



ICORD

International Conference on
Rare Diseases & Orphan Drugs

John Forman

President of ICORD

ICORD - a vehicle to catalyse stakeholders and raise policy awareness in
countries needing policy on rare diseases



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Introducing John

Married to Judith

Twins Timothy and Hollie

Born 14 November 1974

Alpha-Mannosidosis, an
insidiously progressive
Lysosomal storage disease



Introducing John

- Chair of Lysosomal Diseases New Zealand since 1999
- Executive Director of NZ Organisation for Rare Disorders since 2000
- Vice-President of ISMRD, the International advocate for Glycoprotein storage diseases
- Chair of the NZ Carers Alliance
- Has published on health policy, ethics, screening, patient perspectives and animal model research
- Current President of ICORD

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 9 conferences
- USA, Sweden, Japan, Russia, Argentina, Belgium, Italy, Spain, the Netherlands
- Next meeting in Mexico, October 2015
- 2016 – South Africa?? New Zealand??

ICORD aims

- Organise annual conferences
- Promote research, ethics, policies and action on RD
- Provide a global forum for all stakeholders
- Enhance international discussion, cooperation and coordination of policies and actions
- Exchange best practices and develop approaches and tools to address common issues

ICORD past Presidents

- Jan-Inge Henter – Karolinska Institute, Sweden
- Steve Groft – NIH, USA
- Domenica Taruscio – ISS, Italy
- Virginia Llera – Geiser Foundation, Argentina

ICORD's current priorities

- Providing annual conferences and other forums
- Influencing government policies, especially in the developing world
- Providing input to the UN, WHO, World Bank
- Promoting our declaration on rare disease policies and action plans



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ICORD's focus on policy

- Primary prevention and screening
- Early and accurate diagnosis
- Access to clinical care services
- Palliative care, social support
- Promotion of research & drug discovery
- Improving medicine regulation and medicine access

- The whole life course “womb to tomb”



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ICORD's policy statement

- The Yukiwariso Declaration
- Yukiwariso is Japanese for Hepatica, a perennial in the buttercup family. It breaks through the late snow to welcome spring and signal new life and hope. It is a rare beauty
- ICORD's Yukiwariso Declaration on Rare Diseases was launched in February 2012 at the Tokyo meeting
- Published in Acta Paediatrica Vol 101, Issue 8, pp 805-807, August 2012 (summary version)
- Also at www.icord.se (full version)

The Yukiwariso Declaration

- The declaration provides a rationale and framework for legislation, policies, action plans
- Only about 30 of 196 countries have rare disease policies – not all of them are comprehensive
- 166 countries (85%) have nothing



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The Declaration's 6 Principles

- 1. RDs are a significant public health issue – 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



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12 guidance points

- 1. Disparities and actions to address them
- 2. Human rights and actions to protect them
- 3. Equity in resource allocation
- 4. Specific counterbalancing policies for inherent disadvantage
- 5. Health economics must be balanced with equity and justice
- 6. Benefits to common disease knowledge from RD research



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12 guidance points (continued)

- 7. Gain in clinical care and prevention from research towards therapy
- 8. Encourage public good contributions from industry
- 9. Include patient groups at all levels
- 10. Develop and support patient groups
- 11. Review risk/benefit evaluation criteria
- 12. Promote aid for developing nations

Working together for rare diseases

- ICORD welcomes efforts based on international collaboration
 - Rare Disease International – the patient voices
 - IRDiRC – research collaboration
 - Rare Disease Day – international awareness building
 - GRDR – Global registry program
 - and more
- ICORD adds the multi-stakeholder perspective
- The more voices with consistent messages, the better chance of impact



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Thank you

- ICORD website www.icord.se
- Email icord@karolinska.se