Action Plan 2026

R.I.S.E for Rare

Uniting voices, driving change, and building a future of hope for all PLWRD

RARE
DISEASES
INTERNATIONAL

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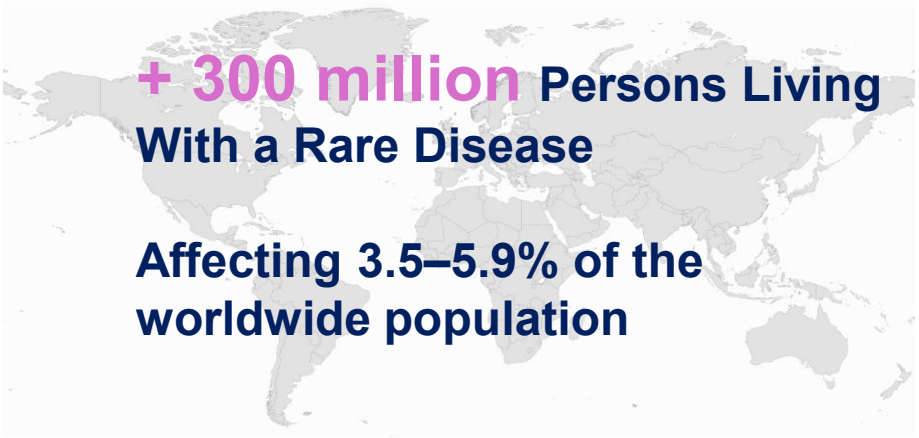
RARE DISEASES INTERNATIONAL

2026

1 About Rare Diseases International

2026

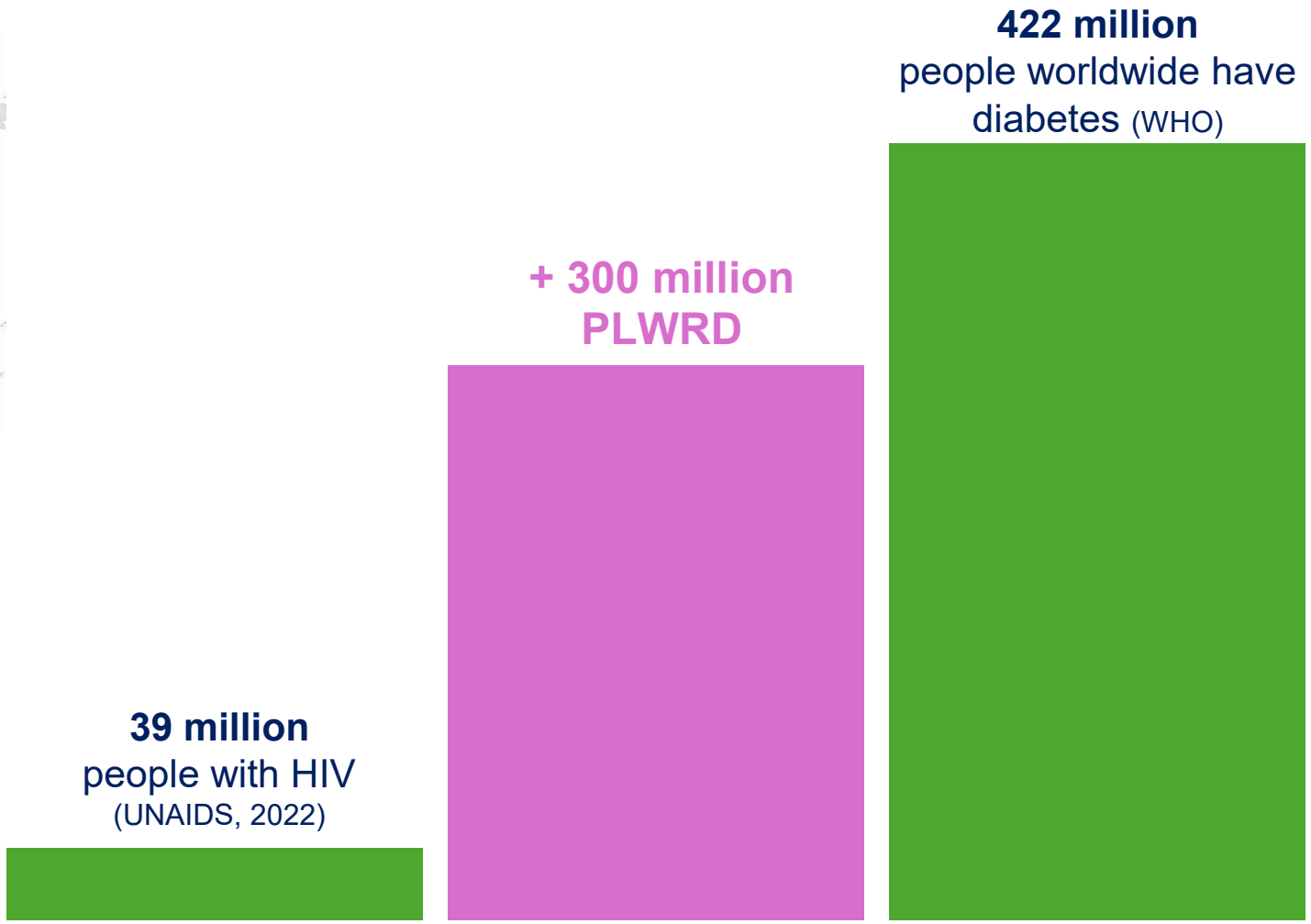
We are few; we are many



+ 300 million Persons Living With a Rare Disease

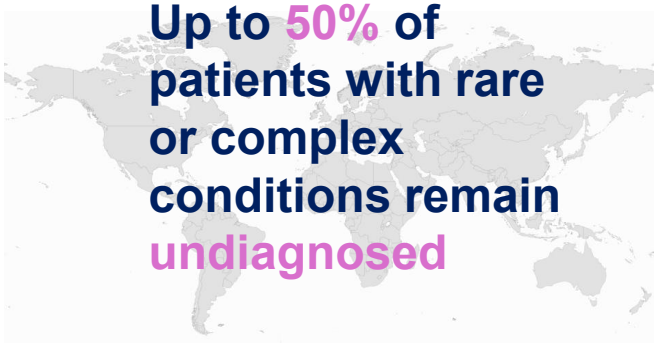
Affecting 3.5–5.9% of the worldwide population

