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Rare Diseases in India: Redesigning the Economics of Treating the Few

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Rare diseases present a fundamental challenge to healthcare economics: small patient populations, high development costs and limited treatment access can push therapies beyond the reach of patients and health systems. For India, the opportunity lies in redesigning this equation through stronger public infrastructure and registries, domestic manufacturing, better diagnostics, innovative financing and regional market scale.



Rare disease sits at the centre of one of healthcare's hardest economic problems. Regulators everywhere draw the line for "rare" at roughly the same order of magnitude, well under one in 2000 people, which sets the scale of the problem: an addressable population of thousands, sometimes hundreds, footing the entire cost of bringing a drug to market. A Reuters analysis of new US drug launches found the median annual list price for drugs launched in 2024 exceeded \$370,000, with orphan indications accounting for 72 per cent of new launches, up sharply from just a few years earlier. Gene therapies sit even further out on the curve, with several now priced above \$3 million per course. That is the bill a small-population model produces when price is the only lever available to recover cost.

India's Multiplier Problem

Government estimates put the country's rare-disease burden at roughly 70 million people, close to one in twenty Indians. Applying international prevalence estimates of 6 to 8 per cent would push that figure as high as 96 million. Individually, these remain rare conditions. Collectively, they add up to one of the largest rare-disease populations in the world. Scale does not translate into affordability, though. Out-of-pocket spending still accounted for 39.4 per cent of India's total health expenditure in 2021–22. That share has fallen sharply, down from 64.2 per cent a decade earlier, but it remains among the highest of any major health system, which means even a therapy priced well below Western benchmarks can sit out of reach for most Indian households.

Where Access Constraints Emerge?

India's rare-disease gap is not only financial. Awareness stays thin at every point in the chain: most families have no reason to suspect a rare condition until symptoms turn severe, and general physicians, usually the first point of contact, get limited exposure to rare-disease recognition during training.

Even once a patient is correctly diagnosed, the barriers do not end. Out-of-pocket costs remain structurally high across India's health system, and specialist treatment capacity sits concentrated in a small number of centres, so an accurate diagnosis does not reliably translate into access.

No single actor can close a gap built from so many layers. It requires a public sector that builds the access architecture funding, registries and notified treatment centres, working alongside a private sector that builds the commercial architecture: discovery models, manufacturing capacity, diagnostic infrastructure and financing structures that make treating small patient populations viable. What follows is best read as two connected halves of one system: what India's public sector has already built and what its private sector still needs to build on top of it.

What the Public Sector Has Already Built

India's National Policy for Rare Diseases, launched in 2021, sorts conditions into three groups by treatment cost and duration rather than applying one uniform definition, and funds up to Rs 50 lakh per patient through Centres of Excellence, which now number twelve, up from eight at launch, covering 63 notified conditions. The National Registry for Rare and Other Inherited Disorders had enrolled 9,675 patients as of July 2023, per government figures. This is useful scaffolding, but it funds access at the margin. It does not change what a therapy costs to discover, manufacture, deliver or pay for, which is where the private sector's job begins.

The Private Sector's Playbook

Discovery. Rare-disease R&D needs to shift from molecule-by-molecule bets to platform-led capability, since no portfolio scales if every programme requires its own discovery engine and its own development playbook. Recursion Pharmaceuticals illustrates the model: its lead rare-disease asset, REC-4881, for familial adenomatous polyposis, was identified through the company's shared AI-driven discovery platform rather than a bespoke research effort built around that single condition, and the same platform now underwrites a broader pipeline across oncology and rare disease. Ultragenyx shows the same logic at the portfolio level, where rare-disease expertise compounds in value when applied across biologics, small molecules, gene therapies and antisense oligonucleotides rather than tied to one asset.

Manufacturing. India already has two working examples of what domestic manufacturing does to therapy cost. Eliglustat, used for Gaucher disease, fell from roughly Rs 1.8–3.6 crore a year as an imported therapy to Rs 3–6 lakh once production moved onshore under the Production Linked Incentive (PLI) scheme. Risdiplam, for spinal muscular atrophy, followed the same path: Biophore India Pharmaceuticals now manufactures the API domestically, and Natco Pharma has launched a generic version. Both cases confirm the underlying mechanism: cost falls when manufacturing capability sits inside the market it serves rather than depending on imports. The task now is to systematise this, building API and formulation capability ahead of commercial launch for the next wave of rare-disease molecules, rather than reacting to an import-price problem after it has already set in.

Diagnosis. The addressable market for any therapy is never the disease burden on paper. It is the number of patients who are diagnosed, findable and clinically eligible for treatment, which makes diagnostic infrastructure a commercial asset, not just a clinical one. India's registry and Centre of Excellence network form an early foundation. CENTOGENE's rare-disease biodata platform, which combines genomic and clinical data from patients across more than 120 countries to support diagnosis and drug development, shows what that infrastructure looks like once built at scale.

Financing. India's most instructive example here comes from an adjacent field. ImmunoACT, an IIT Bombay spin-off working with Tata Memorial Centre, developed NexCAR19, an indigenously manufactured CAR-T cell therapy, and priced it at roughly Rs 30–40 lakh, about one-tenth the cost of comparable therapies abroad. ImmunoACT then partnered with Mango Sciences to layer a zero-interest EMI plan and a value-based pricing option on top of that already-lower price, extending affordability further for clinically eligible patients. The sequence is the template worth replicating in rare disease: bring the manufactured cost down first, then build a financing structure, whether an EMI plan, a value-based agreement, or pooled reimbursement, on top of that lower base rather than trying to finance the imported price.

Scale. A single-country patient pool is often too small to justify commercial investment on its own, even once cost and financing are addressed, and this is where India has a structural advantage it has not yet used. Populations across South Asia, the Middle East and North Africa carry a disproportionate, overlapping burden of the same single-gene disorders, a consequence of high rates of consanguinity in those regions. A therapy developed and manufactured in India for a specific inherited condition is rarely serving an Indian market of a few thousand patients alone. It is serving a regional market spanning several countries with genetically overlapping populations. Building commercial and regulatory pathways that treat this as one addressable market, rather than as isolated national markets, is what turns a subscale opportunity into a viable one.

The Real Test

Viability depends on whether India can lower the recurring cost of finding patients, developing therapies, manufacturing products, paying for treatment, and reaching a patient pool large enough to justify the investment, and do it as one connected system rather than five disconnected initiatives.

India's opportunity is to prove that rare-disease innovation can scale on operating discipline rather than premium pricing. If it succeeds, the model will matter well beyond India, in every health system now confronting the same arithmetic: small patient populations set against unsustainable therapy costs.