

# Orphan Drug Development Policies in India and the United States: A Comparison

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## ABSTRACT

**Background:** A pharmacological agent known as an orphan drug was created to address rare or orphan diseases. The Food and Drug Administration (FDA) claims that the Orphan Drug Act has certainly encouraged the development of treatments for rare diseases. The Orphan Drug Act was passed in the United States in 1983 to encourage pharmaceutical companies to develop treatments for rare diseases. Global sales of orphan drugs are anticipated to increase at a Compound Annual Growth Rate (CAGR) of 12.3% from 2019 to 2024, which is roughly double the rate anticipated for the non-orphan drug industry. India has between 72 and 96 million people who are affected by rare diseases. There are no specific orphan drug laws in India. The Ministry of Health and Family Welfare of India established a National Policy for the Treatment of Rare Diseases in July 2017 (NPTRD). However, there were challenges to the execution of the policy. The Orphan Drug Act, which was passed on January 4, 1983, gave orphan medications legal status in the United States. The lack of orphan drug regulation hurts the Indian pharmaceutical businesses' ability to thrive economically. Contrarily, the US's orphan drug regulations have been effective in bringing therapies to people with rare disorders. The assessment of the scope and impact of orphan diseases, as well as a widespread awareness campaign about orphan diseases, are urgently needed in the current situation. Orphan drug development requires strong policies and initiatives from both public and private institutions. **Materials and Methods:** This review article is based on the information collected from various sources from FDA and NPTRD and the articles mentioned. **Results:** The different guidelines and regulations for the development and manufacture of orphan pharmaceuticals should be put into place to ensure that orphan diseases get the necessary treatment and care. **Conclusion:** The FDA regularly updates and the Orphan Drug Act provides direction for the overall rules and regulations in the United States. In India, being a semi-regulated market, there are no such specific rules and regulations regarding development. The National Policy for Rare Disease in India 2021 helps as a guidance document regarding the policies related to orphan drugs in India.

**Keywords:** Orphan Drugs, Orphan diseases, Rare diseases, Initiative, Treatment.

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## INTRODUCTION

The United States Food and Drug Administration (FDA) defines an orphan drug as one which is "designed for the treatment, prevention or diagnosis of a rare disease or condition, which affects less than 200,000 persons in the US" (equating to about 6 cases/10,000 inhabitants), or one that "meets cost recovery provisions of the act." To incentivize pharmaceutical companies to create cures for rare diseases, the Orphan Drug Act was passed in the US in 1983. In India, there are no rules governing orphan pharmaceuticals, however, the Central Drugs Standard Control Organization (CDSCO) defines orphan drugs as "those meant to cure illnesses that only affect fewer than 200,000 people".<sup>1</sup>

- Challenges in research and development
- Less Known Pathophysiology
- Lacks published data
- Small patient pool

The detailed challenges are given below:

- The pathogenesis and natural history of these disorders are poorly understood.
- The patient population is insignificant, which frequently results in a lack of clinical experience. As a result, medical explanations for unusual illnesses may be misconstrued or fall short.
- Rare diseases are frequently poorly characterized and lack of published evidence on long-term therapy outcomes.



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**Table 1: Examples of Orphan Diseases and Drugs.<sup>6</sup>**

Orphan Diseases	Orphan Drugs
Cystic Fibrosis	Pulmozyme and tobramycin
Neurodegeneration Associated with Phospholipase 2G6	RT001
Hereditary Amyloidosis Related to Transthyretin	Patisiran
Tbi	BHR-100 (progesterone)
Subarachnoid Hemorrhage	Nimodipine, Glyburide
Hemophilia B	Alprolix
Young-onset Parkinson disease	Apokyn
Chronic myeloid leukemia	Bosulif
Systemic primary carnitine deficiency	Carnitor
Prader-Willi syndrome	Genotropin
Multiple myeloma	Thalidomide Celgene

## DISCUSSION

### Challenges in Treatment

#### Unavailability of treatment

Medication availability and access are critical for reducing morbidity and mortality associated with rare diseases. Despite recent advances, the majority of rare diseases continue to lack access to safe and effective treatments. As a result, even if an accurate diagnosis is obtained, the rare disease may lack a treatment option. There are treatments available for less than 5% of the 7000-8000 rare diseases. Less than one in ten people receive treatment specifically for their condition, and more than 95 percent of rare diseases have no approved treatments. Where pharmaceuticals are available, their high-cost places enormous strain on resources.<sup>2</sup> Here are some examples of orphan drugs and diseases Table 1.

