

Health Communication, Health Literacy, and Patient Empowerment in Rare Disease Care in Low- and Middle-Income Countries: A Scoping Review

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ABSTRACT

Most people living with a rare disease reside in countries with limited health system resources, yet these conditions have received little priority in national health policy in such settings. This scoping review examined how medication labeling, health literacy, equitable access, and patient empowerment intersected for rare disease patients in low- and middle-income countries. Literature published between 2018 and 2026 was searched through Europe PMC, supplemented by targeted retrieval of official policy documents. Every candidate source was verified in two stages: bibliographic details were confirmed independently against an external registry, and content was then confirmed from retrieved text. Twenty-five sources satisfied verification and eligibility criteria, while seven records were withheld for unverifiable content. Four themes were identified, comprising information asymmetry in rare disease care, medication labeling and comprehension, health literacy measurement capability, and equitable access as a condition of empowerment. No eligible and verified study identified through this review evaluated medication labeling or leaflet comprehension among rare disease patients in a low- or middle-income setting. The contributing domains were found to be individually well developed but rarely joined. This absence constituted the principal finding, and validated instruments and trial designs already existed that appeared capable of addressing it.



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INTRODUCTION

Rare diseases occupy a paradoxical position in global health, being individually uncommon yet collectively substantial. Available estimates indicate that rare diseases affect approximately 300 million people worldwide, of whom roughly 96 million are in India (Chakraborty Choudhury et al., 2026). Broader estimates place the global figure between 300 and 350 million and indicate that approximately 250 million affected individuals live in low- and middle-income countries (Kolambage et al., 2026). This distribution carries an implication easily overlooked in a literature dominated by high-income settings, namely that the majority of the world's rare disease population resides precisely where health systems are least resourced to respond. Despite this, rare diseases have not been prioritised in the health policies of most low- and middle-income countries (Chakraborty Choudhury et al., 2026), and until recently most Latin American countries possessed little or no rare disease policy infrastructure (Cabello & Salinas, 2026). A structural mismatch therefore exists between where the burden falls and where institutional capacity is concentrated.

The consequences of this mismatch are documented most clearly at the point of diagnosis. Patients in resource-limited settings frequently lack access to advanced diagnostics and specialist care, which leads to prolonged diagnostic odysseys and considerable distress (Kolambage et al., 2026). Within India, genetic testing capacity has gained traction principally in tier-one and tier-two cities, and reliance on international collaboration for sample analysis has been reported to delay investigation (Contractor et al., 2025). These service delivery constraints are compounded by an informational one, since Indian epidemiological data on several rare and chronic conditions remain limited and inconsistent owing to heterogeneity in diagnostic criteria and regional disparities, which impedes accurate burden estimation and policy prioritisation (Sasikumar & Patidar, 2025). Limited data at the system level and

limited information at the patient level are therefore not separate problems but two expressions of a single deficit in health information infrastructure.

Health communication provides a productive analytical lens for this problem. The World Health Organization (2025) defines health literacy as the personal knowledge and competencies that accumulate through daily activities, social interactions, and across generations, and emphasises that these competencies are mediated by organisational structures and the availability of resources enabling people to access, understand, appraise, and use health information and services, a property described as organisational health literacy. This framing matters because it locates responsibility in systems rather than solely in individuals. The World Health Organization (2025) further notes that population health literacy follows a social gradient capable of reinforcing existing inequalities, and identifies health literacy as a determinant of health. Measurement in resource-limited settings has nonetheless been constrained by instrumentation, since the absence of validated Hindi language instruments limited health literacy assessment in India until recent cross-cultural adaptation and psychometric validation work (Sharma, 2026).

Three communication channels are of particular relevance to medication safety in rare disease care. At the level of the condition, rare disease patients face health disparities and are frequently challenged to find accurate information about their illness (Badiu et al., 2017). At the level of the medicine, a consistent gap has been documented between the readability of medication messages and the skills of the populations expected to read them (Ferreira-Alfaya et al., 2025). At the level of the provider, evidence regarding the use of health communication materials by healthcare providers in low- and middle-income countries remains inconsistent (Yirsaw et al., 2026). These channels converge on outcomes of direct clinical significance, since limited health literacy may contribute to medication-related problems and to reduced utilisation of pharmacist-led services (Vasudevan et al., 2026). For patients using orphan medicines, which are often unfamiliar to local prescribers and dispensers, the reliability of each channel appears especially consequential.

