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Legal Analysis of Rare Disease Patients' Rights to Access Therapy in the BPJS Health System Based on Applicable Regulations

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ABSTRACT

This study discusses the legal analysis of patients' rights to access therapy for rare diseases within the Social Health Insurance Administration Body system based on applicable regulations in Indonesia. Jurisdictionally, the right of patients to obtain therapy for rare diseases is guaranteed by Article 28H paragraph (1) of the 1945 Constitution of the Republic of Indonesia, Law Number 17 of 2023, and Presidential Regulation Number 82 of 2018 in conjunction with Presidential Regulation Number 59 of 2024. The analysis highlights that while these guarantees are normatively regulated in a comprehensive manner, their implementation continues to face various challenges: a vacuum of specific norms, financing limitations, facility inequality, and inter-program coordination issues. Managing rare diseases requires regulatory reform, the strengthening of cross-sectoral integration, and the expansion of the Social Health Insurance Administration Body benefit list. These efforts are essential to accelerate the realization of the right to health for groups that have not yet been optimally accommodated. This research recommends the establishment of specific regulations, the harmonization of epidemiological data, and transparency in the health policy process.

INTRODUCTION

Rare diseases require a special approach in medical handling due to their complex characteristics and limited availability of therapy. In various countries, access to treatment for rare diseases often becomes a major concern for national health insurance systems. Patients with rare diseases often face layered obstacles, ranging from delayed diagnosis, very expensive therapy costs, to the limitation of drugs available in the market. This increasingly adds to the psychosocial burden on patients and their families, especially in developing countries (Ibrahim, 2023). This vulnerability reinforces that the health condition of the population is heavily influenced by social determinants and environmental characteristics that surround the daily life patterns of the community (Warin, 2023).

In Indonesia, the National Health Insurance (JKN) system managed by the Social Health Insurance Administration Body (BPJS) aims to ensure all citizens obtain fair and affordable healthcare. However, in practice, the protection provided by Social Health Insurance Administration Body to rare disease patients has not fully met their needs, especially in terms of access to adequate therapy. Often, treatment for rare diseases is expensive and not covered in the JKN benefit list, so patients must seek alternative financing that burdens the family's finances. This accessibility barrier has the potential to trigger public dissatisfaction because ideally, every regulation must provide strong legal protection for patients at all levels of healthcare facilities (Tampil et al., 2023).

Nevertheless, amidst the JKN's great ambition to embrace all levels of society, the reality in the field shows a paradox of health protection for groups with special needs such as rare disease patients. The complexity of the therapy access problem is not merely influenced by medical factors, but also by the overlap between health system capacity, administrative boundaries, and regulations that are not fully adaptive to the characteristics of rare cases. Therefore, the formulation and implementation process of national health insurance policy is inseparable from the dynamics of financing priorities and economic considerations that often clash with the demands for individual rights protection in healthcare

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services. In line with that, the absence of certainty in therapy access guarantees due to weak integration between the normative and operational frameworks within Social Health Insurance Administration Body creates gaps that make it difficult for patients to obtain optimal medical intervention. Furthermore, the absence of a comprehensive list regarding rare diseases and therapies covered, as well as the lack of transparency in benefit determination procedures, further worsens this uncertainty. Not only does this have an individual impact, but this phenomenon also indicates broader structural challenges in realizing fair treatment for all JKN participants. On the other hand, aspects of governance and coordination between institutions often clash, thus opening opportunities for the emergence of new legal problems that impact service access. This complexity is what gives rise to the need to review, more deeply, the position of the rights of rare disease patients in the realm of national health insurance law. Thus, a critical study of the juridical foundation and the practice of patient rights protection becomes crucial as part of the effort to understand the root causes of rare disease therapy access problems in Indonesia (Situmeang & Mile, 2024). This governance arrangement also includes aspects of health institution accountability in providing support services such as dignified palliative care for patients with chronic conditions (Wahyusetiawan et al., 2024).

Apart from administrative challenges, the legal issue of patient rights becomes important to explore. Patient rights have been guaranteed in various laws and regulations, one of which is Law Number 36 of 2009 concerning Health, which asserts the right to access healthcare services for every citizen. However, the reality in the field shows that the application of this guarantee is still far from ideal. The discrepancy between legal norms and reality is a major issue. At the international level, access to rare disease therapy has also become a concern through various agreements and conventions, such as the International Covenant on Economic, Social and Cultural Rights (ICESCR) which has also been ratified by Indonesia. The convention encourages countries to provide health access without discrimination, including towards groups with specific diseases. Even so, the uniqueness of rare diseases demands specific adjustments to regulations and policies, not just proportional ones like other common diseases. This condition gives birth to the need for deep legal analysis of patient rights to rare disease therapy within the Social Health Insurance Administration Body system. This study focuses on access inequality, regulatory implementation constraints, and the development of a legal framework that truly sides with patients with special needs. This normative inequality is in line with the need for legal firmness to prevent all forms of medical professional malpractice, such as the falsification of health certificates which violates codes of ethics and criminal law (Hartika et al., 2023).

