



ICORD

International Conference on
Rare Diseases & Orphan Drugs

**Rare Diseases, Global Challenges and
China's Role**

**世界与中国：罕见病的全球挑战与中
国角色**

Manuel Posada
President of ICORD



icord 2017 China





ICORD

International Conference on
Rare Diseases & Orphan Drugs

Hosts : ICORD (International Conference on Rare Disease and Orphan Drugs)

ICORD is an International Society for all individuals actively involved in rare diseases and/or orphan drugs, including health care, research, academic, industry, patient organizations, regulatory authorities, health authorities, and public policy professionals.

The mission of ICORD is to improve the welfare of patients with rare diseases and their families world-wide through better knowledge, research, care, information, education and awareness.

www.icord.se

Introducing ICORD

- Society set up in 2007 (first ICORD conference 2005 in Stockholm)
- A multi-stakeholder society focused on rare diseases and orphan drugs
- Members include health officials, regulators, researchers, clinicians, industry, academics, patients, advocacy group leaders
- Has held 11 international conferences
- Sweden, Spain, Belgium, USA, Italy, Argentina, Japan, Russia, the Netherlands, Mexico and South Africa
- Beijing, China, 2017 is the number twelve of the ICORD conferences

ICORD Board 2016-2018

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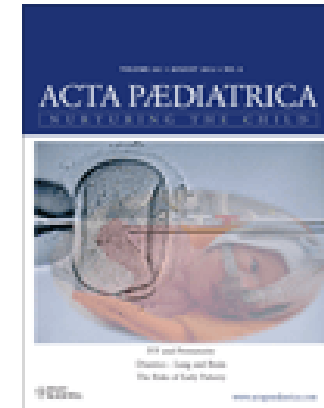
Ramaiah Muthyala, India

Joff Masukawa, USA

Joan X Comella, Spain

ICORD's policy statement

➤ The Yukiwariso Declaration



- Published in Acta Paediatrica Vol 101, Issue 8, pp 805-807, August 2012 (summary version) - Also at www.icord.se (full version)
- The declaration provides a rationale and framework for legislation, policies, action plans



The Declaration's 6 Principles

- 1. RDs are a significant public health issue – 6 to 8%
- 2. Human rights and government duties are involved
- 3. RD research and product development should be supported
- 4. A comprehensive approach to rare diseases should be adopted
- 5. The importance of patient autonomy, consent and information needs
- 6. Include patient groups in policy and services

There are 12 guidance points to assist implementation of these principles



ICORD Call to Action - Extension of the Yukiwariso Declaration: The Protea Declaration for Rare Diseases



A rare flower but beautiful and showing phenotypic variability

by Steve Groft and Safiyya Dharssi

- Protea are South African flowering plants (sugarbushes or suikerbos) representing **change and hope**.
- Diversity of the Protea flowers reflect the **diversity occurring in human populations with rare diseases**.
- **Yukiwariso flowers** were used to direct attention to the **uniqueness, rarity and extra needs** affecting rare diseases patients and their families.
- **Protea flowers** reflect the **complexity and variability** of each of the rare diseases individually and as a group.
- The **first ICORD African** deliberative engagement on rare diseases research and policies identified challenges and opportunities to stimulate discussion and develop actions to accelerate and harmonize research for this public health priority.



ICORD Call to Action: The Protea Declaration for Rare Diseases



A rare flower but beautiful and showing phenotypic variability

by Steve Graft and Safiyya Dharssi

- 1. Access to Diagnosis.** Accessibility to a correct diagnosis should be an achievable goal for all populations in all nations
- 2. Increasing Awareness.** Awareness of rare diseases among health care providers, patients, and the public needs to be raised to improve the understanding of rare diseases
- 3. Information Development and Dissemination.** Countries should utilize the readily accessible existing resources and dissemination channels or create new channels for information dissemination purposes
- 4. Congenital Malformations Identification and Treatment.** Knowledge about congenital malformations is often very limited. Surveillance procedures should aim to achieve much higher reporting rates for Congenital Malformations



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- 5. Access to Care and Treatments.** Access to treatments and care need to be engrained in the routine of the healthcare system to be effective and systematic
- 6. Expanding Role of Patient Advocacy Groups.** Countries should encourage patient advocacy collaboration. The formation of umbrella organizations provides unified voices.
- 7. Research Collaboration.** Data sharing, patient engagement and scalable research models are needed to translate the advances in genomics, imaging procedures, bioinformatics and communications technology to deliver better diagnostics and treatments.



ICORD Call to Action: The Protea Declaration for Rare Diseases in Developing and Developed Nations



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- 8. Quality of Life.** The focus on Quality of Life enables the affected individuals to reach their maximum potential.
- 9. Hearing the Voice of the Patient.** Patients with rare diseases require considerable extensive care.
- 10. Sustainability of Patient Organizations.** Patient organizations fulfill many needs in the rare disease community and require stability, increased membership, and information and support services with social media connections, twitter chats, and websites.



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11. Global Policy Developments. Commonalities of specific needs of patients, families, advocacy groups, health care providers, researchers, industry, payers, government agencies and societies address a significant public health problem despite the uniqueness of each country. National government decisions to meet the needs of the rare diseases community is a long-term commitment to care and treatment of all patients

13. Avoiding Health Inequities. Provide or strengthen coverage of essential interventions and life-saving products to reach poor, underserved and marginalized populations, in existing fragile settings.



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- **Conclusions:**

- The goal of care is for the patient to ***THRIVE, NOT SURVIVE.***
- ***The lack of national legislation or policy remains a significant gap in most countries***
- ***Stigmatization due to rare diseases may extend into limited education, employment opportunities, and limited social interactions including dating, marriage, and limitations on individuals reaching their full potential.***
- ***Establishing a national government emphasis on research and regulatory programs requires implementation of appropriate staff activities and providing financial and personnel resources to reach the stated goals.***
- ***Rare Diseases require specialized clinics at dedicated centers of excellence to provide optimal care and research infrastructure.***

Collaborating together for rare diseases

- ICORD welcomes efforts based on international collaboration
 - UDNI – **Undiagnosed** Diseases Network International
 - IRDiRC – **research** collaboration
 - GRDR/RaDaR, USA **registry** program
 - Rare Disease International – the **patient voices**
 - GEISER; RARE-X; FEMEXER; NORD; EURORDIS;...CORD?
 - and more
- **ICORD adds the multi-stakeholder and global perspective**
- **The more voices with consistent messages, the better chance of impact**

Program Highlights

It is the first time the ICORD, the leading multi-disciplinary organisation in rare disease and orphan drugs, will hold its annual conference in China.

- The first and comprehensive China national registry research
- The top Chinese experts in clinical research in rare disease
- With local and global regulators and policy developers
- Other related stakeholders

will share and explore more collaboration opportunities and partnership in rare disease and orphan drugs.

On behalf ICORD

- Thanks to the local organizers
 - CORD
 - PUNCH
- Welcome to all participants
- CORD adds the multi-stakeholder and global perspective
- The more voices with consistent messages, the better chance of impact

Thank you

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