RARE DISEASES INTERNATIONAL



- Nguengang Wakap et al., 2020 Eur J Hum Genet

Rare Diseases in Number



Up to 50% of patients with rare or complex conditions remain undiagnosed

70-80% are childhood onset

~80% of rare diseases are of genetic origin

More than 6000 diseases are classified as rare

Patients wait an average of 6 years and see 7 doctors before a diagnosis.

84.5% of RDs are extremely rare (<1 / 1 000 000)

Only 5% of RDs have an approved treatment

- Nguengang Wakap et al., 2020 Eur J Hum Genet

RARE DISEASES INTERNATIONAL

The Global Alliance of Persons Living with a Rare Disease and their families across all countries and all rare diseases

OUR VISION, MISSION & STRATEGIC GOALS

VISION

A world where Persons Living With a Rare Disease (PLWRD) and their families experience a better life through full recognition and support

MISSION

A strong common voice for PLWRD and their families around the world
+300 million people globally

STRATEGIC GOALS

1. **EMPOWER & ENGAGE**
   Build capacity and enhance member engagement to increase influence and reach
2. **SHAPE GLOBAL POLICY**
   Influence the global policy ecosystem to prioritize rare diseases and secure sustainable resources
3. **IMPROVE ACCESS TO CARE**
   Strengthen care pathways and networks to ensure treatment and support for PLWRD

IMPROVE EQUITY FOR PLWRD

RARE DISEASES INTERNATIONAL

OUR IMPACT STRATEGY

RARE DISEASES INTERNATIONAL

Our Vision	A world where Persons Living with a Rare Disease (PLWRD) and their families experience a better life through full recognition and support.						
Our Mission	Be a strong common voices for PLWRD and their families around the world						
Our Values	Global	Equitable	Inclusive	Diverse	Person-centered	Independent	Collaborative	Innovative
Our Strategic Goals	Improve **EQUITY** for PLWRD around the world						
	Community Engagement Enhance capacity and engagement of rare diseases community in the response		**Prioritization** of rare disease in policy **Financing** for adequate and sustainable resources for rare disease care			**Care pathways and networks** Improve access to treatment and care for PLWRD	
Our Strategic Pathways	**STRATEGIC ENGAGEMENT** **Building capacity** fellowships, toolkits and resources, trainings, grants **Strengthening regional alliances** regional events, dialogues and collaboration **Raising awareness** Rare Disease Day, communication and awareness raising		**GLOBAL ADVOCACY** **Shaping the global policy ecosystem** UN Resolution on PLWRD and their families UN Political Declaration on UHC **Advocating for PLWRD as a global advocacy priority** WHA Resolution, Global Action Plan **Partnering on a global and regional scale** UHC2030, NGO Committee for Rare Diseases		**GLOBAL PROGRAMMES** **Improving recognition of rare diseases** Operational Description of RD **Supporting timely diagnosis** Newborn screening, WHO EDL, Global Commission **Strengthening health systems** Global Network for Rare Diseases (GNRD) **Advancing equitable access** Access Working Group, GardAccess, WHO EML, IRDiRC	**STRATEGIC PARTNERSHIPS** **Fostering partnerships for impact** Foundations, Philanthropic organizations, public sector donors **Engaging the private sector** RDI Alliance of Companies **Sustainable resource development**	
Our Strategic Enablers	Governance and Membership		Team and organizational culture		Operations and Infrastructure	Financial Sustainability and Efficiency	

A Global Membership Base

140+

MEMBER organizations

Active in over

150 COUNTRIES

covering **6** continents

Who are our members?

- National Rare Disease Alliances
- Regional Alliances
- International Federations
- Organizations for Ultra-Rare Diseases
- National or Regional Organizations for one Rare Condition
- Other NGOs supporting Persons Living with a Rare Disease

RARE DISEASES INTERNATIONAL

Cross-Sector Collaborations and Partnerships

- As of today, RDI counts more than **30 committed partners,** who invest time, resources and expertise to working with us and supporting priority areas of work.
- RDI forges strategic partnerships and collaborations with the **entire RD ecosystem.**
- In turn, a **strong global platform** also supports the empowerment of the entire RD global PLWRD community

RDI’s Unique Role

What makes RDI unique	Impact
Global policy leadership	UN Resolution and WHA Resolution recognition of rare diseases as a global health priority
Direct engagement with WHO & UN, Member States	Forthcoming Global Action Plan development
Unified global PLWRD voice	United in diversity, One Voice for greater impact
Global partnerships	Advance sharing knowledge and equity in diagnosis and care through multistakeholder approach (governments, academia, clinicians & civil society)

RARE DISEASES INTERNATIONAL

RDI TEAM*

* As of 05.05.2026

RDI COUNCIL

* As of 05.05.2026

RARE DISEASES INTERNATIONAL

2 Action Plan 2026

RARE DISEASES INTERNATIONAL

2026

Vision 2026 and Strategic Directions

RISE for Rare: Recognize. Include. Strengthen. Evolve.
Uniting voices, driving change, and building a future of hope for all PLWRD

Vision 2026: R.I.S.E. for Rare

2026 marks a pivotal moment for RDI — a year to **RISE** and transform political recognition into real-world impact for people living with rare diseases.

