RDI ACTION PLAN
2020-2021
STRATEGIC PRIORITIES

1. Strengthen RDI as a well-governed and sustainable organisation
   GOVERNANCE
   FINANCE, HUMAN RESOURCES & ADMINISTRATION

2. Build a legitimate Global RD Patient Alliance
   MEMBERSHIP
   AGM
   PROGRAMMES

3. Build Capacities of Members and Patient Advocates
   CAPACITY BUILDING

4. Advocate for RDs to be an International Public Health Priority and Raise Awareness of RDs worldwide
   AWARENESS
   INTERNATIONAL ADVOCACY

5. Support Global Initiatives to Improve Access to Diagnostics, Treatments & Care
   WHO CGN4RD
   WHO DEFINITION FRAMEWORK & KEY GLOBAL FIGURES
   IRDIRC
   GLOBAL COMMISSION
GOVERNANCE

- F2F Meeting of Council (B/B to RDI 6th Annual Meeting / Level-3 Conferences)
- Officers TC every other month
- **Review By-Laws and revisit Membership Criteria**
- Council Nominations & Elections (March - April 2020)
- Annual Meeting (General Assembly + Membership Meeting + Global Meeting)
  - Adoption Annual Activity & Financial Report 2019
  - Adoption Annual Work Programme: Action Plan & Budget 2020
  - Presentation of By-Laws
  - Presentation of new member of Council (KP Tsang and Jesus Navarro end of mandate)
- **RDI Advocacy Committee**
- Designate leads from the Council for strategic priorities and main projects (ie.Lisa Sarfaty: Capacity Building; Yann Le Cam: International Advocacy, KP Tsang: Membership and Fellowship Programme)

1. Strengthen RDI as a well-governed and sustainable organisation
FINANCE: INCOME STREAMS

- EURORDIS In-Kind contribution
- **Alliance of Companies for Patient-Centred Action (Corporate Partnership):** Engage 11 companies (+2 from 2019) including 2 or more from outside pharma/biotech.
- Fellowships Programme (Corporate Partnership): Aim to support between 30 and 40 fellows
- Conference Programme (Corporate Partnership): Aim to support 3 Conferences *Level 3* and 8 conferences *Level 2*
- Membership fees
- Economic Valorisation of Volunteer time (excluding Council meetings time)
- **Project restricted funding:** aim to secure funding for CGN4RD and expand resources for International Advocacy
RDI ALLIANCE OF COMPANIES

A platform for companies to support the rare disease cause internationally and to increase mutual understanding of issues that are important for rare disease patients and different industries.

The Alliance aims to:

- Build an international, cross-sector network of companies working in areas that affect rare disease patients. Examples include pharmaceutical and biotechnology, medical equipment and devices, research, information technology, data mining and management, artificial intelligence, transport and logistics.

- Exchange perspectives on issues vital to rare disease patients
  - Meet twice (1 virtual and 1 face-to-face if possible) for RDI advocacy update and input on specific projects

- Stimulate companies across many industries to address patient needs
  - Survey results, key findings, member feedback on patient needs

- Provide financial support to advance RDI’s mission
FINANCE: EURORDIS SUPPORT

EURORDIS Support based on 5 years MoU 2019-2023 – 2020 is 2\(^{nd}\) Year

EURORDIS continues to provide human and material resources

a) employ 0.9 FTE staff: RDI Director, Paloma Tejada

b) provide 0.5 FTE staff for RDI international advocacy: Public Affairs Manager, Clara Hervas

c) provide financial & administrative services for management of accounts (Eurordis Chief Finance Officer, Patrice Reigner and his team)

d) provide hosting, maintenance and update of website

e) EURORDIS office space at VOZ office in New York fully provided to RDI

f) EURORDIS volunteer Representatives for International Advocacy to UN System in NY: Claudia Hirawat and Rasha Alnaibari
HUMAN RESOURCES

From EURORDIS Support:
• RDI Director, Paloma Tejada - 0.9 FTE (based Geneva)
• RDI Public Affairs Manager, Clara Hervas - 0.5 FTE (based Brussels)

