Tackling Rare Diseases in India:
Orphan Drugs & Market Opportunities
A WNS Perspective
Rare Diseases: An Emerging Field

With a staggering ~350 million cases worldwide, rare diseases seem anything but rare. India alone accounts for some 72-96 million such patients. This means one in every 20 Indians suffers from a rare disorder. In reality, what we classify as a rare disease may only be rare in certain regions and not rare overall.

In order to facilitate research, development of cures and policy-making, a uniform definition for rare disorders is essential. The World Health Organization (WHO) defines a rare disease as an ‘often debilitating lifelong disease or disorder with a prevalence of one or less per 1000 population.’

Different countries have their own standard definition vis-à-vis their population particulars. The US – the first country to enact the Orphan Drug Act to facilitate drug development for rare diseases – considers a disease rare if it affects fewer than 200,000 patients. In Japan, the figure must be below 50,000 patients, and in the EU, less than five in 10,000 patients.

India is yet to standardize its definition of rare disorders. The country’s ability to classify rare diseases in terms of prevalence (or prevalence rate) has been limited by a lack of epidemiological data. However, this may soon change: since 2017, the National Registry for Rare Diseases has been capturing data on rare and ultra-rare disorders. As of today, data collection is still under progress.

Figure 1: A Snapshot of Rare Diseases in India and the World

- More than 350 Million people worldwide have a rare disease.
- In India, ~72-96 Million people are affected by a rare disease.
- 95% of rare diseases have no approved treatment.
- Diagnosis takes an average of 7 years.
- 70% of rare diseases start in childhood.
- 80% are genetic.
- Estimated number of rare diseases: 7,000
- 450 rare diseases have been recorded in India.

1 Rare Diseases India
2 Rare diseases in India: Still a blind spot | ORF (orfonline.org)
India’s Rare-Disease Burden

Of the roughly 350 million known global cases, one-fifth come from India. It is challenging to determine the precise prevalence of rare diseases in the country for lack of a standard definition. Based on the international estimate that 6-8 percent of the population suffers from rare diseases, India may have about 72-96 million affected people.

Globally, there are an estimated 6,000-8,000 rare diseases, with India recording 450 rare diseases from tertiary hospitals. However, 80 percent of the patients suffer from just about 350 diseases. The most common ones include hemophilia, thalassemia, sickle-cell anemia, primary immunodeficiency, autoimmune diseases, lysosomal storage disorders (such as Pompe disease, Hirschsprung’s disease, Gaucher’s disease, cystic fibrosis, hemangioma) and certain forms of muscular dystrophies.

Management of Rare Disorders

In India, it takes at least seven years for a rare disease to be detected. Often, it is misdiagnosed due to the lack, inaccessibility or complexity of diagnostic procedures and techniques, as well as a lack of awareness among the medical community and the general public.

Furthermore, only one in 10 patients in the country receives targeted treatment. About 95 percent of the world’s rare diseases do not have approved treatments. When they do, the cures can be extremely expensive, with the average price of a drug exceeding USD 100,000 a year. For pharma companies, this can result in revenues equivalent to traditional blockbuster drugs.

However, it also means that many patients do not have access to vital care. To make such drugs more affordable, government measures are needed to lower the price and encourage generic and local manufacturing.

The Ministry of Health and Family Welfare has established a comprehensive National Policy for Rare Diseases, 2021 to address all the issues related to the diagnosis and treatment of rare diseases. It is supported in the collection of epidemiological data by the hospital-based National Registry for Rare Diseases. Through this initiative, institutions across the nation are engaged in the diagnosis and management of rare diseases.

Additionally, the government of the national capital territory of Delhi has appointed an interdisciplinary committee on rare diseases. One of them is the Prioritization of Therapy for Rare Genetic Disorders, which elaborates on the available therapies and prioritizes genetic disorders based on resources, cost of therapy (one-time vs long-term), evidence-based outcomes, quality of life acquired and published guidelines.

Given that ~80 percent of rare diseases are believed to originate in our genes, it can be useful for women to undergo genetic screening in the early stages of pregnancy.

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3Rare Disease Facts | ORD India
4Rare Diseases in India: ‘Orphan’ No More? | ORD India
5www.nhp.gov.in
6Microsoft Word - NPRD FINAL.docx (mohfw.gov.in)
7National Policy for Rare Diseases 2021 | National Portal of India
Orphan Drugs: A Vital Need in India

In addition to the benefits for patients, orphan drugs also represent a significant market opportunity for pharmaceutical companies. The global orphan drug market is projected to reach over USD 250 Billion by 2025, driven by the increasing prevalence of rare diseases and the growing demand for effective treatments.

