# Leveraging India's Patent Regime for Affordable Orphan Drug Development: A Pathway for Not-for-Profit Innovation

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**Abstract.** India's distinctive patent law environment presents unique opportunities for not-for-profit organizations aiming to develop orphan drugs for rare diseases. Section 3(d) of the Indian Patent Act, which restricts the patentability of new forms of known substances lacking enhanced therapeutic efficacy, reduces intellectual property barriers that typically hinder drug repurposing efforts in other jurisdictions. The legal framework explored in this paper combined with India's compulsory licensing regime, offers a strategic advantage for mission-driven entitles seeking to advance treatments for underserved patient populations. This paper explores how not-for-profit organizations can leverage these legal provisions within the broader regulatory eco-system, encompassing 'National Policy for Rare Diseases of 2021', 'Drugs and Cosmetics Act' and patent protection strategies. Through critical analysis of these legal and institutional frameworks, this paper finally proposes actionable strategies for strengthening orphan drug development by not-for-profit entities in India, ultimately aiming to deliver accessible and affordable treatments to individuals affected by rare diseases.

**Keywords:** Orphan drugs, Not-for-Profit, Pharmaceuticals, Patents, Law

## 1 Introduction

Rare diseases, collectively affecting approximately 70 to 90 million individuals in India alone (Rajasimha et al., 2018), present significant challenges for healthcare systems worldwide. These conditions, often genetic in origin, typically lack adequate treatment options due to commercial disincentives in traditional pharmaceutical business models. The high cost of research and development, coupled with small patient populations, has historically resulted in orphan drug development being financially unattractive for profit-driven entitles (Seoane-Vazquez et al., 2008). Consequently, patients with rare


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diseases face substantial barriers to accessing life-improving or life-saving medication, creating critical public health gap.

Not-for-profit pharmaceutical organizations have emerged as important actors in addressing market failures in orphan drug development (Hoffman, 2017). Unlike their commercial counterparts, these organizations prioritize patient access over profit maximization, making them ideally positioned to pursue treatments for neglected conditions. However, their success depends significantly on navigating complex intellectual property landscapes that can either facilitate or hinder affordable drug development.

India's unique patent regime offers distinctive opportunities for not-for-profit drug development. The country's pharmaceutical legal framework includes several provisions that potentially facilitate more accessible development of orphan drugs (Basheer & Reddy, 2008). Central to this framework is Section 3(d) of the Indian Patents Act, which limits evergreening practices by requiring demonstrated improved efficacy of new forms of well-known elements to qualify for patent protection (The Patents Amendments Act, 2005).

This paper employs doctrinal analysis to examine India's patent law framework, supplemented by policy analysis of the regulatory and institutional ecosystem. The research draws upon statutory provisions, landmark judicial decisions, regulatory guidelines, and published policy documents. Illustrative examples are selected based on their relevance to orphan drug development pathways and demonstration of legal principals in practice. This paper also investigates how not-for-profit organizations can strategically leverage India's patent regime to develop affordable treatments for rare diseases. Section 2 analyzes strategic advantages offered by India's patent law framework. Section 3 explores the regulatory ecosystem including the National Policy for Rare Diseases and approval pathways. Section 4 examines collaborative strategies through domestic and international partnerships. Section 5 discusses open innovation models. Section 6 addresses implementation challenges. Section 7 proposes policy recommendations organized under legal reforms, financial mechanisms, and capacity building. The analysis demonstrates how India's patent flexibilities, when combined with supportive institutional arrangements, can enable mission-driven entities to address unmet needs in rare diseases treatment while maintaining affordability objectives

## 2 India's Patent Law Framework: Strategic Advantage for Orphan Drug Development

### 2.1 Section 3(d) and Drug Repurposing Opportunities

India's Patents Act contains distinctive provisions that facilitate orphan drug development, most notably Section 3(d). This provision states that "the mere discovery of a new form of a known substance which does not result in the enhancement of the known efficacy of that substance" is not patentable (The Patent Act, 1970, Section 3(d)). This clause prohibits pharmaceutical companies from securing patents on minor changes to existing medications unless they demonstrate substantial therapeutic enhancement, thus limiting evergreening practices.

