Patient Voice Beyond HTA: A Case-Based Review of Informal Mechanisms Shaping Access in 15 Global Jurisdictions

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Introduction

Patient involvement in access decisions is usually described through formal channels: health technology assessment (HTA) submissions, regulator consultations, and standing committee seats. Across much of Asia Pacific, Latin America, the Middle East, and Africa these channels are narrow, recently established, or absent altogether, and where they do exist participation is often consultative rather than decisive.[1,2]

Despite lacking formal institutional channels, patients, patient advocacy groups (PAGs), and civil society organizations actively shape access outcomes across these markets. They exercise influence through non-traditional mechanisms including strategic litigation, legislative coalitions, patent oppositions, and targeted public campaigns to payers and manufacturers. However, these informal pathways remain poorly characterized and documented. Without a comprehensive taxonomy of these mechanisms, it is difficult to predict when patient-led action will meaningfully alter access decisions or distinguish high-impact engagement from advocacy.

Objectives

To identify and categorise informal mechanisms through which patients, PAGs, and civil society have influenced access outcomes for medicines, therapies, or policy across 15 jurisdictions outside the US and EU.

Methods

1. **Data extraction**: targeted desk review of publicly available sources (2016–2026) across 15 jurisdictions (listed in Figure 1)
   Sources: peer-reviewed literature, news reports, government and HTA records, PAG publications, and legal or policy documents
   Pre-2016 cases: eligible if reported within the search window with complete mechanism and outcome data
2. **Candidate cases required three inclusion criteria:**
   1. Informal mechanism (operating outside formal government channels, standing committees, or routine consultations)
   2. Initiated by patient, PAG or civil society (rather than invited participation)
   3. Linked to an access outcome (product availability or new policy/law/program)
3. **Quantitative analysis**: cases categorized by mechanism type and disease area
   Interpretation: case counts reflect the compiled dataset, not patient-action frequency across markets

Limitations: *reliance on English-accessible public sources and case-level judgement in attributing access outcomes to patient action.*

Results

Six recurring types of mechanisms across 13 jurisdictions identified

Twenty-nine cases met all three criteria, spanning 13 of the 15 jurisdictions reviewed. Within that sample, six mechanism types recur, and none were confined to a single jurisdiction: coalition legislation, campaigns, and litigation each appear in both mature and emerging reimbursement environments (Table 1).

Table 1: Mechanism types and their frequency

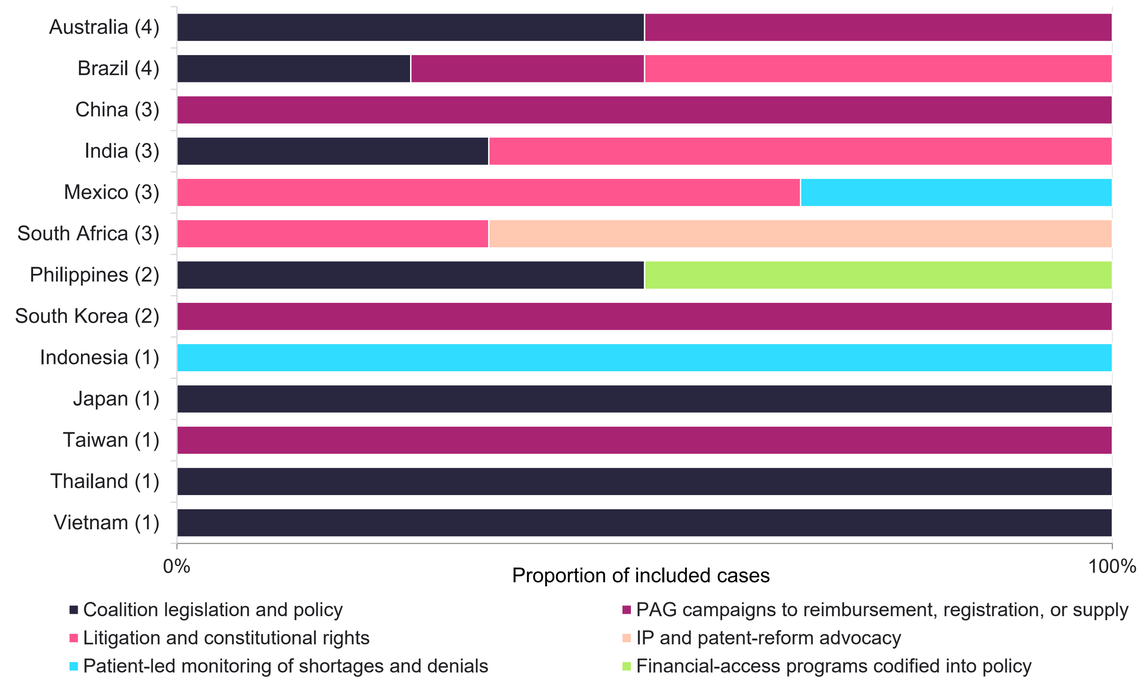
Mechanism type	*Outcome/Impact*	*Indicative examples*	*n*
Coalition legislation and policy	Creates access architecture through law or national policy	*Thailand: 24 rare diseases into the UCS (2019). Philippines: NICCA (2019).*	*12*
PAG campaigns to reimbursement, registration, or supply	Converts sustained public pressure into a listing, registration, or restored supply	*Australia: Trikafta PBS listing cut cost from over AUD 250,000 a year to AUD 31.60 per script (from 2022). South Korea: Kymriah, first CAR-T reimbursed (2022).*	*9*
Litigation and constitutional rights	Court action, including amparo and writ proceedings	*Brazil: STF right-to-health rulings (2019). Mexico: amparo actions on paediatric oncology supply (2019-21).*	*7*
IP and patent-reform advocacy	Patent opposition and IP-policy reform opening generic entry	*South Africa: public trastuzumab at ZAR 6,532 per vial against ZAR 19,015 private (2016-20). India: hepatitis C patent oppositions (2013-18).*	*4*
Patient-led monitoring of shortages and denials	Civil-society surveillance of stockouts and coverage denials	*Mexico: Cero Desabasto documented 7.5 million unfilled prescriptions for 2023. Indonesia: KPCDI referral-rule reform (2022-24).*	*2*
Financial-access programs codified into policy	PAG-operated support later written into statute	*Philippines: ICanServe's Ating Dibdibin written into NICCA Section 12C (2019).*	*1*