#### Prohibitive cost of treatment

Except for Food for Special Medical Purposes (FSMP) for small molecule inborn errors of metabolism, India currently lacks local pharmaceutical manufacturers for uncommon disorders. Because of the high cost of most medicines, the government has been unable to provide them for free. The annual cost of treating some uncommon disorders for a child weighing 10 kg can range from 10 lakhs to more than 1 crore, with the cost and dosage of the drugs increasing with age and weight. Countries have used a variety of strategies tailored to their specific circumstances to address the issue of high costs.<sup>3</sup>

In the United States and Canada, laws such as the Orphan Drug Act (ODA) provide incentives to drug manufacturers to encourage them to develop drugs for rare diseases. The Act's

economic incentives and safeguards ensure that local patients benefit. However, the exorbitant prices of rare disease drugs have raised concerns even in developed countries about the long-term viability of rare disease funding/reimbursement programs. The exorbitant prices have prompted calls for transparency in drug pricing and price control, as well as scrutiny and congressional inquiries.

In 2019 New Drugs and Clinical Trial Regulations (the "New Drugs and CT Rules") say that an orphan drug is a treatment "designed to treat an illness that affects less than five lakhs (500,000) people in India."

With a few exceptions, the rules for a clinical study of an orphan drug are the same as for any other drug. The following are the breaks:

In the case of orphan drugs, India's highest regulatory body, the Central Drugs Control Standards Organization ("CDSCO"), may waive the need for local clinical studies.

The individual who pays for an orphan drug's clinical trials may request that the CDSCO enhance and improve the orphan pharmaceutical approval process.

There is no fee to apply to conduct a clinical trial for an Orphan Drug.<sup>4</sup>

If the Central Drugs Standard Control Organization recognizes a country's marketing authorizers, the drug will not have to go through local clinical trials to get marketing authorization. Still, the new drug won't be able to go on the market until it gets its approval. But, unlike with most other drugs, the CDSCO can change the rules for Orphan Drugs' post-market surveillance.

The orphan drug designation program grants orphan drug designation to pharmaceuticals and biologics that are intended to treat, prevent, or diagnose a rare disease that affects fewer than 200,000 people in the United States, or that meet the act's cost recovery requirement.

### Rare diseases and their associated orphan drugs

An orphan drug-A pharmacological agent known as an orphan drug was created to address rare or orphan diseases. Since production would not be profitable without government support and because they are so uncommon. It is a classification determined by a few factors.<sup>5</sup>

A rare disease-Although the term "rare diseases" is defined slightly differently in each country, the fundamental idea—that there are relatively few patients—remains the same.

### History of Orphan Drugs

Throughout the twentieth century, scientific advances resulted in the expansion of numerous medical products and therapeutic advances for patients. However, it became increasingly obvious

in the late 1970s that many residents were being shut out of these advancements. The tiny size of particular patient populations was one of the main causes of this neglect. Ironically, by the start of the 1980s, there were 20–25 million patients suffering from these "rare diseases," with a total of about 5000 rare diseases, some of which only affected a dozen or so people.

The Orphan Pharma Act of 1983 is regarded as a significant piece of legislation because it addressed issues concerning rare diseases and ailments that had previously gone unnoticed in the pharmaceutical industry.

Waxman and others utilized the concept of "hope" to support their legislative efforts, and it has occasionally been echoed in academic analyses. This concept emphasizes the Act's beneficial effects on the lives of the individuals it affects.