The imbalance between regulations and real access to rare disease therapy causes unrest among patients and health law experts. Although there is a strong legal basis for patient protection, implementation in the field is still hampered by aspects of financing, therapy availability, and drug procurement mechanisms which are often not as fast as the patient's medical needs. A study by Anderson et al. (2019) shows that bureaucracy and resource limitations hinder efforts to guarantee rare disease therapy inclusively and fairly. The Social Health Insurance Administration Body system as the main organizer of national health insurance is not yet fully responsive to the dynamics of rare diseases that continue to develop. Administrative issues, rigid regulations, and the lack of rare disease epidemiological data become significant structural obstacles in providing complete healthcare services. Raharja et al. (2022) emphasize that changes in regulations and Social Health Insurance Administration Body governance are needed to respond to the needs of rare disease patients. Besides that, pharmaceutical policy reform and corporate compliance of drug companies in distributing generic supplies and managing patent rights become determinant factors for realizing equal drug access for patients (Hartono et al., 2024).

The lack of clarity regarding funding mechanisms and the setting of healthcare service priorities make access to rare disease therapy a problematic legal issue. Many patients experience layered obstacles, ranging from the difficulty of obtaining a timely diagnosis, limited expert medical personnel, to the lack of financing guarantees for specific therapies. This reinforces the need for evaluation of regulations and health insurance management to guarantee the realization of patient rights comprehensively. In the era of digitalization, this protection guarantee must also be strengthened with the reliability of information technology operations in hospitals to avoid system disruptions that can threaten patient safety and medical rights (Yatno et al., 2023).

The debate regarding the protection of the rights of patients with rare diseases is becoming increasingly crucial along with the increasing global attention to the issue of equality in healthcare services. In the era of medical science advancement and globalization, public demand for fair healthcare services is increasing. This momentum is both an opportunity and a challenge for Social Health Insurance Administration Body to adjust its system to fulfill the rights of all patients without discrimination. Furthermore, the advancement of therapy technology and the emergence of pharmaceutical innovations present new treatment options for rare diseases. If governance and legal systems are not adaptive, Indonesia has the potential to be left behind, so that patients with infectious diseases will continue to experience limited access. In-depth analysis is needed so that policy and regulation formulation can accelerate according to community needs. These regulatory adjustment efforts must ultimately lead to an improvement in the quality of life of the community inclusively through sustainable policies that place social and health equality as the main priority (Issalillah, 2021). The improvement of this insurance system is also influenced by psychological, sociocultural, demographic factors, as well as public perception towards the image of insurance institutions and the value of premiums offered in attracting public membership interest (Issalillah et al., 2021; Issalillah & Khayru, 2022).

Based on the previous description, the research problems addressed in this study are as follows: 1) How is the patient's right to access therapy for rare diseases regulated and implemented within the Social Health Insurance Administration Body system according to regulations currently in force in Indonesia? 2) What are the legal and implementation challenges in fulfilling the rights of patients with rare diseases regarding access to therapy under the Social Health Insurance Administration Body system?

This study aims to examine and analyze the regulation and implementation of patient rights regarding access to therapy for rare diseases within the Social Health Insurance Administration Body system based on applicable regulations in Indonesia, while providing recommendations based on empirical and legal findings to strengthen the protection of patients with rare diseases.

RESEARCH METHOD

This study employs a normative juridical method with a focus on systematic qualitative literature analysis. This study seeks to understand how the regulation of patients' rights to access therapy for rare diseases is analyzed through prevailing legislation, policy documents, and relevant scientific literature. Thematic synthesis is used as the primary approach to identify patterns, gaps, and dynamics in the implementation of health policy, as well as potential legal obstacles that arise in the fulfillment of the rights of patients with rare diseases under the Social Health Insurance Administration Body system. Each source is examined for validity and critically reviewed, ensuring that the research conclusions are based on authentic evidence supported by prevailing legal norms (Soekanto & Mamudji, 2011).

