



Rare-Disease Drugs Can Become India's Next Pharmaceutical Frontier Through Innovation, Regulation and Affordable Access

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Recent Developments:

- India's pharmaceutical sector has established global strength in **generic medicines**, but rare diseases present a different challenge because extremely small patient populations, high research costs and complex therapies make conventional commercial models difficult to sustain.
- The **National Policy for Rare Diseases, 2021** provides financial and medical support of up to **₹50 lakh per patient** for treatment at designated **Centres of Excellence**. As of 2026, the Government has expanded the number of designated Centres of Excellence to **15**.
- The Government has also promoted domestic manufacturing of medicines for rare diseases. For example, **risdiplam**, used in the treatment of spinal muscular atrophy, is being manufactured domestically, while a generic version has also been launched in India.
- The Department of Biotechnology's **UMMID initiative** is expanding genetic screening, diagnosis, counselling and clinical capacity for inherited disorders. Its recent expansion aims to cover around **22 States/Union Territories** through approximately **70 UMMID components**.
- Globally, rare diseases are increasingly being treated as an **equity and inclusion issue**. The World Health Organization reported in 2025 that more than **7,000 rare diseases** affect over **300 million people**, with around 70% beginning during childhood.

Understanding Rare Diseases:

Definition and Scale:

- **Rare diseases** are conditions that occur infrequently within a population and are often chronic, progressive, disabling or life-threatening.
- There is **no single globally uniform numerical definition**; the World Health Organization notes that rare diseases are often described as conditions affecting fewer than **1 in 2,000 people**.
- The collective burden is substantial because thousands of individually uncommon diseases together affect **hundreds of millions of people globally**.
- India has documented **1,004 rare genetic disorders** in the GenTIGS database, illustrating the considerable genetic diversity relevant to rare-disease research.
- Drugs developed specifically for rare diseases are commonly called **orphan drugs** because the small patient population historically provided limited commercial incentives for pharmaceutical companies.

Why Rare Diseases Create a Distinct Policy Problem:

- A conventional pharmaceutical market depends on a sufficiently large patient population to recover **research, clinical-trial, manufacturing and regulatory costs**.

- Rare diseases reverse this model: the scientific need can be high, but the number of potential buyers for an individual therapy can be extremely small.
- Consequently, even when an effective treatment exists, its **price, availability and distribution** can remain major barriers.

The Global Orphan-Drug Model:

United States Orphan Drug Act:

- The **US Orphan Drug Act, 1983** attempted to correct the market failure by providing incentives for pharmaceutical companies to develop treatments for rare diseases.
- Major incentives included **tax benefits, grants, regulatory assistance and market exclusivity** for qualifying products.
- The policy demonstrated how governments can use **targeted incentives to reduce the commercial risk of rare-disease research**.
- The United States subsequently developed a substantial orphan-drug pipeline, although the exact number of approvals depends on the designation and approval category used.

The Access Paradox:

- Incentives can increase drug development but do not automatically guarantee **affordable access**.
- Small patient populations mean that companies may attempt to recover large development costs from a limited number of patients, contributing to very high treatment prices.
- Advanced therapies, particularly **gene and cell therapies**, can involve exceptionally high one-time or lifelong treatment costs.
- This creates a policy dilemma between **rewarding pharmaceutical innovation and ensuring equitable access to essential treatment**.

India's Current Support System:

National Policy for Rare Diseases, 2021:

- The **National Policy for Rare Diseases, 2021** was introduced to improve prevention, diagnosis, treatment, research and financial support for rare-disease patients.
- The policy provides financial assistance of up to **₹50 lakh per patient** for treatment through designated Centres of Excellence, subject to the applicable guidelines.
- The policy categorizes rare diseases into **three groups** according to the nature, availability and cost of treatment:
- **Group 1:** Diseases amenable to a **one-time curative treatment**.
- **Group 2:** Diseases requiring **long-term or lifelong treatment** that is relatively less costly and has documented benefits.
- **Group 3:** Diseases for which effective treatment exists but is **very expensive and generally lifelong**.
- As health is primarily a **State subject**, implementation requires coordination between the Union Government, State Governments, Centres of Excellence and specialized medical institutions.