Previously "without hope," people with rare diseases now have reason to anticipate that remedies will be developed; this development is also discernible in their transition from "victims" of orphan diseases to "consumers" of orphan products. However, if the medicine is developed and our people are unable to buy it, as Christine Hayes of the Huntington's Disease Society of America stated in 1992, "hope is worthless."<sup>7</sup>

According to the ongoing debate over the exorbitant prices of some orphan pharmaceuticals, some people with rare diseases may discover that the hope they have been given is unfounded. The Orphan Drug Act of 1983 grants 7 years of commercial exclusivity to goods used to treat uncommon illnesses and ailments affecting fewer than 200,000 patients in the United States. Orphan Drug exclusivity, which is available for both drugs and biologics, accomplishes more than simply deferring the use of a shortened approval pathway, i.e., the Abbreviated New Drug Approval process (ANDA). The 1983 Orphan Drug Act fundamentally altered the therapeutic landscape for rare diseases.

By 1990, 49 of the 370 medications given orphan status by the FDA had received approval for orphan indications.

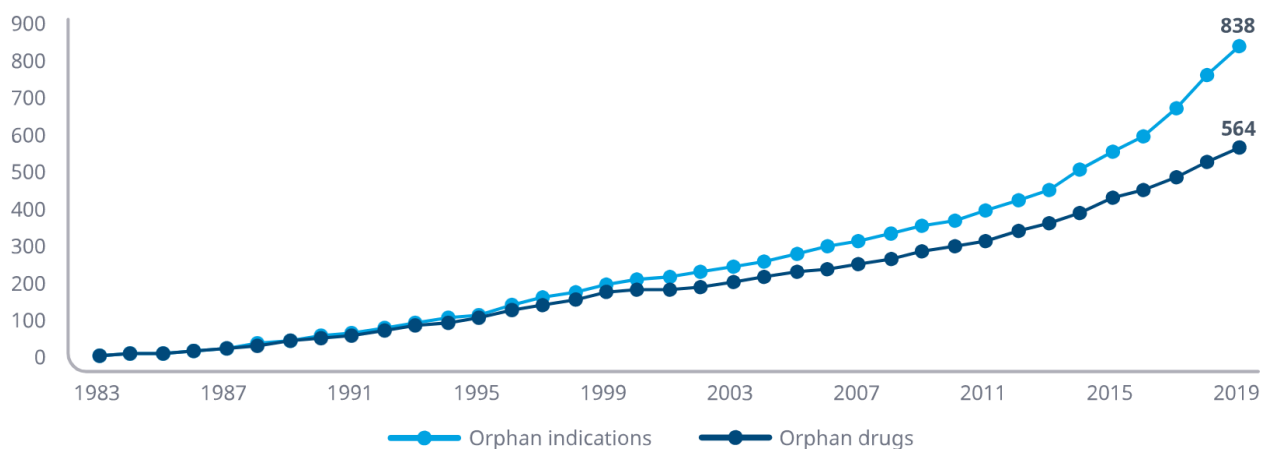
By 2002, there were close to 1100 orphan designations and 232 approvals, providing care for an estimated 11 million patients. Considering how many people suffered from rare ailments, there's still much work to be done. But because of the efforts of numerous people, not the least of whom were those patients and their friends who had long defended the rights of the forgotten patients, the Orphan Drug Act eventually provided for many of those who had been abandoned by popular medicines a chance of their own Figure 1.

### Importance of Orphan Drug

The FDA claims that the Orphan Drug Act has certainly encouraged the development of treatments for rare diseases. Since the statute's enactment in 1983, the Office of Orphan Goods Development has identified approximately one thousand orphan items, of which more than two hundred have subsequently received marketing permission. Technical advances include several orphan medications, including those licensed for hemophilia, cystic fibrosis, and multiple sclerosis.