R – Recognize
We build on the momentum of the 2025 WHA Resolution, acknowledging the global recognition of rare diseases as a global public health priority. This is our foundation for change.
I – Include
We advocate for embedding rare diseases into national health and social systems. Inclusion means empowering our members and amplifying the voices of PLWRD in every region.
S – Strengthen
We will reinforce our role as a trusted global convener — uniting governments, international agencies, patient leaders, academia, and industry. Together, we co-create sustainable solutions and strengthen governance, advocacy, and knowledge systems.
E – Evolve
We aim to support the evolution of the rare disease movement as it enters its next chapter- one of measurable progress, innovation, and hope. Through collective action, we will contribute improving diagnostics, care pathways, data ecosystems, and professional education.

Strategic Directions

- Build **stronger partnerships and governance** for solidity and sustainability
- Consolidate **Team** and Staff Development
- Enhance **evidence-knowledge** to contribute to the development of the GARPD (LCRD, Innovative Financing, Policy Watch/Observatory)
- Translate the WHA Resolution into regional and national action by strengthening **regional advocacy empowerement and Youth Leadership Programme**
- Drive global collaboration to share **expertise,** improve **diagnostics, and care pathways** (GNRD, strengthen **data (**registries in LMICs**),** ensure **patient centricity in research (**Erdera/CRN, IRDiRC**)** ecosystems, and advance **education for healthcare professionals** (Medscape)

RARE DISEASES INTERNATIONAL

Strategic Enabler

Organizational Development

PRIORITIES

1. Elaboration of RDI Strategy – 2026-2030
2. Team Empowerment and Staff Growth
3. Reinforce Governance, Transparency and Compliance
4. Pursue financial stability
5. Organizational Efficiency and Process Optimization

RARE DISEASES INTERNATIONAL

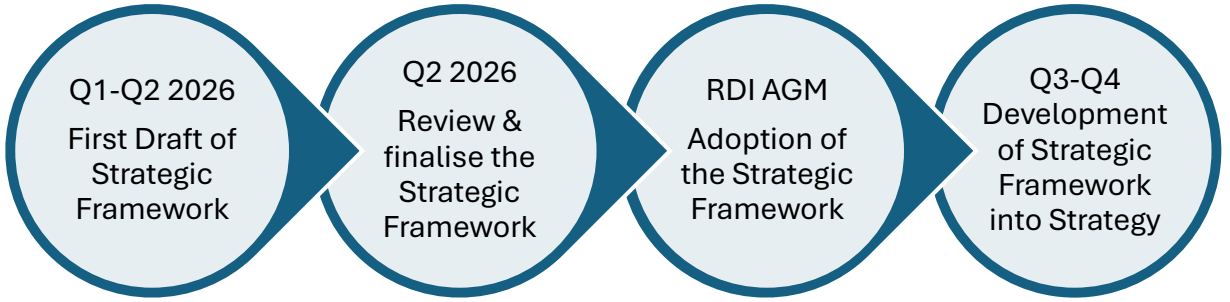
PRIORITY 1

Elaboration of RDI 2030 Strategy – 2026-2030

Objective:

RDI is prioritizing the development of a comprehensive **2030 Strategy** (2026-2030). This strategy will guide RDI's efforts over the next four years, focusing on strengthening global advocacy, expanding capacity-building initiatives, and contributing to improve equitable access to care for PLWRD in parallel and in alignment with the development of the Global Action Plan for Rare Diseases led by the World Heath Assembly Resolution for Rare Diseases. By aligning with the evolving needs of the rare disease community, particularly in underserved regions, RDI aims to pursue the lead of a truly global movement for rare diseases towards 2030.

2026 deliverables:



PRIORITIES 2, 3, 4, 5

Governance and Operations

Objectives

- Governance: Pursue the consolidation of RDI's Organization Governance transparency and compliance to the French Law
- HR: Enhancing Team cohesiveness and staff development
- Finance: Continue monitoring RDI's finances and treasury to ensure RDI financial health and stability
- Operations: Strengthen and professionalise organisational tools development & implementation that meet inclusivity needs (multilingual, accessible)

2026 deliverables:

Governance :

- Update existing policies and introduce new ones as needed
- Implement targeted training to staff in line with Compliance
- Organise a hybrid in person/online membership meeting to enable broader participation and consultation

Operations:

- Externalise an audit of RDI's current working systems and identify Team needs
- Prioritise & plan implementation of appropriate inclusive working tools multilingual, accessible for client management, project management, library database.

Finance :

Regular follow-ups comparing actuals to the budget, to ensure reaching the breakeven point by year-end and sustainability

HR :

- Implement the HR Package Oct. 2025
- Plan for 2 in-person meetings including team-building sessions
- Identify staff development needs and plan for dedicated training

PATHWAY 1

Strategic Engagement Programme

Build capacity and enhance members' engagement to increase influence and reach

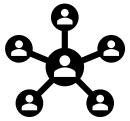
PATHWAY 1

Strategic Engagement Programme

PRIORITIES

1. Awareness: Mapping Rare, Rare Disease Day, Communication Campaigns

2. Building Capacity at the Global and Regional Level



3. Fostering Connections: Bringing our Members Together

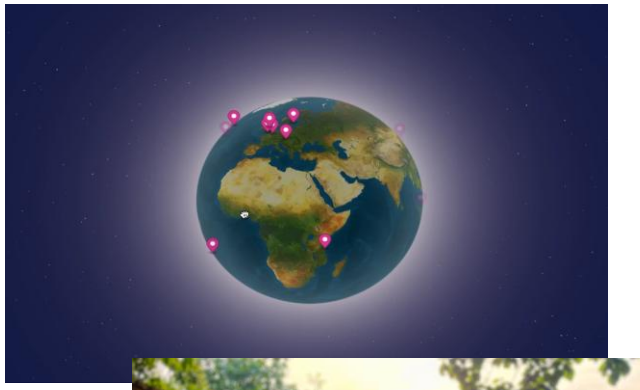
4. Empowering Members: Fellowship Programme

