

PATIENT GROUPS

# A seat at the pricing table

The actions and advocacy of patient groups to gain access to medicines.



**BLEEDING DISORDERS**



## Bleeding disorders at a glance

**63**

RESPONDENT  
PATIENT GROUPS

**67%**

REPRESENT INTERESTS  
"REGULARLY" OR  
"SOMETIMES"

**35**

GROUPS SHARED  
COMMENTARY

REPORT NAVIGATION

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BLEEDING DISORDERS

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AI-use disclosure

AI was used to support:

- checking data
- data analyses
- product design
- translations

**A SEAT AT THE PRICING TABLE**

# Introduction

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Historically, patients and the organisations that represent them were often regarded as little more than passive recipients of healthcare, with limited involvement in shaping the policies, treatment and research priorities that directly affected them. Recently, however, this perception has increasingly been challenged, with patient organisations taking a more active and influential role in shaping healthcare policy at local, regional, national and international levels.

Over recent years, patient organisations have also increasingly advocated for greater involvement in decisions concerning the treatments and care available to the communities they represent, providing invaluable insights into how these decisions directly affect patients and their families. However, relatively little has been known about the extent to which patient organisations are involved in decision-making processes undertaken by regulators, payers and reimbursement bodies.

For the first time, PatientView is seeking to quantify and highlight the extent and impact of patient group involvement in this space, providing greater insight into how patient organisations are contributing to regulatory, reimbursement and healthcare decision-making. This provides policymakers, the private sector and regulators with insight into the activities and perspectives of 2,262 patient groups across disease areas and countries.

While the role of patient groups continues to evolve alongside the wider pharmaceutical and regulatory landscape, one thing is clear: patient groups are increasingly demanding that their voices are heard and meaningfully considered, securing a more prominent and established role in discussions around pricing, and importantly, a seat at the pricing table.

## HOW THE RESEARCH WAS CONDUCTED

# Methodology

The research presented in “Patient Groups: A Seat at the Pricing Table” sought to establish the extent to which patient groups represent patient interests to drug regulators, payers (including through health technology assessments (HTAs) and medical device assessments) and governmental or policy committees.

Between December 2025 and March 2026, a short survey was circulated to key patient organisations and translated into 22 languages. In total, 2,262 patient groups responded, representing 100 countries and 45 therapy areas.

Both the quantitative and qualitative findings were analysed to identify overarching trends and insights. Where sufficient responses (14 or more respondent patient groups) were received for individual countries or therapy areas, these data were also examined separately.

Among patient groups actively engaging with regulators and payers, responses were analysed against 8 overarching goals and 39 sub-categories, ranging from improving access pathways to upholding patients' rights. In total, 35 countries are examined and 26 therapy areas. (Note that responses can fall in more than one goal or sub-category.)

The following pages present:

1. The overall percentages of the 985 unique respondent patient groups that provided commentary mentioning the types of interaction they have with drug regulators, and the actions and advocacy they undertake to gain access to medicines.
2. A brief explanation of the different types of interactions, actions and advocacy undertaken by patient groups to gain access to medicines.

The research presented on the accompanying website ([www.patientview.com](http://www.patientview.com)) and in this report highlights the findings from this analysis, supported by verbatim responses that capture patient organisations' experiences, priorities and perspectives in their own words.

This report also contains:

1. Overall results
2. Therapy-area-specific results
3. Appendix I: Profiles of respondent patient groups
4. Appendix II: Respondent patient groups happy to be named
5. Appendix III: Full set of commentaries from respondent patient groups

For a listing of all respondents to this pricing survey, happy to be named, go to [www.patientview.com](http://www.patientview.com).

To access country- or therapy-specific reports please write to [report@patient-view.com](mailto:report@patient-view.com)

## INTERACTION · PERCENTAGE OF ALL COMMENTS

# How patient groups interact with drug regulators

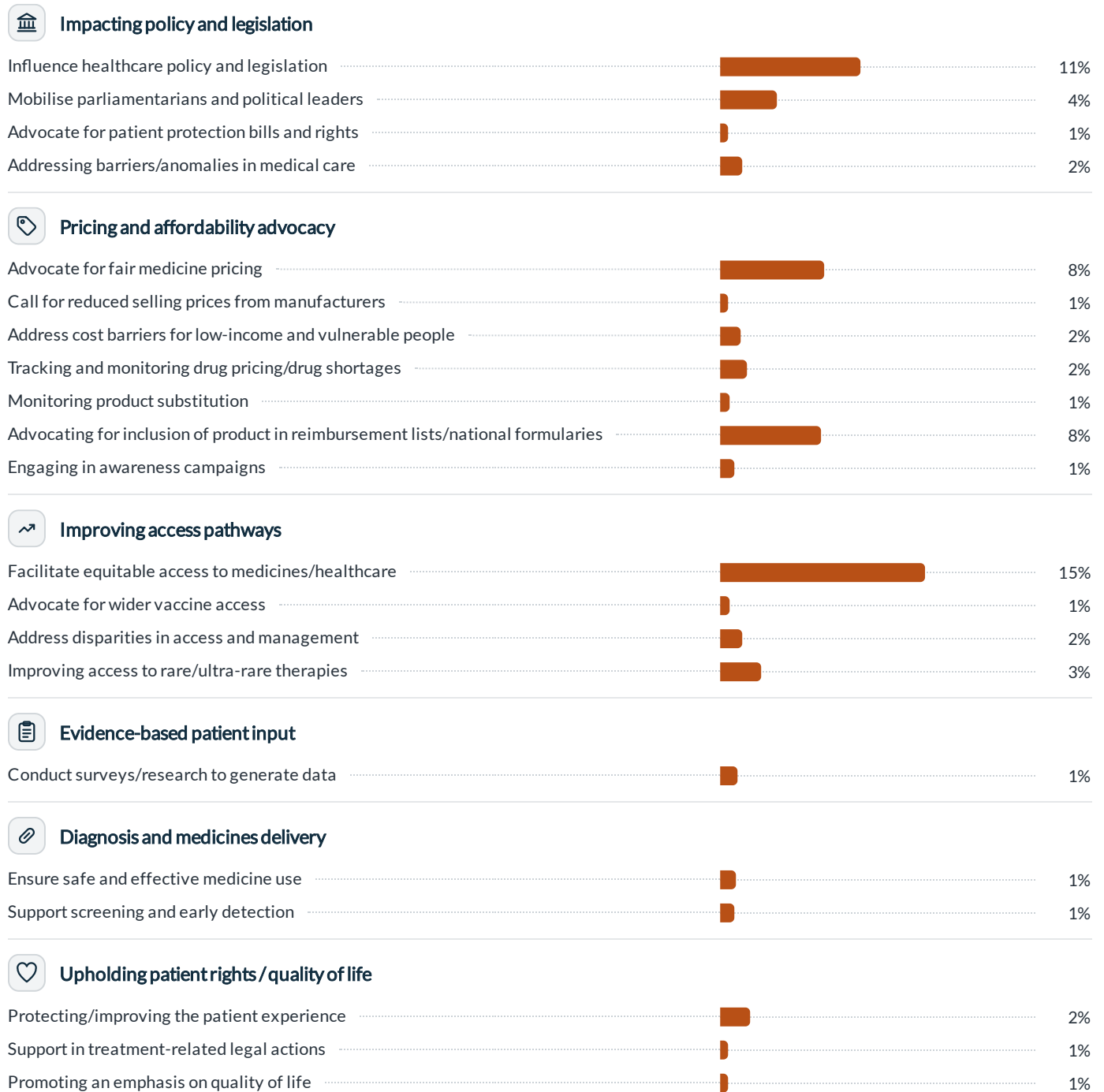
Direct, two-way participation in regulatory, HTA and policy processes.