From RDI Human Resources: employee and self-employed
• RDI Outreach & Engagement Manager, Hlawulani Mkhabela - 1 FTE (based Paris) (employed by RDI as of 01/03/2020)
• RDI Corporate Relations Advisor, Jill Bonjean – 50 to 70 days (self employed)
• RDI HealthCare & CGN4RD Advisor – 96 days (self employed) – TBC / funding
MEMBERSHIP

- **RDI 6th Annual Meeting**: General Assembly and Membership Meeting (for members only) and Global Meeting (open to all stakeholders)
- **Expand membership base from ~ 60 to 70**
  - International Federations
  - Upcoming applications: HAEi, Tuberous Sclerosis International, Duchenne World, IPWSO, OIFE, Global Skin
  - To pursue: CF Worldwide, Rett Syndrome International, FOP, Alpha 1, Dravet Fundation
  - National Alliances
    - Upcoming applications: Kenya, Ivory Coast
    - To pursue: Chile, Uruguay, South Korea, Thailand, Turkey, Algeria, Israel
- Call for Annual Membership fees: aim for 80% fee payment
- **Develop and actively animate RDI virtual space (Facebook Workplace)**

2. Build a legitimate Global RD Patient Alliance
CONFERENCE PROGRAMME
FELLOWSHIP PROGRAMME

Level 1:
Logo, Communication & Promotion
• ReACT Congress and IRDiRC, Berlin (postponed Jan 2021)
• Global Summit on Rare Diseases & Medicine, Dubai, UAE (Nov)
• Geneva Health Forum (postponed Nov)

Level 2:
Logo, Communication & Promotion
Programme Committee and RDI Speakers
• ECRD 2020 Stockholm (moved online)
• African Patient Advocates Meeting
• China Rare Disease Conference
• World Orphan Drug Congress, USA (August) and Europe (Nov)
• APEC Meetings in Malaysia, Japan, China, Korea (tbc)
• ICORD XV, Georgia

Level 3:
Logo, Communication & Promotion
Programme Committee and RDI Speakers
Fellowships for patient advocates to attend
• RDI 6th Annual Meeting, Geneva (moved online)
• APARDO/APEC Meeting in Malaysia or Singapore (tbc)
• ALIBER/APEC Meeting in Peru or Argentina (tbc)
EXPERTS FACULTY

Launch in 2020 to tap into rare disease expertise from outside RDI membership

Specific objectives:

- To provide RDI with a pool of quality speakers for international conferences, meetings and workshops who are relevant to the specific need, topic or country/region

- To provide RDI members with an expert resource for advice, consultation, technical assistance, insight or involvement in local activity or specific project

Activities 2020:

- Identify experts to match RDI mission needs; cultivate pool of potential external experts; invite candidates; review applications; welcome and induct experts; build an online directory of Experts Faculty on RDI website

- Identify members of Experts Faculty most relevant to experience sharing and capacity building need in Conferences Level 2 & 3

- Liaise with members of Experts Faculty to attend relevant meetings on behalf of RDI, contribute to review policy documents or contribute to operational activities
CAPACITY BUILDING

- **Perform a needs Assessments** amongst Members to identify priority areas for capacity building, best instruments (F2F, Online, Fellowships, Mentorship, Twinning, etc), existing best resources to promote, new resource to create

(The assessment performed in 2020 will inform the design of a capacity building programme to be developed over several years, starting in 2021)

- **Information and Networking**
  - Monthly Newsletter
  - Facebook Workplace
  - Targeted Mailouts

- **Training and Exchange** (across all activities)
  - Webinars with members (ie. APEC RD Network Advocacy tool kit; UHC implementation; CGN4RDs)
  - F2F Meetings and Workshops

- **Fellowships** for patient representatives to attend meetings and conferences
UNIVERSAL HEALTH COVERAGE