The literature search strategy is conducted using a structured search methodology across various major scientific databases, such as Scopus, PubMed, Google Scholar, and official Indonesian legislative repositories. Inclusion criteria include: publications with direct relevance to the topic of patient rights and rare disease therapy, as well as explicit linkages to Social Health Insurance Administration Body regulations. Exclusion criteria are applied to publications containing unverified references, opinion pieces without legal basis, or research outside the scope of Indonesian regulations. All identified documents are evaluated in terms of credibility, originality, and temporal relevance before being included in this study (Xiao & Watson, 2019).

Coding is performed manually for every key finding relevant to the selected publications. This process begins with the identification of core issues at the regulatory level and empirical obstacles detected in the field. Each finding is confirmed through cross-referencing between sources. Quality assurance in this research is carried out through internal validation (*peer debriefing*) by involving a team of academics specializing in health law, as well as source triangulation by confirming each citation against official links and academic journal or book databases. This effort is essential to minimize interpretation bias and ensure that all conclusions are scientifically accountable and legally valid.

RESULT AND DISCUSSION

Regulations on the Rights of Patients with Rare Diseases within the BPJS Health System

The fulfillment of the health rights of patients with rare diseases demands the clarity of binding legal principles. Normatively and juridically, the right of patients to obtain access to rare disease therapy in Indonesia is strongly rooted in the constitutional principle that every citizen must be guaranteed the right to receive decent healthcare (Muis et al., 2022).

Specifically, the right to health services and the right to personal autonomy are aspects of human rights in healthcare, meaning that everyone has the right to healthcare and the right to make decisions regarding their health independently (Jatemin & Irawati, 2021). This foundation is explicitly reflected in Law Number 17 of 2023 concerning Health. In this law, the state does not merely assert that healthcare is a citizen's human right, but also specifies the fulfillment of services according to medical needs and professional standards. Patients have the right to receive medical information, undergo health procedures in accordance with professional procedures, and obtain legal protection. These rights are inherent in every stage of service, starting from prevention and diagnosis to the treatment of diseases, including rare diseases. At the implementation level, the state's obligation to ensure the fulfillment of these rights has consequences for the provision of transparent, accountable, and effectively monitored services. Legal instruments such as implementing regulations, standard operating procedures, and technical policies must be designed to provide comprehensive protection in healthcare practice. Furthermore, the formulation of health policies based on epidemiological data and input from professional associations is a strategic element so that every health intervention is truly relevant to the dynamics of needs in the field. The state is obligated to ensure that medical information provided to patients is openly accessible in language that is easily understood by patients and their families to uphold the principle of *informed consent* (Tjandrawinata & Heliany, 2024).

Firm regulation regarding the roles and responsibilities of healthcare workers is increasingly necessary to avoid bias in interpretation during the handling of rare disease cases. Strengthening preventive efforts, early screening, and structured national referrals will reinforce the quality of legal protection for patients throughout Indonesia. The harmonization of regulations between central and regional governments, as well as hospitals, is important to reduce service access disparities and increase the affordability of innovative therapies. Furthermore, periodic supervision of the implementation of rare disease service policies will increase the compliance of all stakeholders with the principles of justice and respect for patient rights. These efforts essentially represent the state's commitment to realizing health as a substantial and tangible universal right for all levels of society, including patients with rare diseases. The strengthening of regulations and supervision is the determinant for achieving equal health coverage for all patients, including those facing rare diseases. This renewal of the governance legal framework is crucial in responding to the increasingly complex challenges of national health development (Harianto et al., 2024).

The strengthening of the national health insurance scheme shows the state's commitment to guaranteeing service access for rare disease patients. The provision of healthcare for rare diseases is increasingly recognized as a state mandate through the national health insurance system. Presidential Regulation Number 82 of 2018 concerning Health Insurance, which has been updated several times and most recently refined through Presidential Regulation Number 59 of 2024, affirms the obligation of universal membership in Social Health Insurance Administration Body. This is Indonesia's effort to achieve *universal health coverage* (UHC) recommended by the World Health Organization (WHO) to ensure that all citizens receive the health services they need without facing financial burdens (Wasir et al., 2019). This regulation explicitly establishes that the scope of health service benefits must be adjusted to the medical indications experienced by participants, covering diagnostic, curative, rehabilitative, and palliative procedures, including for rare diseases. In the regulation, the state places the principle of equity as an operational foundation, ensuring no discrimination occurs based on health conditions, economic status, or disease rarity. This normative protection guarantee becomes an important part of affirming patient rights in the conceptualization of the national health insurance system (Tamaka et al., 2023).