5. Youth Engagement

RARE DISEASES INTERNATIONAL

PRIORITY 1: Awareness

Mapping Rare



Objective:

Launched to mark Rare Disease Day 2024, Mapping Rare is designed to collect and share with the broader community **successful advocacy initiatives from RDI members around the world.**

2026 deliverables:

- 1 additional round of Mapping Rare (8-10 new entries) with an emphasis on geographical balance
- 1 thematic webinar based on the Mapping Rare projects to foster discussion and peer-to-peer learning
- Promotion of the Mapping Rare projects on social media through videos and interviews
- Aurora Awards to celebrate the achievements of our community, with 2 additional categories

PRIORITY 1: Awareness

Rare Disease Awareness

Objective: RDI's global awareness campaigns focus on the messages that rare diseases are not as rare as people think, rare diseases should be a global health priority and that PLWRD must be included in Universal Health Coverage.

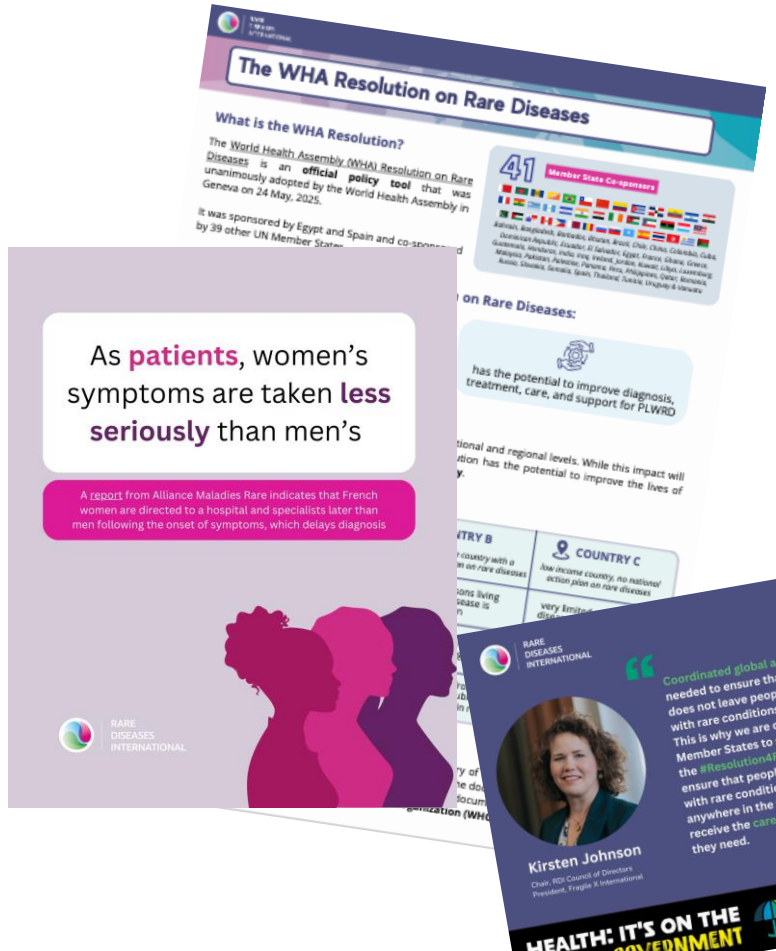
2026 deliverables:

Global Rare Disease Day event on 28 February in conjunction with RDI members & Global Youth

Restructuring of RDI website to highlight resources for and from our global community in an accessible way

2-4 publications on rare diseases on general interest media channels (blogs, newspapers)

Expansion of social media channels. Goal of 23,000 followers on Linkedin and 1,500 followers on Instagram



PRIORITY 2:

Build Capacity Globally and Regionally

Objective: By strengthening and uniting regional alliances, RDI ensures that PLWRD around the world have a strong collective voice and that our objectives are aligned for maximum impact.

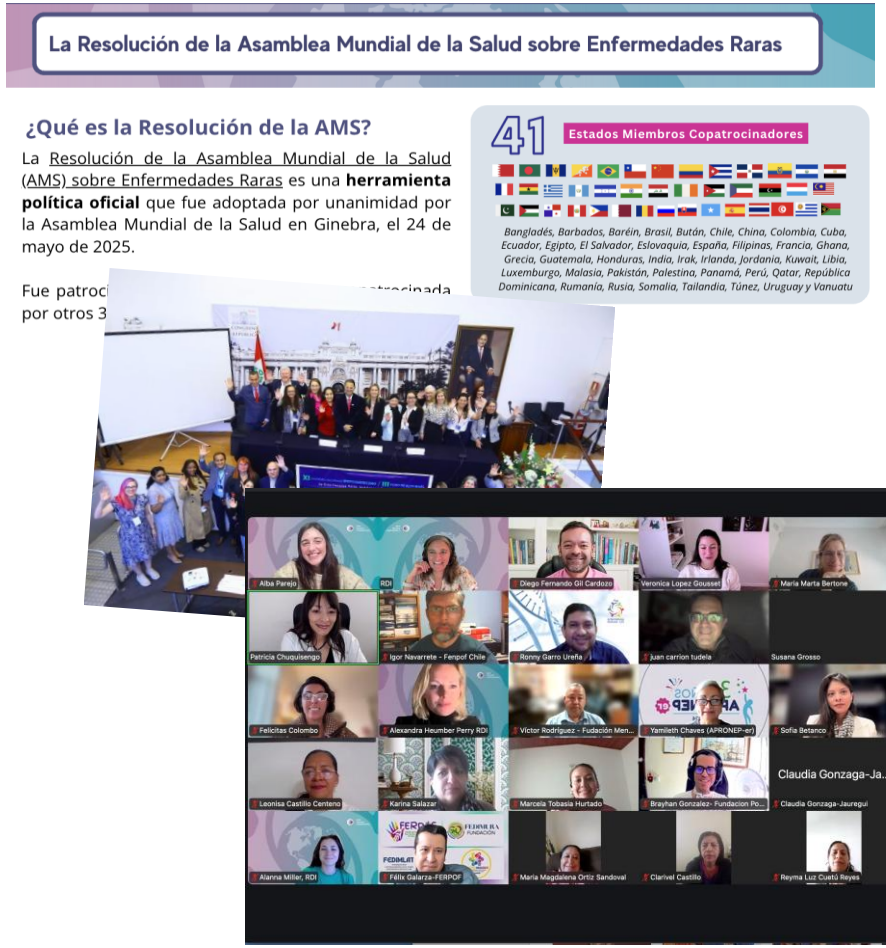
2026 deliverables:

