National Policy for Rare Diseases 2021

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Context

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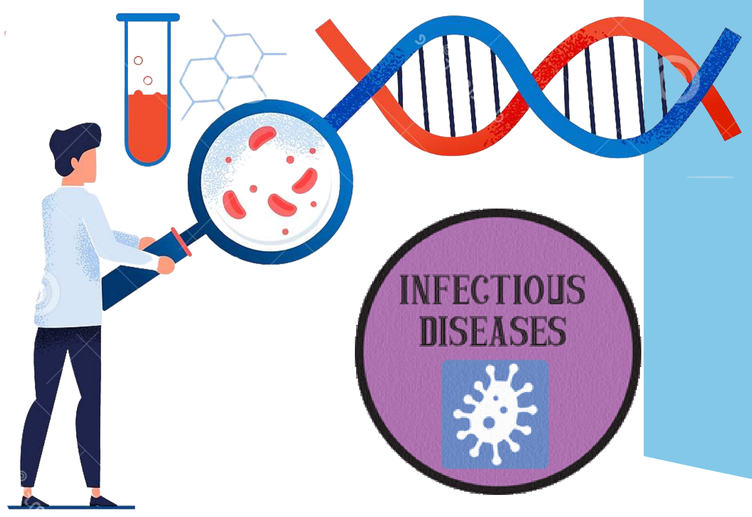
The Union Health & Family Welfare Ministry is taking measures to spread awareness about the “National Policy for Rare Diseases 2021”.

“National Policy for Rare Diseases 2021”.

What is a rare disease?

A rare disease is a health condition of **low prevalence that affects a small number of people** compared with other prevalent diseases in the general population.



Rare diseases include genetic diseases, rare cancers, infectious tropical diseases and degenerative diseases.

80% of rare diseases are genetic in origin and hence disproportionately impact children.

Definition of a rare disease

There is **no universally accepted definition** of rare disease.

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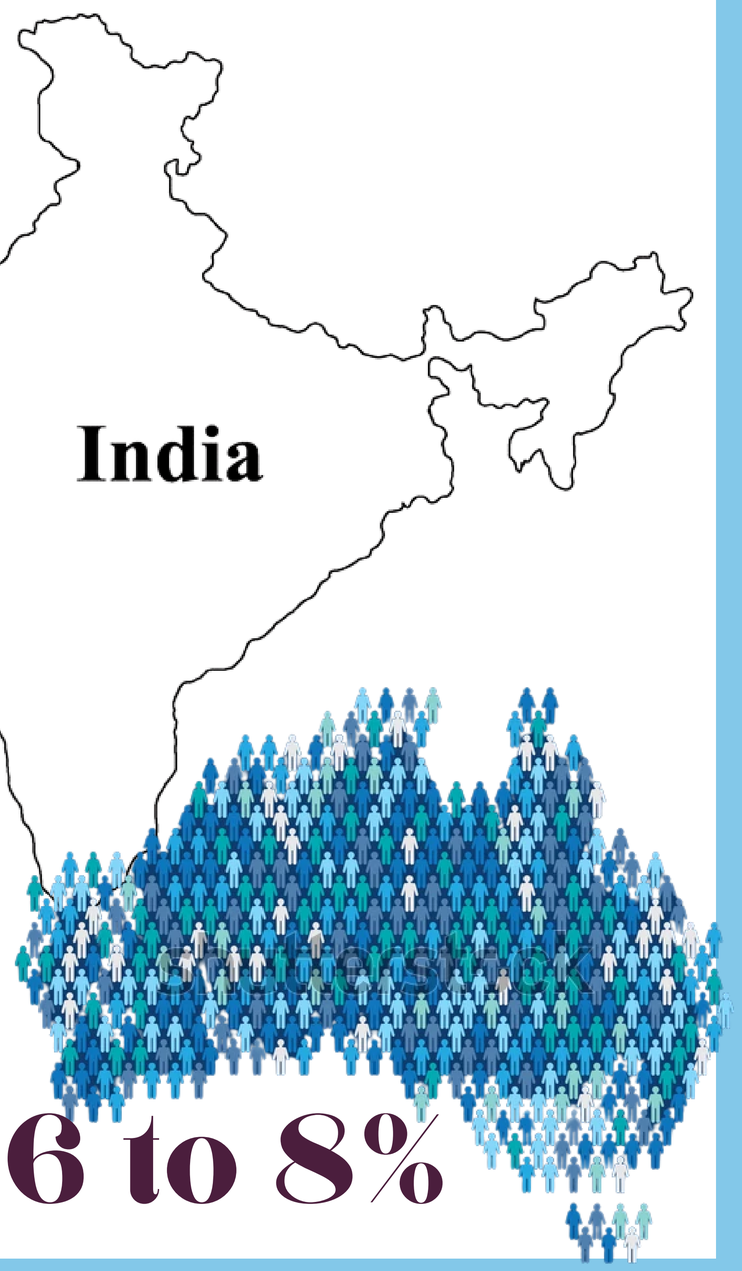
These diseases have **differing definitions** in various countries and range from those that are prevalent in 1 in 10,000 of the population to 6 per 10,000. According to the **Indian Council of Medical Research (ICMR)** registry definition, “A disease or disorder is defined as Rare in India when it **affects fewer than 1 in 2500 individuals”.**

