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IN THE CHANGING WORLD OF INTELLECTUAL PROPERTY RIGHTS: PATENTABILITY AND MARKET EXCLUSIVITY FOR ORPHAN DRUGS

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Legislative Measures and Their Impact

4.1. The Orphan Drug Act of 1983:-

The Orphan Drug Act (ODA) of 1983 is a major piece of legislation in the United States that was enacted expressly to address the lack of incentives for discovering therapies for rare diseases. These uncommon diseases, sometimes known as "orphan diseases," afflict a small percentage of the population—usually less than 200,000 persons in the United States at any given time. Prior to the ODA, pharmaceutical firms had little financial incentive to invest in research and development (R&D) for such tiny patient groups, owing to the high expenses and poor economic returns. The ODA attempted to address these issues by offering a variety of financial and regulatory incentives to support the development of orphan medications.

Objectives of the Orphan Drug Act:-

The ODA's major goal was to encourage innovation in the discovery of therapies for rare diseases that had previously been overlooked by pharmaceutical corporations owing to low profitability. The Act attempted to do this through a mix of incentives designed to lessen the financial risks involved with orphan medication development. Among the primary goals were:

Promote R&D for Rare Diseases:-The ODA aimed to incentivise pharmaceutical firms to invest in R&D for rare disease therapies, ultimately meeting the unmet medical requirements of people with orphan disorders.

Increase Market Exclusivity:- By providing prolonged periods of market exclusivity, the Act hoped to ensure that corporations could maintain a competitive advantage and recoup their expenditures in orphan medicine development.

Financial Incentives: The ODA offered tax credits, grants, and other types of financial aid to help lower the expenses of clinical trials and regulatory processes involved with orphan drug development.

Fast-Track Approval Processes:- The Act established simplified regulatory paths to speed up the approval process for orphan medications, minimizing the time it takes for them to reach the market.

Key Provisions of the Orphan Drug Act:-

1. Market Exclusivity:- One of the most significant features of the ODA is the seven-year market exclusivity granted to firms whose products are designated as orphans. During this time, no other medicine, no matter how chemically unique, can compete with the authorized orphan drug for the same ailment.

36. 6. A. G. Lippmann, "The Orphan Drug Act and the Pricing of Orphan Drugs," Journal of Health Economics, vol. 35, 2020, pp. 131-147.
37. K. R. Mukherjee, "India's National Policy for Rare Diseases: An Overview," Journal of Global Health Policy, vol. 6, no. 1, 2021, pp. 23-30.

This exclusivity provides a significant financial incentive, allowing corporations to increase medicine prices in order to repay R&D expenses.

2. Tax Credits and Financial Assistance:- The Act provides tax credits for up to 50% of clinical trial expenditures, greatly easing the financial strain on pharmaceutical businesses. Furthermore, the ODA offers funds through the Orphan Products Clinical Trials funds Program to further clinical research on orphan medications. These financial incentives were especially important in encouraging smaller biotechnology companies to participate in orphan medication research.

3. FDA Assistance and Fast-Track Approval:- The FDA created the Office of Orphan Products Development (OOPD) to help firms navigate the regulatory processes for orphan pharmaceuticals. The ODA provides for a more rapid approval procedure, allowing orphan medications to reach the market sooner than other pharmaceuticals. This expedited clearance process is important for getting life-saving therapies to patients who would otherwise have limited alternatives.

4. Waivers and Subsidies for Application expenses:- Another significant element of the ODA is the waiver of some expenses involved with the drug approval process, such as Prescription Drug User Fee Act (PDUFA) fees, which can be prohibitively expensive for smaller businesses. By removing these fees for orphan drug applications, the ODA made it easier for biotech startups and smaller companies to reach the market.

The Orphan Drug Act and its Relevance to India:-

While the ODA has been extremely successful in the United States, its structure provides critical lessons for India, where the constraints of orphan drug research are amplified by a bigger population and less healthcare resources. India lacks a comprehensive orphan drug strategy, and there are substantial impediments to accessing rare illness therapies, such as high medication pricing and a lack of R&D funding.