Regional engagement framework to support implementation of the WHA Resolution through creation and support of Regional Task Forces (RTFs)

Partner with regional and national alliances to build capacity among PLWRD groups to ensure a strong common voice

Together with advocacy team, elevate regional voices at the global level by facilitating interactions with WHO and other bodies

Ongoing support for growth and development of regional alliances



PRIORITY 3: Create Connections

Bringing our Members Together

Objective: In-person and Virtual Meetings are an opportunity to consult with our community, align objectives, build capacity, and foster global connections between organizations.

2026 deliverables:

In-person membership meeting in Nairobi co-hosted by Rare Disorders Kenya (30/6/2026 - 2/7/2026)

Quarterly meetings to facilitate exchange of knowledge and find areas of synergy

3 virtual meetings on topics of interest to the community (e.g. mental health, carer support, employment)

Target of 20 new members in 2026, with specific outreach to underrepresented countries and conditions; Strategy for new collaborations with international groups

PRIORITY 4: Empower Members

RDI Fellowship Programme

Objective: In addition to RDI-initiated capacity building activities, RDI Fellowships **offer essential support for RDI members** by enabling patient advocates to benefit from international learning opportunities and fostering **a robust support network** within the PLWRD community.

2026 deliverables:

Partner with EURORDIS Open Academy and EUPATI to provide scholarships to attend in-person training

Explore new capacity building opportunities for members from within and beyond the rare disease community

Facilitate member participation in international panels, meetings, advisory committees and conferences

Continue and expand innovative learning solutions for members

PRIORITY 5:

Youth Engagement Programme

Objective: The RDI Youth Engagement Programme aims to integrate the passion and focus areas of young people into our advocacy efforts. By empowering youth, RDI will drive forward innovative solutions and ensure a brighter future for the rare disease community.

2026 deliverables:

In-person youth participation in RDI Membership meeting

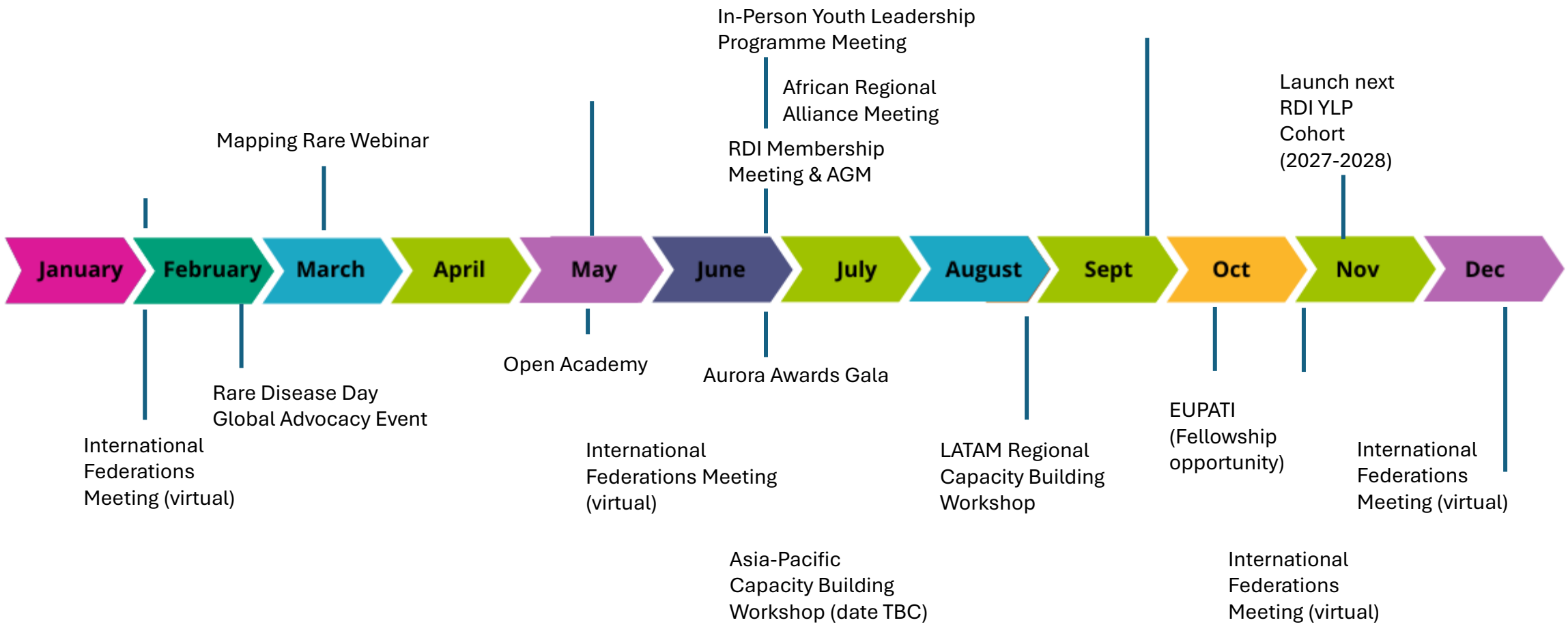
Facilitation of youth participation in international, regional and national fora

