RARE
DISEASES
INTERNATIONAL

ACTIVITY REPORT 2017

Paloma Tejada
Director, Rare Diseases International
RDI Membership Meeting , Vienna
May 10, 2018

GOVERNANCE

- Council elections:

  John Dart – DEBRA International (2016-2018)

  Ramaiah Muthyala – Indian Organsation for Rare Diseases (2016-2017)

  Alfredo Toledo – Alianza Iberoamericana de Enfermedades Raras (2016-2017)

  - 12 Nominations from full members
  - Online Elections (April –May 2017)
  - 29 out of 36 full members voted

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RDI COUNCIL

Alfredo Toledo
ALIBER

Megan Fookes
Rare Voices Australia

Kin Ping Tsang
Retina International

Durhane Wong-Rieger
CORD

Lisa Phelps
NORD

Ritu Jain
DEBRA International

Yann Le Cam
EURORDIS

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MEMBERSHIP

- 49 member organisations end 2017
- 42 Full Members
- 7 Associate Members

Diversity:

  - 17 non-European national alliances
  - 10 European national alliances
  - 12 international disease-specific federations
  - 4 regional networks

NEW MEMBERS IN 2017

- World Alliance of Pituitary Organisation Association
- Nordic hypoPARA Organisation
- Cutis Laxa International
- Organisation for Rare Diseases India

MEMBERSHIP MEETING 2017 BARCELONA
3RD ANNUAL MEETING

- 50 participants
- 23 countries
- June 4th, 2017 (the day before and in the same venue as the EURORIDS ExPRESS Summer School)
- RD Patient leaders from:
    - Asia (India, Iran, Malaysia, Hong Kong, Singapore)
    - Africa (South Africa)
    - Latin America (Argentina, Brazil, Colombia, Mexico, Uruguay, Venezuela)

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BARCELONA 2017

RDI MEMBERS PARTICIPATED IN EURORDIS SUMMER SCHOOL

12 RDI Fellows attended EURORDIS Summer School English & First edition in Spanish

Training patient advocates on drug development and regulatory affairs

India - Malaysia -South Africa

from Latin America

Argentina - Colombia -Mexico - Uruguay -Venezuela

+ 3 members from ALIBER

EURORDIS
RARE DISEASES EUROPE

EXPERT PATIENTS AND RESEARCHERS
EURORDIS SUMMER SCHOOL

“ExPRESS YOURSELF!”

HOTEL SB BCN EVENTS
CASTELLDEFELS,
BARCELONA, SPAIN
5-9 DE JUNIO, 2017

Un programa de capacitación para representantes de pacientes e investigadores.
Sobre la información, la evaluación de la tecnología sanitaria y el acceso a medicamentos huérfanos, pediátricos y terapias avanzadas

#EURORDISSummerSchool

PLATAFORMA
MALALTIES MINORITÀRIES

ALIBER
ALIANZA IBEROAMERICANA DE ENFERMEDADES RARAS

RARE DISEASES INTERNATIONAL

RDI FELLOWS AT SPANISH SUMMER SCHOOL

THE RIGHT TO HEALTH: THE RARE DISEASE PERSPECTIVE

Friday, February 10, 2017

#rdiGeneve

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In partnership and
with the support of:

BLACKSWAN® FOUNDATION
SWISS FOUNDATION FOR RESEARCH ON ORPHAN DISEASES
SCHWEIZERISCHE STIFTUNG FÜR DIE FORSCHUNG SELTENER KRANKHEITEN
FONDATION SUISSE POUR LA RECHERCHE SUR LES MALADIES ORPHELINES
FONDAZIONE SVIZZERA PER LA RICERCA SULLE MALATTIE ORFANE

EURORDIS
RARE DISEASES EUROPE

FIRST RARE DISEASE POLICY EVENT IN GENEVA

- **“The Right to Health: The Rare Disease Perspective” at** Biotech Campus in Geneva
- 20 international speakers and panellists
- 140 participants
- WHO Department of Essential Medicines and Health Products at the World Health Organization
- United Nations Special Rapporteur on the Right to Health
- co-Chair of the United Nations Secretary General’s High-Level Panel on Access to Medicines

Statement of Support from Dr. Tedros Adhanom Ghebreyesus

new Director-General of the World Health

RDI ADVOCACY COMMITTEE

- Terms of Reference
- Calls for Expressions of Interest in September
- RDI Advocacy Committee - 15 patient reps from Member Organisations + IAPO
- 1st Meeting in October
- 2nd Meeting in November
- RDI Policy on developing, recommending

