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Orphan Drug Development in India: Analysing Patent Law Limitations, Challenges and the Need for Reform

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The growth of the pharmaceutical industry shows how important the patent system is, because without it, the industry would not have become as large as it is today. However, the potential of patents to foster innovation is constrained in certain areas where commercial viability is limited. Orphan drug is a pharmaceutical product used for diseases that affect a small number of people (rare diseases). What constitutes a rare disease does not have a uniform definition. Although patents offer exclusivity, they fail to sufficiently incentivize orphan drug development due to the small market size and limited potential to recover investments, in addition to other challenges such as statistical hurdles in clinical trials and lack of awareness.

This paper examines the limitations of the patent system in encouraging R&D in orphan drugs. It examines the orphan drug landscape, which faces significant unmet demand, and critically analyses India's approach to orphan drug development by evaluating existing initiatives to identify gaps. It advocates for a balanced framework benefiting both developers and society. Such an approach would be explored by studying global best practices and how they may be adopted in the Indian context.

Keywords: Orphan Drugs, Orphan Diseases, Patent, Pharmaceutical, Market Exclusivity, Drug Development

The introduction of patents has attracted huge investments in pharmaceuticals, contributing to the industry's growth and glory. Drug discovery and development is a highly expensive endeavour requiring time and resources. Bringing a drug to the market requires satisfying all regulatory conditions of safety and efficacy through various stages of clinical trials. Once it's so made available, it is easy to imitate. These reasons make the industry heavily dependent on patent protection to secure the investments in R&D through profits. However, in certain sectors, even patents fail to adequately incentivise innovation.

Orphan drugs are drugs used for diseases where the number of people affected is very small. The dynamics of these diseases (called rare diseases or orphan diseases) are complex, with many diseases characterized by varying and unidentifiable symptoms. There is no single definition for rare diseases that is accepted globally. In aggregate, a huge population is affected by rare diseases. Only a few diseases have approved treatments. Drug development is also challenging due to a lack of pathophysiological knowledge, challenges in clinical trials, lack of adequate facilities, etc. High costs of development and low potential for recouping investment due to small market size hinder manufacturers from pursuing research in orphan drugs.

The patent system is viewed as the primary legal driver for encouraging technological innovation across all industries. It is seen as a tool that can be leveraged by inventors to recoup expenses associated with the innovation and that safeguards from free riding. The exclusivity granted by patents gives the patent holder complete control over how to use and commercialize the invention, whereby he or she decides its price and availability. Patents thus incentivize investment in the development of an invention, allowing innovators to recover their investment. Critics have questioned this rationale and the capacity of the patent system to promote innovation. According to them, patents alone are not stimulating innovation, and it is dependent on various social and economic factors.

Although patent is an important element, its effect on innovation is still questionable, especially in commercially nonviable sectors. Even in pharmaceuticals, the profit-driven motives of the industry are focused on the market potential. Therefore, even the grant of patents need not guarantee new breakthroughs. For an invention to qualify for a patent, it must be both new and novel. The patent criteria are not

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concerned about the potential social value or the developmental costs of the invention, which has far-reaching effects on public health, access to healthcare, as well as human rights.

The benefits of patents can be seen only when there is a huge market with consumers willing to pay for the patented products. The pharmaceutical industry prioritizes high demand and profit potential. This, therefore, would mean very minimal research in underfunded areas and areas where the market size is small. Moreover, patent criteria render socially valuable drugs unpatentable. Patents are based on novelty and inventiveness, not societal need. This means that even drugs with significant public health value may be denied patents if they don't meet these technical thresholds, deterring investment in their development despite their potential social impact. Thereby, research would not be pursued or would be abandoned in case of the development of many types of drugs.

The current patent regime fails to adequately incentivize the development of orphan drugs, as they face unique challenges in addition to broader market difficulties. Small market size, weak enforcement, lack of supplementary incentives, and strong public health priorities together make innovation financially unviable. Sufficient returns to justify the costs involved in R&D are not gained, and therefore, patent incentives are not enough. Therefore, treatments for rare diseases remain underdeveloped or abandoned without enough support for innovation.