INDIAN COUNCIL OF MEDICAL RESEARCH

So far about 450 rare diseases have been recorded in India and it is estimated that about 6-8% of the country’s population is affected by a rare disease.

450



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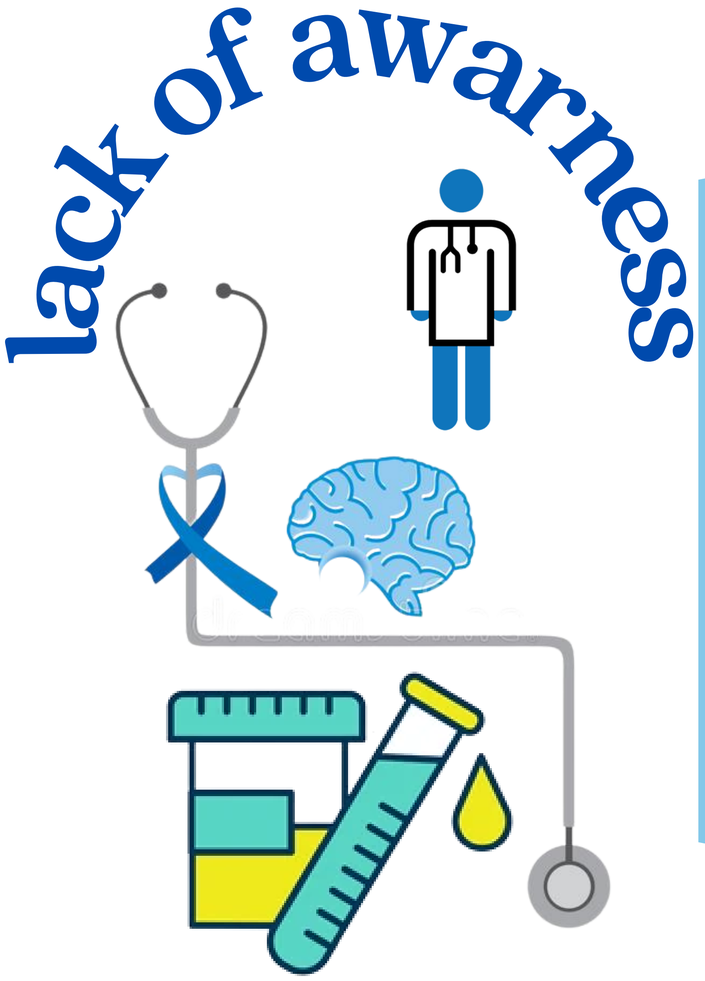
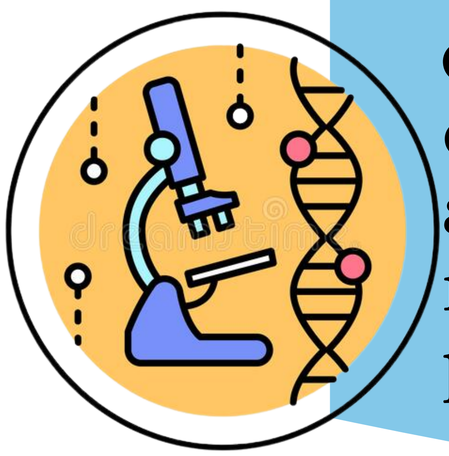
Challenges associated with rare diseases

There are 7,000-8,000 classified rare diseases, but **less than 5% have therapies available to treat them.** About 95% rare diseases have no approved treatment and less than 1 in 10 patients receive disease-specific treatment.