India may learn from the United States' approach by implementing a comparable system of financial and regulatory incentives to support the development of orphan medications inside its own pharmaceutical sector. Provisions like as tax breaks, market exclusivity, and expedited approvals might be tailored to the Indian setting to encourage innovation. Given the socioeconomic constraints that many patients in India confront, any such program must include steps to assure cost and accessibility. Recent policy talks in India have underlined the importance of a national framework for rare illnesses. The National Policy for Rare Diseases (NPRD) 2021 was created to address these issues, however its scope and impact are limited. By implementing more powerful financial and regulatory incentives, India has the ability to establish a more favourable climate for orphan drug research while ensuring that therapies are available to all patients in need.

4.2. International Approaches:-

The United States: The Orphan Drug Act of 1983

In the United States, orphan drug regulation is often widely regarded as a leader in the regulation of similar or related items in other developed markets. By 1983, the Orphan Drug Act was passed to establish a range of incentives designed to encourage the development of treatments for rare diseases. Less than ten medications have been created in the US for uncommon disorders before to 1983. The National Organization for Rare Disorders (NORD) and other patient advocacy groups were instrumental in pushing for legislative action. The "valley of death" in rare illness research—where promising candidates failed to reach the market due to financial constraints—led to the enactment of the ODA.

38 1. U.S. Food and Drug Administration, "Orphan Drug Designations and Approvals," accessed September 12, 2023.
39. M. Kaltenboeck and S. L. Bach, "The Orphan Drug Act at 35: Observations and an Outlook for the Twenty-First Century," American Journal of Law & Medicine, vol. 44, no. 2, 2018.

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The ODA determines that orphan drugs are those targeted at treating diseases that affect fewer than 200,000 individuals in the U.S. Some of the most important incentives provided by the ODA include:

- Market exclusivity for seven years after approval; competitors are not allowed to market generic versions of the drug.

Designation as an Orphan Drug (ODD):

1. A sponsor creating a medication for an illness that affects fewer than 200,000 persons in the US is given orphan drug classification by the FDA.

2. Prior to filing a biologics license application (BLA) or new drug application (NDA), the sponsor must submit a request.

3. Access to a range of development advantages, including as tax breaks and market exclusivity, is made possible by ODD designation.

Market Exclusiveness:

1. Regardless of patent status, the sponsor is granted seven years of market exclusivity for the medicine for the designated indication after approval.

2. A comparable medication for the same indication cannot be approved by the FDA during this time unless it exhibits clinical superiority.

3. For small biotech businesses, this exclusivity is essential since it offers a definite window for income creation and expense recovery.

Tax Credits:

1.The ODA first permitted a 50% tax credit for eligible clinical research costs.
2. Particularly during the expensive Phase II and Phase III studies, this greatly lessens the budgetary load.

3. The Tax Cuts and Jobs Act of 2017 made changes to the tax advantage, lowering the credit to 25%.

Financial aid and grants:

1.Funding for clinical research on orphan medications is made available through the Orphan Products Clinical Trials Grants Program.

2. Academic institutions and small biotech companies doing early-stage research can especially benefit from this.

Waivers of User Fees:

1.The Prescription Drug User Fee Act (PDUFA) does not require sponsors of orphan medications to pay fees.

2. As of 2023, this can save businesses more than $2.9 million every application.
3. Waivers also cover product and production expenses.

Protocol Support:

1.The FDA directly advises sponsors on statistical techniques, endpoints, and study design.
2. Clinical trial success rates and regulatory compliance are enhanced by this assistance.

Support for Research Infrastructure:

1.A cooperative platform for multi-site clinical trials is offered by the NIH-funded Rare Diseases Clinical Research Network (RDCRN).

2.Helps with rare illness translational research, biomarker discovery, and data exchange.

Amendments to the Law

To improve its efficacy, expand its scope, and handle new issues, the ODA has undergone a number of revisions throughout time. The foundation for orphan drug development has been strengthened by these legal revisions, which have also included measures to fight against abuse.

The 2002 Rare Diseases Act:

1.Created the National Institutes of Health's (NIH) Office of Rare Diseases Research (ORDR).
2.Increased federal funding for epidemiological studies, public-private collaborations, and research coordination.