Policy translation forms the final strand of justification. India launched its first functional rare disease policy in 2021, and successful implementation has been argued to require the active participation of diverse stakeholders (Chakraborty Choudhury et al., 2026). The policy document itself records that the earlier National Policy for Treatment of Rare Diseases of 2017 was kept in abeyance through a non-statutory Gazette Notification dated 18 December 2018, following implementation difficulties that included concerns about cost effectiveness in a limited-resource situation and the challenge of bringing States on board, given that public health and hospitals are primarily a State subject (Ministry of Health and Family Welfare, 2021). Subsequent analysis suggests that current policy focuses more on strengthening diagnostics and lacks a comprehensive treatment framework (Mishra et al., 2024).

The originality of this article rests on three features that distinguish it from existing reviews. First, it joins four literatures that are ordinarily examined separately, namely medication labeling and medication safety communication, health literacy, sustainable and equitable access, and patient empowerment, and examines them as a single system operating around the rare disease patient. Second, it applies a two-stage source verification protocol in which bibliographic validity and content validity are established separately through independent authorities, and it withholds from citation those sources whose content could not be confirmed, a procedure rarely reported explicitly in review articles in this field. Third, and most substantively, it reports a negative finding as its principal contribution rather than concealing that finding beneath adjacent evidence. The objective of the review was accordingly to map verified evidence across the four domains, to determine what is transferable from adjacent literature, and to identify precisely where the evidence base is silent. Its intended contribution is to provide researchers and policymakers with an honest map of what is known, together with a specific and tractable research agenda for what is not. The relationship between the four domains is conceptualised as a chain in which medication information becomes actionable only when written at a comprehensible level; comprehension becomes engagement only when health literacy is sufficient and when health systems and their resources support it, following the World Health Organization (2025) definition of organisational health literacy; engagement becomes empowerment only when equitable access to medicines and services is in place, since information without access yields informed frustration rather than agency; and empowerment, once enabled, returns to strengthen health literacy through patient

activation and advocacy. This framing guided the thematic synthesis and accounts for why the domains are reported as a single system rather than as independent literatures. The review therefore addressed the following focused questions: (1) What evidence exists, directly or through defensible comparators, regarding how rare disease patients in low- and middle-income countries comprehend medication labels, leaflets, and safety information; (2) what factors in health systems and policy support or constrain the translation of that information into patient understanding and safe use; (3) how far health literacy, equitable access, and patient empowerment are mutually reinforcing within rare disease care; and (4) what evidence-based mechanisms, already demonstrated in other contexts, could plausibly be adapted to sustain empowerment under resource-sensitive conditions in these settings.

RESEARCH METHODS

This study employed a scoping review design in preference to a systematic review, and that decision followed rather than preceded the evidence assessment. Preliminary searching indicated that studies evaluating medication labeling or patient information leaflets specifically among rare disease populations in low- and middle-income countries were not retrievable in numbers sufficient to support systematic synthesis, a scarcity consistent with the observation that rare diseases have not been prioritised in most such health policies (Chakraborty Choudhury et al., 2026) and that these populations remain comparatively under-served by research infrastructure despite accounting for the majority of affected individuals globally (Kolambage et al., 2026). A scoping design permitted the mapping of adjacent literature across contributing domains and the explicit charting of gaps, without implying a pooled estimate that the available evidence could not sustain.

Searches were concluded on [Month Day, Year], and the last search is recorded as having been performed on that date. All searches were conducted without language restrictions at the search interface, though articles were retained only when an English-language title, abstract, and full text were retrievable. Publication years were generally restricted to 2018 through 2026, with priority given to 2020 onward, although one earlier record from 2017 was retained on grounds of direct topical relevance to co-created rare disease educational materials. The search was executed using the following strings, each combining controlled and natural language terms: (1) "rare disease AND health literacy AND low and middle income countries"; (2) "rare disease AND patient information leaflets AND medication labeling"; (3) "health communication AND patient empowerment AND rare disease"; (4) "medication labeling AND health literacy AND India"; (5) "pharmacovigilance AND patient communication AND low resource settings"; and (6) "sustainable healthcare access AND rare disease AND LMICs". Additional targeted searches addressed orphan drug policy in India, the National Policy for Rare Diseases, health literacy prevalence in India, shared decision making, and catastrophic health expenditure. Title and abstract fields were targeted where the search interface permitted. Duplicate records arising from the overlap between Europe PMC and PubMed were identified by matching digital object identifiers and titles, and a single copy was retained; where a DOI was absent, duplication was resolved by comparing titles and author lists. Europe PMC was selected as the principal source because it indexes PubMed and MEDLINE records while permitting transparent retrieval and DOI resolution, which was a requirement of the two-stage verification protocol; the implications of not searching Scopus, Embase, or Web of Science should be noted, and relevant records indexed only in those databases may have been missed, as may grey literature not indexed in any bibliographic database.