The most powerful incentive provided by the Orphan Drug Act, marketing exclusivity, which takes effect on the date of marketing approval, continues to limit competition by prohibiting the FDA from approving a different formulation of the same orphan drug for the same indication unless the new medication is clinically superior. The promise of marketing exclusivity, which is especially important for small businesses seeking public and private capital, is far more important than other incentives such as tax breaks and waivers of user fees. The lack of exclusivity, however, does not preclude businesses from gaining market share through traditional channels.<sup>8</sup>

The other advantages are as follows:



**Figure 1:** Explaining the Orphan indications and Orphan Drugs in the particular year.  
**Source:** IQVIA institute (Aug 2020:FDA Orphan Drug Designation and Approvals)

## Shortages rarely occur

Patients can obtain even the most expensive orphan products.

## The market for orphan drugs

From 2019 to 2024, it is predicted that orphan drug sales will grow globally at a CAGR of 12.3%, which is about twice as fast as that predicted for the non-orphan drug sector. Orphan medication markets are anticipated to generate \$242 billion by 2024, accounting for one-fifth of all prescription drug sales worldwide.

## Orphan Drug Development in India

In India, rare diseases constitute a hazard to the general public's health. The universe of rare diseases is complicated, diversified, ever-changing, and medically and scientifically uneducated. In India, over 450 rare diseases have been reported. Because of the following factors, rare diseases pose a serious challenge to public health systems worldwide and in India: it is difficult to collect epidemiological data, which prevents estimations of the disease burden and costs; it is difficult to conduct research and development; it is difficult to make an accurate and timely diagnosis; The illness is costly and untreatable, and it necessitates complex tertiary level management requiring long-term care and rehabilitation. Despite a country's size and population, rare diseases create a large economic burden owing to increasing healthcare expenditures. Limited resources present a macroeconomic allocation conundrum because of the opportunity cost of funding the treatment of rare diseases: On the one hand, a far higher number of people's health difficulties can be resolved with a relatively lower amount of money, whereas the health issues of a relatively smaller number of people will require significantly more resources. India, like many other developing countries, currently lacks a common definition of rare diseases as well as prevalence data. There are no statistics on the burden of rare diseases or the morbidity and mortality associated with them because there are no epidemiological data.<sup>3</sup>

In comparison to the international average of 6 to 8% of the population affected by rare diseases, India has between 72 and 96 million people affected by rare diseases. However, because there are no current statistics on the prevalence of rare diseases, India will need to develop its estimate and definition of rare diseases.

Only 450 uncommon diseases have been found in India to date, according to tertiary care facilities. Certain actions are required by the government's and Indian pharmacists' initiative to enact laws that would improve the nation's health system and provide relief to the numerous individuals suffering from rare diseases and illnesses.

A group of pharmacologists requested that the Indian Government implement the Orphan Drug Act in India at a

meeting held by the Indian Drugs Manufacturers Association in 2001.

In July 2017, the Indian Ministry of Health and Family Welfare established a National Policy for the Treatment of Rare Diseases (NPTRD). However, there were challenges in implementing the policy. Getting states on board, as well as a lack of clarity about federal funding for tertiary care, hampered its implementation.

To analyze the NPTRD, 2017, the Ministry of Health and Family Welfare appointed a Committee of Experts in November 2018. The followings are the Terms and References for the Expert Committee:

- Consider making any required adjustments to the 2017 National Policy for the Treatment of Rare Diseases.
- To give India's definition of "Rare Diseases."
- Creating a National Rare Diseases Policy.
- To offer an idea as well as a plan within the limitations of the country.

## The National Policy for Rare Disease in India 2021

In July 2017, the Government of India's Ministry of Health and Family Welfare established a National Policy for the Treatment of Rare Diseases (NPTRD). However, there were numerous problems with the policy's implementation. Its implementation was hampered by two factors: securing state cooperation and a lack of information about the potential scope of government funding for tertiary care. Some of the issues raised by State Governments when the policy was shared with them included the cost-effectiveness of interventions for rare diseases in comparison to other health priorities, the division of costs between the Central and State Governments, and the State Governments' flexibility to accept or modify the policy in light of their circumstances.

To review the NPTRD, 2017, the Ministry of Health and Family Welfare established an Expert Committee in November 2018. The Expert Committee's Terms and References are listed underneath:

The 2017 National Policy for Treating Rare Diseases should be examined, and any necessary updates or modifications should be advised.

