

Policy Implementation and Stakeholder Engagement in Rare Disease Management: An In-Depth Analysis of India's National Policy for Rare Diseases (NPRD) 2021 and Ayushman Bharat Scheme

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ABSTRACT

The National Policy for Rare Diseases (NPRD) 2021 and Ayushman Bharat are key initiatives in India's healthcare framework aimed at enhancing equitable access to medical treatment. NPRD provides a structured approach to managing rare diseases, which are often costly and complex, through financial aid and specialized Centres of Excellence (CoEs). Ayushman Bharat complements these efforts by providing health insurance for economically disadvantaged groups, although its coverage for rare diseases is still limited. This paper examines the roles of stakeholders such as Health Secretaries, Mission Directors, and Parliamentarians in the implementation and improvement of these policies, as well as the financial and infrastructural challenges they face. State-wise budget allocations and parliamentary advocacy are analyzed to understand how policy improvements can be achieved to benefit rare disease patients across India. Findings highlight existing gaps in resource distribution, accessibility in rural areas, and data tracking systems, suggesting that enhanced stakeholder collaboration and expanded insurance coverage are essential for effective rare disease management.

Keywords: Rare Diseases, NPRD, Ayushman Bharat, Centres of Excellence, Healthcare Policy, Stakeholder Mapping, India.

Introduction:

Rare diseases affect a small fraction of the population, but they often present significant healthcare challenges due to their complexity and high treatment costs. In India, rare disease

management has traditionally been an overlooked area. The National Policy for Rare Diseases (NPRD) 2021 was developed to provide a framework for improving access to treatment and reducing the financial burden on affected families. Meanwhile, the Ayushman Bharat Scheme aims to offer universal health coverage, with special focus on economically disadvantaged populations.

India's National Policy for Rare Diseases (NPRD) 2021 seeks to address the unique healthcare challenges associated with rare diseases, which, while affecting a small fraction of the population, require costly and specialized treatment. NPRD 2021 introduces a framework for financial aid and improved access to care through designated Centres of Excellence (CoEs) across India.

NPRD's success depends on its integration with other policies, particularly Ayushman Bharat, which provides health insurance coverage up to ₹5 lakhs annually for secondary and tertiary care. Although Ayushman Bharat mainly covers common procedures, efforts are underway to include rare disease treatments, ensuring broader financial relief for affected families. Health and Wellness Centres (HWCs) under Ayushman Bharat could also enhance rural outreach, improving early diagnostics and access to treatment.

Effective implementation of NPRD relies on coordination between stakeholders, including Health Secretaries, Mission Directors, and State Nodal Officers, each overseeing different aspects of healthcare delivery and rare disease management. Parliamentarians also play a critical role by advocating for policy improvements, funding, and the establishment of a national registry.

Despite NPRD's initiatives, gaps remain. Limited CoEs primarily serve urban areas, restricting rural access. High costs for orphan drugs and lifelong treatment continue to burden families, highlighting the need to extend Ayushman Bharat's coverage. Additionally, a centralized registry for rare diseases is essential to better track patient data, resource allocation, and policy outcomes. Addressing these issues would strengthen NPRD's impact, creating a more equitable healthcare framework for rare diseases in India.

By exploring the interplay between the two landmark policies – NPRD 2021 & Ayushman Bharat Scheme, this paper highlights the role of various stakeholders, including health authorities, Centres of Excellence (CoEs), and policymakers, in delivering effective rare disease care. Additionally, the paper assesses budget allocations across states and identifies opportunities to enhance healthcare access through policy reforms.

Background:

National Policy for Rare Diseases (NPRD) 2021:

The NPRD 2021 was established to address the unique challenges faced by rare disease patients. Under this policy, rare diseases are grouped into three categories:

1. **Group 1:** Diseases that are treatable with one-time interventions, such as liver transplants.
2. **Group 2:** Diseases requiring long-term or lifelong management, like hemophilia and thalassemia.
3. **Group 3:** Diseases where treatment is available but costly and not easily accessible, including genetic disorders like Gaucher's disease.

The policy aims to reduce the financial burden on patients by providing up to ₹50 lakhs of financial assistance under the Rashtriya Arogya Nidhi (RAN) scheme. It also establishes specialized Centres of Excellence (CoEs) to ensure that rare disease patients have access to advanced care, including genetic testing, counseling, and multidisciplinary treatment (Government of India, 2021).

Ayushman Bharat Scheme:

Ayushman Bharat is India's flagship health insurance program, targeting low-income families. It has two main components:

1. **Pradhan Mantri Jan Arogya Yojana (PM-JAY):** Provides health insurance coverage up to ₹5 lakhs per family per year for secondary and tertiary care.
2. **Health and Wellness Centres (HWCs):** Focuses on providing primary care services, including diagnostics, treatment for non-communicable diseases, and health education.