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3.Required the creation of databases and registries for rare illness research.

The 2012 FDA Safety and Innovation Act (FDASIA):

1.Expedited approval processes and allowances for innovative treatments, which will help rare illness medications.

2.Through the Rare Pediatric illness Priority Review Voucher program, pediatric rare illness programs have been strengthened.

The 2016 21st Century Cures Act:

1.Focuses on developing drugs with patients in mind.

2.Mandated that the FDA take into account real-world data and patient viewpoints while making approval decisions.

3.Promoted the use of surrogate endpoints and adaptive trial designs in rare illness research.

The 2017 Tax Cuts and Jobs Act:

1.Decreased the tax credit for orphan drugs from 50% to 25%.

2.Investment in orphan pharmaceuticals was maintained by other methods even if this reduced one of the financial incentives.

Updates to Guidance and Regulatory Refinements (Ongoing):

1.The FDA regularly revises its guidance guidelines on clinical endpoints, post-marketing responsibilities, and orphan medication designation.

2.Drug assessments for uncommon diseases are increasingly incorporating digital health technology and decentralized trial methodologies.

- Tax credits to cover up to 50% of the cost of conducting clinical trials.

- Grant programs to aid research and development for orphan drugs.

- Forfeits FDA fees, which help alleviate the financial hardship on developers.

Since the passage of the ODA, orphan drug development in the United States has increased dramatically with over 600 orphan drugs approved by the Food and Drug Administration as of 2021. But while progress has been made in drug development, critics argue that the exclusivity period has brought orphan drugs to such exorbitant prices that they have become unaffordable for patients, even in affluent healthcare systems.

European Union Regulation of Orphan Drugs (1999):-

In the European Union (EU), the Orphan Medicinal Products Regulation was introduced in 1999 (EC No 141/2000), largely mirroring the U.S. model but revised to fit the European market. EU Orphan Drugs are defined as drugs that treat conditions that affect no more than 5 in 10,000 persons across the member states. A significant step in the EU's endeavor to promote the development of medications for uncommon illnesses, orphan pharmaceuticals, was taken in December 1999 when the European Parliament and the Council of the European Union enacted the Orphan Medicinal Products Regulation (Regulation (EC) No 141/2000). The U.S. Orphan Drug Act of 1983 served as a major inspiration for this law, which went into effect on January 27, 2000. The EU law, however, includes region-specific provisions that are adapted to the demands and complexity of its multi-national healthcare environment, even if it closely resembles the U.S. model in many ways:

-Ten years market exclusivity after approval.

-Protocol support for the developers from the European Medicines Agency, providing such developers with guidelines on designing their clinical trials.

-Fee reductions for marketing authorization applications and post-approval variations.

-Research grants from the European Commission.

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Definition of Orphan Drugs in the EU Context

A medication is classified as a "orphan drug" under the rule if it satisfies the following requirements:

Prevalence-based definition: At the time of the application for designation, the medicine must be used to diagnose, prevent, or treat a persistently debilitating or life-threatening illness that affects no more than 5 out of 10,000 people in the EU.

Lack of adequate return on investment: A medicine may be eligible even if the prevalence criteria is surpassed if it is improbable that the drug's marketing will yield enough revenue to cover the costs of development.

No adequate current therapy: The medication must either provide a substantial benefit to persons afflicted by the ailment, or there must be no adequate means of diagnosis, prevention, or treatment.

Important Rewards Provided to Developers

The EU rule provides a number of important incentives to pique pharmaceutical firms' interest, as many of them could otherwise overlook rare illnesses because of the little market potential:

1. Ten Years of Market Exclusiveness

Ten years of commercial exclusivity after the orphan medication is approved is arguably the biggest inducement. No comparable product may be released for the same therapeutic indication during this time unless:

1.It has been demonstrated that the second product is clinically superior.
2.The holder of the marketing permission agrees to the competitive product's launch.
3.The initial holder is unable to provide enough of the medication.
4.According to the Paediatric Regulation (EC No 1901/2006), this exclusivity term is extended to 12 years for orphan pharmaceuticals that contain findings from pediatric research carried out in accordance with an established Pediatric Investigation Plan (PIP).