## ACTIONS &amp; ADVOCACY · PERCENTAGE OF ALL COMMENTS

## How patient groups get access to medicines

Advocacy, campaigning and activities to secure access to medicines.



# How patient organisations engage on securing access to medicines



## Interaction



### Regulatory partnership

#### Working with regulatory bodies

Patient organisations engage directly with national medicines regulators and health authorities, submitting contributions, proposals and formal requests. This work establishes them as recognised interlocutors in regulatory processes, and many report actively seeking closer, more formalised relationships with these bodies.

#### Participate in Health Technology Assessment (HTA) and reimbursement reviews

Groups take part in health technology assessment and reimbursement evaluations, responding to consultations and preparing submissions that accompany reimbursement applications. By feeding into the bodies that appraise new treatments, they help ensure the patient perspective shapes which therapies are ultimately funded. This is the single most frequently reported form of engagement.

#### Contribute to Marketing Authorisation submissions

Organisations provide input into marketing-authorisation processes for new and orphan medicines, offering opinions to regulators and supporting applications for approval. Their contributions range from scientific-advice committees and fast-track designations to formal position statements backing a product's introduction.

#### Involvement with clinical guideline-setting bodies

Patient representatives sit on guideline and expert committees, helping to shape clinical standards. Involvement is typically through formal membership of the committees that develop national clinical guidelines and quality standards.

#### Attend/facilitate meetings with policymakers/regulators

Groups actively request and attend meetings, roundtables and consultative sessions with regulators, ministries and health authorities. Whether convened by invitation or sought proactively, these encounters give patients a direct channel to decision-makers.

#### With other healthcare advocates

Much engagement happens collectively, through umbrella and federation bodies that advocate on members' behalf. Joint position statements and shared representation amplify the voices of smaller organisations in reimbursement and regulatory forums.



### Impacting policy and legislation

#### Participate in public hearings and consultations

Groups submit formal responses to public consultations and take part in parliamentary hearings, forums and working groups. They use these open processes to convey the patient perspective directly to decision-makers.

#### Contribute to the drafting of public health legislation

Groups actively help draft and refine health laws, commenting on legislative proposals and, in some cases, sponsoring bills. They contribute technical input and amendments across a range of access, equity and medicines legislation.



### Pricing and affordability advocacy

#### Engage in price-review board negotiations

Groups participate directly in the bodies that set prices and reimbursement terms — completing questionnaires, sitting on advisory councils and joining negotiation roundtables. Their input brings patient priorities into otherwise technical pricing decisions.



### Evidence-based patient input

#### Use patient insights to inform decisions

Organisations systematically gather and convey patient views to inform regulatory, reimbursement and policy decisions — through advisory councils, questionnaires, nominated committee members and formal submissions. This ensures decisions reflect real patient priorities.



## Evidence-based patient input *(continued)*

### Provide Real-World Evidence (RWE) to regulatory agencies

Groups generate and submit structured real-world evidence and patient-reported outcomes to inform HTA and reimbursement, translating lived experience into policy-relevant data. This articulates treatment burden, unmet needs and value beyond clinical endpoints.

### Leverage patient stories and lived-experience narratives

Advocates bring patient and caregiver stories into appraisals, consultations and media to demonstrate the human impact of treatment and access decisions. Lived-experience submissions give qualitative weight to reimbursement cases.

### Translate evidence and insights into policy input

Groups convert technical, clinical and scientific evidence into structured recommendations and position papers for decision-makers. Through scientific committees, technical briefs and formal submissions, they recommend which medicines and supplies should be incorporated into health systems.



## Diagnosis and medicines delivery

### Participate in developing clinical guidelines

Organisations contribute to clinical guideline and care-pathway development through technical working groups and committees. Their involvement helps ensure guidelines reflect patient needs and the realities of everyday care.

### Collaborate on clinical trial design

Groups advise on trial design and endpoints, sitting on scientific advisory councils and working with regulators and sponsors. Their input covers issues such as eligibility, trial burden and improving studies for both sponsors and patients.



## Institutional governance

### Ensure representation within governmental advisory bodies

Patient organisations secure formal seats on government advisory boards, committees and working groups. This embedded representation gives patients standing in ongoing health governance.

### Working effectively within multi-stakeholder networks

Groups operate within broad networks linking patients, clinicians, industry, regulators and umbrella bodies. Collaboration across these platforms strengthens their collective influence.

### Maintain formal status as public interest or charitable entity

Organisations establish and maintain formal legal standing — public-interest designation, charitable or non-profit registration, or listing on official interest-group registers — to legitimise their advocacy. This status underpins their recognition as social partners to the state.



## Actions & Advocacy



### Impacting policy and legislation

#### Influence healthcare policy and legislation

Organisations campaign to shape health policy through petitions, position statements, ministerial engagement and lobbying. Efforts span narrowly targeted reforms through to broad representation of patient interests before ministries and parliaments.