For orphan drug development, Section 3(d) creates an advantageous environment for drug repurposing strategies. Drug repurposing, the identification of new therapeutic uses for existing medications, represents an efficient approach for rare disease treatment (Pushpakom et al., 2019). By circumventing extensive early-stage research, repurposing can reduce development costs by up to 75% compared to de novo drug discovery (Nosengo, 2016).

The Indian Supreme Court's 2013 decision in *Novartis AG v. Union of India* (2013) established that efficacy in pharmaceutical context refers specifically to therapeutic efficacy. This interpretation creates a higher bar for patentability affecting polymorph patents, salt forms and esters, formulation modifications, and combination products containing known active ingredients.

**Table 1.** Key Indian Patent Provisions for Orphan Drug Development

| Provision & Section | Relevance to Orphan Drugs | Strategic Advantage |
|---|---|---|
| Anti-evergreening 3(d) | Restricts secondary patents on known substances | Enables repurposing of existing compounds without infringement |
| Compulsory Licensing 84 | Permits licensing when public needs unmet | Access to patented technologies at affordable prices |
| Pre-grant Opposition 25(1) | Public challenge before patent grant | Cost-effective mechanism to prevent problematic patents |
| Bolar exemption 107a | Research use of patented inventions | Enables early development work without infringement |

For not-for-profit organizations, this interpretation creates opportunities to work with existing compounds that might be patent-protected elsewhere. For example, thalidomide, which has shown efficacy against multiple myeloma and leprosy complications, faced patent barriers in many countries through secondary patents (Stirner & Thangaraj, 2013). In India, many such secondary patents would likely fail Section 3(d) scrutiny, enabling not-for-profit developers to pursue these applications without infringement concerns.

### 2.2 Compulsory Licensing Provisions and Public Health Priorities

India's patent framework includes robust compulsory licensing provisions under Section 84, permitting licenses when reasonable public requirements are not satisfied, when the invention is not available at reasonably affordable prices, or when the patent is not worked in India (The Patent Act, 1970, Section 84). These provisions, aligned with TRIPS Agreement flexibilities, provide mechanism for accessing patented technologies when public health needs are inadequately addressed.

The *Bayer Corporation v. Natco Pharma Ltd*. (2013) case established precedent when India's Controller of Patents granted a compulsory license for 'sorafenib tosylate', citing affordability concerns and insufficient working. For rare diseases, where patented treatments often command prohibitively high prices, compulsory licensing serves as both a direct access mechanism and a negotiation lever for not-for-profit developers. However, the strategic use of these provisions requires careful consideration of international relations, as aggressive compulsory licensing has historically faced pushback from pharmaceutical patent holders and their home governments (Baker, 2008).

### 2.3 Pre-grant Opposition and Patent Quality Control

The Indian Patent Act's Section 25(1) enables any person to submit written opposition to a patent application before grant (The Patent Act, 1970, Section 25(1)). This mechanism allows public scrutiny on grounds including lack of novelty, obviousness, non-patentability under Section 3, insufficient disclosure, and wrongful acquisition.

For not-for-profit organizations, this system offers strategic value by providing cost-effective challenges to weak pharmaceutical patents, enabling early intervention before patent establishment, and allowing collective action with patient groups and academic institutions. The successful challenge to Gilead's sofosbuvir application demonstrates this system's effectiveness (Amin, 2018).

### 2.4 Bolar Exemption and Research Exceptions

Section 107A offers a comprehensive research exemption permitting use of patented inventions for research and regulatory approval activities (The Patents Act, 1970, Section 107A). This exemption enables not-for-profit organizations without infringement liability, allows for rare disease application without infringement liability, allows early development of manufacturing processes, and facilitate data generation for regulatory filings. India's research exemption scope is relatively broad compared to other jurisdictions, creating a favorable environment for early-stage orphan drug development (Mueller, 2007).

