National Policy for Rare Diseases, 2021

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In News: Recently, Union health and family welfare minister Harsh Vardhan has approved the National Policy for Rare Diseases 2021.

Key points about National Policy for Rare Diseases, 2021

- **Policy Aim:** To bring down the high cost of treatment for rare diseases.
- It is proposed to have a national registry for a database of rare disease
- Along with lowering the cost of treatment of rare diseases, the policy aims to increase focus on indigenous research and local production of medicines.
- Those who are suffering from rare diseases (diseases listed under Group 1 in the rare disease policy) that require one-time treatment will have the financial support of up to Rs20 lakh under the umbrella scheme of Rashtriya Arogya Nidhi.
- Beneficiaries for such financial assistance would not be limited to BPL families, but the benefit will be extended to about 40% of the population, who are eligible under Pradhan Mantri Jan Arogya Yojana.
- The policy will make use of a crowdfunding mechanism to cover the cost of treatment of rare diseases. Corporates and individuals will be encouraged to extend financial support through a robust IT platform.
- 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.
- Through the help of Health and Wellness Centres, District Early Intervention Centres and counselling, the

policy aims to screen and detect rare diseases early at early stages, which will in turn help in their prevention.

The Policy classifies rare diseases into three groups:

- **Group 1**: Disorders controllable by one-time curative treatment, including osteopetrosis and Fanconi anaemia.
- **Group 2** : Diseases requiring long-term or lifelong treatment with a relatively lower cost of treatment and benefit has been documented in literature, including galactosemia, severe food protein allergy, and homocystinuria.
- **Group 3:** Diseases for which definitive treatment is available, but challenges are to make optimal patient selection for benefit, and very high cost and lifelong therapy, covering diseases such as spinal muscular atrophy (SMA), Pompe disease, and Hunter syndrome.

Rare Disease

- Also referred to as an orphan disease, is any disease that affects a small percentage of the population.
- Most rare diseases are genetic, and are present throughout a person’s entire life, even if symptoms do not immediately appear.
- Rare diseases recorded in India are Haemophilia, Thalassemia, sickle-cell anaemia and primary immunodeficiency in children, auto-immune diseases, Lysosomal storage disorders such as Pompe disease, Hirschsprung disease, Gaucher’s disease, Cystic Fibrosis, Hemangiomas and certain forms of muscular dystrophies