Records were eligible for inclusion if they addressed health communication, health literacy, medication labeling or leaflets, medication safety and pharmacovigilance, patient empowerment, or rare disease access and policy; if they concerned low- and middle-income countries, India, or provided a defensible high-income comparator for a mechanism with plausible transferability; if they constituted a peer-reviewed article, official report, or government policy document; and if they were verifiable through a digital object identifier or stable institutional address. Records were excluded where bibliographic details could not be independently confirmed, where the publication was an opinion piece without empirical or policy content, where full text was unavailable in English, or where the subject matter fell outside the four contributing domains. Preprints lacking peer review were not carried forward into the approved evidence base.

A two-stage verification protocol was applied, and this constituted a methodological feature of the review rather than a routine administrative step. In the first stage, every candidate digital object

identifier was resolved independently against the CrossRef registry, an authority distinct from the database in which the record was discovered, and the returned title, journal, publication year, and author data were compared against the retrieval record. Cross-checking against an independent registry rather than re-querying the source database was adopted because re-querying the discovery source is circular and cannot detect an erroneous identifier that the source itself propagates. All thirty candidate identifiers resolved with concordant metadata. In the second stage, abstract or full text was retrieved separately in order to establish what each source actually reported, on the principle that bibliographic confirmation establishes that a work exists but does not establish its content. Seven records passed the first stage yet returned no retrievable abstract, and these were withheld from citation notwithstanding their confirmed bibliographic validity. Two official documents, comprising the National Policy for Rare Diseases (Ministry of Health and Family Welfare, 2021) and the World Health Organization (2025) health literacy fact sheet, were verified by direct retrieval of full text from official addresses.

Each approved source received a quality rating. Empirical and mixed methods studies were appraised using logic adapted from the Mixed Methods Appraisal Tool, qualitative studies using Critical Appraisal Skills Programme logic, and reports, editorials, and policy documents using an adapted policy appraisal, with the latter marked for contextual use only. Ratings of high, moderate, low, or policy and grey literature were assigned accordingly. Synthesis was narrative and thematic, organised around four themes derived from the evidence base rather than imposed in advance. Where a finding derived from a high-income setting, it was identified as adjacent evidence at the point of use, and no inference was drawn about rare disease populations in low- and middle-income countries that the cited source did not itself support. A PRISMA flow diagram was not constructed, because record counts at each screening stage were not captured in a manner that would support an accurate diagram, and reporting unrecorded counts would have breached the verification standard applied throughout this review.

RESULTS AND DISCUSSION

Twenty-five sources satisfied both verification stages and the eligibility criteria, and their distribution across themes, regions, and designs is summarised in Table 1. The composition of that evidence base is itself informative, since studies conducted within low- and middle-income settings clustered in the domains of health literacy measurement and policy analysis, whereas evidence concerning medication labeling and leaflet comprehension derived exclusively from high-income countries.

Table 1 Distribution Of The Verified Evidence Base By Theme And Setting

Theme	Sources	LMIC setting	High-income setting	Predominant design
Information asymmetry in rare disease care	4	0	4	Qualitative and mixed methods
Medication labeling and comprehension	5	1	4	Trial, survey, scoping review
Health literacy measurement capability	4	3	1	Cross-sectional and psychometric
Equitable access and policy	12	8	4	Policy analysis and perspective
Total	25	12	13	Mixed

A recurring observation across the verified literature was that rare disease patients encountered difficulty obtaining accurate information about their own condition, and that this difficulty accompanied broader health disparities affecting the group (Badiu et al., 2017). The scale of qualitative inquiry into

this experience is now considerable, since a narrative synthesis identified 317 qualitative studies of rare genetic diseases published between 2004 and 2024, analysed through reflexive thematic analysis, and situated the understanding of patient, caregiver, and provider lived experience as essential to addressing the challenges these populations face (Gush & Moosa, 2026). The coexistence of so substantial a qualitative corpus with the persistence of the information problem it documents suggests that the deficit is not attributable to an absence of research attention, but may instead reflect a translational failure between what is known about patient information needs and what health systems routinely deliver.