#### Mobilise parliamentarians and political leaders

Advocates engage directly with parliamentarians, ministers and parliamentary health groups — through hearings, budget submissions and face-to-face meetings — to win political backing. Sustained political mobilisation is repeatedly credited with delivering concrete policy change.

#### Advocate for patient protection bills and rights

Organisations pursue dedicated legislation to protect patients' rights. Where formal legislative routes stall, some turn to constitutional and legal mechanisms to defend fundamental rights.

#### Addressing barriers/anomalies in medical care

Advocates identify and challenge specific inequities and gaps in care, such as treatments approved or reimbursed for some patient groups but not others. They press authorities to correct these anomalies and extend access where clinical need is otherwise unmet.



### Pricing and affordability advocacy

#### Advocate for fair medicine pricing

Organisations campaign for affordable, well-reimbursed medicines, raising pricing and out-of-pocket costs with parliaments, insurers and manufacturers. Activity ranges from formal advocacy submissions and reimbursement calls to public and media campaigns highlighting the burden of high drug costs.

#### Call for reduced selling prices from manufacturers

Some organisations appeal or negotiate directly with pharmaceutical companies to lower prices. Others escalate to pricing alliances and procurement bodies when costs remain out of reach.

#### Address cost barriers for low-income and vulnerable people

Advocates work to remove financial barriers for those least able to pay, supporting assistance and discount schemes. Efforts focus on low-income patients, welfare recipients and marginalised communities facing prohibitive out-of-pocket costs.

#### Tracking and monitoring drug pricing/drug shortages

Groups actively monitor prices, availability and shortages, alerting regulators and mobilising their communities when supplies fail. Activity includes civic oversight of pricing compliance, position statements on specific shortages, and engagement with authorities and manufacturers to secure continuity of supply.

#### Monitoring product substitution

Organisations watch for unilateral substitutions — such as biologic-to-biosimilar switches or altered prescriptions — that may disadvantage patients, and intervene to protect appropriate treatment. Several advocate for legislation requiring explicit prescriber consent before substitution.

#### Advocating for inclusion of product in reimbursement lists/national formularies

A major focus is getting specific medicines and devices added to reimbursement lists and national formularies. Groups submit applications, complete reimbursement questionnaires and advocate at both regional and national level.

#### Engaging in awareness campaigns

Organisations run public awareness and prevention campaigns — through media, events and dedicated awareness days — to build understanding and support for their cause. These efforts often accompany calls for better access and encourage public participation in consultations.



## Improving access pathways

### Facilitate equitable access to medicines/healthcare

This broad, high-volume activity centres on ensuring patients can actually obtain the treatments, tests and services they need. Groups advocate on availability, guide families through access routes, join procurement processes and press for consistent, nationwide provision of appropriate therapy.

### Advocate for wider vaccine access

Organisations campaign to expand vaccine access and funding through consumer comments, awareness activity and price-review interventions. Vaccination and equitable access are recurring priorities, often pursued alongside partner networks.

### Address disparities in access and management

Advocates highlight and act on regional, socioeconomic and system-level inequities — such as differences in waiting times, or treatments available in one area but not another. They press states and agencies to close these gaps and standardise access.

### Improving access to rare/ultra-rare therapies

Given the prohibitive cost of orphan drugs, groups focus specifically on securing access to rare-disease therapies through early-access and compassionate-use programmes and dedicated rare-disease commissions and laws. Cross-border alliances and national rare-disease committees are central to this work.



## Evidence-based patient input

### Conduct surveys/research to generate data

Organisations run surveys, interviews and research studies to build an evidence base on patient needs, which they share with policymakers and regulators. Instruments range from structured questionnaires to patient-focused research collaborations.



## Diagnosis and medicines delivery

### Ensure safe and effective medicine use

Advocates engage on medicine safety, quality and appropriate use — raising adverse-event concerns, contributing to pharmacovigilance committees and challenging withdrawals or substandard products. This embeds the patient perspective in safety policy.

### Support screening and early detection

Advocates campaign for screening and early-detection programmes, including newborn screening and multi-condition early detection. They provide expertise to funding and regulatory bodies on adding conditions to screening panels.



## Upholding patient rights / quality of life

### Protecting/improving the patient experience

Advocates defend and improve the patient experience beyond the clinic — across employment, disability support, discrimination and social inclusion — and provide social and legal guidance. They help patients assert their rights and resolve barriers with insurers and authorities.

### Support in treatment-related legal actions

Some organisations pursue or support legal action to secure access to treatment, including constitutional and administrative proceedings and litigation for approved therapies. This judicial route is used to compel coverage of high-cost medicines where other channels fall short.

### Promoting an emphasis on quality of life

Groups advocate for quality of life and quality of care as ends in themselves — spanning palliative care, accessibility and broader wellbeing. Their input keeps unmet needs and lived quality of life on the agenda alongside clinical and cost considerations.

## BLEEDING DISORDERS

# Summary

Of the 63 patient groups responding in bleeding disorders, 67% represent patient interests to drug regulators either “regularly” or “sometimes”. 35 gave detail on how they interact with regulators, and on the actions and advocacy they undertake to improve access to medicines.

The most common form of engagement is attending meetings with policymakers and regulators (23%), followed by participating in HTA and reimbursement reviews and securing representation within governmental advisory bodies (both 20%); participating in public hearings and consultations (14%); working directly with regulatory bodies (9%); engaging in price-review board negotiations (6%); and contributing to marketing-authorisation submissions, drawing on patient stories and lived-experience narratives, contributing to the development of clinical guidelines, contributing to the drafting of public-health legislation and working within multi-stakeholder networks (each 3%). Engagement here centres on direct meetings with policymakers and regulators.

“ Participation in the G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee], RKI [Robert Koch-Institut – Robert Koch Institute], and Arbeitskreis Blut [Advisory Committee on Blood].”

National bleeding-disorders patient group, Germany

On actions and advocacy, patient groups in bleeding disorders concentrate on access and pricing and reimbursement. The leading activity is facilitating equitable access to healthcare (23%), followed by advocating for fair medicine pricing (11%); ensuring safe and effective medicine use and influencing healthcare policy and legislation (both 6%); and mobilising parliamentarians and political leaders, tracking and monitoring drug pricing and shortages, addressing disparities in access and management and improving access to rare and ultra-rare therapies (each 3%).

“ The first procurement and registration of factor VIII in our country was made possible through the efforts of our organisation in collaboration with the IGSS [Instituto Guatemalteco de Seguridad Social – Guatemalan Social Security Institute].”

