous research and local production of medicines.**
- **Lower the cost of treatment** of rare diseases.
- **Screen and detect rare diseases early** for prevention.

▪ **Major Provisions of the Policy:**

- Categorization:
 - Group 1: Disorders amenable to **one-time curative treatment.**
 - Group 2: Diseases requiring **long-term or lifelong treatment.**
 - Group 3: Diseases with **available treatment but challenges in patient selection, high cost, and lifelong therapy.**

▪ **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, outside the Umbrella Scheme of Rashtriya Arogya Nidhi.
 - Financial support of **up to Rs. 20 lakhs under Rashtriya Arogya Nidhi** for rare diseases listed under Group 1.
 - Rashtriya Arogya Nidhi provides assistance to patients with major life-threatening diseases, regardless of their poverty status.
- Voluntary crowdfunding for treatment through a **digital platform for individual and corporate contributions.**

▪ **Centres of Excellence:**

- Designation of **eight health facilities as 'Centres of Excellence'.**
- One-time financial support of up to Rs. 5 crore for upgrading diagnostic facilities.

▪ **National Registry:**

- Creation of a **national hospital-based registry of rare diseases.**
- Ensuring comprehensive data and definitions for research and development purposes.

▪ **Concerns Raised:**

- Lack of **sustainable funding for patients with Group 3 disorders.**
- **Prohibitive costs of drugs for rare diseases.**
- Limited global and domestic manufacturers of drugs for rare diseases.

UPSC Civil Services Examination, Previous Year Questions (PYQs)

Q. Appropriate local community-level healthcare intervention is a prerequisite to achieve 'Health for All' in

[Source: TH](#)

PDF Refernece URL: <https://www.drishtias.com/printpdf/national-rare-diseases-committee>