Centres of Excellence:

- The Government has progressively expanded the network of designated **Centres of Excellence**, reaching **15 centres by 2026**.
- These centres provide specialized diagnosis, treatment, multidisciplinary care and access to financial assistance under the national policy.
- Concentrating expertise in designated centres can improve **diagnostic accuracy, treatment protocols, clinical research and patient referral systems**.

Crowdfunding and Financial Gaps:

- Government assistance can substantially reduce treatment costs but may remain inadequate for therapies whose total cost runs into several crores of rupees.
- This has resulted in dependence on **crowdfunding, charitable support and manufacturer assistance programmes** for some high-cost therapies.
- For example, in 2025, a child in Kolkata received a gene therapy costing about **₹16 crore**, after approximately **₹9 crore was raised through crowdfunding**, supplemented by support through the manufacturer's early-access programme.
- The case illustrates the broader problem of relying on **individual fundraising for treatments that may be medically necessary but commercially unaffordable**.

Major Challenges in Developing Rare-Disease Drugs:

Commercial and Financial Challenges:

- The small number of patients with an individual disease **reduces the potential market**, weakening conventional commercial incentives for pharmaceutical research.
- Rare-disease development often involves high expenditure on **genomic research, preclinical studies, clinical trials, regulatory compliance and specialized manufacturing**.
- High treatment prices can further reduce effective demand and create a cycle in which low patient numbers and high prices reinforce each other.

Clinical-Trial Challenges:

- Identifying sufficiently large and geographically distributed **patient cohorts** is difficult because individual rare diseases affect relatively few people.
- Small sample sizes make it difficult to establish statistically robust evidence using conventional clinical-trial designs.
- Researchers must also identify appropriate **clinical endpoints**, particularly when diseases progress slowly or involve multiple organ systems.
- International patient registries, natural-history studies and adaptive trial designs can help address these evidence-generation challenges.

Manufacturing and Distribution Challenges:

- Rare-disease therapies often require **small-batch, highly specialized manufacturing**, which can have high per-unit costs.
- Advanced therapies such as gene and cell therapies require specialized laboratories, trained personnel, quality-control systems and appropriate logistics.
- Even after regulatory approval, **manufacturing scale-up, cold-chain requirements and specialized administration** can restrict access.

India's Potential as a Rare-Disease Pharmaceutical Hub:

Large and Diverse Patient Population:

- India's large population means that even diseases with low prevalence can produce substantial absolute numbers of patients.
- Certain genetic disorders may also have relatively higher prevalence in particular communities because of **population structure and endogamy**, creating opportunities for disease-specific research and cohort identification.

- A coordinated national patient registry can therefore convert India's large population into an advantage for **clinical research and natural-history studies**.

Pharmaceutical and Biotechnology Capacity:

- India possesses strong capabilities in **generic medicines, biosimilars, vaccines and biotechnology**, providing an industrial foundation for expanding into rare-disease therapeutics.
- Emerging capabilities in **gene therapy and cell therapy**, including CAR-T technology, create opportunities to move from generic-drug manufacturing towards higher-value biomedical innovation.
- Lower manufacturing costs and a large pool of scientific and technical professionals could support **cost-efficient small-batch production** if appropriate regulatory and procurement systems are developed.

Regulatory and Policy Measures:

Streamlined Regulatory Pathways:

- The **New Drugs and Clinical Trials Rules, 2019** provide the regulatory framework for new drugs and clinical trials in India.
- India has provisions for regulatory consideration of drugs already approved in specified foreign jurisdictions, reducing unnecessary duplication in some circumstances while retaining regulatory oversight.
- Such mechanisms can reduce development time for rare-disease therapies, particularly where patient populations are too small to justify repeating extensive trials without scientific necessity.

Production and Industrial Support:

- The **Production Linked Incentive Scheme for Pharmaceuticals** seeks to strengthen domestic pharmaceutical manufacturing and promote higher-value pharmaceutical products.
- Greater domestic production can reduce dependence on imported rare-disease medicines and potentially improve **price stability, supply security and treatment availability**.
- Public procurement can further create a predictable market for manufacturers by aggregating demand across government hospitals and designated Centres of Excellence.