2. Support for Protocols

The European Medicines Agency (EMA) offers protocol help to orphan medication developers. In order to ensure that the trials are appropriate for regulatory approval and customized to the difficulties associated with rare illnesses (such as limited patient populations, ethical problems, and endpoint selection), this involves scientific assistance on the design of preclinical and clinical research.

3. Waivers and Fee Reductions

Reduced fees or waivers are advantageous to applicants for:

1.Applications for marketing permission

2.Examinations
3.Changes after permission

4.Annual charges

5.Assistance with protocols and scientific guidance

The size and nature of the business determine how much of a fee reduction is offered, with small and medium-sized businesses (SMEs) getting the most financial assistance.

4. Public Funding and Research Grants

1. Additionally, EU projects like the Horizon Europe framework and earlier initiatives like FP7 or Horizon 2020 might provide direct financing for orphan medication research.
2. Coordination and grant distribution for the development of orphan medications, especially for diseases for which there are currently no therapies, are functions of the European Commission.

Comparing the Orphan Drug Act of the United States

The regulatory environments in the EU and the US differ significantly, although being based on the US system:

Feature	EU Regulation	U.S. Orphan Drug Act
Prevalence	≤ 5 per 10,000	≤ 200,000 people

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Feature	EU Regulation	U.S. Orphan Drug Act
threshold	persons	in the U.S.
Market exclusivity	10 years (up to 12 with PIP)	7 years
Regulatory agency	European Medicines Agency (EMA)	Food and Drug Administration (FDA)
Protocol assistance	EMA provides scientific advice	FDA provides similar guidance
Fee reductions	Extensive, especially for SMEs	Yes, but generally fewer
EU coordination	Emphasis on inter-member state access	U.S. has single healthcare system

Obstacles and Remarks

The EU orphan drug policy still has implementation issues despite the incentives and cooperative structure, especially with regard to fair access across its 27 member states. Important concerns include:

1.Disparities in National Reimbursement and Pricing:

(a) Orphan medications can be lawfully marketed throughout the EU thanks to centralized permission granted by the EMA. Pricing and reimbursement choices, however, are handled at the federal level. The availability and cost of orphan medications vary greatly between nations as a result.

(b) Compared to lower-income nations (like Bulgaria, Romania, and Hungary), wealthier member states (like Germany, France, and the Netherlands) frequently accept orphan medications more quickly and provide broader reimbursement.

2. Variability in Health Technology Assessment (HTA)

(a) Different member states use different HTA criteria to assess the **cost-effectiveness** and **therapeutic benefit** of new orphan drugs. Because of the high per-patient cost of many orphan drugs and limited clinical trial data, this can lead to **negative funding decisions** in some regions.

3. Disjointed Execution

(a) Despite being EU-wide, patient access and practical implementation are still dispersed. Due to financial limitations or legal obstacles, certain nations may postpone or refuse access to orphan medications.

4. Criticisms of Market Exclusivity

(a) Critics contend that pharmaceutical corporations may abuse the market exclusivity clause to uphold monopolies and establish exorbitant pricing. This is particularly true when businesses prolong exclusivity by making just slight changes to already-approved medications.

(b) The possibility of "salami slicing"—the technique of purposefully dividing illnesses into smaller subgroups in order to continually achieve orphan status—has also drawn criticism.

The other great difference between the EU and the U.S. system is the duration of market exclusivity, with the EU offering 10 years, as compared to 7 years in the U.S. Another significant approach adopted by the EU is that it shows greater co-ordination between the member states in making orphan drugs available all over the region. The EU model has widely been appreciated for innovation and collaboration but, there are still problems accessing orphan drugs, especially different countries' member states, with different healthcare systems and vastly different budgets.

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Innovation and Beneficial Effects

Notwithstanding the difficulties, the EU's orphan drug law has had a major benefit:

(a) Since the rule's inception, more than 2,000 orphan drug designations have been given out.

(b) Over 200 orphan medications, which provide life-altering or life-saving medicines for illnesses for which there were previously no effective treatments, have been approved for sale.