National bleeding-disorders patient group, Guatemala

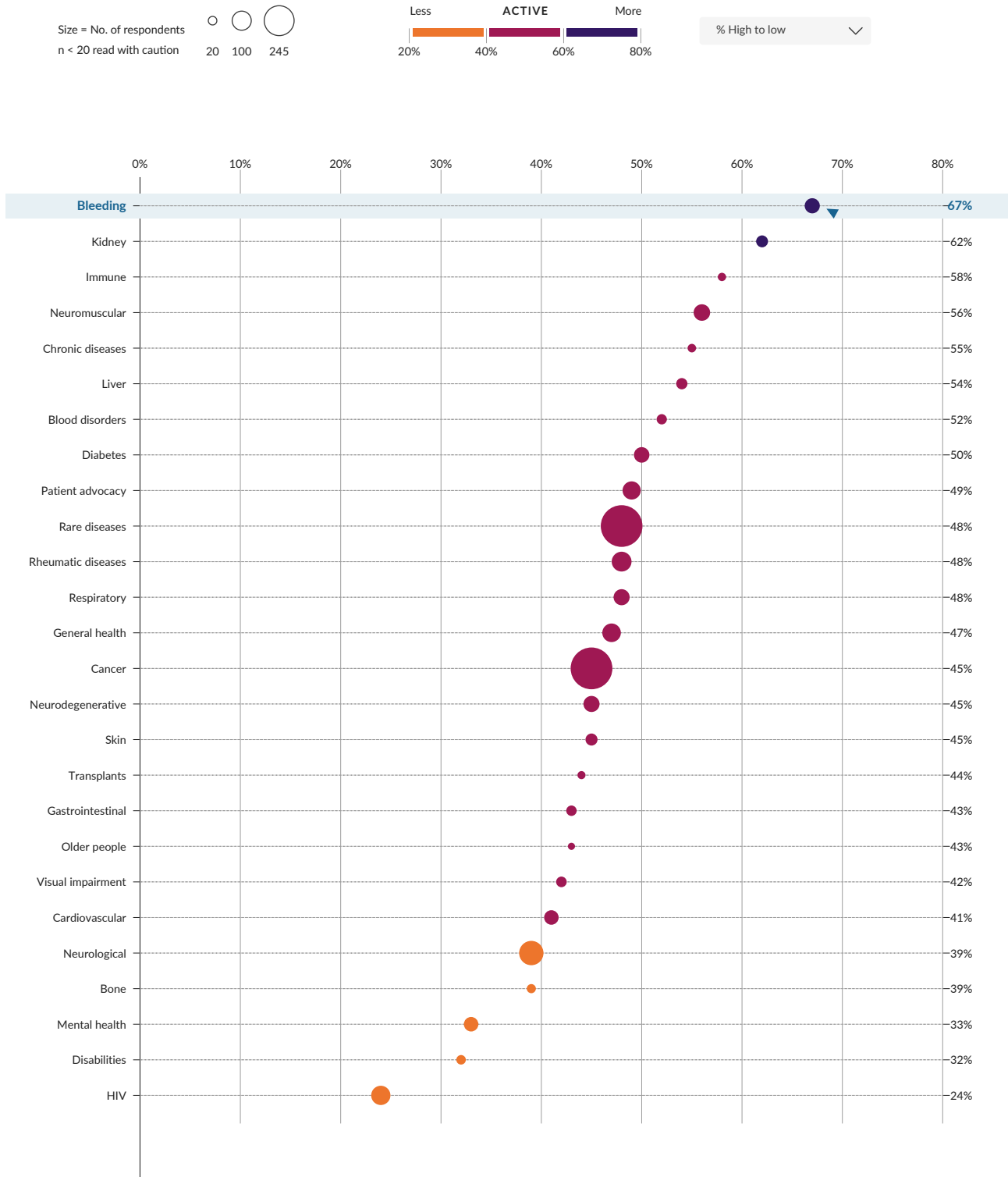
In summary, patient groups in bleeding disorders are most active in attending meetings with policymakers and regulators and, on the access side, facilitating equitable access to healthcare.

# Share of patient groups engaging with drug-pricing regulators 'regularly' or 'sometimes'

Total responses = 2,262

Excludes 'don't know' responses and where n < 14 are omitted

## SURVEY RESPONSES



# **Therapy area analyses and results**



# Bleeding disorders

63
RESPONDENT PATIENT GROUPS

67%
"REGULARLY" OR "SOMETIMES" REPRESENT INTERESTS TO REGULATORS

35
GROUPS SHARED COMMENTARY

**SUMMARY**

Of the 63 patient groups from bleeding disorders, 67% represent patient interests to drug regulators. The most common form is attending meetings with policymakers/regulators (23%). When advocating patient groups concentrate on access and pricing/reimbursement.

**How bleeding-disorders patient groups interact with drug regulators**
N = 35

| GOAL                                                                                                                          | INTERACTION                                                                 | %   |
|-------------------------------------------------------------------------------------------------------------------------------|-----------------------------------------------------------------------------|-----|
|  <b>Regulatory partnership</b>               | Attend/facilitate meetings with policymakers/regulators                     | 23% |
|                                                                                                                               | Participate in Health Technology Assessment (HTA) and reimbursement reviews | 20% |
|                                                                                                                               | Working with regulatory bodies                                              | 9%  |
|                                                                                                                               | Contribute to Marketing Authorisation submissions                           | 3%  |
|  <b>Evidence-based patient input</b>       | Leverage patient stories and lived-experience narratives                    | 3%  |
|  <b>Diagnosis &amp; medicines delivery</b> | Participate in developing clinical guidelines                               | 3%  |
|  <b>Impacting policy &amp; legislation</b> | Participate in public hearings and consultations                            | 14% |
|                                                                                                                               | Contribute to the drafting of public health legislation                     | 3%  |
|  <b>Institutional governance</b>           | Ensure representation within governmental advisory bodies                   | 20% |
|                                                                                                                               | Working effectively within multi-stakeholder networks                       | 3%  |
|  <b>Pricing &amp; affordability</b>        | Engage in price-review board negotiations                                   | 6%  |

Pill shade = share of comments – darker is higher

*Participation in the G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee], RKI [Robert Koch-Institut – Robert Koch Institute], and Arbeitskreis Blut [Advisory Committee on Blood].*