✓Response to the open consultation on Draft Concept Note towards WHO's 13th General Programme of Work 2019–2023 – submitted on October 13, 2017

✓ Response to open consultation on the Draft 13th General Programme of Work 2019–2023. RDI Contribution to Draft GPW 13 – submitted on November 2017

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ADVOCACY COMMITTEE


Yan Lee Cam
Eurordis
Chair


Maureen Smith
CORD-Canada


Angela. Chaves
FECOER-Colombia


Kelly du Plessis
RDSA-South Africa


Ramaiah
Muthyala
IORD-India


Paul
Melmeyer
NORD-EEUU


Ritu Jain
DEBRA
International


Lara Bloom
EDS-UK


Sanja Peric
RD-Croatia


Migdalia Denis
PHLS


Lieven
Bauwens
IFSBH


Alba
Ancochea
FEDER-Spain


Mark Brooker
WFH


Chiara
Ciriminna
BLACKSWAN
FOUNDATION

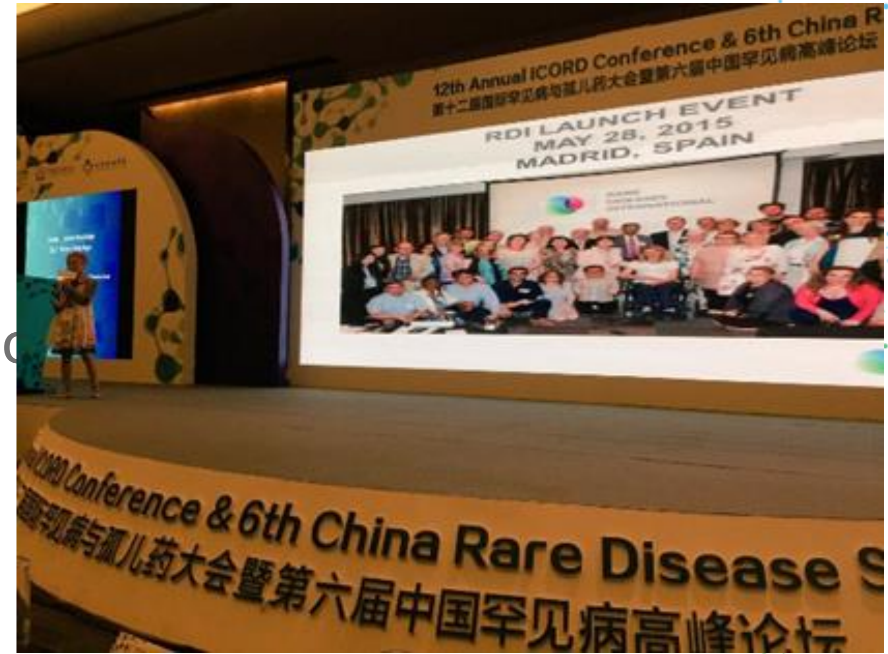

Kawaldip
Sehmi
IAPO


Simone
Boselli
EURORDIS –
Rare
Diseases
Europe

ICORD CHINA

XII ICORD Conference and 6th China Rare Diseases Summit in Beijing, China, in September 7-10, 2017.

- MoU with ICORD
- Programme Committee
- Stronger patient dimensi
- Presentation of RDI
- Hosted by RDI Member:



Chinese Organization for Rare Diseases

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NGO COMMITTEE FOR RARE DISEASES

- RDI officially accepted as a Member by the Inception Executive Board of the NGO Committee for Rare Diseases.
  - EURORDIS
  - Agrenska
  - World Federation of Hemophilia
  - International Federation for Spina Bifida and Hydrocephalus
  - International Alliance of Patients' Organisations
  - International Aalliance of Women
- Durhane Wong-Rieger represents RDI in the Executive Board

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2017 FINANCIAL STATEMENT

Expenses	
Staff	85 300
Annual Meeting	7 327
Policy Event	25 825
Fellowship EURORDIS Summer School	12 963
Communications	4 886
Miscellaneous	2 184
Overheads (25%)	34 621
Total Expenses	**173 106**
Income	
Members' contributions	500
Health Sector Corporates – direct to RDI	75 862
Health Sector Corporates – through EURORDIS International Initiatives	96 744
Total Income	**173 106**