(c) The rule has raised awareness of rare illnesses, promoted international collaboration, and improved funding for research.

Japan: The Orphan Drug Designation (ODD) Program:-

Japan's orphan drug regulation strategy is also comprised of the strong package of incentives aimed at supporting the creation of treatments for rare diseases. The Orphan Drug Designation (ODD) Program, established in 1993, offers a number of incentives to pharmaceutical companies.

Context and Regulatory Background

Recognizing the particular difficulties in creating therapies for rare diseases—which sometimes only impact a tiny number of patients—Japan created its Orphan Drug Designation (ODD) system in 1993. As a result, pharmaceutical firms have little financial motivation to explore cures for these disorders. In addition to promoting research and development in underrepresented therapeutic areas, the program was created to align with international norms, such as those enacted in the US (Orphan Drug Act, 1983) and the EU (Regulation (EC) No 141/2000). The Ministry of Health, Labor, and Welfare (MHLW) and the Pharmaceuticals and Medical Devices Agency (PMDA) work together to manage the program.

-Ten years of market exclusivity.

-Priority review and expedited approval via the Pharmaceuticals and Medical Devices Agency (PMDA).

-Subsidies covering up to 50% of clinical trial costs.

-Tax incentives and grant programs to reduce the financial burden of R&D.

-Guidance from regulatory agencies throughout the development process.

Japan's Orphan Drug Designation Criteria

For a product to be designated as an orphan drug, it must fulfill all of the following requirements:

1. Prevalence Threshold: In Japan, the illness must impact less than 50,000 people.

2. High Medical Need: The medication must treat a serious illness for which there is currently no effective treatment, or, in the event that there is, it must provide a substantial improvement.
3. Justification for Development: The drug's potential efficacy must be supported by solid scientific data and reasoning.

4.Development Plan: A workable plan outlining the sponsor's strategy for product development and commercialization in Japan must be supplied.

Rewards for the Development of Orphan Drugs

A comprehensive set of incentives is part of Japan's ODD program, which aims to lessen the financial and regulatory difficulties related to the development of drugs for rare diseases:

1. Exclusiveness in the Market

(a) Orphan medications have a ten-year commercial exclusivity period when they are approved.

(b) No other medication with the same indication may be licensed during this time unless it proves to be more effective or safe.

41. J. Sakai and T. Yamaguchi, "Japan's Orphan Drug System: A Review of Current Policy and Incentives," Journal of Pharmaceutical Policy and Practice, vol. 12, no. 3, 2021.

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2. Faster Approval and Priority Review

(a) The PMDA gives priority review to orphan medications.

(b) The approval time is greatly decreased, which enables businesses to launch items more quickly.

(c) Rolling submissions and more design freedom for clinical trials are examples of expedited processes.

3. Financial Support and Subsidies:

(a) Public subsidies from the Japanese government can pay for up to 50% of clinical trial expenses, which lessens the total financial burden, especially during the expensive clinical development stage.

4. Tax Breaks:

(a) Businesses that invest in orphan drug research and development are eligible for corporation tax incentives.

(b) These consist of deferred taxation advantages and deductions from taxable income.

5. Regulatory and Scientific Guidance: Early and continuing consultation is offered by the PMDA and includes:

(a) Advice on protocol

(b) Support for the design of clinical trials

(c) Alignment of regulatory strategies

This ongoing assistance reduces regulatory risks and enhances submission quality.

6. Funding and Grant Initiatives:

(a) Other R&D assistance schemes are offered by organizations such as the Japan Agency for Medical Research and Development (AMED), which provides academic institutions and early-stage companies with non-dilutive financing sources.

Pathway for Regulatory Approval

Following orphan designation, a sponsor usually takes the following course of action:

(a) Because patients are rare, clinical trials are frequently smaller and more adaptable in their design.

(b) NDA Submission: Businesses may create a more efficient submission with PMDA's help.

(c) Review Process: The typical 12-month review period may be reduced to around 6 to 9 months under the priority track.

(d) Post-Market Surveillance: To track long-term safety and efficacy, businesses may be obliged to carry out post-marketing research (Phase IV trials).