This paper analyses the challenges of the patent system in adequately incentivising orphan drug development. It examines the initiatives in India to promote orphan drug development to highlight the drawbacks in the regulatory framework and patent system to promote R&D in orphan drugs. The paper points out the need for a new regime in light of global best practices and explores their adoption in the Indian context. Such an approach is essential to cater to the public health demands and to balance the interests of drug developers.

Orphan Diseases and Orphan Drugs

As the name indicates, a rare disease or orphan disease is a medical condition that affects a small population. Rare diseases are defined differently across countries. The threshold for determining rare diseases varies globally.[1]The definitions are mainly based on the disease prevalence. However, other factors have also been considered.

In the United States, a disease affecting less than 200,000 people is labeled as rare. Diseases that affect more than 200,000 people would also come within this ambit if the drugs for such conditions would not generate enough sales so as to recover the cost of development (Orphan Drug Act, 1983). Under the EU Regulation, a life-threatening or chronic condition not affecting more than 5 in 10,000 persons would come under this category (Regulation (EU) 141/2000). Australia also sets the same threshold to mark rare diseases (Therapeutic Goods Regulations 1999). Under the Japanese Law, the number of people affected by the disease has to be less than 50,000 (Pharmaceutical Affairs Law). The criteria for classifying rare diseases, therefore, differ considerably worldwide. Some countries rely on disease prevalence, whereas others take into account factors such as disease severity, availability of alternate treatment, or the potential of recovering the investment in drug development.

Collectively, the scope of rare diseases is quite big. Almost 5000 to 8000 rare diseases have been identified, which span genetic disorders, rare cancers, neurological disorders, respiratory diseases, autoimmune diseases, blood disorders, kidney diseases, etc.[2] In aggregate, rare diseases affect a substantial population around the world.[3] They may be acute or chronic. The majority of them are serious or life-threatening, and a considerable number of patients are children. Although many diseases have been identified, treatment is available for very few. Where there are approved treatments, it is placed at exorbitant prices, making them unaffordable for those who need them the most. The prevalence of orphan diseases varies among populations and geographical territories. For example, diseases such as tuberculosis are common in developing countries, but are rare in developed countries. Further, certain diseases initially would be rare, but later may move out of the category since the patient pool has increased.[3]

Thus, the landscape of orphan diseases is vast and complex, encompassing a wide range of conditions with varying characteristics. The causes for such diseases could be genetic or due to exposure to infections or toxins. Some may have multiple causes.[4] They have varied and unidentifiable symptoms, making their diagnosis very difficult. Accurate diagnosis would take years and is dependent on the doctor’s evaluation and availability. Lack of specialisation and geographic dispersion of patients make timely examination and diagnosis difficult.[5]

Orphan Drugs present various unique challenges in the already arduous process of drug discovery and development. Natural history studies provide important information about a disease, such as demographic, genetic, environmental, and other variables that correlate development of the disease and outcomes in the absence of treatment.[4] In the case of rare diseases, limited knowledge due to the lack of natural history studies complicates the identification of therapeutic targets, biomarkers, and clinical endpoints.[6]

Suitable animal models may not exist, hindering pre-clinical testing. Conducting clinical trials is difficult due to low prevalence, geographic dispersion of patients, and ethical concerns, especially for vulnerable populations. Moreover, there would be statistical hurdles as well.[7] Safety evaluation depends on the data and sample size, which would not be adequate for rare diseases, thus not providing comprehensive results. High costs and low success rates deter investment, as the small market makes recouping R&D expenses unlikely, leading to many potential treatments being abandoned in the early stages of basic research and preclinical studies.[8]The limited potential for market exploitation, therefore, hinders the discovery and development of orphan drugs. The same models of trial design as those for common diseases may not be fruitful. Due to incomplete understanding of the disease, the standard of care and disease management are also serious concerns. Cost of development, lack of funding, and absence of any sort of protection or incentive therefore attract very little attention to the domain of orphan drugs.[4]

The serious nature of rare diseases, therefore, requires more research on strategies for prevention, diagnosis, and treatment. The disproportion in the levels of prevalence and available therapies indicates that the unmet demand is very high, thus making it a significant public health issue. The lack of available medicines and limited access to treatment significantly harm patients with orphan diseases. Without proper medication, they often rely on expensive therapeutic interventions that may not be effective. This is not a minor issue, as a considerable number of people are affected. As a result, society as a whole suffers, with many individuals losing their lives to rare diseases each year.