The determination of financing schemes and guarantee procedures is set out in detail in the articles governing the implementation of social health insurance, making it mandatory for all related parties to comply, from service providers to regulators. To achieve success in the health insurance program, Social Health Insurance Administration Body needs to ensure the availability of healthcare workers, facilities, and infrastructure with adequate quality (Wahyuni et al., 2023). The regulation also requires the updating of benefit lists and medical interventions based on population need data and clinical audit results, which serve as references in determining financing ceilings. Legal protection for beneficiaries is supported by provisions regarding complaint mechanisms, remediation, and the guarantee of fair treatment regarding medical decisions. The affirmation of administrative sanctions up to the revocation of operational permits serves as an instrument to ensure that the implementation of regulations remains consistent and accountable. These provisions also convey the necessity for Social Health Insurance Administration Body to conduct regular public education so that patients and their families understand their rights, access procedures, and service evaluation mechanisms related to rare diseases. The state is continuously obligated to review and harmonize policies to ensure that all regulatory content is relevant to the development of advanced medical science and community needs. Adjustments to provisions in implementing rules are needed to accelerate the integration of innovative therapies into the National Health Insurance system. The obligation for periodic supervision by authorized authorities serves as a guarantee that all healthcare facilities comply with the standards stipulated in the regulations. All these arrangements explicitly show that equal treatment for rare disease patients has been accommodated systemically, providing legal certainty and strengthening the main goal of health insurance for all citizens. All these provisions confirm that the national health insurance system is designed to ensure equal and certain protection for rare disease patients throughout Indonesia. The presence of this legal certainty is also crucial for minimizing and strictly prosecuting all forms of potential fraud or forgery in the health insurance ecosystem (Setiawan et al., 2023).

Cost considerations are an important factor affecting the expansion of service coverage for rare disease patients. However, the principle of financial sustainability is one of the main considerations in Social Health Insurance Administration Body benefit coverage. The benefit list covered by Social Health Insurance Administration Body is compiled based on medical effectiveness and the state's financial capacity. In the case of rare disease therapy, the constraints of availability and high therapy costs mean that not all innovative therapies can be immediately included in the benefit catalog. Therefore, the provision of therapy often has to go through additional mechanisms, such as special guarantees or cross-sectoral collaboration between Social Health Insurance Administration Body, the Ministry of Health, and other government entities. The arrangement of additional guarantee mechanisms shows that access to rare disease therapy requires a financing strategy that is more adaptive and measurable. Strict evaluation of this budget governance is needed to identify root causes and map preventive steps for financing dispute risks and health fund misuse issues (Firmansyah et al., 2022).

Increased access to rare disease therapy depends on the effectiveness of detection efforts from the earliest stages of life. The state's effort in guaranteeing access to rare disease therapy is not limited to financing but is also directed at early detection through health screening programs. Since the newborn stage, various programs have been carried out by the Ministry of Health to diagnose congenital diseases and rare conditions. It is hoped that with early intervention, rare disease management can run effectively and efficiently, thereby creating opportunities for broader therapy access, although the financing system still requires strengthening and development. This approach shows that the success of rare disease handling is heavily determined by early intervention that is structured and sustainable. This commitment to providing interactive handling also includes the accountability of health facilities in optimizing palliative care for terminal-phase patients (Wahyusetiawan et al., 2024).

The principle of equality is an important foundation for all health service policies. One of the main principles in all these arrangements is non-discrimination. The prevailing legal norms ensure that patients, regardless of the type of disease, have the right to access health services without distinction. This makes every health service regulation, including Social Health Insurance Administration Body, mandatory to be oriented toward the fulfillment of human rights as guaranteed by the constitution and health laws. This principle asserts that every patient must obtain equal health services in accordance with human rights guarantees. This equal treatment and fulfillment of human rights must also be felt inclusively by all vulnerable groups, including persons with disabilities (Subiakso et al., 2023).

The reality of health services shows that access to rare disease therapy is not yet fully berjalan without obstacles. Even so, implementation in the field still faces challenges. Rare disease patients often encounter administrative obstacles or limitations on the types of therapy covered. This occurs because not all rare disease therapies or drugs have sufficient validation of effectiveness, or because of very high prices that cannot yet be accommodated in the national insurance system. Consequently, the state seeks to balance the responsibility of health protection and the financing capacity of the national health sector. This situation confirms the need for policy strategies capable of maintaining a balance between the medical needs of patients and the state's financing capacity. Accessibility barriers caused by sociocultural factors as well as limitations in urban areas also influence health determinant gaps in the community (Warin, 2023).