The organisational dimension of this deficit was illustrated by evidence on care coordination. Rare diseases are frequently complex, chronic, and in many instances life shortening, and where care is organised through specialist expert centres while patients simultaneously depend on local general practitioners for routine care, information exchange between sectors becomes highly relevant to the quality of that care (Inhestern et al., 2024). Research examining patient and caregiver perspectives on such arrangements indicated that the interface between local and specialist providers was itself an object of patient concern rather than a purely administrative matter (Inhestern et al., 2024). This evidence derived from Germany, a high-income setting with an established expert centre network, and is therefore adjacent rather than direct. Its relevance to resource-limited contexts remains a matter of inference rather than demonstration, although the underlying structural condition, namely the dispersal of a patient's care across facilities of differing specialist capability, appears likely to be at least as pronounced where diagnostic capacity is geographically concentrated.

Direct measurement of health literacy within a rare disease population proved uncommon, which lends particular value to the small number of studies attempting it. Health-related quality of life and health literacy were assessed together in cohorts with systemic mastocytosis and mast cell activation syndrome using validated instruments developed by the European Organisation for Research and Treatment of Cancer (Schmidt et al., 2022), demonstrating that such measurement is methodologically feasible in a rare disease context. Rare disease patient organisations have in parallel been characterised as potentially enhancing patient experience, informing expectations, and promoting health literacy, while their own perspectives remain under-examined (Nguyen et al., 2022). Both formal measurement and organised patient intermediation therefore appear to be established components of the rare disease communication landscape in high-income settings. No study identified in this review measured health literacy in a rare disease population within a low- or middle-income country, and no claim regarding the health literacy levels of such populations is consequently advanced here.

Turning to medication information itself, clear patient instruction is a foundational requirement of pharmaceutical care, yet the literature revealed a consistent gap between the readability of medication messages and the skills of the populations expected to interpret them (Ferreira-Alfaya et al., 2025). This finding carries particular weight for the present review, because it indicates that the labeling problem is not primarily one of missing information but of information supplied in a register that its intended recipients cannot reliably decode. Interventional evidence on remedies exists, since a multicentre randomised controlled trial assessed comprehension of information in three prescription medication leaflets, with and without supplementary United States Pharmacopeia pictograms, among adolescents completing secondary education in Spain (Ferreira-Alfaya et al., 2025). The design of that trial, testing a visual supplement against text alone, is directly pertinent to any setting in which textual literacy cannot be assumed uniform across the patient population.

Format constitutes a second axis of the labeling question. Medicines are legally mandated to include a patient information leaflet in physical form, and this leaflet serves as a vital source of information about the medicine (Forsberg et al., 2026). Electronic leaflets offer a distinct advantage in ensuring the availability of the most current information, although the prevailing preference between traditional and electronic formats remains uncertain and has been the subject of survey investigation among patients in Sweden (Forsberg et al., 2026). The currency advantage of electronic formats appears particularly relevant to orphan medicines, for which safety information may be revised as post-marketing experience accumulates in small populations. This connection is nonetheless interpretive,

since the cited survey concerned general medicines in a high-income European setting, and its findings on patient preference should not be assumed to transfer to populations differing in device access, connectivity, or language.

The delivery side of medication communication in resource-limited settings was characterised more by inconsistency of evidence than by demonstrated adequacy, since a scoping review of health communication material use by healthcare providers in low- and middle-income countries reported the existing evidence base as inconsistent (Yirsaw et al., 2026). Where health literacy was limited, it appeared to contribute to medication-related problems and to reduced utilisation of pharmacist-led services (Vasudevan et al., 2026), indicating that comprehension deficits translate into disengagement from precisely the professional resource best positioned to remedy them. The consequences extend into medication safety surveillance, since adverse drug reactions contribute to morbidity, mortality, compromised quality of life, and increased healthcare costs, and underreporting persists despite regulatory efforts, with knowledge gaps and time constraints identified among the contributing reasons (Palanisamy et al., 2026). The severity of this reporting deficit in peripheral Indian settings was illustrated concretely by an adverse drug reaction monitoring centre established at Karaikal, where hospitals in the surrounding area had generated no reports at all in the preceding two years prior to targeted intervention (Palanisamy et al., 2026). For rare disease patients receiving therapies with limited post-marketing experience, the reliability of such reporting infrastructure appears consequential, although no verified source examined pharmacovigilance participation among rare disease patients specifically in a low- or middle-income setting, a silence that appears to characterise the evidence base rather than to indicate that participation is absent.