**PARTICIPATE IN HEALTH TECHNOLOGY ASSESSMENT (HTA) AND REIMBURSEMENT REVIEWS**

**National bleeding-disorders patient group, Germany**

*The Federation participates in dialogue and collaboration forums with institutions within Mexico's healthcare system – including IMSS [Instituto Mexicano del Seguro Social – Mexican Social Security Institute], ISSSTE [Instituto de Seguridad y Servicios Sociales de los Trabajadores del Estado – Institute for Social Security and Services for State Workers], and other health authorities – to give voice to the needs of people living with coagulation disorders.*

**ENSURE REPRESENTATION WITHIN GOVERNMENTAL ADVISORY BODIES**

**National bleeding-disorders patient group, Mexico**

# Bleeding disorders

## How bleeding-disorders patient groups get access to medicines

N = 35

| GOAL                                                                                                                        | ACTIONS & ADVOCACY                                  | %   |
|-----------------------------------------------------------------------------------------------------------------------------|-----------------------------------------------------|-----|
|  <b>Diagnosis &amp; medicines delivery</b> | Ensure safe and effective medicine use              | 6%  |
|  <b>Impacting policy &amp; legislation</b> | Influence healthcare policy and legislation         | 6%  |
|                                                                                                                             | Mobilise parliamentarians and political leaders     | 3%  |
|  <b>Pricing &amp; affordability</b>        | Advocate for fair medicine pricing                  | 11% |
|                                                                                                                             | Tracking and monitoring drug pricing/drug shortages | 3%  |
|                                                                                                                             | Facilitate equitable access to medicines/healthcare | 23% |
|  <b>Improving access pathways</b>          | Address disparities in access and management        | 3%  |
|                                                                                                                             | Improving access to rare/ultra-rare therapies       | 3%  |

Pill shade = share of comments — darker is higher

“

The first procurement and registration of factor VIII in our country was made possible through the efforts of our organisation in collaboration with the IGSS [Instituto Guatemalteco de Seguridad Social – Guatemalan Social Security Institute].

**FACILITATE EQUITABLE ACCESS TO MEDICINES/HEALTHCARE**

**National bleeding-disorders patient group, Guatemala**

“

We are constantly vigilant in ensuring that the pharmaceutical industry sells its products at international prices and does not exceed those price thresholds — in other words, we engage in civic oversight to verify proper compliance and prevent any irregularities, in order to safeguard our annual state budget.

**ADVOCATE FOR FAIR MEDICINE PRICING**

**National bleeding-disorders patient group, Ecuador**

# Appendices

Appendix I: Profiles of patient groups

Appendix II: Patient group respondents that gave permission to be named

Appendix III: Full list of commentaries – categorised

## APPENDIX • PATIENT GROUP PROFILES

## Profiles of respondent patient groups focusing on bleeding disorders

**63**

RESPONDENT PATIENT GROUPS

**201,800**

PATIENTS REACHED

**29**

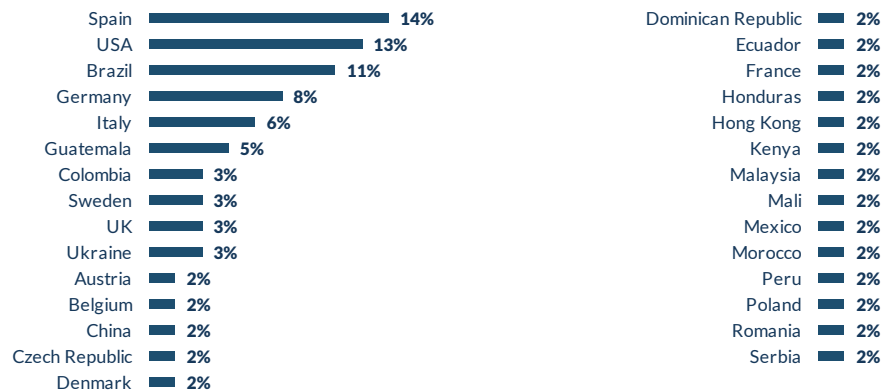
COUNTRIES

**GEOGRAPHIC REACH**

Total respondent bleeding-disorders patient groups

**COUNTRIES**

Total respondent bleeding-disorders patient groups

*n = 63 respondent patient groups • 29 countries represented*

## APPENDIX II • RESPONDENTS THAT GAVE PERMISSION TO BE NAMED

## Respondent patient groups in bleeding disorders that gave permission to be named

Patient groups that consented to be named, listed alphabetically.  
Where a group provided a website, its name links to it.

ABHE - Associação Baiana dos Hemofílicos  
 AHES - Associação dos Hemofílicos do Espírito Santo  
 APH - Associação Paranaense dos Hemofílicos  
 ASPAHC - Associação Paraense de Portadores de Hemofilia e Coagulopatias Hereditárias  
 CHESP - Centro dos Hemofílicos do Estado de São Paulo  
 FBH - Federação Brasileira de Hemofilia  
 山西博爱血友病关爱中心  
 Liga Antioqueña de Hemofílicos  
 Liga Colombiana de Hemofílicos y Otras Deficiencias Sanguíneas (Colhemofílicos)  
 Hemojunior, z.s.  
 FUNDHEC - Fundacion Hemofilica Ecuatoriana  
 AFH - Association Française des Hémophiles  
 Bluter Betreuung Bayern (BBB) e.V.  
 Deutsche Hämophiliegesellschaft (DHG) e.V.  
 Hämophilie 2000 e.V.  
 Sociedad Hondureña de Hemofilia (SHH)  
 香港血友病會  
 ATE - Associazione Toscana Emofilici  
 Kenya Haemophilia Association (KHA)  
 Hemophilia Society of Malaysia (HSM)  
 Association Malienne de Lutte contre l'Hémophilie et les autres Coagulopathies (AMALHEC)  
 FHRM - Federación de Hemofilia de la República Mexicana, A.C.  
 Association Marocaine des Hémophiles (AMH)  
 Asociación de Hemofilia Región Norte del Perú  
 Fundacja Sanguis Hemofilia i Pokrewne Skazy Krwotoczne  
 Asociația Română de Hemofilie (ARH)  
 Udruženje hemofiličara Srbije (UHS)  
 AHEMAS - Asociación de Hemofilia de Asturias  
 ASHECOVA - Asociación de Hemofilia de la Comunidad Valenciana  
 ASHEMADRID - Asociación de Hemofilia de la Comunidad de Madrid  
 Förbundet Blödarsjuka i Sverige  
 Всеукраїнське Товариство Гемофілії  
 Bleeding Disorders Council of California (BDCC)  
 Bleeding Disorders Foundation of North Carolina  
 Coalition for Hemophilia B, Inc., The  
 Hemophilia Foundation of Michigan (HFM)

# BLEEDING DISORDERS

What patient groups told us – commentaries by category (57 categorised entries)

Interaction with drug regulators

## INTERACTION

### ✓ Regulatory partnership

#### Working with regulatory bodies

“Participation in advisory councils, forums, and public manifestos.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

“We engage with government bodies in forums where matters relating to our cause are discussed.”