## 3 Regulatory Ecosystem for Orphan Drug Development in India

### 3.1 National Policy for Rare Diseases: Evolving Framework

The National Policy for Rare Diseases 2021 established a three-tiered categorization: Group 1 disorders amenable to one-time curative treatment, Group 2 diseases requiring long-term treatment with relatively lower cost, and Group 3 diseases with available but high-cost treatment (Ministry of Health and Family Welfare, 2021a). The policy provides financial support up to 20 lakh rupees for Group 1 diseases treated at Centers of Excellence (Ministry of Health and Family Welfare, 2021b, p. 15)

**Table 2.** Regulatory and Policy Framework for Orphan Drugs in India

| Framework Component | Key Agents | Current Status | Gap/Opportunity |
|---|---|---|---|
| Rare Disease Policy | NPRD 2021 | Three-tier classification; limited funding (20 Lakh cap) | Lacks drug development incentives |
| Drug Approval | CDSCO under Drugs and Cosmetics Act 1940 | Standard pathways; no orphan-specific route | Opportunity for dedicated pathway |
| Pricing Control | NPPA under DPCO 2013 | Case-by-case intervention for specialized drugs | Potential for orphan drug pricing framework |
| Manufacturing Incentives | PLI Scheme 2021 | Supports complex generics production | Eligibility for orphan drug manufacturers |

For not-for-profit organizations, this policy opportunities through formal recognition of rare diseases as a public health priority. However, its focus on treatment access rather than drug development leaves significant gaps. The emphasis on indigenous development signals potential support for domestic innovation that not-for-profit organizations can leverage.

### 3.2 Regulatory Pathways and Accelerated Approval

India's drug regulatory framework, governed by the Drugs and Cosmetics Act (1940) and New Drugs and Clinical Trial Rules (2019), lacks specific orphan drug approval pathways. Unlike the United States, Europe Union, and Japan, India's Central Drugs Standard Control Organization applies standard approval processes regardless of disease prevalence. However, provisions for accelerated approval based on surrogate end points for severe conditions could benefit rare disease treatments (New Drugs and Clinical Trial Rules, 2019)

Not-for-profit developers can navigate this landscape by engaging early with regulatory authorities through pre-submission consultations, leveraging global data through provisions allowing waiver of local trials for drugs approved in specified countries, and utilizing the academic clinical trial pathway with simplified requirements for non-commercial studies.

### 3.3 Pricing Regulations and Accessibility Considerations

India's pharmaceutical pricing through the National Pharmaceutical Pricing Authority plays a crucial role in medication accessibility. The Drug Price Control Order of 2013 empowers price fixing for scheduled formulations. In 2018, the authority capped prices of select anti-cancer drugs, establishing precedent for intervention in specialized therapeutic areas (National Pharmaceutical Pricing Authority, 2018). The Production Linked Incentive scheme for pharmaceuticals offers potential manufacturing incentives for domestically produced orphan drugs (Department of Pharmaceuticals, 2021).

## 4 Strategic Collaborations for Enhanced Capacity

### 4.1 Domestic Research Networks and Institutional Partnerships

Not-for-profit organizations can enhance capabilities through strategic domestic partnerships. India's public research institutions, including the Council of Scientific and Industrial Research, Indian Council of Medical Research, and Institutes of National Importance, maintain advanced facilities and specialized knowledge (Department of

Science and Technology, 2020). The National Centre for Cell Science in Pune has expertise in cellular models of rare genetic disorders, while the Centre for Cellular and Molecular Biology in Hyderabad maintains advanced genomic analysis capabilities.

Successful collaboration models include joint research programs with shared intellectual property frameworks, exemplified by the Open-Source Drug Discovery initiative, technology transfer arrangements, shared clinical research networks, and formal Material Transfer Agreements. The Science, Technology, and Innovation Policy of 2020 encourage public-private partnerships in health research (Department of Science and Technology, 2020).

### 4.2 Patient Organization Collaborations

Patient Advocacy organizations provide unique insights into disease manifestations, treatment priorities, and meaningful outcome measures. Groups such as the Organization for Rare Diseases India (ORDI) and Lysosomal Storage Disorders Support Society have emerged as significant voices (Choudhary & Nagarajan, 2020). Strategic collaborations offer access to patient registries and natural history data, participation in patient-centered trial design, and powerful advocacy partnerships.

The Patient Centered Outcomes Research Institute model from the United States provides a template for structured patient engagement that could be adapted to the Indian context, formalizing patient input throughout the drug development process (Frank et al., 2014).

### 4.3 International Technology Transfer and Research Collaborations

International partnerships offer crucial access to technologies and expertise. Pathways include voluntary licensing arrangements exemplified by the Medicines Patent Pool (‘t Hoen et al., 2018), North-South research partnerships, and Product Development Partnerships such as the Drugs for Neglected Diseases Initiative (Pecoul et al, 2021). The initiative's development of fexinidazole for sleeping sickness demonstrates successful collaborative approaches (DNDi, 2019).