- To explain rare illnesses in India.
- To establish a national rare disease policy.
- To put forth a vision and plan of action for the nation.<sup>9</sup>

The draught National Policy for Rare Diseases was finalized and made available to the public on January 13, 2020, based on the Expert Committee's report and with the approval of the relevant authority, inviting comments/views from all stakeholders, the general public, organizations, and States/UTs. For review and recommendation, the DGHS (Directorate General of Health Services) received comments and suggestions from the general



**Table 2: Comparison of parameters between the US and India.**

Parameters	USA	India
Legal framework	Orphan Drug Act (1983) <sup>16</sup>	NPRD-2021
Administrative authorities involved	FDA / OOPD	Ministry of Health India <sup>17</sup>
Disease prevalence (per 10,000 people), supporting the designation as an orphan	7.5	1 to 6
Estimation of the impacted population and incidence rate (per 10,000 individuals)	20 million	-
Marketing exclusivity <sup>18</sup>	7 years	-
Tax credit	Yes: It is 50% for clinical studies	Yes: It is a 25% Tax Credit for qualified clinical studies.
Grants for research	Programs of NIH and others	Ministry of Health India
Applications for orphan designation are being re-examined.	No	No
Assistance with technical aspects of developing the application file.	Yes	Yes
Expedited marketing process.	Yes	Yes

public, organizations, stakeholder groups, states, and UTs. The DGHS formed an Expert Committee to review the comments and proposals submitted. The National Policy for Rare Diseases has been finalized after taking into account the feedback/suggestions received, the recommendations of the same Expert Committee, and additional consideration.

The absence of epidemiological data limits India's ability to classify rare diseases based on prevalence or prevalence rate, as other countries have done. To combat this, the Indian Council of Medical Research (ICMR) has established a hospital-based National Registry for Rare Diseases, which includes organizations from across the country involved in the diagnosis and treatment of Rare Diseases. This will provide critical epidemiology information for rare diseases.<sup>10</sup>

## Support for treatment from the Indian government

### *The following actions will be made to benefit people with rare diseases*

Under the Rashtriya Arogya Nidhi Umbrella Scheme, the Central Government will provide financial assistance of up to Rs. 20 lakhs for the treatment of such rare diseases that require a single medication (diseases listed under Group 1). Not only would BPL households be eligible, but so would approximately 40% of the general public who meet the 23 eligibility conditions of the Pradhan Mantri Jan Arogya Yojana for treatment exclusively at government tertiary institutions.

State governments have to think about providing financial assistance to those with such rare diseases that are treatable with specialized diets, hormone supplements, or other reasonably priced therapies (Diseases listed under Group 2).

The government will attempt to build an alternative funding source by developing a digital platform for voluntary individual and corporate contributors to contribute to the treatment costs of patients with rare diseases. To maximize the health benefits for the community and people, this is done while keeping resource constraints and the urgent need to prioritize existing resources in mind.

## Orphan Drug Act 1983

Congress enacted the Orphan Drug Act in 1983 to promote the creation of drugs for rare diseases. There was little incentive for the commercial sector to create drugs for tiny patient populations before the passing of this historic statute because these treatments were thought to be ineffective. The law offers three advantages:

Sponsors of approved orphan products will have seven years of market exclusivity; A tax break equivalent to 50% of the price of carrying out human clinical trials; and Clinical trials of innovative medications to treat and/or diagnose rare illnesses are eligible for federal research funding.

Although the 1985 amendment expanded commercial exclusivity to include both patentable and unpatentable drugs, the 1988 amendment required sponsors to seek orphan status before submitting a marketing authorization application.<sup>11</sup>

By exempting businesses creating orphan pharmaceuticals from the Food and Drug Administration's usual drug application or "user" fees in 1997, Congress added another incentive (FDA). These fees will come to around \$500,000 for the 2001 fiscal year. If a company's product cures a life-threatening ailment, its marketing authorization application may be eligible for expedited review. Numerous orphan drugs treat fatal or life-threatening conditions. The money supports clinical research on the safety and efficacy of therapies for uncommon diseases. Requests for marketing permission are evaluated by the Center for Drug

Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER).