The regulation of rare disease services also emphasizes the importance of precision in the therapy guarantee process. In addition to financing aspects, claim submission procedures and the validation of innovative therapies for rare diseases are also strictly regulated. Medical and legal evaluation processes are conducted to ensure that the therapy provided can be professionally accounted for and does not burden the long-term financial system. Therefore, the inclusion of new drugs and special rare disease therapies into Social Health Insurance Administration Body benefits takes place gradually, based on the results of special studies by experts. This gradual approach ensures that every therapy accommodated by Social Health Insurance Administration Body meets professional standards while maintaining the sustainability of the health insurance system. This principle of operational caution requires the reliability of hospital digital systems, because disturbances or disruptions in information systems can cause serious legal impacts on patient services (Yatno et al., 2023).

Improving health service governance is an important step to meet the needs of rare disease patients. The Social Health Insurance Administration Body system continues to adapt in responding to the needs of rare disease patients. Data integration and the utilization of health technology are among the efforts to improve recording, monitoring, and service evaluation systems, so that rare disease epidemiological data can serve as a basis for future policies. With system improvements, it is hoped that the coverage of therapy and services for rare diseases will become wider. These efforts show that the expansion of therapy access is heavily dependent on systems capable of providing accurate data and supporting effective decision-making. A responsive service system at the primary level such as community health centers plays a major role in ensuring maximum legal protection and patient safety (Tampil et al., 2023).

Collaboration between various stakeholders is an important element in expanding support for rare disease management. Cross-sectoral cooperation is also very crucial in providing additional financing for rare disease management. The government partners with various sectors, such as the pharmaceutical industry, professional associations, and international organizations to provide therapy access and education more comprehensively. This is an effort so that the patient's legal right to therapy access does not stop at the normative aspect but is truly realized in healthcare practice. This collaborative approach ensures that the fulfillment of therapy rights for rare disease patients can be realized tangibly in health services. The quality of this cooperation contributes linearly to the public satisfaction index regarding the quality of services provided by health agencies (Darmawan et al., 2022).

The responsiveness of health policy is an important prerequisite for the fulfillment of the rights of rare disease patients. Public policy regulations that always prioritize social justice serve as a foundation that the Social Health Insurance Administration Body system must be responsive to the dynamics of public health needs. Patient rights to access rare disease therapy should not be ignored simply because the number of sufferers is a minority or because the handling costs are very high. Thus, the state continues to be urged to strengthen policies and financing mechanisms to adjust to the development of medical science and the demands of patient rights. This point confirms the need for policy strengthening so that therapy access remains guaranteed despite the limitations of cost challenges and the number of patients.

Information transparency is an important element in ensuring health policy can be understood by all related parties. The government also ensures that policy communication runs transparently and accountably. Every change or update to the benefit catalog, especially for rare disease therapy, is announced openly and involves socialization processes so that all stakeholders, especially patients and their families, obtain clarity on rights and the financing mechanisms for specific therapies needed. This approach asserts that clarity in policy communication is a primary requirement for the effective fulfillment of rare disease patient rights.

The strengthening of national health policy reflects the state’s commitment to the protection of patients with rare diseases. The national commitment to expanding access to rare disease therapy through the Social Health Insurance Administration Body indicates a policy direction aligned with constitutional principles, namely social justice and the equalization of rights in the health sector. The realization of these efforts affirms the state's presence in providing protection for even the most vulnerable groups in society, particularly individuals facing the most severe obstacles in obtaining medical services. The process of integrating rare disease benefits into the National

Health Insurance (JKN) scheme marks a shift from a health service paradigm based on majority interests toward the recognition of the specific needs of small yet highly affected groups. Furthermore, the presence of progressive regulations indicates the strengthening of legal legitimacy for patients' rights to innovative medical interventions, even as the harmonization of regulations and their implementation remains ongoing. The continued improvement of the financing system, therapy validation, and the updating of benefit list determination mechanisms demonstrate the government's seriousness in addressing the issue of rare diseases as an integral part of national health development. The realization of this commitment also simultaneously encourages the creation of cross-sectoral coordination so that policy formulation remains responsive to the dynamics of societal needs. The state's central role in ensuring service affordability and quality is increasingly visible through cross-ministerial involvement, the strengthening of health facility capacity, and the optimization of human resources. It is also important to emphasize that the roadmap for policy implementation is always accompanied by updates based on epidemiological data and consultations with patient groups. Adjustments to the benefits catalog and operational procedures are essentially directed so that social protection mechanisms effectively reach rare disease patients throughout all regions of Indonesia. These efforts send a positive signal to the public that equal treatment in the health sector is not merely a normative rhetoric, but a principle realized through concrete and measurable actions in state policy. These overall steps confirm that the principle of social justice is tangibly realized through the health insurance system.