Drug developers and sponsors are already selective in their approach due to high costs and low chances of success. Out of the hundreds of potential candidates, only a few might qualify for clinical trials, and this number will further drop while reaching the market approval stage. Therefore, developers would emphasize on those drugs that assure good financial returns on their investment. With a small market, the likelihood of recouping the investment through sales would not be possible. Consequently, orphan drug R&D is either not initiated or is discontinued in its early stage. Moreover, commercial interest would also be lost due to concerns of eligibility for patents. Thus, despite the patent system, orphan drug development remains insufficiently promoted. The following section explores these issues in detail.

Limitations of Patent Law in Encouraging Orphan Drug Development

The foundations of patent law justify its relevance primarily on the ground that it provides an opportunity for innovators to retrieve the time and resources spent in bringing out something that is beneficial for the whole society. In the absence of such exclusivity, the incentive to innovate is greatly weakened. Moreover, patents facilitate public disclosure of the invention, leaving scope for further research. Hence, patent law aims to stimulate innovation and facilitate the dissemination of knowledge in the public domain by rewarding the innovator. As a result, society enjoys new, innovative, and efficient technologies.[9]

The TRIPS agreement mandated that any invention meeting the required criteria must be patentable. Therefore, any product or process, including pharmaceuticals, could be patented. This brought a radical change in many nations since earlier they did not grant patents to health technologies and pharmaceuticals to promote health objectives. Product patents give the discretion to patent holders to decide the price of their goods during the patent term, and in case of pharmaceuticals, this means high prices, thereby making them inaccessible to patients, especially those in underprivileged sections. However, the need to protect public health is mandated within the TRIPS agreement itself[10]and has been reiterated in the DOHA Declaration, according to which members are not prevented from taking measures for the protection of public health.[12] Further, the TRIPS also provides flexibilities to balance innovation and public health, such as compulsory licensing, parallel importation, research exception, early working exception, limiting test data protection, measures to promote competition, etc.[15] These flexibilities, however, remain underused in many countries, creating barriers to affordable access, limiting the ability to fully address public health needs.

India also changed its patent law to comply with the TRIPS agreement with the 2005 Amendment to the Indian Patents Act by introducing product patents. In addition to the three standard criteria, namely novelty, non-obviousness, and industrial application, the product should also pass the test of patentable subject matter.[13] They should not be excluded from patenting under the Act. Moreover, the application must be supported with sufficient enabling disclosure of the invention.[14] After the amendment, pharmaceutical products could be patented, giving the holder exclusive rights for 20 years. The generic industry in India was affected by this change since they could no longer freely reverse engineer branded drugs to provide cheaper versions.[15] The TRIPS flexibilities have also been incorporated in the Indian Patents Act.[12]

Indian law also prohibits patenting of small changes or minor improvements of existing products unless there is a significant difference in the property in terms of efficiency.[16] In the pharmaceutical context, this translates to enhanced therapeutic efficiency.[17] This requirement aims to prevent the practice of evergreening and ensure prompt entry of generics so that drugs can be made affordable by encouraging innovation rather than repeated patent extensions. Through the lens of public health, generic drugs can mitigate the health demands in society.

The role of patents in realizing the aim of stimulating innovation potential has always remained uncertain. The theory and practice of patent law have a huge gap. It has worked differently for different industries, and in many of them, there would be innovation even in its absence.[18] Moreover, though patents aim to benefit the world at large, the benefits are received by the rich, creating economic disparities. The pharmaceutical industry is considered one that is highly dependent on patent protection. There are huge developmental costs involved in the invention, but it is easy and cheap to replicate once it is brought out. Patents, therefore, provide an edge to the first mover of the drug to recoup all R&D investments by delaying the entry of imitators.[19]

However, the reality is that patent is market-dependent, thus creating monopolies. Manufacturers, therefore, would invest only where there is high potential to return their investment. Research would be pursued where there is high demand and willingness to pay.[20]Another effect of this is that there is increased R&D but no innovation, meaning that although the total number of patents has grown, few are for original inventions. The majority protect incremental changes to existing products, casting doubt on whether patents effectively encourage meaningful innovation.[21]Therefore, what is brought out will be ‘me too’ drugs and variations of existing compounds, creating a dearth of “new entities” and those with therapeutic value. Moreover, due to the market-driven approach, developers would be interested in drugs for diseases of high-income countries, thus ignoring diseases with higher prevalence in lower and middle-income countries.[22]