### Orphan Drug Development in the USA

The term "orphan drug" has a broader definition in the US than just pharmaceuticals and biologicals. It also includes dietary supplements and medicinal gadgets. Within the FDA, the OOPD (Office of Orphan Products Development) was established (Food and Drug Administration). Its job is to encourage the development of secure and effective treatments for uncommon diseases. The "orphan" classification of these treatments allows the drug sponsor to benefit from incentives for their development up until commercial approval.

All phases of the drug development process must follow these guidelines

### Tax credits for clinical research are available

Technical support during the file's elaboration and the streamlining of administrative processes are required for an application file that is required for marketing approval (shortening the waiting period and lowering the registration costs).

Marketing: Seven years of exclusivity following the granting of the marketing approval.<sup>12</sup>

### American public policy regarding orphan pharmaceuticals

The Orphan Drug Act, which was passed on January 4, 1983, gave orphan medications legal status in the United States. Orphan pharmaceuticals are prescribed for ailments or illnesses that are so rare in the USA that it is unrealistic to expect that the cost of developing and distributing them in the nation will be covered by sales of the treatments there. The goal of the following adoption of other modifications was to specify the requirements orphan medications must adhere to:

- A 1984 amendment established the terms low incidence and low prevalence.
- A rare condition or sickness by definition:
- Affects fewer than 200 000 people in the USA;
- More than 200 000 persons in the USA are affected by it, but the cost of development and distribution cannot be recouped through domestic sales.
- The amendments of 1985 and 1990:<sup>13</sup>
- The term "orphan product" was expanded to include goods besides medicines, including biologicals, medical equipment, and foods used for parenteral nourishment and nutraceuticals.

### The amendment of 1988

Before applying orphan drug classification, the product must be the foundation of a Marketing Authorization. The drug cannot have previously obtained approval following a New Drug Application (NDA) or a Product Licence Application for the ailment or condition for which the applicant requests orphan drug classification.

Orphan drug designation: for a disease or condition

### The modification of 1992

The applicant must demonstrate that the medication is clinically superior to one of the orphan medications already approved for the same uncommon condition, in which case it will be treated in the same manner as a new active component. In terms of the diagnosis, prevention, or treatment of this condition, the drug's efficacy must be proven.

The designation for the same medicine for the same user may be granted to more than one sponsor; however, the first sponsor to submit a completely New Drug Application (NDA) will be granted seven-year marketing exclusivity. Competitors are not prohibited from making the medicine available for numerous uses during the seven-year exclusivity period. In the United States, orphan drugs can be used to treat 6.5 million patients. Since the law was enacted by the USA in 1983, more than 700 orphan drugs have been developed by pharmaceutical companies in the USA.

### Labeling and legal status of orphan drugs

Orphan drugs are given a special status by a particular individual legal system that covers them. The Office of Orphan Products Development's (OOPD) application dossier is what determines whether medicine is given orphan drug classification.

Common administrative information (name and address of the manufacturer, name, and address of the sponsor, trade name of the drug, international non-proprietary name of the drug).

The FDA has the sponsor's consent to publish information about the product and the approved indication.

Regarding the size and other key characteristics of the population most likely to need medical attention in the United States, all available information, whether published or not, must be disclosed.

A summary of the drug's risks and benefits, a simple paperwork summary as well as an overview of the key preclinical and clinical data about the usage of the product for the relevant indication.

The medicine's estimated development and distribution costs and prospective sales in the USA both support the conclusion that distributing the treatment in particular circumstances would not be commercially viable.

The FDA must respond to the sponsor within 60 days of receiving the application. The FDA posts this information in the Federal Register when a medicine is given the "orphan" designation. Before an orphan medicine can be commercialized, two steps must be completed: orphan designation and a marketing authorization application. An exclusive FDA entity makes each decision.

Research and Development, intellectual property, and marketing incentives for orphan drug manufacturers.

