Rare-Disease Drugs

Mains: *GSII – Health*

Why in News?

India has the potential to become a global hub for rare-disease drug development and manufacturing by leveraging its pharma capabilities and large patient base.

What is the Status of Rare Diseases and Orphan Drugs?

- **Rare Disease Drug** – It, referred as an **orphan drug**, is a pharmaceutical agent specifically developed to diagnose, prevent, or treat a rare disease or condition.
- **Definition** – A rare disease is a condition affecting fewer than 65 to-100 per 100,000 people, As per **World Health Organization (WHO).**
- **Global status** –
  - Around 8,000 rare diseases affect hundreds of millions globally.
  - Orphan drugs face limited commercial incentives due to small patient populations and high R&D costs.
  - The U.S. Orphan Drug Act, 1983 introduced incentives such as tax benefits, grants and market exclusivity.
- **India** –
  - Around 1,004 rare genetic disorders have been reported in India.
  - High treatment costs often exceed government support, leading to dependence on crowdfunding.
  - India has strong capabilities in generics, biosimilars and advanced therapies, but rare-disease drug development remains limited.

Some expensive drugs

For rare diseases

Drug	Generic name	Therapy type	Disease	One-time/ Annual treatment	Price (USD)
Lenmeldy	Atidarsagene autotemcel	Stem cell gene therapy	Metachromatic leukodystrophy	One-time	4.25 million
Hemgenix	Etranacogene dezaparvovec	Gene therapy	Haemophilia B	One-time	3.5 million
Elevidys	Delandistrogene moxeparvovec	Gene therapy	Duchenne muscular dystrophy	One-time	3.2 million
Zokinvi	lonafarnib	Small molecule	Hutchinson-Gilford progeria and ultra-rare laminopathies	Annual	1–1.2 million/year
Skyclarys	Omaveloxolone	Small molecule	Friedreich's ataxia	Annual	370,000/year
Trikafta	Combination of elexacaftor, ivacaftor, tezacaftor	Small molecule	Cystic fibrosis	Annual	200,000–300,000/year

What is the Significance of Developing Rare-Disease Drugs?

- **Improving healthcare access** – Domestic production can reduce treatment costs and improve access to expensive medicines.
- **Strengthening self-reliance** – Indigenous drug development can reduce dependence on imported therapies and strengthen pharmaceutical capabilities.
- **Promoting research and innovation** – India's large patient population, genetic diversity and patient registries provide opportunities for clinical trials and drug discovery.
- **Enhancing global competitiveness** – Cost-effective manufacturing can expand pharmaceutical exports and strengthen India's position in specialised medicines.
- **Ensuring healthcare equity** – Affordable treatments can reduce the financial burden on patients and improve access to advanced healthcare.
- **Generating investment and employment** – Research centres, biotechnology facilities and specialised manufacturing can attract investment and create skilled jobs.

What are the Key Areas of Opportunity Developing These Drugs?

- **Clinical research** – Large patient pools and disease registries can support clinical trials and drug development.
- **Domestic manufacturing** – Generic, biosimilar and advanced-therapy capabilities can enable affordable small-batch production.
- **Global collaboration** – Partnerships can promote technology transfer, research and local manufacturing of existing orphan drugs.

What are the Challenges Associated?

- **High R&D costs** – Drug discovery and clinical trials require significant investment despite limited patient populations.
- **Limited commercial viability** – Small markets and uncertain demand reduce incentives for private investment.
- **High treatment costs** – Advanced therapies, particularly gene therapies, can cost crores of rupees.
- **Clinical-trial difficulties** – Identifying suitable patients, maintaining registries and determining trial endpoints remain challenging.
- **Limited treatment availability** – Most rare diseases lack specific approved therapies.
- **Regulatory complexities** – Unclear procedures and lengthy approval processes can delay access to medicines.
- **Manufacturing constraints** – Specialised infrastructure, quality control and small-scale production increase costs.
- **Limited financial protection** – Inadequate insurance coverage and dependence on crowdfunding increase the burden on patients.

What are the Initiatives taken in this Regard So far?

- **National Policy for Rare Diseases, 2021** – Provides financial assistance of up to ₹50 lakh per eligible patient for specified treatments through designated Centres of Excellence.
- **New Drugs and Clinical Trials Rules, 2019** – Rule 101 allows certain drugs approved in six specified countries, including the U.S. and the U.K., to be considered for Indian approval.
    - These drugs doesn't require repeated clinical trials, subject to applicable regulatory requirements.
- **Centres of Excellence** – These provide specialised diagnosis, treatment and care while supporting research and coordination of rare-disease services.
- **Government Crowdfunding Platform –** Enables eligible patients to mobilise additional financial support for expensive treatments.
- **Global Orphan Drug Frameworks –** The U.S. Orphan Drug Act and other countries similar frameworks provide incentives for orphan-drug research and development.

What Measures can be Taken?

- **Streamline regulations** – Establish transparent approval procedures while maintaining strict standards for safety, efficacy and quality.
- **Provide financial incentives** – Introduce tax benefits, research grants and production-linked incentives for orphan-drug development and manufacturing.
- **Strengthen clinical research** – Expand patient registries and promote collaboration between pharmaceutical companies, hospitals and research institutions.
- **Promote public-private partnerships** – Encourage multinational companies to establish drug-development centres and transfer technology to Indian manufacturers.
- **Ensure affordable access** – Introduce government procurement and advanced market commitments to create predictable demand and improve affordability.
- **Strengthen domestic manufacturing** – Support specialised infrastructure, small-

batch production and high-quality manufacturing of orphan drugs.

- **Promote fair pricing** – Develop pricing and benefit-sharing arrangements that ensure Indian patients participating in research can access resulting treatments.
- **Expand international collaboration** – Encourage joint research, clinical trials and technology transfer to strengthen India's role in global rare-disease treatment.

What lies ahead?

- India can build on its strength in generic medicines to develop a globally competitive rare-disease pharmaceutical sector.
- The ultimate objective is to ensure that pharmaceutical innovation translates into affordable and equitable treatment for rare-disease patients.

Reference

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