The conceptual foundation for the third theme was supplied by the World Health Organization (2025), which holds that personal health literacy competencies are mediated by organisational structures and by the availability of resources enabling people to access, understand, appraise, and use health information and services. This formulation relocates a substantial portion of the analytic burden from the individual patient to the institution serving them. Read alongside the labeling evidence considered above, it suggests that a leaflet written above the comprehension level of its readership is not a neutral document, but one that may actively distribute benefit along the same gradient that already structures health disadvantage. Measurement capability is a precondition for evidence-based communication policy, and in the Indian context that capability was demonstrably incomplete, since the absence of validated Hindi language instruments limited assessment until the translation, cultural adaptation, and psychometric validation of three internationally recognised tools for Hindi-speaking adults in rural northern India (Sharma, 2026). The significance of this development is structural rather than substantive, because where validated vernacular instruments are recent or absent, the apparent scarcity of health literacy data may partly reflect the unavailability of the means to measure it rather than an absence of scholarly interest. Empirical assessment in Indian community settings, where undertaken, framed health literacy explicitly as an instrument of equity, with particular importance attached to adolescence on the reasoning that investment during formative years supports informed decision making in adulthood (Rachel & Sudha, 2026). A notable asymmetry therefore emerges, in that demand-side capability is beginning to be measured while the quality and deployment of the materials that capability is meant to engage remain comparatively uncharacterised.

Sustainability in rare disease care proved, in the Indian policy record, to be explicitly a question of competing priorities under resource constraint. The National Policy for Treatment of Rare Diseases of 2017 was kept in abeyance through a non-statutory Gazette Notification dated 18 December 2018, following implementation difficulties that the policy document attributes in part to concerns regarding cost effectiveness in a limited-resource situation, and in part to the challenge of bringing State Governments on board, given that public health and hospitals are primarily a State subject (Ministry of Health and Family Welfare, 2021). The revised draft was placed in the public domain on 13 January 2020 for comment from stakeholders, the general public, organisations, and States and Union Territories, and the resulting policy provides for Centres of Excellence and *Nidan Kendras* (Ministry

of Health and Family Welfare, 2021). The federal structure of health governance is therefore not incidental to rare disease communication, since the institutions that would deliver patient information operate under State authority while policy direction is set centrally.

Analysis of the resulting framework identified persistent structural gaps, since rare disease management and orphan drug development in India have been reported to face hurdles concerning the implementation of comprehensive policies, the absence of dedicated regulatory frameworks, and a lack of epidemiological data, with current policy focusing more on strengthening diagnostics than on a comprehensive treatment framework, and with Indian patients largely excluded from global orphan drug clinical trials (Mishra et al., 2024). The epidemiological deficit was corroborated in a related disease area, where Indian data were described as limited and inconsistent owing to heterogeneity in diagnostic criteria and regional disparities (Sasikumar & Patidar, 2025). Service capacity showed a parallel concentration in tier-one and tier-two cities, with reliance on international collaboration reported to delay investigation (Contractor et al., 2025). Diagnosis, data, and treatment provision thus appear constrained in a mutually reinforcing pattern rather than independently.

The household-level consequences of these system-level constraints were quantified in at least one Indian rare disease population, through a societal perspective cost of illness analysis of thirty Gaucher disease patients attending a genetic clinic at a public hospital in Mumbai between January 2019 and January 2021, which estimated direct, indirect, and intangible costs and compared treated with treatment-naïve groups (Mhatre et al., 2023). Such evidence connects rare disease care to the wider equity literature, in which the concept of pharmacoequity holds that all individuals, regardless of race, ethnicity, geography, or socioeconomic status, should have access to high-quality evidence-based therapies (Jayachandran et al., 2026). Measured against that standard, access to agents accepted as standard of care in many regions remains unequal globally, with the high cost of novel medications combined with complex research and regulatory pathways constraining delivery (Scott et al., 2026). The pattern is not confined to Asia, since people living with inherited metabolic diseases depend upon orphan drugs developed and available primarily in high-income countries, and patients across much of Latin America face stark inequities in access in a region where most countries until recently possessed little rare disease policy infrastructure (Cabello & Salinas, 2026).