- Tool kit to support RDI members to advocate UHC integration of RD in national policies and regional policies (ie. APEC Action Plan on RD, EU RD Policy Framework)
- Coordinate a campaign with National Alliances to reach out to their PM in Geneva, MoH and MoFA on the lead to UHC Day (Dec 12)
- Communicate success stories of RDs in UHC at national level on UHC Day
- Inject UHC in National Conferences (China, Georgia), Regional Conferences (APARDO; APEC; ECRD 2020) and International Conferences (WODC 2020)
- Leverage RDI membership in UHC2030

4. Advocate for RDs to be an International Public Health Priority and Raise Awareness of RDs worldwide
UN GENERAL ASSEMBLY RESOLUTION ON RARE DISEASES

• “Main Asks” for the UN GA Resolution on Rare Diseases drafted by RDI Advocacy Committee, reviewed and validated by RDI Members; reviewed and adopted by the NGO Committee for Rare Diseases

• Rationale and Outline of UN GA Resolution on RDs drafted by RDI Advocacy Committee, reviewed and validated by RDI members at large; reviewed and adopted by the NGO Committee for Rare Diseases

Post-COVID 19 crisis activities:

• Outreach and networking campaign to Permanent Missions in New York (Council members, volunteers, Secretariat), form a core group of Member States, establish Friends of Rare Diseases

• Coordinate an outreach campaign with National Alliance to Permanent Missions in New York, MoH and MoFA

• Leverage RDI membership in NGO Committee for Rare Diseases
RARE DISEASE DAY

• Organise a **RDI Rare Disease Day Event in Geneva**
  • Photo Exhibit at UN Office during Human Rights Council 43rd Session (Feb 24 – March 6, 2020)
  • Objective: Raise awareness amongst Permanent Missions, UN officials and Member State delegations attending the Human Rights Council High Level Segment
  • In collaboration with RD National Alliances of Italy (UNIAMO) and Cyprus rare disease national alliance of disease patient organisation (Unique Smiles)
  • Sponsored by Permanent Mission of Cyprus and Permanent Mission of Brazil
• Engage **RDI member National Alliances to join as Official Partners of RDD 2020**
• Promote RDI members events in RDD via FB Workplace to learn from each other
OPERATIONAL ACTIVITIES WITH WHO

WHO - RDI MoU two main specific areas of collaboration 2020:

1. **WHO Collaborative Global Network for Rare Diseases**: Needs Assessment Study (2020-2021)

2. **WHO Framework of operational descriptions for rare diseases and key figures**, to inform WHO and Member states when considering policy shaping and implementation (starts 2020)

Other areas of collaboration between WHO and RDI:

3. **Lists of Essential In Vitro Diagnostic Tools (EDL) and of Essential Medicines (EML)** (starts 2020)

4. **Support Global Initiatives to Improve Access to Diagnostics, Treatments & Care**
WHO CGN4RD: NEED ASSESSMENT

Vision:

- WHO Collaborative Global Network for Rare Diseases (WHO CGN4RD) connect major university research hospital centres (hubs) that have multi-disciplinary rare disease specialist capacities at regional and global levels.

- Structure grouping of rare diseases under thematic areas (under “global programmes of care”), inclusive of all rare diseases, leaving no one behind.

- Global RD Hubs will be internationally-recognised University Medical Centres in the field of rare disease, who provide healthcare, research, education and technology

- Selection and identification of Global RD Hubs will be through endorsement of Member States and an independent, volunteer international accreditation system

WHO RD DEFINITION FRAMEWORK

**Objective**: RDI and WHO lay down a framework of operational functional description of RD and prevalence and incidence figures

**Activities 2020**:

- Put together an RDI Ad hoc group composed of some Experts Faculty, ICD-11 RD Task Force and RDI Advocacy Committee (under the RDI Council)
- RDI Ad hoc group to define the scope of a paper which provides the operational functional description, terminology and prevalence and incidence figures
- Partner with INSERM, a collaborative centre of WHO for ICD, and Orphanet, leader of ICD-11 RD Task Force
- Bibliographic review and analysis
- State of play in term of definitions, prevalence, incidence, population
- Review and adoption by Ad hoc group
- Consultations with WHO at each step
WHO ESSENTIAL MEDICINES AND DIAGNOSTICS LISTS