Program Achievements and Difficulties

(a) Early patient access to cutting-edge treatments and quicker medication development have both been made possible by the Japanese ODD system.

(b) Multinational corporations now have more unified global development plans because to the harmonization with US and EU frameworks.

Continuous Difficulties

1. Affordability and Access

(a) Many orphan medications are expensive despite incentives, which prevents them from being fully reimbursed or from being accessed through national insurance programs.

(b) During pricing discussions, the Central Social Insurance Medical Council (Chuikyo) is paying more and more attention to cost-effectiveness.

2. Recruitment of Patients

(a) Clinical studies may be delayed or statistical significance may be limited due to Japan's tiny patient population.

(b) International collaboration is becoming more and more necessary for multicenter investigations.

3. After-Approval Promise

(a) The same scarcity of patients that hampered previous phases of research may also make it difficult for sponsors to carry out necessary post-marketing studies.

4. Insufficient Innovation at Home

(a) Although the orphan medication industry is dominated by multinational pharmaceutical corporations, there are relatively few native

Japanese biotechs working on rare illness treatment development, however this is gradually changing.

Prospects for the Future

Japan is anticipated to keep improving its ODD structure, concentrating on the following: (a) To expedite the development of orphan drugs worldwide, international harmonization should be improved.

(b) Rising support for cell and gene treatments, which are being utilized more and more to treat uncommon disorders.

(c) Investigating outcomes-based pricing schemes to deal with issues related to reimbursement and costs.

(d) Fostering the expansion of biotech companies in Japan by means of public-private partnerships and innovation clusters.

Japan's system has been particularly effective in streamlining the approval process for orphan drugs and reducing the time and cost involved in bringing a drug to market. Much like the U.S. and EU, access to orphan drugs remains an issue, particularly given the high prices often associated with these treatments.

India: Emergent Framework for Orphan Drug Development:-

India thus offers a unique and also evolving perspective on orphan drug regulation, even though the country has not yet developed a comprehensive regulatory framework of the sort that is observed in the U.S., EU, or Japan. Yet there has been growing recognition of the need to address rare diseases. India's Ministry of Health and Family Welfare has issued the National Policy for Treatment of Rare Diseases in 2017. It is intended to help facilitate the development of treatments for rare diseases. However, challenges abound for the orphan drug landscape in India There isn't a unique, all-encompassing legal or regulatory structure in India that deals with orphan pharmaceuticals. In contrast to the Pharmaceutical and Medical Device Act of Japan, the EU's Regulation (EC) No 141/2000, and the U.S. Orphan Drug Act of 1983, India lacks:

(a) A legal definition of an orphan medication.
(b) Formal processes for designating drugs as orphans.
(c) Regulatory rewards including priority review, fast-track approvals, or market exclusivity.

Notwithstanding this, the Indian government has made some first efforts to address the problem of rare illnesses; the National Policy for Treatment of Rare illnesses (NPTRD) represents a significant turning point.

Major Obstacles in the Orphan Drug Situation in India

Despite good intentions, India's orphan drug ecosystem encounters a number of obstacles:

(a) Absence of Particular Law:
Pharmaceutical firms are reluctant to participate in the development of orphan drugs in India due to the absence of a defined legal and regulatory framework. The lack of systems such as

(a) Credits for taxes
(b) Exclusivity in the market
(c) Requirements for clinical trial waivers … This diminishes the commercial feasibility of creating such medications.

(b) **Expensive Treatment**:
Because of their tiny patient populations and hefty R&D expenses, orphan medicines are famously costly. In India:

(a) The majority of therapies are paid for out of pocket.

(b) Rare illness insurance is either non existent or very limited.

(c) There are still very few government subsidies available.

Families are severely impacted financially, and access to treatments that might save lives is restricted.

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(c) Fragmented Healthcare Infrastructure

It is challenging to determine the illness burden in India since there is no centralized rare disease registry. Delays in diagnosis are frequently caused by:

(a) Physicians' ignorance.
(b) Inadequate diagnostic facilities, particularly in remote regions.
(c) Referral systems that are disjointed.