«Participamos junto aos órgãos governamentais onde são discutidas pautas sobre nossa causa.»

*National bleeding-disorders patient group, Brazil*

“Administrative procedures and communications are conducted with the relevant authorities to ensure that patients receive the support and resources they require.”

«Se realizan trámites y comunicaciones con autoridades para que puedan brindar el apoyo y requerimiento que necesitan los pacientes.»

*Regional bleeding-disorders patient group, Peru*

#### Participate in Health Technology Assessment (HTA) and reimbursement reviews

“Advisory board of RIZIV [Rijksinstituut voor Ziekte- en Invaliditeitsverzekering – National Institute for Health and Disability Insurance] in connection with applications for reimbursement modalities for new medicines.”

“Adviesraad RIZIV bij aanvraag terugbetalingsmodaliteit van nieuwe geneesmiddelen.”

*National bleeding-disorders patient group, Belgium*

“CONITEC [Comissão Nacional de Incorporação de Tecnologias no SUS – National Committee for Health Technology Incorporation] meetings, Health Committees and Councils, and the Blood and Blood Products Coordination.”

«Reuniões da CONITEC, Comitês e Conselhos de Saúde, Coordenação de Sangue e Hemoderivados.»

*National bleeding-disorders patient group, Brazil*

“We have patient representatives on the specialist committees of Medicinrådet [Danish Medicines Council].”

»Vi har patientrepræsentanter i fagudvalg i Medicinrådet, og foreningen har, når relevant, kontakt med Amgros.»

*National bleeding-disorders patient group, Denmark*

“Participation in the G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee], RKI [Robert Koch-Institut – Robert Koch Institute], and Arbeitskreis Blut [Advisory Committee on Blood].”

„Teilnahme im G-BA, RKI, Arbeitskreis Blut.“

*National bleeding-disorders patient group, Germany*

“G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee].”

„Gemeinsamer Bundesausschuß (G-BA).“

*National bleeding-disorders patient group, Germany*

“We participate in TLV [Tandvårds- och läkemedelsförmånsverket – Dental and Pharmaceutical Benefits Agency] assessments relating to bleeding disorders.”

”Vi deltar i TLVs utredningar vad gäller blödningsrubbningsar.”

*National bleeding-disorders patient group, Sweden*

“NICE [National Institute for Health and Care Excellence] Technology appraisal committee submissions.”

*National bleeding-disorders patient group, UK*

#### Contribute to Marketing Authorisation submissions

“HAS [Haute Autorité de Santé – French National Authority for Health], contributions to the AMM [autorisation de mise sur le marché – marketing authorisation] processes for new medicines.”

«HAS, contributions pour les AMM des nouveaux médicaments.»

*National bleeding-disorders patient group, France*

#### Attend/facilitate meetings with policymakers/regulators

“Participation in councils and forums; endorsement of position statements; mobilisation of parliamentarians; submission of legislative proposals and regulatory recommendations; and engagement of public opinion and regulatory authorities.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

“CONITEC [Comissão Nacional de Incorporação de Tecnologias no SUS – National Committee for Health Technology Incorporation] meetings, Health Committees and Councils, and the Blood and Blood Products Coordination.”

«Reuniões da CONITEC, Comitês e Conselhos de Saúde, Coordenação de Sangue e Hemoderivados.»

*National bleeding-disorders patient group, Brazil*

“Participation in the G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee], RKI [Robert Koch-Institut – Robert Koch Institute], and Arbeitskreis Blut [Advisory Committee on Blood].”

„Teilnahme im G-BA, RKI, Arbeitskreis Blut.“

*National bleeding-disorders patient group, Germany*

“Meetings with health systems, the Ministry, national hospitals, and pharmaceutical companies that support our work.”

«Reuniones con sistemas de salud, Ministerio y hospitales nacionales, y con farmacéuticas que nos apoyan.»

*National bleeding-disorders patient group, Guatemala*

“We request meetings with regional authorities and hospital management bodies responsible for pharmaceutical distribution.”

"Chiediamo confronti con le autorità regionali e aziendali-ospedaliere che si occupano di distribuzione dei farmaci."

*Regional bleeding-disorders patient group, Italy*

“We engage in dialogue with the Regional Authority and with the ASP [Aziende Sanitarie Provinciali – Provincial Health Authorities].”

"Ci confrontiamo con la Regione e con le ASP."

*Regional bleeding-disorders patient group, Italy*

“Meetings at the MZ [Ministerstwo Zdrowia – Ministry of Health].”

„Spotkania w MZ.”

*National bleeding-disorders patient group, Poland*

“Community of Madrid, Department of Education, Department of Health.”

«Comunidad de Madrid, Consejería de Educación, Consejería de Sanidad.»

*Regional bleeding-disorders patient group, Spain*

## Impacting policy and legislation

**Participate in public hearings and consultations**

“By participating in public consultations.”

«Participando das consultas públicas.»

*Regional bleeding-disorders patient group, Brazil*

“... The Federation takes part in meetings, consultations, and collaborative projects aimed at strengthening timely access to medicines and innovative therapies for the community.”

«La Federación participa en espacios de diálogo y colaboración con instituciones del sistema de salud en México, como el IMSS, ISSSTE y otras autoridades sanitarias, para expresar las necesidades de las personas que viven con trastornos de la coagulación. A través de estas colaboraciones se comparten experiencias de pacientes, se identifican barreras de acceso al tratamiento y se promueven mejoras en los modelos de atención. Asimismo, la Federación participa en reuniones, consultas y proyectos colaborativos que buscan fortalecer el acceso oportuno a medicamentos y terapias innovadoras para la comunidad.»