5%



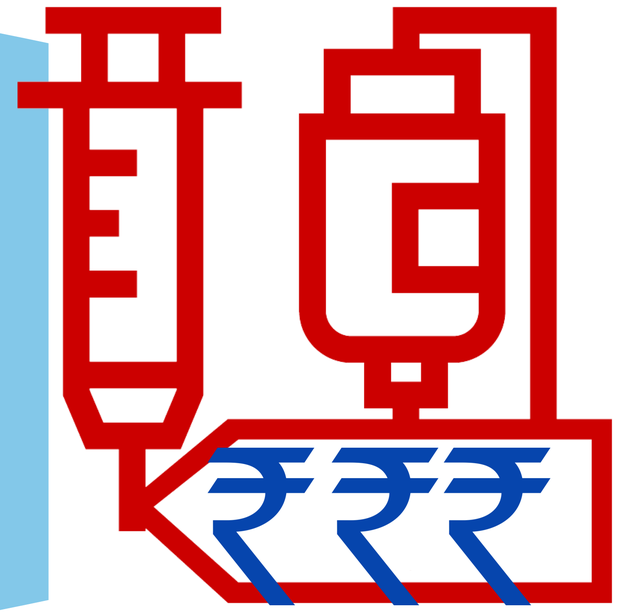
he field of rare diseases is very complex and heterogeneous and prevention, treatment and management of rare diseases have multiple challenges. **Early diagnosis** of rare diseases is a major challenge owing to a variety of factors that include **lack of awareness among primary care physicians, lack of adequate screening and diagnostic facilities etc.**



There are also fundamental challenges in the **research and development** for the majority of rare diseases as relatively little is known about the pathophysiology or the natural history of these diseases particularly in the Indian context.

Rare diseases are also **difficult to research upon** as the patients pool is very small and it often results in inadequate clinical experience. **Availability and accessibility to medicines** are also important to reduce morbidity and mortality associated with rare disease.

The **cost of treatment of rare diseases is prohibitively expensive.** Various High Courts and the Supreme Court have also expressed concern about lack of a national policy for rare diseases.



National Policy for Rare Diseases 2021

To address all these challenges, the Union Health & Family Welfare Ministry approved the "National Policy for Rare Diseases 2021" in April 2021.

Highlights of the Policy

In the new policy, rare diseases have not been defined but **classified into three groups.**

Group 1 has disorders amenable to one-time curative treatment, including osteopetrosis and Fanconi anaemia.

Group 2 has diseases requiring long-term or lifelong treatment with relatively lower cost of treatment and benefit has been documented in literature, including galactosemia, severe food protein allergy, and homocystinuria.