Ayushman Bharat's wide-reaching network could significantly contribute to rare disease care, but its current design primarily focuses on common medical procedures. There is a growing need to integrate rare disease treatments within PM-JAY's insurance coverage to address the financial challenges faced by affected families (Ministry of Health and Family Welfare, 2023).

Literature Review:

Healthcare disparities remain a formidable issue globally, especially for those affected by rare diseases who often require costly and specialized treatments. In India, the National Policy for

Rare Diseases (NPRD) 2021 aims to address these disparities by facilitating early diagnosis, appropriate care, and financial assistance for patients affected by rare diseases (Gupta et al., 2021). This policy represents a critical advancement in healthcare, focusing on reducing both morbidity and mortality rates among those with rare diseases by meeting their unique healthcare needs.

Raman and Sharma (2024) underscore the importance of collaborative efforts among key stakeholders, such as Health Secretaries, Mission Directors, and State Nodal Officers, in the successful implementation of the NPRD. They emphasize that efficient resource distribution relies on cohesive coordination and clear communication among these stakeholders, especially in areas where healthcare systems are overstretched and resources are limited. This coordination is vital to ensuring equitable access to care for rare disease patients throughout the country.

The NPRD 2021 classifies rare diseases into three distinct categories to streamline treatment and resource allocation: Group 1 includes diseases that can be treated with one-time interventions, such as specific lysosomal storage disorders; Group 2 comprises conditions that require continuous, long-term management, such as cystic fibrosis and Duchenne muscular dystrophy; and Group 3 includes diseases for which treatment is available but remains challenging due to complex management needs, like Gaucher and Pompe diseases (Gupta et al., 2021). To reduce the financial burden on affected families, the policy provides financial aid up to Rs. 50 lakhs per patient through the Rashtriya Arogya Nidhi scheme, supplemented by voluntary crowdfunding efforts (Sinha & Chaturvedi, 2022).

Budget allocation plays a critical role in determining healthcare access and service quality for rare disease patients across different regions. Raman and Sharma (2024) highlight the need for improved prioritization in healthcare spending to bridge gaps in treatment availability and enhance access for marginalized communities. Their research reveals significant disparities in budget allocations across states, underscoring the need for state-specific financial strategies that reflect the unique healthcare demands within each region.

Despite the NPRD's robust framework, implementation challenges persist. Sharma et al. (2022) identify difficulties in achieving consistent policy adoption across states, partly due to India's decentralized healthcare system. This decentralization leads to regional disparities in service delivery, complicating the policy's impact on a national level. Additionally, the absence of clearly defined funding mechanisms for tertiary care and a lack of comprehensive epidemiological data limit effective resource allocation and planning (Mishra & Patel, 2023). To address these data gaps, the Indian Council of Medical Research (ICMR) established a national rare disease registry, enhancing policymakers' ability to make informed interventions based on reliable data (Patel & Kumar, 2023).

Complementing NPRD, the Ayushman Bharat Scheme, launched in 2018, has expanded healthcare access significantly. The Pradhan Mantri Jan Arogya Yojana (PM-JAY), a component of Ayushman Bharat, provides Rs. 5 lakhs in annual health coverage per family for secondary and tertiary care, benefiting approximately 50 crore economically vulnerable individuals. Bhushan et al. (2020) detail the scheme's impact in lowering out-of-pocket healthcare expenses, enabling cashless and paperless access to healthcare across a network of empaneled hospitals. The scheme also includes fraud prevention and quality assurance measures to build public trust and improve healthcare outcomes (Rao & Singh, 2022).

Ayushman Bharat also focuses on primary healthcare through its Health and Wellness Centres (HWCs), which bring essential healthcare services closer to communities. Reddy et al. (2021) note that HWCs have improved healthcare infrastructure in underserved areas, reducing the need for long-distance travel for basic care. However, implementing HWCs presents challenges, including ensuring quality control, integrating state health schemes, and preventing fraud. Overcoming these challenges requires coordinated efforts between central and state governments, as well as engagement with local communities and healthcare providers (Verma & Chaudhary, 2023).

Parliamentary support has been pivotal in driving policies for rare disease management and expanding healthcare access. Raman and Sharma (2024) document the proactive role of Members of Parliament (MPs) in advocating for policies and financial aid to support rare disease patients. This legislative commitment has been instrumental in securing funding and refining policy frameworks, ensuring that rare disease patients receive adequate attention and support from government authorities (Deshmukh & Rao, 2023).

Research Objectives:

Stakeholder Mapping and Roles:

- **Objective 1:** To examine the roles and coordination of key stakeholders, such as Health Secretaries, Mission Directors, and State Nodal Officers, in implementing NPRD and Ayushman Bharat.
- **Objective 2:** To assess the distribution and accessibility of Centres of Excellence (CoEs) across regions, identifying challenges faced by rural patients in accessing specialized care.