Mentorship and Capacity Building opportunities through our network, including support for youth-led projects

Development of the Youth Academy and framework for engagement of YLP alumni. Launch of the Next Cohort (2027-2028).

STRATEGIC ENGAGEMENT PROGRAMME

Member engagement opportunities in 2026



PATHWAY 2

Global Advocacy & Evidence Knowledge

Influence the global ecosystem to achieve sustainable resources and greater equity for PLWRD

PATHWAY 2

Global Advocacy & Evidence Knowledge

PRIORITIES

1. Support the Implementation of the World Health Assembly (WHA) Resolution
2. Facilitate the development of the Global Action Plan for Rare Diseases (GAPRD)
3. Develop evidence knowledge to enhance policy and health systems strengthening
4. Enhance integration of rare diseases into transversal policies and disease areas

RARE DISEASES INTERNATIONAL

PRIORITY 1: Support the WHA Resolution Implementation

Objective:

- Ensure that progress and political momentum around the WHA Resolution does not stall and that implementation proceeds efficiently and effectively, both at the national level (Member States) and at the global level (WHO).

2026 deliverables:

Collaboration with Member States & cosponsors and offer support through written communications & bilateral meetings

Formalization & activation of the Coalition for Advocacy in Rare Disease Equity (CARE) + public webinars

Support regional task forces (RTFs) to engage with their governments and WHO regional offices to advocate for implementation at the national level

Ongoing advocacy at WHO EB158/ WHA 2026 to ensure the Resolution and GAPRD remain a priority

World Health Organization

Seventy-eighth World Health Assembly

Agenda item 13.3 — WHA78.11

27 May 2025

Rare diseases: a global health priority for equity and inclusion

The Seventy-eighth World Health Assembly,

Having considered the report by the Director-General;[1]

Recognizing that a rare disease is often described as a specific health condition affecting fewer than 1 in 2000 individuals in general population, and that there are currently more than 7000 known rare diseases impacting more than 300 million people globally, with 70% of these conditions starting in childhood;[2] and that, while the frequency of most rare diseases can be described by prevalence, some rare diseases can be more precisely described by incidence;[3]

Noting that rare diseases are often complex and multisystemic, affecting multiple organs and leading to comorbidities, and that many of these conditions are chronic, progressive and can consequently result in serious disabilities and premature death;

Recognizing that some persons living with a rare disease have disabilities, which may have a greater impact on their health, and that they may also face various barriers, which may hinder their full and effective participation in society on an equal basis with others;[4]

Recognizing also that, in addition to the physical impact, some persons living with a rare disease, their families and caregivers may experience discrimination and psychosocial consequences, such as isolation, stigmatization and limited opportunities for social inclusion, which are often intensified by a lack of public awareness and knowledge and the absence, limit scope or poor implementation of policies and social support;

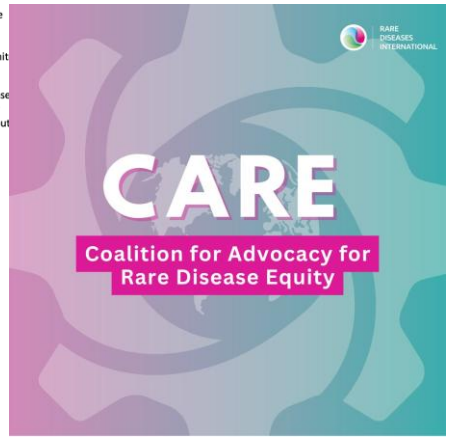
Recognizing further that persons living with a rare disease (including those whose disease undiagnosed), their families and caregivers may be psychologically, socially and economically vulnerable throughout their life course, facing specific challenges in several areas, including but not limited to physical and mental health, education, employment, financial well-being and leisure;

[1] Document A78/4.

[2] The Lancet Global Health. The landscape for rare diseases in 2024. Editorial (accessed 3 March 2025).

[3] Wang et al. Orphanet Journal of Rare Diseases. Operational description of rare diseases: a reference to improve the recognition and visibility of rare diseases (2024), pp. 19–334 (accessed 3 March 2025).

[4] Resolution WHA74.8 (2021).

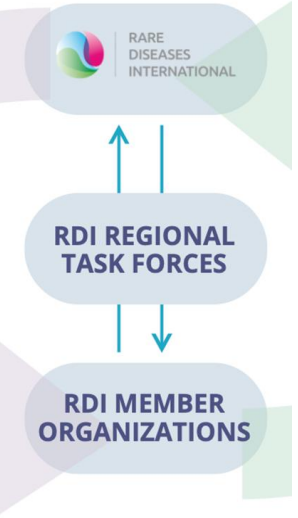


PRIORITY 2: Facilitate the development of the Global Action Plan for Rare Diseases (GAPRD)

Objective: Lead and coordinate the facilitation of the development of the GAPRD among the rare disease community, notably RDI members, CARE members, partners, and other experts. This will include advocating for an acceptable consultation process and preparing the content of the GAPRD from the **perspectives of the RDI community**, engaging with stakeholders to develop **key pillars.**

2026 deliverables:

- Facilitate input from PLWRD community to the GAPRD consultations through Regional Engagement Framework
- Involvement of CARE members in the development of the GAPRD through the steering group, RTFs, etc
- Publication of RDI position paper(s) (endorsed by the CARE) on the GAPRD consultations & input
- Collaboration with WHO Secretariat & experts to lend support and expertise



PRIORITY 3: Develop evidence knowledge to enhance policy & health systems strengthening

LCRD, Innovative Financing, RD Policy Observatory

Objective: Ensure that advocacy and policy efforts are grounded in best available evidence, through generation of robust evidence knowledge to fill current gaps and support health systems strengthening.