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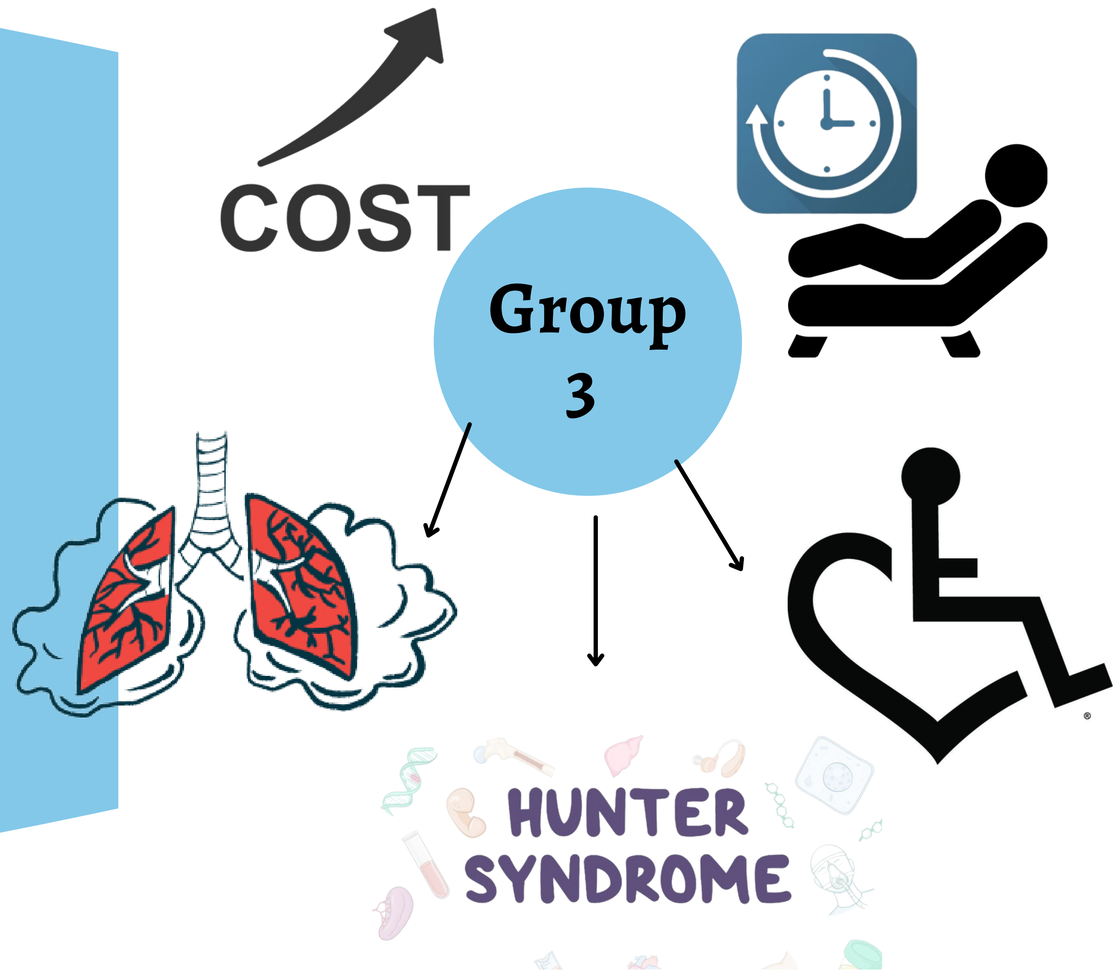
Group 3 has diseases for which definitive treatment is available, but challenges are to make optimal patient selection for benefit, and very high cost and lifelong therapy, covering diseases such as spinal muscular atrophy (SMA), Pompe disease, and Hunter syndrome.



Lowering the Cost of Treatment

The Rare Diseases Policy aims to lower the high cost of treatment for rare diseases with **increased focus on indigenous research** with the help of a **National Consortium** to be set up with the Department of Health Research, Ministry of Health & Family Welfare as convenor.

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Ministry of Health & Family Welfare
Government of India

increased focus on indigenous research

Increased focus of research and development and local production of medicines will lower the cost of treatment for rare diseases.

National Hospital Based Registry

The policy also envisages creation of a **national hospital based registry of rare diseases** so that adequate data is available for definition of rare diseases and for research and development related to rare diseases within the country.

Screening, Prevention & Treatment

The Policy also focuses on **early screening and prevention through primary and secondary health care infrastructure** such as Health and Wellness Centres and District Early Intervention Centres (DEICs) and through counselling for the high-risk parents.

WELLNESS CENTER

primary and secondary health care

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Screening will also be supported by **Nidan Kendras** set up by the Department of Biotechnology.

Nidan Kendras

Policy also aims to **strengthen tertiary health care facilities** for prevention and treatment of rare diseases through **designating 8 health facilities as Centre of Excellence (CoEs)** and these CoEs will also be provided one-time financial support of up to Rs 5 crores for upgradation of diagnostics facilities.

Crowd Funding Mechanism

Besides, the Policy also envisages a **crowd funding mechanism** in which corporates and individuals will be encouraged to extend financial support through a robust IT platform for treatment of rare diseases.

Funds collected will be utilized by Centres of Excellence for treatment of all three categories of rare diseases as first charge and then the balance financial resources could also be used for research.