Although patents aim to secure investments in development and to produce socially relevant drugs, these aspects remain distant from reality. Patents are granted to any invention that is new and nonobvious for a fixed, uniform term, irrespective of other considerations. Therefore, inventions with small developmental costs are patentable and generate huge revenue, while those that have high developmental costs, like drugs, would be unpatentable. Firms would therefore divert their investment to those drugs with the potential of granting strong patents and would not be interested in taking up research on drugs that may have the potential to solve a health crisis but are not patentable. As a result, patents fail to consider both the societal impact and the costs involved in developing the invention.[23]

Access is another concern, since many would not be able to afford patented products. The presence of adequate reimbursement schemes and purchasing capacity is necessary to determine the success of the invention. Availability and affordability of drugs are important determinants of access, which can be blocked by patents, thereby failing in its aim to maximise social welfare.[24]

Orphan drugs are the best illustration of all the above-mentioned limitations. The market size being small, firms’ capacity to recoup investment is directly affected, making R&D efforts unlikely.[7] Geographical disparity in disease prevalence makes certain diseases orphan in some countries and non-orphan in others. Moreover, firms would be focused on drugs for common diseases, which may already have existing treatments, since they may generate profits.[25] Lack of adequate knowledge of pathophysiology and natural history makes drug discovery challenging. Orphan drugs, therefore, remain an underfunded and underdeveloped domain receiving no commercial interest. The scope of the patent system revolves around the invention and not the upliftment of sectors that require investment and support, such as orphan

drugs. Patent criteria may render socially valuable drugs unpatentable and lead to abandonment of research. Such discontinued projects might have led to the development of drugs capable of treating life-threatening diseases.[26] The varied symptoms and lack of specialisation in orphan diseases make it difficult to identify targets and endpoints, making basic research complicated and expensive. Similarly, the challenges in clinical trials enhances the risk taken by manufacturers with low chances of return.[27]

Therefore, this has larger public health implications, creating unmet demand for rare disease patients, thus requiring government attention. Novel approaches ought to be explored so that the needs of rare disease patients are not sidelined. From a human rights standpoint, individuals with rare diseases are entitled to the same protections and care as those with more common illnesses, placing an obligation on the state to safeguard their rights.

Beyond Patents: Global Best Practices in Promoting Orphan Drugs

Considering the inadequacies in the regulatory framework, an approach beyond the traditional patent outlook is essential. Various nations have enacted legislation and policies to boost the orphan drug landscape. The US Orphan Drug Act of 1983 was the first such legislation. Australia's Therapeutic Goods Act and the Pharmaceutical Affairs Law in Japan have also incorporated provisions to encourage R&D in orphan drugs. European Union Regulation 141/2000, Medicines (Orphan Drugs) (Exemption) Order of Singapore, Taiwan's Rare Disease and Orphan Drug Act, 2000 are examples of other legislations.

One of the most important common incentives is market exclusivity. This is the time period during which similar products will not be granted market approval. The period of market exclusivity varies in these countries. Japan restricts the submission of similar applications for market approval for 10 years during the re-examination period. Other incentives include fee Reductions, tax grants, accelerated review, protocol assistance, etc. To receive these benefits, a drug must be officially designated as an "orphan" by the relevant authority. The procedure for market approval is the same as non-orphan drugs in most countries. The EU, in this case, provides for a distinct centralised process.[28]

These legislative interventions have led to increased research in orphan drugs and an increase in the number of applications for designations and market approval.[29] The market exclusivity is independent of patent term, and the orphan designation is strictly based on criteria as provided in the statute. This has increased the commercial interest in orphan drugs, with many companies beginning to take up research, including big pharma.[4] This has also raised concerns about access, as manufacturers often set extremely high prices to recover their investment from a limited market. In fact, many of the world's most expensive drugs are orphan drugs.[30]

Orphan Drug Landscape in India

Rare diseases and orphan drugs have rarely been considered in policy formulation. Until the National Policy of Rare Diseases (NPRD) 2017, the field was largely overlooked by the Government, with most support coming from patient advocacy groups and NGOs. Due to implementation challenges, the 2017 policy was withdrawn. The National Policy for Rare Diseases 2021 was introduced with necessary changes to address the unmet demand.[31]