If orphan drug status is granted, the sponsor may profit from the following for the development of the product:

A 50% tax credit off the cost of clinical studies carried out in the USA.

A period of seven years for commercial exclusivity following marketing approval.

The FDA offered some written guidelines on the clinical and preclinical research that must be done to register the new medicine.

A quick review process for registration submissions by the FDA.

Before receiving marketing approval, orphan medications may be made accessible to patients. A Treatment Investigational New Drug (t-IND) may be obtained for compassionate use in some circumstances provided certain requirements are met.

The medication needs to be used to treat a serious or life-threatening illness;

There must be no substitute medication or therapy;

The product must be undergoing clinical testing and must be actively pursuing commercial approval.

INDs are issued for a specific amount of time due to the latter reason.<sup>1</sup>

## **Policies on the orphan drug in the USA and India**

### ***Comparison between India and the United States***

Such an act is required by the government's and the Indian pharmacists' initiative to pass laws that would improve the nation's healthcare system and provide relief to countless individuals suffering from rare diseases. Pharmacologists urged the Indian government to put the Orphan Drug Act into effect in India during a meeting organized by the Indian Drugs Manufacturers Association in 2001. In India, there are between 6,000 and 8,000 rare diseases, some of which include Cystic Fibrosis, Wilson Disease, Arthrogryposis, Leishmaniosis, Norrie Disease, etc. The majority of these illnesses are hereditary in origin and are untreatable. In India, 72, 611, 605 persons are affected by these rare diseases. The lack of orphan drug regulation hurts the Indian pharmaceutical businesses' ability to thrive economically Table 2.

Numerous patients with uncommon diseases shouldn't be left in their dreadful circumstances without any care or treatment. The Indian government needs to act right away to solve the issue of uncommon diseases. A relevant piece of legislation should be created to control orphan pharmaceuticals.<sup>15</sup>

## **CONCLUSION**

Due to inadequate diagnosis and treatment, rare diseases require extra attention. Rare disease treatment and prevention are seen as "no man's land." Since biologics make up more than 50% of the orphan medicine market, the arrival of biogenerics will have a significant impact on the industry's future. As more governments take steps to encourage this industry, particularly in Asia, it can be anticipated that the orphan medication market will continue to grow favorably. In terms of the Indian situation, 450 rare diseases have so far been found there. Similar regulatory frameworks are being established in India. The market potential is anticipated to grow as more nations pass legislation along these lines. The issue of orphan pharmaceuticals is becoming more and more important, especially for developing countries like India, which are the most affected. India still lacks a law governing the creation of orphan drugs. Governments all across the world offer pharmaceutical companies incentives for creating orphan medications. Several governments passed laws to address this issue. India does have a sizable population of patients with rare diseases, which might provide a sizable market for domestic pharmaceutical firms. Contrarily, the US's orphan drug regulations have been effective in bringing therapies to people with uncommon disorders. The first orphan drug law in the world was the US Orphan Drug Act, passed in 1982. It is a law built on financial incentives that enable pharmaceutical companies to develop treatments for specific patient populations. Since its passage, several other nations have created orphan drug programs and a large number of pharmaceutical companies have grown up around it. Today, the United States has produced more than 500 medications for the treatment of rare disorders. Over the past few years, there has been a significant increase in orphan medication designations. To assist both patients and the pharmaceutical industry, the Indian government should pass legislation regulating orphan medications and providing financial incentives to drug manufacturers. Additionally, many consumers with genetic problems who are unable to buy imported medication will benefit from this. For orphan pharmaceuticals, there is a great need for commercialization and incentive-based innovation.

The assessment of the scope and impact of orphan diseases, as well as a widespread awareness campaign about orphan diseases, are urgently needed in the current situation. Orphan drug development requires strong policies and initiatives from both public and private institutions.

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## CONFLICT OF INTEREST

The authors declare that there is no conflict of interest.

## ABBREVIATIONS

**FDA:** Food and Drug Administration; **CAGR:** Compound annual growth rate; **NPTRD:** National Policy for the Treatment of Rare Diseases; **DGHS:** Directorate General of Health Services.

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