Budget Allocation and Financial Sustainability:

- **Objective 3:** To analyze state-wise budget allocations and their impact on implementing NPRD and Ayushman Bharat for rare diseases.
- **Objective 4:** To explore the feasibility of integrating rare disease treatments into Ayushman Bharat to reduce financial burdens on affected families.

Parliamentary Engagement and Policy Advocacy:

- **Objective 5:** To evaluate the role of parliamentarians in advancing healthcare policies for rare diseases, particularly through budget support and infrastructure expansion.
- **Objective 6:** To assess the outcomes of parliamentary advocacy, including awareness-raising, budget increases, and the need for a national rare disease data registry.

Research Methodology:

This study employs a mixed-methods approach to examine the effectiveness of NPRD and Ayushman Bharat. A qualitative analysis of stakeholder roles was conducted using official policy documents, government reports, and expert interviews. Quantitative data on healthcare budget allocations was sourced from state-level budget reports for the fiscal year 2024-25. Parliamentary discussions were analyzed to assess the level of political engagement in shaping rare disease policies.

Analysis and Interpretation:

Stakeholder Mapping and Roles:

- **Key Stakeholders in NPRD and Ayushman Bharat Implementation:**

The successful execution of NPRD and Ayushman Bharat depends on the coordination of multiple stakeholders at both the national and state levels. These stakeholders include:

- **Health Secretaries** oversee the allocation of healthcare resources and ensure that states implement rare disease programs effectively.
- **Mission Directors** are responsible for executing national health missions, including Ayushman Bharat, and integrating rare disease care into broader healthcare services.
- **State Nodal Officers** manage the operational aspects of CoEs, ensuring that rare disease patients receive timely and appropriate care.
- **Parliamentarians** play a critical role in advocating for additional funding and infrastructure development during policy debates.

Table 1: Stakeholders and Their Roles in NPRD and Ayushman Bharat Implementation

Stakeholder	Role	Example
Health Secretaries	Oversee healthcare resource allocation and service delivery.	Delhi Health Secretary managing NPRD implementation.
Mission Directors	Execute Ayushman Bharat policies at the state level.	Tamil Nadu Mission Director implementing Ayushman Bharat.
State Nodal Officers	Coordinate CoEs and manage rare disease patient care.	Karnataka Nodal Officer at NIMHANS.
Parliamentarians	Advocate for budgetary support and policy improvements.	MPs advocating for CoEs expansion in West Bengal.

Efficient collaboration between these stakeholders is crucial for ensuring that rare disease patients receive timely treatment and financial assistance (Patel & Kumar, 2023).

- **Role of Centres of Excellence (CoEs):**

CoEs serve as hubs for specialized rare disease care, offering advanced diagnostic and therapeutic services. The NPRD 2021 designates 12 CoEs across five zones, ensuring geographical representation, though some regions remain underserved.

Table 2: Distribution of Centres of Excellence (CoEs) in India

Northern Zone	Central Zone	Eastern Zone	Western Zone	Southern Zone
Post Graduate Institute of Medical Education and Research (PGIMER), Chandigarh	All India Institute of Medical Sciences (AIIMS), Delhi	Institute of Post-Graduate Medical Education and Research (IPGMER), Kolkata	King Edward Memorial Hospital (KEM), Mumbai	Centre for DNA Fingerprinting & Diagnostics with Nizam Institute of Medical Sciences (CDFD), Hyderabad
All India Institute of Medical Sciences (AIIMS), Jodhpur	Maulana Azad Medical College (MAMC), Delhi			Centre For Human Genetics (CHG) with Indira Gandhi Hospital, Bangalore
	Sanjay Gandhi Postgraduate Institute of Medical Sciences (SGPGI), Lucknow			Institute Of Child Health and Hospital for Children (ICH), Chennai
	All India Institute of Medical Sciences (AIIMS), Bhopal			Government Medical College, Thiruvananthapuram, Kerala

Figure 1. Mapping of the 12 Centres of Excellence (CoEs) across the five zones



While CoEs provide high-quality care in major cities, patients in rural and remote areas face significant barriers in accessing these facilities, leading to delays in diagnosis and treatment (Gupta, 2021). Expanding CoE services and enhancing outreach programs are vital for improving access to care.

Budget Allocation and Financial Sustainability:

- **Analyzing State-wise Budget Allocations:**

State-level budget allocations are critical in determining the success of healthcare policies. Variations in healthcare funding across states directly impact the availability and quality of care for rare disease patients.