Major Government Initiatives:

UMMID Initiative:

- The **Unique Methods of Management and Inherited Disorders initiative** was launched by the Department of Biotechnology to strengthen genetic-disease prevention, diagnosis and management.
- Its major components include:
- Establishment of **NIDAN Kendras** for genetic diagnosis, counselling and comprehensive clinical care.
- Training of government medical professionals in **biochemical genetics, cytogenetics, molecular genetics and clinical genetics**.
- Screening of pregnant women and newborns for selected inherited disorders.
- The initiative links rare-disease policy with **early diagnosis and prevention**, rather than relying exclusively on expensive treatment after disease progression.

Research and Development:

- The Government has supported research through the **Indian Council of Medical Research** and other institutions for development of indigenous therapies for rare diseases.
- A national research ecosystem can reduce dependence on imported technologies and improve the availability of **India-specific genetic and clinical evidence**.
- Current research should increasingly focus on **genomic medicine, gene therapy, cell therapy, precision medicine and affordable biologics**.

What Should India Do Next?

Build a National Rare-Disease Ecosystem:

- Establish interoperable **national patient registries and disease-specific databases** linking hospitals, research institutions and Centres of Excellence.
- Use genomic and clinical data to identify patient cohorts, understand disease progression and improve clinical-trial recruitment.

Reduce the Cost of Therapies:

- Combine **public procurement, pooled purchasing, domestic manufacturing and technology transfer** to reduce treatment costs.
- Encourage Indian manufacturers to develop biosimilars, generic versions and improved manufacturing processes wherever intellectual-property and regulatory conditions permit.
- Develop sustainable financing mechanisms for therapies whose costs exceed existing government assistance.

Improve Regulatory Predictability:

- Create **disease-specific regulatory guidance**, scientific advice mechanisms and proportionate evidence requirements for ultra-rare conditions.
- Encourage international clinical-trial collaboration so that Indian patients can participate in global studies without unnecessary duplication of trials.

Strengthen Prevention and Early Diagnosis:

- Expand **newborn screening, prenatal testing, genetic counselling and molecular diagnostics**.
- Integrate rare-disease screening into appropriate public-health programmes, particularly where early diagnosis can prevent irreversible complications.
- Strengthen genetic counselling to address **carrier detection, reproductive choices and family-level risk assessment**.

Value Addition for UPSC:

GS-II Linkages:

- **Health as a State subject:** Requires Centre-State coordination in financing, infrastructure and service delivery.
- **Equity and access:** Rare-disease treatment raises questions of distributive justice because high treatment costs can exclude low-income households.
- **Public procurement:** Government demand aggregation can correct market failures created by small patient populations.

GS-III Linkages:

- **Pharmaceutical self-reliance:** Rare-disease therapeutics can move India from generic manufacturing towards high-value biotechnology.
- **Innovation ecosystem:** Genomics, gene therapy, cell therapy and precision medicine represent emerging areas of biomedical research.
- **Intellectual property:** India must balance innovation incentives with affordable access to essential therapies.

Key Conceptual Framework:

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- **Rare disease ? Small patient population ? Weak market incentive ? Limited R&D ? Few therapies ? High prices ? Poor access**
- **Policy response: Regulatory incentives + public procurement + domestic manufacturing + research funding + financial protection**
- **Long-term objective: Shift from merely importing expensive orphan drugs to building an **affordable, innovation-driven and globally competitive rare-disease ecosystem.****

Relevant SDGs:

- **SDG 3:** Good Health and Well-being — ensuring access to safe, effective and affordable healthcare.
- **SDG 9:** Industry, Innovation and Infrastructure — strengthening biotechnology and pharmaceutical innovation.
- **SDG 10:** Reduced Inequalities — preventing rare-disease patients from being excluded because of treatment costs.
- **SDG 17:** Partnerships for the Goals — promoting international research and technology collaboration.

Conclusion:

- Rare diseases represent a classic **market-failure problem in healthcare**: the medical need is significant, but the small number of patients with each individual disease limits conventional commercial incentives.
- India can address this gap by combining its **pharmaceutical manufacturing capacity, large and diverse patient population, biotechnology capabilities and expanding regulatory infrastructure.**
- The policy objective should extend beyond providing financial assistance after diagnosis to creating an integrated ecosystem covering **early screening, diagnosis, research, clinical trials, domestic manufacturing, public procurement and sustainable financing.**
- A successful strategy can simultaneously improve **patient access, pharmaceutical self-reliance, biomedical innovation and India's position in the global rare-disease therapeutics market.**