For Indian not-for-profits, engagement with international initiatives including the International Rare Disease Consortium provides access to global resources. These collaborations leverage India's strengths in clinical research and manufacturing while accessing complementary international capabilities in specialized areas such as gene therapy manufacturing and advanced formulation technologies.

## 5 Open Innovation Models for Cost-Effective Development

### 5.1 Open-Source Drug Discovery Approaches

Open-source approaches apply principles from software development to pharmaceutical research, emphasizing transparent sharing of data and methods without restrictive intellectual property barriers (Balasegaram et al.,2017). The Open-Source Drug Discovery project coordinated by the Council of Scientific and Industrial Research demonstrated this approach's potential in tuberculosis research (Maiti & Mukherjee, 2015).

For orphan drug development, open-source approaches distribute research costs across participants, accelerate knowledge generation through transparent sharing, create commonly accessible resources, and facilitate distributed problem-solving. Implementation strategies include establishing precompetitive research consortia, creating open-access databases, developing shared computational platforms, and implementing modular research workflows.

Results can be protected through patent-left approaches preventing downstream patenting or through strategic patenting with humanitarian licensing terms (Rai & Reichman, 2016). The Structural Genomics Consortium provides a model requiring all outputs remain in the public domain.

### 5.2 Data Sharing Platforms and Collaborative Resources

Shared data resources address knowledge gaps in rare disease drug development. For many rare conditions, limited understanding of natural history, biomarkers, and outcome measures presents significant barriers. Collaborative platforms can consolidate scattered information and generate insight through collective analysis.

The National Digital Health Mission creates infrastructure potentially supporting rare disease data initiatives (Ministry of Health and Family Welfare, 2020). Not-for-profit organizations can leverage this ecosystem to establish disease-specific data collaboratives. Governance frameworks must address patients' privacy, data security, and appropriate consent mechanisms for sensitive genetic and health information.

For genetic rare disease constituting approximately 80% of rare conditions, the GenomeAsia 100K initiative provides valuable population-specific data (GenomeAsia100K Consortium, 2019). Not-for-profit developers can utilize these resources to identify India-specific genetic variants and therapeutic targets.

### 5.3 Shared Clinical Trial Networks and Infrastructure

Clinical trials for rare diseases present unique challenges due to geographical patient dispersion, limited disease expertise, and difficulties achieving statistical power with small samples. Collaborative networks address these challenges by creating shared infrastructure for patient identification, standardizing protocols across sites, and building specialized expertise.

The Department of Biotechnology's Clinical Development Services Agency supports clinical trial capacity building and could partner for rare disease-focused trial networks (Clinical Development Services Agency, 2020). Innovative trial designs including adaptive platform trials, N-of-1 studies, and Bayesian approaches maximize information from limited patient populations (Saville & Berry, 2016). Not-for-profit organizations can coordinate these specialized clinical research capabilities in partnership with academic medical centers and patient organizations.

## 6 Implementation Challenges and Mitigation Strategies

### 6.1 Funding Constraints and Sustainable Models

Despite legal and regulatory advantages, not-for-profit orphan drug development faces significant funding challenges. Traditional venture capital is rarely available for non-commercial entities, and philanthropic funding remains limited. To address constraints, not-for-profit developers can pursue diversified funding models including blended finance combining philanthropic grants with impact investments and government funding, tiered pricing maintaining affordability in India while generating revenue from higher-income markets, cross-subsidization while revenues from common indications support rare disease work, milestone-based funding reducing investor risk, and social impact bonds designed for rare disease development outcomes.

The Wellcome Trust's Affordable Innovations in Healthcare Initiative demonstrates how blended finance can support health innovation for underserved populations (Wellcome Trust, 2020). A particularly promising approach combines delinkage, separating research and development financing from product pricing, with sustainable business models. Research and development costs are covered through grants, prizes, and push-pull mechanism, allowing products to be sold at near-marginal production cost

### 6.2 Technical Expertise and Capability Gaps

Drug development requires specialized expertise across medicinal chemistry, preclinical testing, clinical trial design, regulatory affairs, and manufacturing. Many not-for-profit organizations lack comprehensive in-house capabilities. Strategic approaches include hub-and-spoke organizational models centralizing specialized functions, shared service platforms providing access to technical capabilities, and capability building partnerships with academic institutions.