*National bleeding-disorders patient group, Mexico*

“In the Community of Madrid, we are consulted on medicines and their importance for our patient community.”

«En la Comunidad de Madrid nos preguntan sobre fármacos y su importancia para nuestro colectivo.»

*Regional bleeding-disorders patient group, Spain*

“Testify before prescription drug affordability boards.”

*Local bleeding-disorders patient group, USA*

“We work with the state agencies that oversee our Medicaid program, our exchange and private insurance. We hold quarterly meetings with them to discuss patient access issues. We also provide letters and testimony to the board that oversees health care costs in our state.”

*Regional bleeding-disorders patient group, USA*

**Contribute to the drafting of public health legislation**

“Participation in councils and forums; endorsement of position statements; mobilisation of parliamentarians; submission of legislative proposals and regulatory recommendations; and engagement of public opinion and regulatory authorities.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

** Pricing & affordability advocacy****Engage in price-review board negotiations**

“Advisory board of RIZIV [Rijksinstituut voor Ziekte- en Invaliditeitsverzekering – National Institute for Health and Disability Insurance] in connection with applications for reimbursement modalities for new medicines.”

“Adviesraad RIZIV bij aanvraag terugbetalingsmodaliteit van nieuwe geneesmiddelen.”

*National bleeding-disorders patient group, Belgium*

“... meetings with state office of CMS [Centers for Medicare & Medicaid Services].”

*Local bleeding-disorders patient group, USA*

** Evidence-based patient input****Leverage patient stories and lived-experience narratives**

“The Federation participates in dialogue and collaboration forums with institutions within Mexico's healthcare system — including IMSS [Instituto Mexicano del Seguro Social – Mexican Social Security Institute], ISSSTE [Instituto de Seguridad y Servicios Sociales de los Trabajadores del Estado – Institute for Social Security and Services for State Workers], and other health authorities — to give voice to the needs of people living with coagulation disorders. Through these collaborative efforts, patient experiences are shared, barriers to treatment access are identified, and improvements to care models are promoted.”

«La Federación participa en espacios de diálogo y colaboración con instituciones del sistema de salud en México, como el IMSS, ISSSTE y otras autoridades sanitarias, para expresar las necesidades de las personas que viven con trastornos de la coagulación. A través de estas colaboraciones se comparten experiencias de pacientes, se identifican barreras de acceso al tratamiento y se promueven mejoras en los modelos de atención. Asimismo, la Federación participa en reuniones, consultas y proyectos colaborativos que buscan fortalecer el acceso oportuno a medicamentos y terapias innovadoras para la comunidad.»

*National bleeding-disorders patient group, Mexico*

** Diagnosis & medicines delivery****Participate in developing clinical guidelines**

“Participation in the G-BA [Gemeinsamer Bundesausschuss – Federal Joint Committee], RKI [Robert Koch-Institut – Robert Koch Institute], and Arbeitskreis Blut [Advisory Committee on Blood].”

„Teilnahme im G-BA, RKI, Arbeitskreis Blut.“

*National bleeding-disorders patient group, Germany*

## Institutional governance

### Ensure representation within governmental advisory bodies

“Participation in advisory councils, forums, and public manifestos.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

“We engage with government bodies in forums where matters relating to our cause are discussed.”

«Participamos junto aos órgãos governamentais onde são discutidas pautas sobre nossa causa.»

*National bleeding-disorders patient group, Brazil*

“We have patient representatives on the specialist committees of Medicinrådet [Danish Medicines Council].”

»Vi har patientrepræsentanter i fagudvalg i Medicinrådet, og foreningen har, når relevant, kontakt med Amgros.»

*National bleeding-disorders patient group, Denmark*

“Meetings with health systems, the Ministry, national hospitals, and pharmaceutical companies that support our work.”

«Reuniones con sistemas de salud, Ministerio y hospitales nacionales, y con farmacéuticas que nos apoyan.»

*National bleeding-disorders patient group, Guatemala*

“We maintain regular contact with the relevant offices of the Tuscany Regional Authority.”

“Prendiamo contatti regolari con gli uffici preposti della Regione Toscana.”

*Regional bleeding-disorders patient group, Italy*

“The Federation participates in dialogue and collaboration forums with institutions within Mexico's healthcare system — including IMSS [Instituto Mexicano del Seguro Social – Mexican Social Security Institute], ISSSTE [Instituto de Seguridad y Servicios Sociales de los Trabajadores del Estado – Institute for Social Security and Services for State Workers], and other health authorities — to give voice to the needs of people living with coagulation disorders.”

«La Federación participa en espacios de diálogo y colaboración con instituciones del sistema de salud en México, como el IMSS, ISSSTE y otras autoridades sanitarias, para expresar las necesidades de las personas que viven con trastornos de la coagulación. A través de estas colaboraciones se comparten experiencias de pacientes, se identifican barreras de acceso al tratamiento y se promueven mejoras en los modelos de atención. Asimismo, la Federación participa en reuniones, consultas y proyectos colaborativos que buscan fortalecer el acceso oportuno a medicamentos y terapias innovadoras para la comunidad.»

*National bleeding-disorders patient group, Mexico*

“With public authorities.”

«Ante las administraciones públicas.»

*Regional bleeding-disorders patient group, Spain*

### Working effectively within multi-stakeholder networks

“The Federation participates in dialogue and collaboration forums with institutions within Mexico's healthcare system — including IMSS [Instituto Mexicano del Seguro Social – Mexican Social Security Institute], ISSSTE [Instituto de Seguridad y Servicios Sociales de los Trabajadores del Estado – Institute for Social Security and Services for State Workers], and other health authorities — to give voice to the needs of people living with coagulation disorders.”

«La Federación participa en espacios de diálogo y colaboración con instituciones del sistema de salud en México, como el IMSS, ISSSTE y otras autoridades sanitarias, para expresar las necesidades de las personas que viven con trastornos de la coagulación. A través de estas colaboraciones se comparten experiencias de pacientes, se identifican barreras de acceso al tratamiento y se promueven mejoras en los modelos de atención. Asimismo, la Federación participa en reuniones, consultas y proyectos colaborativos que buscan fortalecer el acceso oportuno a medicamentos y terapias innovadoras para la comunidad.»