2026 deliverables:

RDI-LRCD

- Progress of LCRD working groups
- Drafting, consultations, and submission of LCRD final report to the Lancet
- Incorporation of the patient perspective in LCRD produts
- Profiling of the LCRD

Innovative financing solutions

- Analytical report on innovative financing models
- Webinar series
- Preparing hackathon
- Drafting policy recommendations

Global RD Observatory

- Collection of policy-relevant data in collaboration with Orphanet and other partners with validation by MoH
- An online platform (web interface)
- Launch Q4 2026/Q1 2027 with ~24 countries, scale-up in 2027

PRIORITY 3: Develop evidence knowledge to enhance policy & health systems strengthening

LCRD Timeline

PRIORITY 4: Enhance the integration of rare diseases into transversal policies

UHC, PHC, mental health, human rights, rare NCDs

Objective: In addition to RD-focused events, initiatives, and activities, RDI is aiming to **increase the profile of RDs and RDI** itself with other key organizations and in key events in the **broader health fields** such as Human Rights, UHC, NCDs, Rare NCDs, Primary Care, Genomics, etc.

2026 deliverables:

- Engagement with key organisations such as OHCHR, UHC 2030, NCDA, UICC, WEF
- Participation and networking at conferences and summits on broader health topics to bring rare disease considerations to the discussion (WHA side events, WHS)
- Engagement with patient organizations representing diverse diseases / health topics
- Connections through the WHO relevant technical departments

PATHWAY 3

Global Programmes

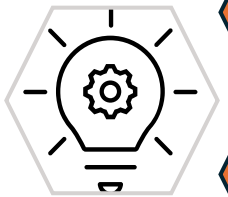
Lead and partner in global projects that advance equity for PLWRD and their caregivers

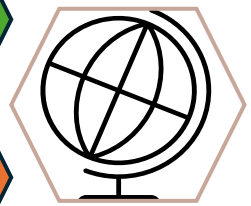
Global Programmes: Cuts across all RDI strategic directions
Central to all points of the GAPRD

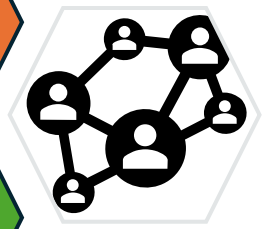
RDI Strategic Directions

- Translate WHA Resolution to action plan
- Evidence-based knowledge
- Stronger partnerships
- Drive Global Collaboration (Access, diagnosis, care, treatment)

- Education and Training
- YLP
- Pairing Model
- Care Pathway
- LCRD
- Registry Task Force
- Collaborations: ERDERA, IRDiRC, PQMD
- Engagement with Global Nursing Network, IHF
- Innovative Financing







- Comprehensive framework to foster equitable access to timely, cost-effective, affordable, available, accurate diagnosis and evidence-based treatments and adequate management
- Strategies for improving data collection, research and surveillance
- Guidelines for the establishment of national and regional registries
- Guidelines to improve access to affordable and equitable healthcare services, the essential health products needed for accurate diagnosis and effective treatment, as well as an accompanying process for accountability and monitoring to track implementation progress

WHA Global Action Plan on Rare Disease

PATHWAY 3

Global Programmes

PRIORITIES

1. Improve equitable access to diagnosis and care

2. Advance participation in rare disease registries for LMIC

3. Ensure patient-centricity in the ecosystem for acceleration of research

4. Raise awareness and knowledge of rare diseases with primary care providers

RARE DISEASES INTERNATIONAL

PRIORITY 1: Improve equitable access to diagnosis and care

Objective: Generate and share knowledge to foster global collaboration to improve diagnosis and care.

2026 deliverables and outcomes:

- GNRD: Produce a Patient Care Pathway Framework (WG)
- GNRD: Launch of Pairing Model & case discussions
- GNRD: Launch and conduct a Task Force on Rare Disease Centers
- GNRD: Scalability / sustainability plan development for Cross border collaboration
- GNRD: Dissemination Webinars for members, multi-stakeholders and public
- GNRD: Virtual platform development

Cross-project activities

- Participate in the PQMD RD work group and activities
- Development of new partnerships and initiatives for access
- Contribute to the LCRD and implement recommendations
- Inform the GAPRD through task forces' recommendations
- Participate in the Innovative Funding project

PRIORITY 2: Advance participation in rare disease registries for LMIC

Objective: Develop a framework for creation of, and participation in, rare disease registries for LMICs and under-represented countries

2026 deliverables and outcomes:

Convene a task force to create recommendations for RDI's role in facilitating creation of, and linking, registries (new partnerships)

Summarize task force recommendations and develop action plan

Creation of linkages between existing registries and participants (new partnerships)

PRIORITY 3: Ensure patient-centricity in the ecosystem for acceleration of research

Objective: To facilitate the involvement of PLWRD in clinical research, with a focus on LMIC

2026 deliverables and outcomes:

Summary of CRN conference and action plan for 2026

ERDERA: Continued leadership on WP25 and participation in WP1, 10, 23 and 24
IRDiRC: Participation in work groups (TBD)