Rashtriya Arogya Nidhi

A provision for **financial support up to Rs. 20 lakhs under the Umbrella Scheme of Rashtriya Arogya Nidhi** is proposed for treatment of those rare diseases that require a one-time treatment (diseases listed under Group 1 in the rare disease policy).

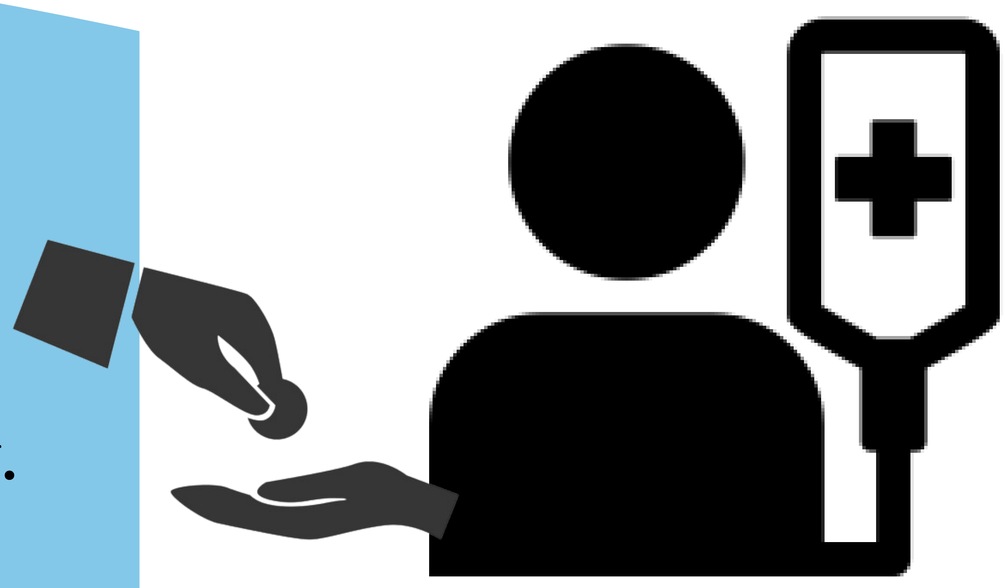
Beneficiaries for such financial assistance would **not be limited to BPL families,** but the benefit will be **extended to about 40% of the population**, who are eligible under Pradhan Mantri Jan Arogya Yojana.

The **state governments** would be asked to undertake treatment of diseases covered under **Group 2** which largely include disorders managed with special dietary formulae or food for special medical purposes (FSMP) and disorders that are amenable to other forms of therapy.

For diseases classified under **Group 3**, which require life-long expensive treatments, the government would create a **digital platform to bring together Centres for Excellence, patients undergoing treatment and corporate donors or prospective voluntary individuals who could help fund treatment.**

Criticisms

Families of rare diseases patients and their advocacy groups are unhappy with the **lack of financial support for diagnosed Group 3 diseases patients** in the new policy.