*National bleeding-disorders patient group, Mexico*

Actions & advocacy to access medicines

## ACTIONS & ADVOCACY

### Impacting policy and legislation

#### Influence healthcare policy and legislation

“Participation in councils and forums; endorsement of position statements; mobilisation of parliamentarians; submission of legislative proposals and regulatory recommendations; and engagement of public opinion and regulatory authorities.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

“The association submits recommendations.”

«L’association fait des suggestions.»

*National bleeding-disorders patient group, Morocco*

#### Mobilise parliamentarians and political leaders

“Participation in councils and forums; endorsement of position statements; mobilisation of parliamentarians; submission of legislative proposals and regulatory recommendations; and engagement of public opinion and regulatory authorities.”

«Participação em conselhos, fóruns, manifesto, mobilização de parlamentares, proposição de projetos de lei e regulação, mobilização da opinião pública e órgãos reguladores.»

*Regional bleeding-disorders patient group, Brazil*

### Pricing & affordability advocacy

#### Advocate for fair medicine pricing

“We are constantly vigilant in ensuring that the pharmaceutical industry sells its products at international prices and does not exceed those price thresholds — in other words, we engage in civic oversight to verify proper compliance and prevent any irregularities, in order to safeguard our annual state budget.”

«Estamos siempre pendientes que la industria venda sus productos al precio internacional y no se extralimite en el precio, es decir, hacemos veeduría ciudadana en que se aplique bien y no haya suspicacia, para cuidar nuestro presupuesto estatal cada año.»

*National bleeding-disorders patient group, Ecuador*

“Advocacy for reducing the economic burden.”

«Plaidoyer pour l’allègement du poids économique.»

*National bleeding-disorders patient group, Mali*

“Testify before prescription drug affordability boards.”

*Local bleeding-disorders patient group, USA*

“We work with the state agencies that oversee our Medicaid program, our exchange and private insurance. We hold quarterly meetings with them to discuss patient access issues. We also provide letters and testimony to the board that oversees health care costs in our state.”

*Regional bleeding-disorders patient group, USA*

#### Tracking and monitoring drug pricing/drug shortages

“We are constantly vigilant in ensuring that the pharmaceutical industry sells its products at international prices and does not exceed those price thresholds — in other words, we engage in civic oversight to verify proper compliance and prevent any irregularities, in order to safeguard our annual state budget.”

«Estamos siempre pendientes que la industria venda sus productos al precio internacional y no se extralimite en el precio, es decir, hacemos veeduría ciudadana en que se aplique bien y no haya suspicacia, para cuidar nuestro presupuesto estatal cada año.»

*National bleeding-disorders patient group, Ecuador*

## ↗ Improving access pathways

### Facilitate equitable access to medicines/healthcare

“Regarding access to treatments and medicines.”

«Sobre acesso a tratamentos e medicamentos.»

*Local bleeding-disorders patient group, Brazil*

“We have patient representatives serving on the specialist sub-committees of Medicinrådet [Danish Medicines Council] and the organisation maintains contact with Amgros [the Danish public hospital medicines procurement authority] where relevant.”

»Vi har patientrepræsentanter i fagudvalg i Medicinrådet, og foreningen har, når relevant, kontakt med Amgros.«

*National bleeding-disorders patient group, Denmark*

“When we recommend the procurement of medicines that have been established as safe and efficacious.”

«Cuando sugerimos que se compren medicamentos seguros y eficaces.»

*National bleeding-disorders patient group, Dominican Republic*

“Adverse events and medication availability.”

„Nebenwirkungen, Verfügbarkeit von Medikamenten.“

*National bleeding-disorders patient group, Germany*

“The first procurement and registration of factor VIII in our country was made possible through the efforts of our organisation in collaboration with the IGSS [Instituto Guatemalteco de Seguridad Social – Guatemalan Social Security Institute].”

«La primera compra y registro de factor VIII en nuestro país fue gracias al trabajo realizado por nuestra organización con el IGSS, Instituto Guatemalteco de Seguridad Social.»

*National bleeding-disorders patient group, Guatemala*

“We request meetings with regional authorities and hospital management bodies responsible for pharmaceutical distribution.”

"Chiediamo confronti con le autorità regionali e aziendali-ospedaliere che si occupano di distribuzione dei farmaci."

*Regional bleeding-disorders patient group, Italy*

“... The Federation takes part in meetings, consultations, and collaborative projects aimed at strengthening timely access to medicines and innovative therapies for the community.”

«La Federación participa en espacios de diálogo y colaboración con instituciones del sistema de salud en México, como el IMSS, ISSSTE y otras autoridades sanitarias, para expresar las necesidades de las personas que viven con trastornos de la coagulación. A través de estas colaboraciones se comparten experiencias de pacientes, se identifican barreras de acceso al tratamiento y se promueven mejoras en los modelos de atención. Asimismo, la Federación participa en reuniones, consultas y proyectos colaborativos que buscan fortalecer el acceso oportuno a medicamentos y terapias innovadoras para la comunidad.»

*National bleeding-disorders patient group, Mexico*

“We work with the state agencies that oversee our Medicaid program, our exchange and private insurance. We hold quarterly meetings with them to discuss patient access issues. We also provide letters and testimony to the board that oversees health care costs in our state.”

*Regional bleeding-disorders patient group, USA*

### Address disparities in access and management

“We work with the state agencies that oversee our Medicaid program, our exchange and private insurance. We hold quarterly meetings with them to discuss patient access issues. We also provide letters and testimony to the board that oversees health care costs in our state.”

*Regional bleeding-disorders patient group, USA*

### Improving access to rare/ultra-rare therapies

“The first procurement and registration of factor VIII in our country was made possible through the efforts of our organisation in collaboration with the IGSS [Instituto Guatemalteco de Seguridad Social – Guatemalan Social Security Institute].”

«La primera compra y registro de factor VIII en nuestro país fue gracias al trabajo realizado por nuestra organización con el IGSS, Instituto Guatemalteco de Seguridad Social.»

*National bleeding-disorders patient group, Guatemala*

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## Diagnosis & medicines delivery

### Ensure safe and effective medicine use

“When we advocate for the procurement of safe and efficacious medicines.”

«Cuando sugerimos que se compren medicamentos seguros y eficaces.»

*National bleeding-disorders patient group, Dominican Republic*

“Adverse events and medication availability.”

„Nebenwirkungen, Verfügbarkeit von Medikamenten.“

*National bleeding-disorders patient group, Germany*

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