ERDERA
EUROPEAN RARE DISEASES RESEARCH ALLIANCE

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

PRIORITY 4: Raise awareness and knowledge of rare diseases with primary care providers

Objective: Facilitate the education and training of HCPs on diagnosing and caring for PLWRD

2026 deliverables and outcomes:

Global Nursing Network
Rare Diseases

Participation in the development and delivery of the Medscape Master Class for RD in 2 regions (MENA, Latam – 2026/2027)

Participation in the development and delivery of the Rare Disease Winter School (collaboration with NewGiza University, Egypt and Heidelberg University Hospital, Germany)

Strengthen engagement with the Global Nursing Network

PATHWAY 4

Partnerships

Mobilizing diverse resources and partnerships to deliver a sustainable impact for PLWRD

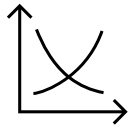
Strategic Partnerships

Objective: Foster global and cross-sector partnerships for impact and develop sustainable resource streams

PRIORITIES

1. **Global Network Growth**: leverage our current network and forge new strategic partnerships

2. **Collaborative Projects**: Develop joint initiatives with RD foundations, governments, and NGOs to enhance policy development, capacity-building and more RD-related programs



3. **Funding and Resources**: Secure additional funding streams and resources to scale up and diversify support for RDI's activities

4. **Nurture and expand the Alliance of Companies**: Maintain the support of the AoC partners while deepening the existing partnerships and seeking new partners

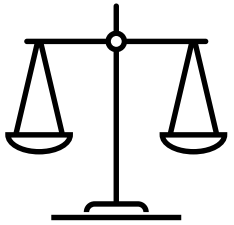
Cross-sector Collaborations and Partnerships

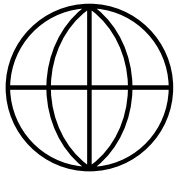
- As of today, RDI counts more than **30 committed partners,** who invest time, resources and expertise to working with us and supporting priority areas of work.
- RDI forges strategic partnerships and collaborations with the **entire RD ecosystem.**
- In turn, a **strong global platform** also supports the empowerment of the entire RD global PLWRD community

Our partners are part of our story

Relationship-centric approach

Balanced funding model



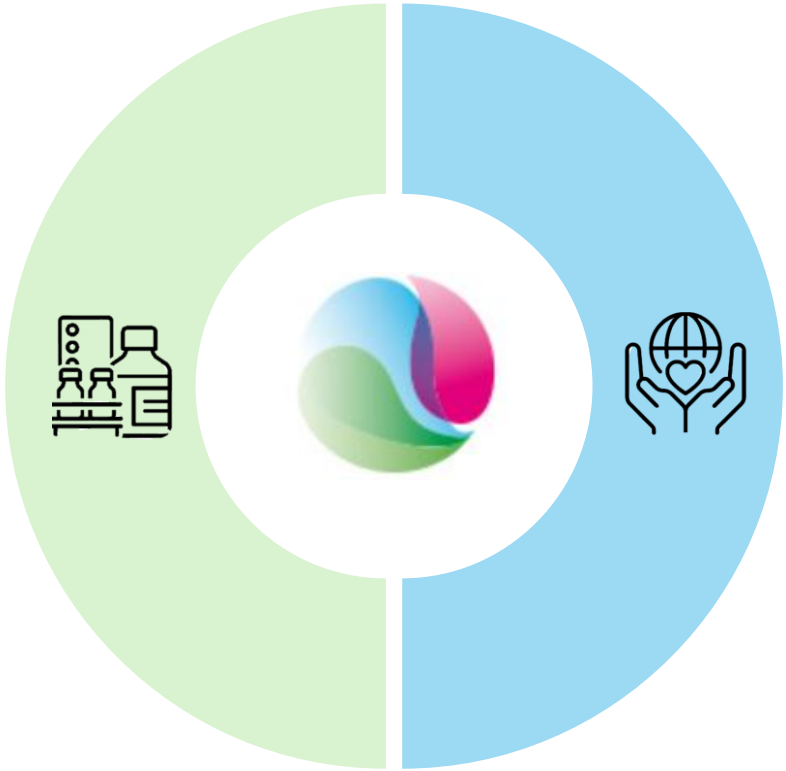


A strong and independent global voice for PLWRD & their families

Fundraising Model

Alliance of Companies

- Core support
- Pharmaceutical and biotech industry
- Annual Membership
- Support other RDI Programmes (SEP/GNRD)



In-Kind donations

From non-for-profit sector, institutions & academics

Non-for-profit sector partners

-Strategic mid/long-term partners
-Project-based partners

Tech and other industries

New sector to explore in 2026

Partnerships 2025

Alliance of Companies (private sector)

- 18 Partners (Private sector)
- CORE support + SEP/GNRD
- No support for Advocacy/Lancet

Occasional Partnerships

- For certain events (example WHA side-event co-hosts)
- Non-healthcare organizations (Instituts, foundations, NGOs)

Mid-Long term partners

- Institutes/Foundations/NGOs
- In development: secure 3 long-term partnerships

Strategic Partnerships

Objective: Foster global and cross-sector partnerships for impact and develop sustainable resource streams

2026 deliverables:

Deepening Impact with AoC partners and expand the alliance
Increase bilateral meetings with existing partners
Establish 2 -3 new partners (20/21 AoC)

Improve the diversification of funds
By establishing 1 new long-term partner

Administrative & Comms
Develop/update policies & procedures on fundraising
Communicate on partnerships

Explore innovative fundraising and partnership models
A partnership with the tech/other industry
Participate in networking events

Maintaining sustainable partnerships
By involving long-term partners with projects, and establishing quarterly meetings

Thank you!

RARE
DISEASES
INTERNATIONAL