The policy acknowledges the absence of comprehensive empirical data needed to assess rare disease prevalence in India and the need to formulate a context-specific definition. To address this, a National Registry has been initiated by ICMR.[32] Rare diseases have been categorized into three groups based on existing knowledge, and the policy aims to enhance prevention, diagnosis, and treatment through the establishment of Centres of Excellence. It also includes provisions for financial support to patients and encourages crowdfunding efforts. Research and drug development for rare diseases are also emphasized, with funding agencies being involved to promote innovation. The National Consortium for Research and Development on Therapeutics for Rare Diseases has been established under the policy for the improvement of research and development. The policy also advocates for an integrated approach, engaging multiple ministries to boost local orphan drug production.

According to NPRD, 450 rare diseases have been identified to have incidence in India. Applying international estimates, 70 – 90 million people are affected by rare diseases in the country.[33] ICMR identifies 4001 rare diseases in India.[34] However, the exact prevalence is unknown due to a lack of adequate epidemiological data. This makes it difficult to accurately evaluate the morbidity and mortality. Therefore, the economic burden cannot be adequately

assessed, thus affecting policy-orienteddecision-making. The prevalence of rare diseases in India is presumed to be high due to cultural practices such as consanguineous marriages, which contribute to the accumulation of genetic traits, as most of these diseases are genetic in nature.[35]

The drug regulatory regime in India has evolved under the supervision of the Central Drugs Standard Control Organization and various legislative reforms. The pharmaceutical industry in India is rapidly expanding, with generic medicines dominating the market. Though traditionally fragmented, the sector is rapidly professionalizing with stronger regulations and innovation efforts. However, challenges such as regulatory inconsistencies and limited original drug discovery exist.[15] The focus on orphan drugs was also limited until the New Drugs and Clinical Trial Rules, 2019. An orphan drug under the Rules means a drug used for the treatment of a disease affecting less than 5 lakh people.[36] The Rules waive local clinical trials under prescribed conditions, accelerate approval, and offer a fee waiver for clinical trials in case of orphan drugs.[37]

National initiatives like Ayushman Bharat Pradhan Mantri Jan Arogya Yojana, Rashtriya Arogya Nidhi, Ayushman Bharat Digital Mission, National Sickle Cell Anaemia Digital Mission, and National Health Mission have attracted increased attention towards the management and treatment of rare diseases.[38] Under the 'Aatmanirbhar Bharat' (Self-reliant India) Campaign, the Indian government introduced a Production Linked Incentive Scheme for domestic manufacturing of pharmaceuticals wherein financial incentives are provided for the manufacturing of certain products under three categories. The scheme provides for the support of orphan drugs in Category 1.[39]

While all these are positive measures, significant gaps remain. A definition of rare diseases suited to the Indian demography is yet to be developed, for which there has to be data regarding prevalence and diversity. Lack of adequate diagnostic facilities makes a timely diagnosis difficult. The awareness of rare diseases among both medical professionals and the general public is very low. Other challenges include, lack of expertise and specialised institutions, lack of sufficient documentation, unavailability of treatment, prohibitive costs of existing treatment, inadequate funding for research as well as treatment, and a fragmented healthcare system.[40] Rare diseases not only affect the patient but also place a financial, emotional, and societal burden on families, medical professionals, and caregivers. The policy also suffers drawbacks in terms of a lack of an effective implementation strategy and funding.[41]

The policy does not offer sufficient incentives for orphan drug development to drive large-scale research and overcome existing challenges. Domestic Manufacture of drugs can bring down the cost of development and treatment, which requires the introduction of incentives for manufacturers. Greater support and intervention are essential to protect the efforts of developers and fund research institutions. The definition in the Drugs and Clinical Trials Rules, 2019, does not fully account for the actual prevalence and diversity of rare diseases in India. Moreover, the practical utilisation of these provisions needs to be thoroughly assessed. While these steps mark progress, the growing unmet demand underscores the need for more comprehensive and inclusive measures to effectively address the challenges in orphan drug development.[42]

There is no legislative framework that effectively balances the interests of both patients and drug developers or manufacturers. The absence of enforceable provisions reflects a lack of legal recognition for orphan diseases and orphan drugs. For patients, this translates not only to a scarcity of medicines but also to prohibitively high treatment costs that remain out of reach for most people. Funding for therapeutic and research activities primarily relies on the efforts of voluntary groups and nonprofit organizations. In addition, patent law presents limitations in incentivizing orphan drug development. The criteria for patentability, such as the requirement for inventive step and the exclusion of incremental innovations under Section 3(d), often discourage investment in repurposed or modified treatments.