A final line of evidence indicated that these inequities are shaped by selection decisions as much as by underlying epidemiology. Health needs are relatively similar across the world, since few conditions are genuinely unique to a single region, and differences in health needs therefore do not explain observed differences between national essential medicines lists, implying that factors other than need influence which medicines countries select (Ul Haq et al., 2026). The World Health Organization Model Lists of Essential Medicines serve as global references for medicines that should be universally available, accessible, and affordable, yet persistent economic and regulatory barriers to equitable access remain (Fattah et al., 2025). Placed against the estimate that approximately 250 million of those living with rare disease reside in low- and middle-income countries where advanced diagnostics and specialist care are frequently unavailable (Kolambage et al., 2026), the implication for sustainability is direct, in that communication interventions operate within, and cannot substitute for, the access architecture determined by these selection and financing decisions.

Considered together, these four themes describe a body of evidence in which the contributing domains are individually well populated yet rarely joined. The demographic case for attention is not in doubt, and the communication case is established in general terms, yet the intersection is absent, since no eligible and verified study identified through this review evaluated medication labeling or leaflet comprehension among rare disease patients in a low- or middle-income country. Three categories of evidence are therefore distinguished. Direct evidence, derived from studies reporting measurable outcomes for rare disease patients in low- and middle-income country settings, was confined to the domains of health literacy measurement and access and policy. Partial evidence, addressing a contributing mechanism within, but not specific to, that population, was available for medication safety surveillance and pharmacovigilance awareness, and for health communication material use by

providers, although none of these studies examined rare disease patients directly. Adjacent evidence, drawn from high-income comparators, was available for medication labeling and leaflet comprehension and informed the analysis as a mechanism that may transfer, provided each use was flagged as such at the point of occurrence. The principal finding of this review is therefore the shape of an absence, and it would be a misrepresentation to present it otherwise. This finding neither agrees nor conflicts with prior reviews in the conventional sense, because no prior review appears to have posed the question in precisely these terms; rather, it is consistent with the broader observation that rare disease research capacity concentrates in high-income settings while the affected population concentrates elsewhere (Kolambage et al., 2026). Where the present synthesis does align with earlier work is in its account of responsibility, since the finding that comprehension failures are produced by organisational conditions rather than individual deficiency corroborates the organisational health literacy construct advanced by the World Health Organization (2025) and is consistent with evidence that limited health literacy predicts disengagement from pharmacist services (Vasudevan et al., 2026).

Empowerment, on the evidence assembled, appeared less an attribute conferred upon patients than a capacity conditioned by surrounding structures. Patient organisations have been characterised as promoting health literacy while remaining under-examined as actors (Nguyen et al., 2022), and educational materials for rare conditions have been successfully co-created by expert clinicians together with patients and subsequently evaluated for end-user acceptability (Badiu et al., 2017), indicating that patients function as producers of health information and not solely as its recipients. The policy literature converges on the same point in holding that implementation requires active stakeholder participation (Chakraborty Choudhury et al., 2026). Read against the principle of pharmacoequity (Jayachandran et al., 2026), empowerment and access appear mutually dependent rather than sequential, since information without access yields informed frustration, while access without information yields therapy that may be used unsafely or abandoned.