The Normative Foundation and Jurisprudential Framework for Rare Disease Patients' Rights

The fulfillment of health rights for patients with rare diseases demands a strong and consistent legal foundation. Juridically, the fulfillment of rare disease patients' rights within the Social Health Insurance Administration Body system is rooted in constitutional principles concerning human rights (Khalid et al., 2023). Article 28H paragraph (1) of the 1945 Constitution of the Republic of Indonesia affirms the state's obligation to ensure decent healthcare for all citizens without exception. This constitutional mandate is further clarified at the statutory level through Law Number 17 of 2023 concerning Health, which asserts that every patient, including those suffering from rare diseases, has the right to access healthcare in accordance with medical needs and scientific developments. These rights include access to medication, medical devices, and adequate medical services.

In terms of regulation, the state's commitment is manifested through the National Health Insurance (JKN) scheme, which seeks to systematically integrate financing and service availability. This mechanism includes ensuring continuity of access, efforts to equalize the distribution of facilities, and support for therapeutic innovations that adapt to the progress of medical science. Although healthcare services in Indonesia are managed decentrally meaning that some findings regarding patient risks may not be fully representative of all regions the centralized nature of the national insurance system means its impacts tend to be uniform across the country (Hasnida et al., 2021). Progressive legislative efforts provide a strong legal basis for drafting health policies that are responsive to the needs of vulnerable groups, especially those facing limited access due to the rarity of their diseases and the high cost of therapy (Ariyanto et al., 2023). These provisions are also binding on all stakeholders to ensure the consistent application of justice principles in healthcare throughout Indonesia. Inter-agency policy harmonization is essential to ensure that the availability of essential services and medicines is accessible to the broader community. The establishment of minimum service standards for rare disease patients in government regulations serves as a crucial instrument in avoiding service inequality. The formulation of regulations based on actual needs and scientific studies ensures that health policies are not merely formalistic, but substantial and impactful for beneficiaries. Furthermore, strengthening the monitoring and evaluation systems for health insurance implementation will ensure that patient rights are continuously and sustainably protected. The synergy of all actors in the health sector including the government, service facilities, and patient communities is a decisive factor in the successful implementation of justice principles in protecting rare disease patients. This entire framework demonstrates that the protection of rare disease patients' rights can only be realized through regulations that are aligned, measurable, and implemented sustainably. This normative framework aligns with the importance of conducting a comprehensive juridical review of the status of patient rights within the national social health insurance system (Tamaka et al., 2023).

Strengthening the mandate of Social Health Insurance Administration Body indicates an increasingly inclusive policy direction for all participants. Within the social security system framework, Presidential Regulation Number 82 of 2018 concerning Health Insurance, alongside its latest amendment through Presidential Regulation Number 59 of 2024, expands the mandate of Social Health Insurance Administration Body as the executor of comprehensive health service guarantees. These regulations emphasize the principle of non-discrimination, ensuring that all Social Health Insurance Administration Body participants including rare disease patients are entitled to services tailored to their medical indications (Fajri & Sarwo, 2022). This affirmation of non-discrimination ensures that rare disease patients receive services according to their medical needs on an equal basis. This principle of inclusivity also demands equal protection for other marginalized groups, such as the juridical rights of poor or underprivileged patients, ensuring they are not ignored within the service system (Noor et al., 2023).

The absence of detailed regulations regarding rare diseases creates fundamental issues in the implementation of health insurance. At the implementation level, there are several structural and substantive legal challenges. One primary challenge is the lack of specific and firm regulations governing the list of rare diseases and the scope of therapy covered by Social Health Insurance Administration Body. This lack of clarity in definitions and coverage creates uncertainty for patients, families, and healthcare workers. The determination of therapy or medication for rare diseases often depends heavily on internal administrative decisions, the proposal process from the benefits panel, and the availability of budgets. Consequently, patient families frequently face lengthy administrative processes or even rejections because the therapy is not yet listed in the benefits catalog. This situation underscores the need for regulatory clarity so that therapy access for rare disease patients is not hindered by administrative uncertainty. Such regulatory gaps and administrative governance uncertainties are prone to triggering legal irregularities and risks of fraud at the hospital operational level (Jamiri et al., 2023).