Need for a New Approach

There is a pressing need for a sustainable regulatory framework that not only promotes research and innovation but also enhances domestic manufacturing and encourages active engagement of patients in the process. Such a framework should ensure adequate insurance coverage and reimbursement mechanisms, making treatments more accessible. This approach promotes innovation while ensuring that life-saving drugs are accessible to populations. A collaborative, multi-stakeholder approach involving the government, industry,

healthcare providers, and patient advocacy groups is essential. Incentivizing pharmaceutical companies to invest in orphan drug development is crucial, but it must be balanced with measures to curb exorbitant pricing. To create smoother pathways for drug approval, dedicated R&D funding is vital, along with clear regulatory guidelines and effective price control mechanisms to ensure affordability without stifling innovation. Additionally, the strategic use of TRIPS flexibilities can play a significant role in enhancing access to orphan drugs, especially when high costs hinder availability.

The current Indian regulatory framework lacks sufficient incentives for orphan drug R&D. The inherent challenges in this field impede drug development, regardless of patent protection. A fresh approach is needed, beginning with a definition of rare diseases tailored to India's demographics and healthcare landscape. Establishing a dedicated body for orphan drug designation and market approval could streamline entry and reduce bureaucratic hurdles. Drawing from global best practices, India must implement economic incentives while ensuring that they align with local needs. The effective implementation of TRIPS flexibilities and the Aatmanirbhar Bharat schemes within the national policy framework can significantly enhance the affordability and accessibility of orphan drugs. These policies, when integrated with well-structured funding, reimbursement, and accessibility mechanisms, can ensure the equitable distribution of medicines. Moreover, the generic industry also has a crucial role in enhancing access by providing cost-effective alternatives to branded medications, ensuring treatments are available to a larger population.

Beyond regulatory protection, adequate funding is crucial for orphan drug development. Given the unique challenges of rare diseases, many institutions and agencies hesitate to invest in research due to limited commercial returns. Funding is essential not only for patient treatment but also for drug discovery and development. However, with government resources already constrained, the feasibility of allocating sufficient funds remains a key concern. To address this, alternative funding sources must be explored, and legislative policies should incorporate mechanisms to attract investment from diverse stakeholders. A collaborative approach involving multiple ministries, public institutions, private entities, and the medical community is necessary. Additionally, the regulatory framework should prioritize awareness campaigns, patient advocacy, and the promotion of user-driven innovations. Intervention in terms of a well-designed policy is essential to establish structured funding, regulatory support, and sustainable market exclusivity models. Such an approach reduces the gap between profit-making and social welfare.

Conclusion

Patents and innovation are only weakly connected, as already highlighted by the dominance of patents on incremental modifications and the capacity of some industries to innovate without substantial reliance on patent protection. Various alternative mechanisms exist to encourage innovation, such as direct investment protection through market exclusivity and incentives like research grants and prizes. These approaches are particularly crucial for orphan drugs, where traditional patent incentives granting exclusive rights to encourage innovation often fall short due to limited market opportunities. Market exclusivity can help ensure a return on investment, even if a drug does not meet patent criteria. However, exclusivity mustn't come at the cost of access and affordability.

Orphan drugs pose a significant burden on healthcare budgets, regardless of a country's economic or developmental status. Balancing health priorities is essential, especially in resource-constrained settings like India, where state funds are limited. Although many patients have approached the courts to seek free treatment for rare diseases, the state faces challenges in allocating substantial resources, especially for conditions that require ongoing treatment, where even a single dose is extremely expensive. Nevertheless, public-private partnerships and international collaborations hold great potential to advance research, development, and access to orphan drugs. Leveraging such partnerships, along with innovative funding models and policy support, can reduce the burden on the public health system. Achieving an adequate balance where the needs of all patient populations are addressed without discrimination requires a holistic, inclusive approach that aligns affordability, accessibility, and innovation.

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