While this market remains largely untapped in India, launching orphan drugs can be challenging due to the complex regulatory landscape, limited healthcare infrastructure and high treatment cost.

Figure 2: Spotting Opportunities and Overcoming Challenges – A SWOT Analysis of the Rare Diseases Market in India for Pharmaceutical Companies

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<th>Strengths</th>
<th>Weaknesses</th>
<th>Opportunities</th>
<th>Threats</th>
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<td>- There are significant unmet medical needs, creating potential demand for pharma companies.</td>
<td>- Lack of awareness and understanding among the general public leads to delayed diagnoses and treatments.</td>
<td>- The potential for growth in this sector will rise as more patients get diagnosed and treated.</td>
<td>- There is competition from local and international pharma companies.</td>
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<td>- The large patient population for certain rare diseases offers a significant market opportunity.</td>
<td>- Limited diagnostic facilities and infrastructure in many areas give rise to challenges in identification and treatment.</td>
<td>- There is an increasing focus on rare diseases in both the public and private sectors, creating a supportive environment for pharma firms.</td>
<td>- Limited healthcare budgets and resources can lead to challenges in accessing and paying for treatments.</td>
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<td>- There are favorable government policies and initiatives, such as tax exemptions and fast-track approvals for orphan drugs.</td>
<td>- The high cost of treatment and limited insurance coverage create barriers to patient access.</td>
<td>- The opportunities for innovation and research indicate a potential for new and improved treatments.</td>
<td>- There are complex and time-consuming regulatory approval processes for orphan drugs.</td>
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<td>- There is access to a pool of skilled healthcare professionals and researchers.</td>
<td>- India’s complex and evolving regulatory landscape can pose a challenge for pharma companies launching orphan drugs.</td>
<td>- The increasing use of telemedicine and digital healthcare solutions is creating new channels for patient access and treatment.</td>
<td>- Intellectual property issues and legal challenges can be expected in the Indian pharmaceutical industry.</td>
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https://www.recordati.com/resources/Pubblicazione/___ea24749bd98d4e02a003259f7af40d73_/3y-plan-presentation.pdf
Launching Orphan Drugs in India: How Strategic Partnerships Can Pave the Way

Consulting firms and strategic partners can play a crucial role in supporting pharmaceutical companies in launching orphan drugs in India. In turn, this accelerates improvements in access to treatments and healthcare outcomes for rare disease patients. Here are a few key ways in which a strategic partner can provide vital information and support for business insights in this area:

**Market Research**
- Provide research and analysis on the Indian healthcare market and the needs of rare disease patients
- Acquire and analyze market data to help identify potential opportunities and challenges

**Regulatory Compliance**
- Help navigate India’s complex regulatory landscape and ensure that the orphan drugs in question meet all the necessary requirements

**Pricing and Reimbursement Strategy**
- Help develop pricing and reimbursement strategies that are both affordable for patients and profitable for the company

**Patient Access**
- Help craft efficient market-access strategies to ensure orphan drugs are accessible to more patients

**Stakeholder Engagement**
- Support engagement with key stakeholders, such as patient groups, healthcare providers and policymakers, to raise awareness about rare diseases and the need for orphan drugs
The research and treatment of rare diseases in India are poised to make great strides. This fairly untapped market poses huge revenue opportunities for pharmaceutical leaders, especially in the field of orphan drugs. The potential market size is large, with an estimated 70 million people in need of therapy. Launching orphan drugs can provide these patients vital treatment options.

Collaboration between drug makers and consulting firms is critical to the success of new orphan drugs. Strategic research partners can provide invaluable information and insights to navigate the complex regulatory landscape, develop the right pricing and reimbursement strategies, and create roadmaps for widespread market access.

The impact of orphan drugs on rare disease patients cannot be overstated. For many, these medicines represent the only chance for effective treatment and an improved quality of life. It is, therefore, necessary to accelerate the launch of orphan drugs.

As a first step, it is essential to raise greater awareness about rare diseases and the need for orphan drugs. Collaboration between stakeholders, including patients, healthcare providers, policymakers and pharmaceutical companies, will be critical to ensure such dissemination and to plan holistic, accessible patient care.

To explore more about how consulting firms can provide crucial assistance in overcoming the challenges of launching orphan drugs in India, click here.
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