Lack of Special Orphan Drug Legislation: At present, no specific legislation related to orphan drugs is available in India, unlike the U.S. Orphan Drug Act or the EU Orphan Regulation. This factor mainly has withheld investment by pharmaceutical companies in this space because there is no regulatory clarity and incentives for market exclusivity.

Cost Burden on Patients: With the high price of orphan drugs along with poor government subsidies, most Indian patients cannot afford to carry on treatments for rare diseases.

Emerging Initiatives: The NPTRD, along with some state-level initiatives, initiated a limited funding for the treatment of rare diseases, which are in very early stages. Furthermore, there is an ongoing dialogue in order to form a more defined orphan drug approval and designation process, but a lot is still in the works. The NPTRD, which was initially published in 2017 and revised in 2021, is India's first coordinated attempt to combat rare illnesses. It divides uncommon diseases into three categories according to the expense and viability of therapy, and it seeks to:

(a) Give each qualifying patient up to ₹20 lakhs (about $24,000) in financial help.
(b) Encourage domestic research and development for treatments for uncommon diseases.
(c) Create diagnosis and treatment Centers of Excellence (CoEs).
(d) Encourage crowd-funding strategies to help pay for expensive medical care.

Funding limitations, administrative roadblocks, and an ambiguous medication research and approval process, however, continue to restrict implementation.

India in the Global Ecosystem of Orphan Drugs

By 2030, the global orphan drug industry is expected to reach a value of over $300 billion, propelled by advancements in sophisticated biologics, personalized medicine, and gene therapy. Even with an estimated 70–96 million people suffering from uncommon diseases, India is still a market that is underdeveloped and poorly governed. There is increasing pressure on India to follow the examples set by countries like:
(a) The United States: Providing fast-track approvals, a 25% tax credit on clinical studies, and seven years of commercial exclusivity.

(b) The EU: 10 years of protocol support and market exclusivity.
(b) Japan: Priority reviews, tax incentives, and clinical trial subsidies.

Including comparable clauses could:

(a) Draw in foreign investment.
(b) Help domestic pharmaceutical companies investigate specialized markets.
(c) R&D in precision and customized medicine should be accelerated.

Global orphan drug development market: India is one such market which has unmet medical needs but still, there is an ample lack of regulation, and this acts as a major hindrance to progress. The increasing demand for inclusion in a practical orphan drug policy with provisions such as market exclusivity, tax benefits, and R&D grants, which are suited to models adopted in other jurisdictions.

Comparative Insights:-

Comparative Insights Compare the approaches of orphan drug regulation across the U.S., EU, Japan, and India. Some patterns and differences emerge. Each of the U.S. and the EU provides extended market exclusivity along with

42. 5. Government of India, Ministry of Health and Family Welfare, "National Policy for Rare Diseases 2017," accessed September 15, 2023.
43. K. Chaudhuri, "Developing a Framework for Orphan Drugs in India: Current Challenges and Future Prospects," Indian Journal of Pharmacology, vol. 53, no. 1, 2021.

financial incentives for impetus to rapid growth in the number of approvals during the orphan drug era. In this case, Japan's approach is equivalent, offering market exclusivity, tax benefits, with a fasttrack approval mechanism and integration into the national healthcare system.

The country is still very nascent in developing an orphan drug policy, and current initiatives revolve more around financial assistance to patients rather than incentivizing pharma companies. One thing that these jurisdictions share is the understanding that orphan drug development demands strong support from regulation and finance since drugs targeting these small groups are, on average, very costly and high-risk. However, the incentive packages are quite varied, with developed markets like the U.S. and EU being a cut above others in light of the emerging markets like India.

Trends and Important Distinctions:

Criteria	U.S.	EU	Japan	India
Exclusivity Period	7 years	10–12 years	10 years	Not clearly defined
Regulatory Body	FDA (OOPD)	EMA (COMP)	PMDA/MHLW	CDSCO
Definition of Rare Disease	<200,000 people	<5 in 10,000	<50,000 people	<500,000 people
Financial Incentives	Tax credits, grants	Fee waivers, grants	Tax subsidies	Minimal
R&D Support	Strong	Strong	Moderate to strong	Weak
Market Maturity	Highly developed	Developed	Developed	Nascent