Health Sector Corporates

Rare Diseases International:
Achillion
Biogen
Novartis
Sanofi-Genzyme
Vertex

EURORDIS International Initiatives:
Celgene
CSL Behring
Pfizer
Shire
Takeda

ACTION PLAN & BUDGET 2018

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STRATEGIC PRIORITIES

1. Strengthen RDI as a well-governed and sustainable organisation

2. Build a legitimate Global RD Patient Alliance

3. Advocate for RDs to be an International Public Health Priority and Raise Awareness of RDs worldwide

4. Advocate in favour of PLWRD worldwide in key areas

5. Build Capacities of Members and Patient Advocates

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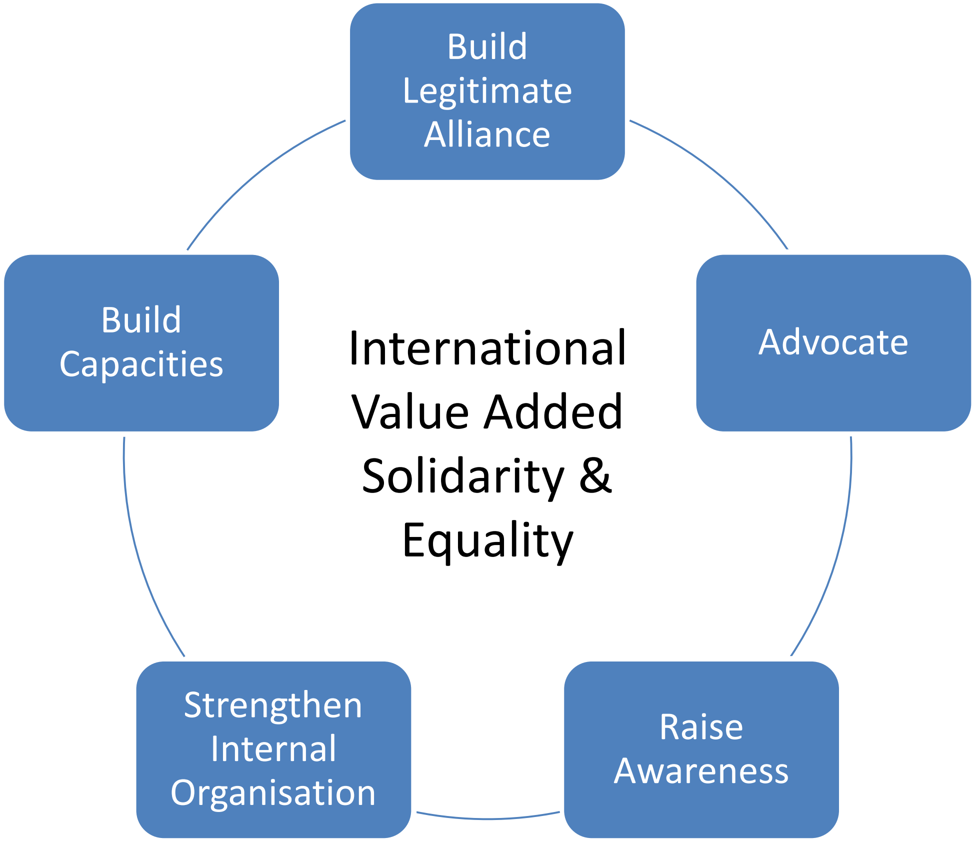
STRATEGIC APPROACH



Build Legitimate Alliance

Advocate

Raise Awareness

Strengthen Internal Organisation

Build Capacities

International Value Added Solidarity & Equality

RARE DISEASES INTERNATIONAL

1. STRENGTHEN RDI AS A WELL-GOVERNED AND SUSTAINABLE ORGANISATION

- Legal incorporation
- Statutes
- MoU with EURORDOS
- Financial sustainability
- Transition plan

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1. STRENGTHEN RDI AS A WELL-GOVERNED AND SUSTAINABLE ORGANISATION: *COUNCIL*

- Council elections -April 2018
- 6-7 Council Meetings (2F2 and 4 to 5 Teleconferences)
- Annual Activity & Financial Report 2017
- Annual Work Programme: Action Plan & Budget 2018
- Revision of By-Laws after adoption of Statutes
- Develop policy of declaration of potential Conflict of Interest

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2. BUILDING A LEGITIMATE GLOBAL ALLIANCE: *MEMBERS*

- Expand membership base
  - ✓ 53 Member Organisations at present
  - ✓ Recruit more members in 2018
  - ~ 10 National Alliances (eg. Ukraine, Macedonia, Chile, Peru, Brazil, Uruguay, Korea, Philippines, Algeria, Turkey)
  - ~ 10 International Federations (Cystic Fibrosis, IPWSO, Dravet, Rett, Duchenne, Pulmonary Hypertension, OIFE, Tuberous Sclerosis, Fibrodysplasia Ossificans Progressiva, Hereditary Angioedema, TIF)
  - ✓ Proactive direct approach through their