Cost is a determining factor heavily influencing the availability of therapy for rare disease patients. In terms of financing, rare disease therapy is synonymous with exorbitant costs, as it often involves drugs that are rarely available in the market and frequently must be imported. The Social Health Insurance Administration Body system limits financing to services listed in the national e-catalog benefits. Innovative drugs or therapies that are not yet in the catalog, even if medically proven to be essential, cannot be fully guaranteed by the state. Financing mechanisms outside the catalog typically require cross-ministerial collaboration, special requests, or separate financial assistance from Social Health Insurance Administration Body. This creates a dilemma between the principle of the right to health and the limited financial support capacity of the national social security system. This condition highlights the need for more flexible financing strategies to ensure that patients' health rights remain protected despite the extremely high therapy costs. If not anticipated through robust regulations, this uncertainty in funding schemes could lead to criminal legal disputes due to failures in settling health insurance claims (Ambarwati et al., 2024).

The gap in healthcare service capacity is a factor that significantly influences the effectiveness of handling rare diseases in Indonesia. Service implementation is hampered by limited facilities, human resources, and inter-regional disparities. High demand for health services combined with limited health facilities leads to increased congestion in healthcare services (Handayani et al., 2021). Currently, rare disease therapy services are almost entirely concentrated in central referral hospitals or primary health facilities in large cities. Patients in remote areas face access gaps in terms of diagnosis, referrals, and therapy implementation that cause inequality in the fulfillment of health rights. In practice, obtaining access to services and medicines for rare diseases requires high-level advocacy and administrative support. This reinforces the need for strengthening infrastructure and service governance so that access for rare disease patients is no longer dependent on location or administrative advocacy capabilities. The location of facilities and the equitable quality of health service infrastructure are proven to be key variables determining the level of patient satisfaction and safety (Mardikaningsih, 2022).

Policy integration is a key factor in ensuring service continuity for rare disease patients. Coordination between regulations and government agencies remains a distinct challenge. Although rare disease screening programs have been implemented early on, the integration between screening results and the financing program within Social Health Insurance Administration Body remains insufficiently solid. The lack of comprehensive coordination results in legal and administrative gaps, meaning that follow-up processes after diagnosis often do not run smoothly. Patients identified with rare diseases from an early age are not automatically enrolled in continuous therapy guarantee programs. This condition demonstrates the need for stronger regulatory and administrative integration so that patients diagnosed early can obtain sustainable therapy guarantees. This weak operational integration risks creating legal loopholes that may be exploited for document forgery or insurance claims to cover costs (Setiawan et al., 2023).

The discrepancy between legal norms and actual healthcare practices is a crucial issue in fulfilling the rights of rare disease patients. Normatively, current law places the principle of non-discrimination as a foundation every patient, regardless of their disease type, must receive equal treatment and service within the national health insurance framework. However, in practice, there is a gap between norms and facts: rare disease patients often face various administrative, financial, and technical medical limitations. Frequently, patient families must seek their own solutions to obtain necessary therapy, whether by writing special request letters, filing for *judicial review*, or utilizing *crowdfunding*. This image confirms the need for regulatory and administrative improvements so that the fulfillment of health rights no longer depends on the individual efforts of patients or their families. This implementation disparity demands strict supervision, including the regulation of medical advertising so that it remains unbiased and does not disadvantage health consumers (Sahidu et al., 2023).

The health insurance guarantee framework is frequently tested when responding to highly specific therapy needs. Provisions within Social Health Insurance Administration Body, which are flexible in principle, remain too general in practice when facing the dynamics of rare diseases that require quick and *tailor-made* decisions. Policy changes and the expansion of benefit lists generally require long periods, extensive inter-agency consultation, and in-depth studies on medical effectiveness and financial sustainability. Benefit determination requires a more agile mechanism to ensure clinical needs are met on time. In addition to financial aspects, this administrative guarantee constraint has a linear negative impact on the satisfaction level of Social Health Insurance Administration Body participants regarding the quality of service they receive (Issalillah et al., 2021).

The availability of adequate information is an important prerequisite for formulating targeted health policies. Similarly, regulatory challenges arise due to the limited epidemiological data on rare diseases in Indonesia. The lack of accurate data makes it difficult for policymakers to formulate priorities, budget allocations, and health insurance program designs that are inclusive of rare diseases. Inconsistent or invalid data hinders evidence-based policy responses. Improving data quality is a key step so that policies can be designed more accurately and effectively. In a modern context, leveraging the *big data* ecosystem and predictive analysis is crucial to prevent discriminatory actions while strengthening protection for insurance customers (Bashori et al., 2024).