The Council of Scientific and Industrial Research network offers potential infrastructure and expertise supporting not-for-profit developers through collaboration agreements (Council of Scientific and Industrial Research, 2021). Retired pharmaceutical professionals represent an untapped resource, potentially providing experienced mentorship through structured volunteer programs.

### 6.3 Intellectual Property Management and Freedom to Operate

Despite advantages offered by India's patent system, not-for-profit developers must navigate complex IP landscapes. Effective management requires comprehensive freedom-to-operate assessments early in development programs, strategic decisions about patent filing and licensing strategies balancing revenue generation with access objectives.

The Medicines Patent Pool provides a model managing intellectual property through voluntary licensing approaches (Medicines Patent Pool, 2021). Not-for-profit developers can adapt these approaches, potentially establishing specialized patent pools for rare disease technologies. Protection strategies include humanitarian licensing terms ensuring affordable access in low- and middle-income countries, territory-specific patent strategies seeking protection only where commercial returns are necessary, and open innovation approaches placing certain outputs in the public domain while protecting specific applications.

## 7 Policy Recommendations and Future Directions

The following recommendation enhances the legal, financial, and institutional environment for not-for-profit orphan drug development in India.

### 7.1 Legal and Regulatory Reforms

i. **Orphan drug designation and regulatory pathways**: Establish formal orphan drug designation with regulatory accommodations adapted to India's healthcare context. This should include accelerated review provisions, modified clinical trial requirements appropriate for small patient populations, and fee waivers or reductions for qualifying not-for-profit developers.
ii. **Adaptive licensing frameworks**: Develop an adaptive licensing pathway for rare disease treatments allowing progressive authorization based on evolving evidence. This facilitates earlier patient access while continuing to gather real world data on safety and efficacy.
iii. **Research exemption expansion**: Explicitly expand research exemptions to cover all activities related to orphan drug development for non-commercial purposes, providing clear legal certainty for not-for-profit developers working on patented compounds for new indications.
iv. **Integration with pharmaceutical pricing policy**: Integrate orphan drug considerations into pharmaceutical pricing policies, including special provisions for demonstrated not-for-profit development efforts prioritizing affordability and access.

v. **The Draft New Drugs, Medical Devices and Cosmetics Bill of 2022**: The Bill presents a timely opportunity for incorporating orphan drug-specific provisions (Central Drugs Standard Control Organization, 2022). Stakeholder advocacy during consultation could ensure rare disease treatment needs receive appropriate attention in this legislative overhaul.

### 7.2 Financial Support Mechanisms and Incentives

i. **Dedicated orphan drug innovation fund:** Establish a dedicated fund supported by government allocations and potentially a small levy on pharmaceutical sales. This fund should prioritize projects led by not-for-profit organizations with demonstrated commitment to affordable access.
ii. **Tax incentives and corporate social responsibility provisions:** Create tax incentives for donations to qualified not-for-profit drug development organizations. Expand corporate social responsibility guidelines to explicitly recognize rare disease drug development as a qualifying activity under the Companies Act 2013.
iii. **Advanced market commitments and milestone prizes:** Implement advanced market commitments for orphan drugs addressing priority rare diseases, providing assured demand and pricing for successfully developed treatments. Complementary milestone prize mechanisms can reward achievement of specific development objectives.
iv. **Social impact investment vehicles:** Create social impact investment vehicles specifically designed for rare disease drug development, potentially modelled on social impact bonds tying returns to achievement of predefined public health outcomes.
v. **The Biotechnology Industry Research Assistance Council:** It could be expanded with programs targeting orphan drug development by not-for-profit organizations (Biotechnology Industry Research Assistance Council, 2021). The Promoting Innovations in Individuals, Start-ups and Micro, Small and Medium Enterprises scheme could be modified to include special provisions for rare disease-focused innovations.