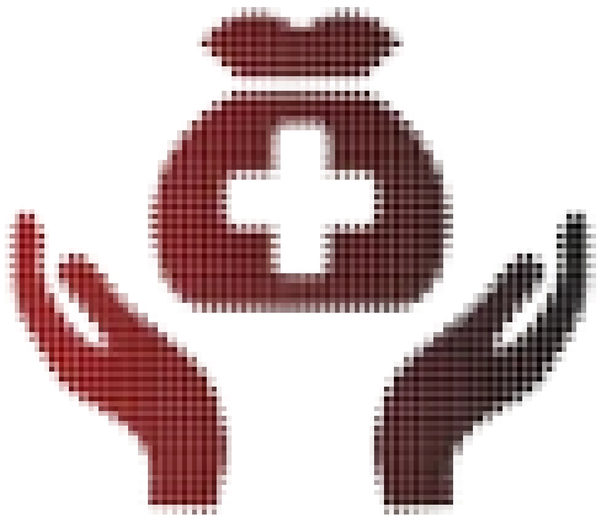


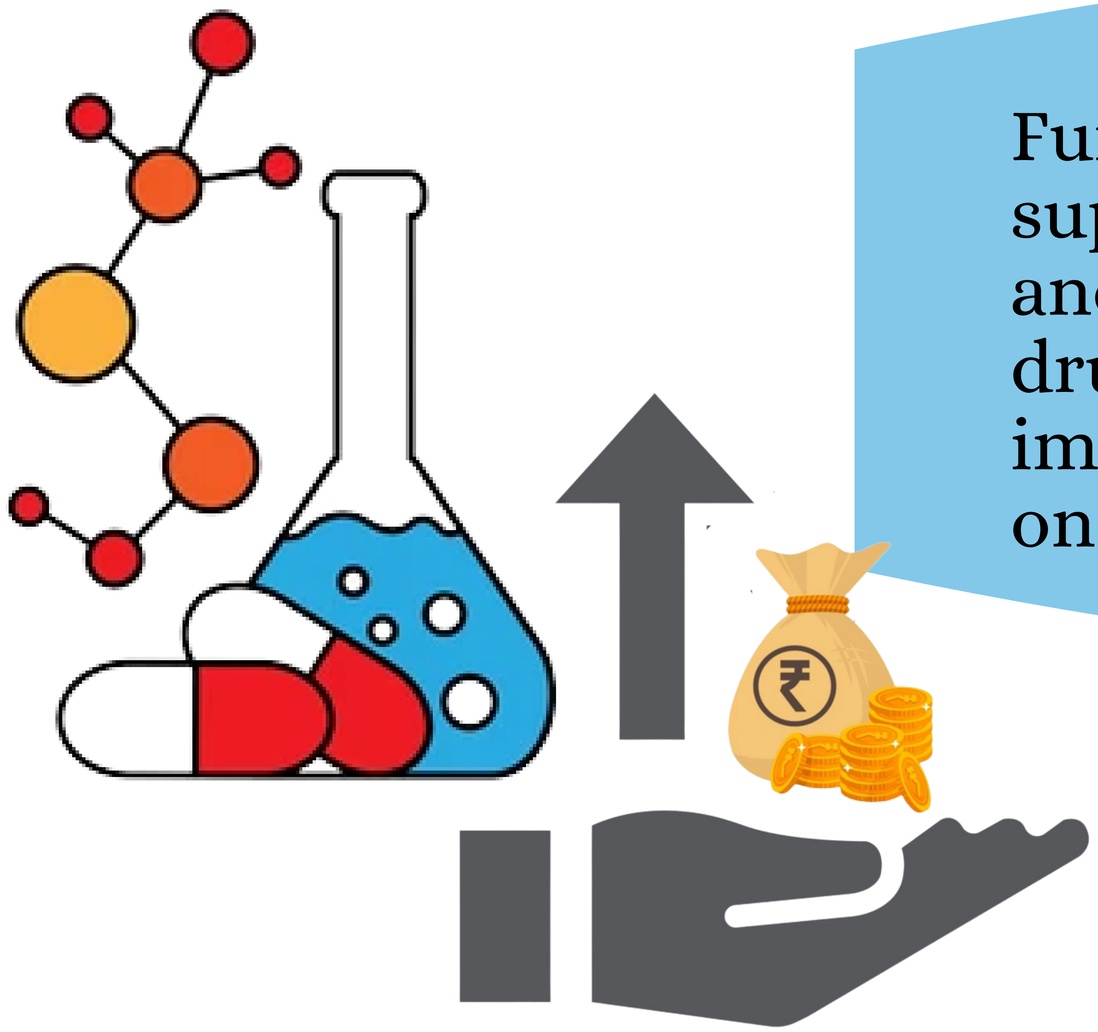
Rs 80-100 crore

Patients and their support groups had recently written to the health ministry seeking **creation of a seed-funding of Rs 80-100 crore** while rolling out the national policy so that life-saving therapies to all those patients with treatable Group 3 disorders can be provided, thereby reducing any further loss of life.

Way Forward

The Centre can set aside a **substantial corpus to fund life-saving treatments**, instead of leaving patients at the mercy of crowdfunding.





Further, there is a need to support the development of and commercialisation of drugs for treatment, and improve funding for research on rare diseases.

Reference:

https://pib.gov.in/PressReleasePage.aspx?PRID=1780141