**Objective:** Inclusion of more in vitro diagnostics and therapies for RD, considered essential by relevant disease communities, into the WHO Essential List

**Method:** in liaison with WHO EDL, EMP, Qualification units and building on RDI members:

- Promote the opportunity to all potential applicants (ie. patient groups, clinical leads, hospitals and company market holders.
- Present and explain EML and EDL programme, application and processes to RDI members at large

**Activities 2020:**

- 2 webinars with WHO EDL and EML Secretariat
- 2 webinars with case studies / patient groups with experience
- Progressively involve relevant Expert Faculty members into analysis of current essential lists, guidance to RDI members, peer to peer learning, liaison with WHO
- Attendance by RDI members and Expert Faculty of EDL and EML Expert Committees open to stakeholders

**Coordination** with IRDIRC Rare Disease Treatment Access Working Group
Support RDI as a member of the International Rare Disease Research Consortium (IRDIRC) Consortium Assembly, which is the main governing body of the Consortium

- Durhane Wong Rieger to replace Ritu Jain as RDI representative in IRDIRC

Support RDI as a member of IRDiRC Patient Advocacy Constituent Committee (PACC) which aims to address systemic issues that apply to all members and all rare diseases, articulates points in the diagnostic and therapeutic development process where patient involvement is crucial, measures the success and impact of that patient involvement (particularly in areas contributory to the IRDiRC goals)

- Coordination between 13 out of 15 patient representatives which are RDI members

Support RDI Chair as the Co-Chair of IRDIRC Access to Medicines Working Group, which aims to create a list of standard-of-care products for rare diseases and to identify the barriers to accessing rare disease drugs, particularly in LMIC populations, in:

- Liaison with RDI work on WHO EML and EDL
- Follow-up work to RDI -IFPMA Round Table on Access to Medicines in LMICs
GLOBAL COMMISSION TO END THE DIAGNOSTIC ODYSSEY FOR CHILDREN WITH RARE DISEASES

The Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease is a multi-disciplinary group of experts committed to shorten the time to diagnosis for children with rare diseases. Based on 2019 set of recommendations, the Global Commission implements awareness campaign and pilot projects for faster and more accurate diagnostics for children and adults.

- 5 out of 15 members of the Global Commission are RDI member organisations

**Objective:** promote greater integration of RDI in Global Commission and contribute more to its activities

**Activities 2020:**
- Form an RDI extended team of RDI members, staff and members of Experts Faculty
- Promote engagement of RDI members within Global Commission activities, including pilots
- Promote engagement of RDI members into the Patient Empowerment Education & Awareness Campaign (Track 1 Policy Recommendations: Empowering Patients and Families)
- Promote the Technical Proposal in support of the RDI-WHO need assessment and conceptual framework for WHO CGN4RD to become a new pilot of Global Commission
RDI MILESTONES 2020

**January**
- 1st Alliance of Companies online meeting

**February**
- RDI Rare Disease Day Event: A Rare Photo Exhibit at UN Geneva during 43 HRC

**March**
- CGN4RD Webinar Internal Communication
- May/June Kick off CGN4RD Webinar series 6 WHO Regions
- May RDI General Assembly, Membership Meeting & RDI Global Meeting Online (May 18 & 19)

**April**
- UHC main messages & National Implementation Advocacy Committee

**May**
- June 2nd Alliance of Companies online meeting
-—who-who
- August/Sept RD Definition Framework & Global figures Internal Communication
- August—Sept launch UHC tool kit and NA Advocacy Campaign to PMs in GVA and MoH

**June**
- September—October/November—RDI submits CGN4RD Needs Assessment and RD Definition Framework & key figures paper to WHO
- October/Nov Level-3 Supported Conferences APARDO Meeting ALIBER Meeting