### 7.3 Institutional Capacity and Human Resource Development

i. **Specialized training and fellowship programs:** Develop specialized training programs in orphan drug development within pharmaceutical education curricula. Establish fellowship programs to develop expertise in rare disease clinical research, emphasizing both scientific and regulatory aspects.
ii. **Knowledge transfer and international exchange:** Implement knowledge transfer initiatives bringing international expertise to the Indian context

through visiting scientist programs, collaborative research exchanges, and participation in global rare disease research networks.

iii. **Infrastructure support for collaborative research:** Provide infrastructure grants for shared laboratory and clinical research facilities focused on rare diseases. This should include support for establishing Centers of Excellence serving as hubs for rare disease research and clinical care.

iv. **The Skills India initiative** could incorporate specialized pharmaceutical development skills with attention to orphan drug development needs (Ministry of Skill Development and Entrepreneurship, 2015). Partnerships between government training programs, academic institutions, and not-for-profit organizations can create pipelines of skilled professionals dedicated to rare disease research.

### 7.4 Responsible Innovation and Data Governance

As not-for-profit organizations develop orphan drugs utilizing advanced technologies and patient data, responsible innovation frameworks become essential. Digital health platforms and rare disease registries must incorporate robust governance mechanisms ensuring patient privacy, informed consent, and data security. For biologics and genetic technologies, appropriate biosafety protocols and ethical oversight committees should guide development activities. Transparent stakeholder engagement, including patients, clinicians, and ethicists, ensures that innovation pathways align with societal values and patient interests while maintaining scientific rigor and regulatory compliance.

## 8 Conclusion

India's unique patent regime, characterized by Section 3(d)'s restrictions on secondary patents, robust compulsory licensing provisions, and accessible patent opposition mechanisms, creates distinctive opportunities for not-for-profit orphan drug development. When leveraged strategically within the broader regulatory ecosystem and supported by collaborative approaches to research and development, these legal advantages can facilitate cost-effective innovation addressing rare disease treatment needs.

The analysis carried out in the paper demonstrates that India's patent system offers specific advantages for orphan drug development particularly well-suited to not-for-profit models. Section 3(d) restrictions facilitate drug repurposing strategies, critical for rare diseases given reduced development costs and timeframes. Compulsory licensing provisions and Bolar exemption create pathways for accessing technologies and developing affordable treatments that might otherwise be blocked by patent barriers.

However, the legal framework alone is insufficient. Effective orphan drug development requires an integrated approach combining legal strategies with collaborative research models, innovative financing mechanisms, and supportive regulatory pathways. Open innovation approaches, including open-source drug discovery and collaborative data platforms, offer particularly promising models for addressing resource constraints faced by not-for-profit developers.

While significant challenges remain regarding sustainable funding, technical capacity and clinical development infrastructure, targeted policy interventions can strengthen the environment for mission-driven pharmaceutical innovation. The recommendations outlined under legal and regulatory reforms, financial support mechanisms, and institutional capacity development provide a roadmap for enhancing the ecosystem supporting not-for-profit innovation.

By combining legal advantages with collaborative models and specialized support mechanisms, India has the capacity to become a global leader in affordable orphan drug development led by not-for-profit organizations. This leadership role would benefit rare disease patients within India and provide a model for other developing nations facing comparable challenges in addressing rare disease treatment needs.

The approach recognizes that meaningful progress in rare disease treatment requires not just scientific innovation but also legal, regulatory, and institutional innovation. Through strategic use of India's patent system, coupled with collaborative development models and supportive policies, not-for-profit organizations can play a transformative role in addressing needs of rare disease patients historically underserved by commercial drug development paradigms. As the global community increasingly recognizes the importance of equitable access to medications, India's experience with not-for-profit orphan drug development could offer valuable lessons for achieving both innovation and accessibility in pharmaceutical development more broadly.

## 9 Declarations

### 9.1 Conflict of Interest

The authors declare that they have no conflict of interest.

### 9.2 Funding

This paper did not receive any specific grant from funding agencies in the public, commercial or not-for-profit sectors.

### 9.3 Institutional Ethical Committee Approval

The research study carried out in the paper is based exclusively on secondary data and publicly available sources. It did not involve human participants, animals, or clinical data, and therefore did not require approval from an institutional ethical committee.

### 9.4 AI Declaration Statement

Generative AI tools were used solely to support language editing and/or formatting of author-written text (e.g., clarity, grammar, structure) for conclusion and references. No confidential, sensitive, or identifiable personal data were entered into these systems. All content was reviewed and verified by the authors, who remain fully responsible for the manuscript.

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