These observations carry practical implications that follow from the evidence without being stated by any single source. Given the documented readability gap and the availability of a randomised design for testing pictogram-supplemented leaflets (Ferreira-Alfaya et al., 2025), together with newly validated Hindi language instruments (Sharma, 2026), orphan medicine labeling in India might reasonably be developed with vernacular text supplemented by standardised pictograms and pre-tested for comprehension before wide deployment rather than assumed adequate. Since the national policy already provides for Centres of Excellence and *Nidan Kendras* (Ministry of Health and Family Welfare, 2021) while diagnostic capacity remains geographically concentrated (Contractor et al., 2025), those institutions might be conceived explicitly as communication nodes carrying a defined information transfer function toward local prescribers and dispensers, which extends existing architecture rather than requiring new institutions. Given documented reporting deficits (Palanisamy et al., 2026), patient-facing pharmacovigilance information for orphan medicines might be integrated into dispensing encounters in vernacular form, although no verified source evaluates such an intervention in a rare disease population and the proposal is therefore offered as a hypothesis for testing. Finally, since registry development addresses both epidemiological and communication deficits simultaneously, and since patients cannot be informed about a burden that has not been characterised (Sasikumar & Patidar, 2025), registry investment warrants consideration as communication infrastructure and not solely as surveillance.

Several limitations qualify these observations, and the most consequential is that the review's central question could not be answered directly, so that adjacent evidence from Germany, Spain, Sweden, Australia, and Saudi Arabia was used to characterise mechanisms rather than to establish findings for rare disease populations in low- and middle-income countries, with each such use flagged at the point of occurrence, though the accumulation of adjacency remains a genuine weakness. Seven records whose bibliographic details were independently confirmed were withheld from citation because their content could not be verified, and several of these, particularly those addressing Indian rare disease policy and patient support organisations, would probably have strengthened the synthesis had full text

been obtainable. Searching was conducted principally through Europe PMC without Scopus, Embase, or Web of Science, so that relevant records indexed only in those databases may have been missed, and no grey literature from national patient organisations was retrieved, nor was any non-English source assessed. Low-income countries as distinct from middle-income countries are effectively unrepresented, so conclusions should be read as pertaining primarily to middle-income settings with India most fully represented.

The gaps identified are nonetheless tractable with existing methods, since validated vernacular health literacy instruments now exist (Sharma, 2026), a transferable trial design for pictogram-supplemented leaflets is available (Ferreira-Alfaya et al., 2025), and health literacy measurement within a rare disease cohort has been shown feasible (Schmidt et al., 2022). A study combining these approaches, measuring comprehension of orphan medicine labeling among rare disease patients in an Indian setting using validated vernacular instruments, would address the central gap directly, and parallel investigation of pharmacovigilance participation appears warranted given documented underreporting (Palanisamy et al., 2026).

CONCLUSION

This scoping review examined the intersection of medication labeling, health literacy, equitable access, and patient empowerment for rare disease patients in low- and middle-income countries, and found the four contributing domains to be individually well populated yet rarely joined, since no verified study evaluated medication labeling or leaflet comprehension among rare disease patients in such a setting. That absence is reported as the principal finding rather than obscured beneath adjacent evidence, and the adjacent literature drawn upon was flagged as such at each point of use. The evidence reviewed indicated that comprehension failures arise substantially from organisational conditions rather than individual deficiency, that empowerment and equitable access operate as mutually dependent rather than sequential conditions, and that communication interventions cannot substitute for the access architecture determined by financing and medicine selection decisions. The identified gap appears tractable, because validated vernacular health literacy instruments now exist for Hindi-speaking populations, a randomised design for testing pictogram-supplemented leaflets is available, health literacy measurement within a rare disease cohort has been demonstrated as feasible, and an existing policy architecture already provides for Centres of Excellence and Nidan Kendras. Whether rare disease patients in these settings become genuinely empowered participants in their own care may therefore depend less upon new discovery than upon the deliberate application of methods that are already available. Sustained empowerment in rare disease care appears to depend on four conditions that the evidence approached individually but did not jointly evaluate. Understandable information, written at a level matched to measured health literacy, is a necessary precondition, since a label unread remains a safety risk unmitigated. Responsive health organisations, characterised through the World Health Organization (2025) as health-literate systems, are the context that translates legibility into action, because no individual can act consistently on information whose presentation varies across providers. Continuity of communication across services matters because rare disease care is necessarily distributed among specialist, primary, and pharmaceutical interfaces, and fragmented information is the proximate cause of the delays and errors that erode confidence. Meaningful patient participation, rather than information delivery alone, completes the circuit, since the policy literature converges on stakeholder involvement as a condition of implementation (Chakraborty Choudhury et al., 2026), and patients co-create educational materials when invited to do so (Badiu et al., 2017). Together these suggest that empowerment should be treated as the observable outcome of a system designed for it, rather than as an attribute presumed to follow from access alone.

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