**July**
- July UNGA Resolution on RD—Key Asks & Outline
- November RDI submits CGN4RD Needs Assessment and RD Definition Framework & key figures paper to WHO

**August**
- August—Sept Launch UHC tool kit and NA Advocacy Campaign to PMs in GVA and MoH

**September**
- October/Nov Level-3 Supported Conferences APARDO Meeting ALIBER Meeting
- October/Nov Launch UHC tool kit and NA Advocacy Campaign to PMs in GVA and MoH

**October**
- November RDI submits CGN4RD Needs Assessment and RD Definition Framework & key figures paper to WHO

**November**
- November RDI submits CGN4RD Needs Assessment and RD Definition Framework & key figures paper to WHO

**December**
- December 12 UHC Day
- WHO EDL March: info next EDL update postponed
- July/Aug: EDL 4th submission cycle
- WHO EML Nov/Dec : Info next EML update March/Apr 2021 23rd Experts Committee on EML
GOVERNANCE
RDI Council Directors
RDI Council Officers

EXECUTIVE
RDI Secretariat

ADVISORY COMMITTEE
RDI Advocacy Committee

CORPORATE ENGAGEMENT
RDI Alliance of Companies for Patient-Centered Action

OPERATIONAL AREAS
WHO Collaborative Global Network for Rare Diseases (CGN4RD)
International framework for an operational description of rare diseases and key figures
WHO List of Essential In Vitro Diagnostics
WHO List of Essential Medicines

PROGRAMMES
Conference Programme
Fellowship Programme

PARTNERSHIPS
EURORDIS
Orphanet
Inserm

REPRESENTATIONS
NGO Committee for Rare Diseases
IRDiRC
APEC Rare Disease Network
Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease
UHC 2030

CORPORATE ENGAGEMENT
RDI Alliance of Companies for Patient-Centered Action
## Expenses

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<td>Director</td>
<td>86 000</td>
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<td>Manager of Relations with POs</td>
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<td>Public Affairs Manager (part-time)</td>
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<td>Corporate Relations Manager</td>
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<td>Freelance communications manager</td>
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<tr>
<td>Volunteers</td>
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<td>Office in NY (in kind)</td>
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<td>2 Council F2F (Geneva and Singapore/Kuala Lumpur)</td>
<td>11 700</td>
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<td>WHO WHA Geneva side meeting (Intercontinental Hotel)</td>
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<td>RDI 6th Annual Meeting - online</td>
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<tr>
<td>Communications</td>
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<td>Photo exhibit (Feb in Geneva)/ Inaugural event</td>
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<td>Travel and Subsistence</td>
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<td>Multi-stakeholder Event (eg. 2nd Round Table Access, WHO ATMP)</td>
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<td>WHO MoU Activities Semester 1 (CGN4RD)</td>
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<td>Miscellaneous</td>
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<tr>
<td><strong>Overheads (25% of all expenses except in kind)</strong></td>
<td><strong>69 925</strong></td>
<td><strong>13 500</strong></td>
<td><strong>83 425</strong></td>
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<td><strong>Sub-total Core Expenses</strong></td>
<td>224 925</td>
<td>377 200</td>
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## RDI Conference Programme

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<td>4 x Conference level-2</td>
<td>12 000</td>
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<td>2 x Conference level-3</td>
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## RDI Fellowship Programme

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<td>Conferences Level-3</td>
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| Overheads (10%)                               | 4 600    | 0     | 4 600  |
| Sub-total Programmes                          | 4 600    | 46 000| 50 600 |

## Total Expenses

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<td>423 200</td>
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## Income

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<td>Health Standard Organisation (NPO)</td>
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<td>Other donations (NPO)</td>
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<td>EURORDIS International Initiatives</td>
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## Total Income

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## Expected surplus

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<td></td>
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<td>71 300</td>
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Breakdown of RDI's revenues - 2020

- 52% Patient organisations and volunteers
- 37% Corporate Health Organisations
- 11% Non Profit Organisations