Biomedical advancements demand that the health insurance system be able to adapt continuously. Handling rare diseases requires regulatory adaptation that is progressive and responsive to the latest medical science and therapeutic technology developments. Without continuous regulatory updates, there is a concern that the Social Health Insurance Administration Body system will become rigid and lag in responding to patient needs in the current era of biotechnology. The success of services for rare disease patients is highly dependent on the ability of regulations to follow modern therapy developments. This responsiveness is important, considering that optimizing the quality of basic and referral healthcare services comprehensively is a major pillar of patient comfort (Khayru & Issalillah, 2022).

Efforts to fulfill the rights of rare disease patients require collective support from various stakeholders. The success of protecting rare disease patients' rights also depends on cross-sectoral collaboration, involving the central government, regional governments, legislative bodies, professional associations, and patient communities. This cooperation is necessary to advocate for change and benefit inclusion, evidence-based data compilation, and better health policy advocacy in the future. Inter-sectoral synergy is an important foundation for achieving more inclusive and sustainable health policies. This commitment to strategic partnership will ultimately lead to increased legal protection that is fair for all levels of society without discrimination, including for persons with disabilities (Subiakso et al., 2023). This comprehensive handling contributes significantly to positive public evaluation regarding the performance of health service institutions (Darmawan et al., 2022), while simultaneously minimizing legal disputes over financing management cases (*fraud*) that often harm the national social security system (Firmansyah et al., 2022).

Various structural obstacles indicate that the fulfillment of the rights of patients with rare diseases still faces fundamental issues. Consequently, the primary legal challenges in fulfilling the rights of patients with rare diseases are the vacuum of specific norms, limitations in financing coverage, and implementation gaps in the field. The dynamics of regulatory formulation show that the absence of technical regulations explicitly governing treatment standards and lists of therapies for rare diseases contributes to ambiguity in the healthcare service claims process for patients. The limitations of state funding capacity in the health sector, especially in facing the needs of high-cost therapies, further increase the risk of access inequality among participants of the National Health Insurance (JKN). The gap between regulation and application at health facilities often hinders the equitable distribution of benefits, forcing many patient families to navigate lengthy administrative mechanisms to obtain basic rights. Moreover, the fragmentation of responsibilities among government authorities leads to suboptimal coordination in ensuring the continuity of treatment for rare diseases. Delays in updating the benefits catalog and the lack of a service monitoring system also add to bureaucratic burdens. Implementation based on empirical evidence is highly necessary so that policy formulation is more targeted and adaptive to the evolving medical needs of rare disease patients. The consistency of implementing the non-discrimination principle must be maintained throughout the entire service chain, from the screening stage to rehabilitation. Accountability in the decision-making process regarding the determination of financing schemes will strengthen the program's legitimacy in the eyes of the wider community. By paying attention to all these aspects, the commitment to fulfilling the right to health can be achieved fairly and sustainably. The effectiveness of health protection is heavily dependent on the clarity of regulations and the precision of their implementation.

CONCLUSION

The fulfillment of the rights of patients with rare diseases within the Social Health Insurance Administration Body system is explicitly regulated within the framework of prevailing legislation in Indonesia. The Constitution, health laws, and Social Health Insurance Administration Body regulations position the right to healthcare as a human right guaranteed by the state. Nevertheless, the realization of access to therapy for rare diseases has not yet been fully effective due to the vacuum of specific norms, financing limitations, and uneven implementation. Resolving these obstacles requires the strengthening of regulations and cross-sectoral program integration for the sake of justice and the welfare of patients with rare diseases.

This analysis indicates the necessity for the renewal and strengthening of regulations that specifically govern rare diseases, as well as the expansion of Social Health Insurance Administration Body benefit coverage. These efforts will bolster legal protection and reinforce referral and financing systems, ensuring that patients' rights are truly guaranteed. The active involvement of all stakeholders is required to synergize various programs, ensure non-discriminatory services, and improve the quality of life for patients with rare diseases.

There is an urgent need for the government to immediately draft technical rules that define rare diseases and clarify the scope of therapy under Social Health Insurance Administration Body coverage. Policy communication must be conducted transparently with the involvement of professional associations and patient communities. The harmonization of epidemiological data and the strengthening of cross-sectoral program integration are also highly urgent to support the effectiveness of program implementation and the realization of patient rights in accordance with the principle of non-discrimination.

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