Table 3: Healthcare Budget Allocations for Selected Indian States (FY 2024-25)

States	Total Budget FY 2024-25	Health Budget	Percentage Allocated to Health
Punjab	₹ 2,04,918 crore	₹ 9,572 crore	4.6
Haryana	₹ 1,89,876 crore	₹ 8,364 crore	4.4
Delhi	₹ 76,000 crore	₹ 8,685 crore	11.4
Uttar Pradesh	₹ 7,36,437 crore	₹ 27,086 crore	3.6
Madhya Pradesh	₹ 3,48,986 crore	₹ 16,299 crore	4.6
Rajasthan	₹ 4,86,615 crore	₹ 22,064 crore	4.5
Maharashtra	₹ 6,00,522 crore	₹ 25,015 crore	4.1
Karnataka	₹ 3,27,747 crore	₹ 14,950 crore	4.5
Tamil Nadu	₹ 3,91,000 crore	₹ 20,198 crore	5.1
Kerala	₹ 1,66,501 crore	₹ 9,935 crore	5.9
West Bengal	₹ 3,04,689 crore	₹ 20,053 crore	6.5
Telangana	₹ 2,75,891 crore	₹ 11,500 crore	4.1

Delhi's higher allocation of 11.4% of its total budget to healthcare allows for better infrastructure and access to specialized treatments. By contrast, states like Uttar Pradesh allocate only 3.6%, limiting their capacity to establish and maintain CoEs and offer financial aid to patients under NPRD (Patel & Kumar, 2023).

- **Integrating Rare Disease Treatments with Ayushman Bharat:**

While Ayushman Bharat covers a range of medical procedures, it has yet to fully integrate rare disease treatments under its insurance schemes. Given the high cost of treating genetic disorders, incorporating these treatments under PM-JAY could relieve families of the financial burden associated with rare disease management. However, the complexity of treatment and the cost of

orphan drugs pose significant challenges to integrating rare disease care into Ayushman Bharat (Rao & Patel, 2022).

Parliamentary Engagement and Policy Advocacy:

- **Role of Parliamentarians in Advocating for Healthcare Reforms:**

Parliamentarians have played a pivotal role in shaping India's healthcare policies, particularly in advocating for the expansion of CoEs and increased budget allocations for rare diseases. MPs from states like Tamil Nadu, Delhi, and Maharashtra have raised concerns about the inequitable distribution of healthcare resources and the need for a national registry to track rare disease cases.

- **Outcomes of Parliamentary Engagement:**

Parliamentary debates have contributed to several positive outcomes, including increased awareness of rare diseases and expansions in healthcare budgets. The advocacy of MPs has led to discussions on the need for a centralized data repository for rare diseases, which could help policymakers allocate resources more effectively and track disease prevalence across the country (Sharma & Deshmukh, 2023).

Challenges and Recommendations:

Key Challenges in Policy Implementation:

- **Geographical Barriers:** CoEs are concentrated in major cities, leaving patients in rural areas with limited access to specialized care.
- **Lack of Data Infrastructure:** The absence of a national rare disease registry makes it difficult to track patient outcomes and allocate resources efficiently.
- **Funding Constraints:** Although financial aid is available, the high costs of orphan drugs and lifelong treatments create sustainability concerns for both NPRD and Ayushman Bharat.

Recommendations:

1. **Establish a National Rare Disease Registry:** A centralized data registry would enable policymakers to track the prevalence of rare diseases, monitor treatment outcomes, and allocate resources more effectively.

2. **Expand Ayushman Bharat Coverage:** Integrating rare disease treatments into PM-JAY would reduce out-of-pocket expenditures for patients and provide comprehensive financial protection for families affected by rare diseases.
3. **Strengthen Public-Private Partnerships:** Collaborating with private healthcare providers and pharmaceutical companies can help bridge funding gaps and improve access to advanced treatments.
4. **Develop Rural Outreach Programs:** CoEs should partner with HWCs to extend diagnostic and referral services to rural areas, ensuring that rare disease patients receive timely care.

Conclusion:

The National Policy for Rare Diseases (NPRD) 2021 and the Ayushman Bharat scheme represent a significant step forward in making healthcare more accessible to India's rare disease patients. By addressing financial burdens, establishing Centres of Excellence (CoEs), and involving stakeholders at various levels, these policies aim to provide comprehensive support to affected individuals. However, success hinges on overcoming several challenges, including the geographical concentration of CoEs, funding limitations, and the absence of a centralized rare disease registry. Through enhanced stakeholder collaboration, increased budget allocations, and the integration of rare disease treatments within Ayushman Bharat, India can create a more equitable healthcare system for vulnerable populations. Continued commitment to policy improvements, rural outreach, and data infrastructure development will be essential for ensuring the long-term impact of these initiatives, ultimately contributing to a more inclusive and resilient healthcare framework.

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