Representative cases are presented for each mechanism type (n = 2 per type). Total assignments (n = 35) exceed the total case count because six cases demonstrated dual mechanisms; all counts describe the compiled sample rather than quantification of patient action.

Mechanism follows institutional design

Cases were identified in every studied jurisdiction except the Gulf, where neither jurisdiction yielded a qualifying case (Figure 1). The dominant mechanism type in a market tracks the strength of its formal access machinery rather than its income level. Where HTA is institutionalized and predictable, patient action targets named listing decisions and works through campaigns. Where the courts offer a faster or more certain route than the payer, as in Brazil, Mexico, and India, litigation substitutes for submission and yields precedent that binds the system rather than a single product. Where neither route is established, coalitions build the access architecture itself, through statute and benefit-package design.

Figure 1: Distribution of cases by jurisdiction and primary mechanism type



Each case is plotted once, by primary mechanism type. Saudi Arabia and the UAE yielded no qualifying case.

Jurisdiction spotlight

- Australia has institutionalised patient engagement and therefore has higher representation of organized PAG campaigns
- Brazil guarantees constitutional right to universal healthcare but the national payer, faces persistent budget constraints and administrative delays resulting in high rates of litigation to access treatment.
- India and South Africa show patent-opposition routes that formal HTA capacity alone would not predict, reflecting mature IP infrastructure and experienced legal NGOs.
- In Taiwan national insurer operates a formal platform for patient and PAG input into reimbursement decisions which is excluded from this research therefore resulting in fewer number of cases identified in a market with high patient involvement

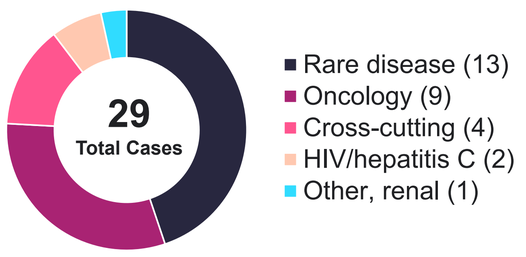
Coalitions build architecture; campaigns win products

Single-organization campaigns delivered product-level results, typically within two to three years (one listing, one registration, one restored supply). Multi-actor coalitions delivered structural change over five years or more: statutes, benefit-package expansion, and IP policy. The distinction matters because the two differ in durability. A campaign win binds the product; a coalition win shapes the market and changes the conditions facing every subsequent product in that category.

Rare disease and oncology recur, reflecting community mobilization

Rare disease and oncology account for 13 and 9 of the 29 included cases (Figure 2), and both appear in every where cases were identified. High per-patient cost and established patient organizations are common to the two areas. In rare disease specifically, populations are small enough for individual patients to become public narratives. Other conditions carrying significant disease burden in these markets are largely absent from the sample.

Figure 2: Cases by disease area



Cross-cutting refers to system-level access issues not specific to one disease area (n=4); oncology includes one paediatric case. The five categories sum to all 29 included cases.

Discussion and Conclusion

Outside the US and EU, informal patient action routinely determines access, taking forms largely predicted by institutional architecture.

Strategic resource allocation requires distinguishing between coalition and campaign support. Coalition investments yield durable institutional reform through multi-year commitments with indirect attribution, whereas campaign support delivers immediate, product-specific access.

Across observed cases, the effective patient contribution served as strategic leverage rather than preference data, specifically via price benchmarking, supply disruption tracking, legal arguments, and policy-aligned testimony. While manufacturers generate much of these data, their impact increases significantly when owned and presented by patient organizations.

Operationally, stakeholders must map dominant regional pathways and develop corresponding strategies. For HTA bodies, informal patient mechanisms signal unmet institutional capacity rather than a replacement for formal engagement.

These conclusions are bounded by three limitations inherent to the publicly available evidence: represented disease areas do not reflect population burden in the scoped jurisdictions, distinguishing formal from informal pathways was not always feasible, and isolating the distinct contributions of patient organizations from other stakeholders within coalition models could not be reliably determined.

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