2. BUILDING A LEGITIMATE GLOBAL ALLIANCE: *INTERNAL*

- RDI Annual Meeting back to back to ECRD Vienna - May 10
- Information stand - ECRD May11
- Regular e-mailings
- Webinars on specific topics (eg. Human Rights, Disability, WHO / UN strategy)
- Facebook Workplace – virtual forum for members to share and exchange information

2. BUILDING A LEGITIMATE GLOBAL ALLIANCE: *EXTERNAL*

- Pursue strategic partnerships with:
- Orphanet
- International Rare Diseases Research Consortium - Patient Advocates Constituency Committee
- International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) Working Group on RD
- No renewal of MoU with ICORD
- No renewal of MoU with BLACKSWAN FOUNDATION

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3. ADVOCATE FOR RD TO BE AN INT'L PUBLIC HEALTH PRIORITY & RAISE AWARENESS: *CURRENT FOCUS*

- Place Rare Diseases in UN Sustainable Development Goals 2030 Agenda with the relevant political institutions (UN bodies):
    - Economic and Social Council (ECOSOC)
    - Human Rights Council
    - Convention of State Parites to the Convention on the Rights of Persons with Disabilities (CRPD)
    - World Health Organization
    - UN General Assembly Third Committee (Social, Cultural & Humanitarian)
- Be active member of NGO Committee for Rare Diseases

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3. ADVOCATE FOR RD TO BE AN INT’L PUBLIC HEALTH PRIORITY & RAISE AWARENESS: *ADVOCACY COMMITTEE*

- Consolidate RDI Advocacy Committee
- Policy on developing, recommending & adopting advocacy initiatives
- Adopt first Position Papers, Contributions, Statements
- Organise Ad Hoc Groups to work on specific topics
  - Human Rights
  - Disability
  - Others to be determined

3. ADVOCATE FOR RD TO BE AN INT’L PUBLIC HEALTH PRIORITY & RAISE AWARENESS: *TOWARDS AN EVIDENCE-BASED ADVOCACY*

- Orphanet: Agree on more robust RD figures (global) to communicate widely
- State of the Art of Rare Diseases: Develop a survey based on and using the tool of, the ‘State of the Art of Rare Disease Activities in Europe’ to collect and benchmark policy information from countries outside the EU
- Rare Voice Barometer: Extend the survey to collect data on specific topics from countries outside Europe

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4. ADVOCATE FOR PLWRD IN KEY AREAS : *ACCESS TO MEDICINES*

- Propose a Collaborative Framework with WHO that includes actions to improve access to medicines in LMICs
- Mobilise political support from Member States through Permanent Representations in Geneva to include access to medicines for rare diseases in WHO agenda
- Organise a Roundtable of Innovative Access Programs with the IFPMA, WHO, HTAi and other global actors

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4. ADVOCATE FOR PLWRD IN KEY AREAS : *RESEARCH*

- RDI becomes member of International RD Research Consortium (IRDIRC)
- IRDIRC Patient Advocates Constituency Committee (PACC) : composed of US Genetic Alliance, EURORDIS, NORD, CORD, China ORD, RVA, JPA , I-ORD (7/8 are members of RDI)
- Contribute to IRDIRC's goals
- Contribute to PACC's policy work by bringing patients perspective (eg. Survey on patient engagement in research)

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4. ADVOCATE FOR PLWRD IN KEY AREAS : DIAGNOSTIC

- Tie into the Global Commission

High level expert Panel led by Shire, Microsoft and EURORDIS to build road map to improve diagnostic pathways for children with rare diseases

- RDI Council Members sitting in the Commission (Yann Le Cam and Durhane Wong-Rieger) to secure RDI representation
- Involve RDI Members through:
  - Topic groups
  - Consultations with all Members on Recommendations
- Participate in determination of the

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5. BUILD CAPACITIES OF MEMBERS & PATIENT ADVOCATES

- Develop an “RDI Conference Program”
- Develop a Capacity Building Programme with fellowships and/or other knowledge exchange and training opportunities

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2018 BUDGET

Expenses	**Budget**	**Optional**
Staff	86 000	0
Annual Meeting	11 000	0
Conferences	4 000	5 000
Fellowship Summer School	0	0
Fellowships Annual Meeting	0	0
Communications	1 750	3 000
RDI Geneva side meetings	4 000	5 000
UN NGO CfRD meetings	3 000	0
Miscellaneous	2 850	
Overheads (25%)	28 150	3 250
Total Expenses	**140 750**	**16 250**
Income		
Membership fees	6 650	
Health Sector Corporates	134 100	
Alternative funds	16 250	
Total Income	**157 000**	

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