ICORD

International Conference on
Rare Diseases & Orphan Drugs

John Forman – ICORD President

Supporting countries emerging to rare diseases

Rare Diseases International – Edinburgh 25 May 2016

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About me

With Judith, Timothy and Hollie Twins, age 40

Alpha-Mannosidosis, a rare Lysosomal storage disease with progressive and severe impacts

Involved in many rare disease groups in NZ and world-wide

Current President of ICORD

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A 41 year journey that never ends

After 50 days in hospital and 42 days on IV antibiotics

A possible additional rare disease for Hollie

XGP kidney disease

No Orphanet or GARD entry

Introducing ICORD

ICORD
International Conference on Rare Diseases & Orphan Drugs

- 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 10 international conferences
- Sweden, Spain, Belgium, USA, Italy, Argentina, Japan, Russia, the Netherlands and Mexico
- 2016 – Cape Town, South Africa.
- 2017 – China? 2018 – India?

ICORD's policy statement

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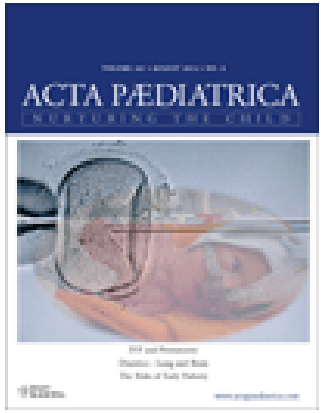
- The Yukiwariso Declaration



- 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

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- 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

The developing world

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- Advice on implementation of rare disease policies includes reference to the developing world
- But “light” on analysis of that point
- How to effectively promote RD policy and action plans in developing world?
- A major challenge for ICORD, RDI, WHO and UN agencies

Rare diseases in the developing world

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- Easier to argue when primary care and public health are well established
- Easier for politicians and officials when resources are plentiful
- Many countries rely on linear development, i.e. when the basics are in place
- But the right to health applies to all, including those with rare diseases

Rights and equity

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- Progressive realisation of the right to health means working from where you are and providing for all sections of the community
- Rare disease patients in developing world suffer double or triple disadvantages of poverty, limited basic healthcare, and lack of provision for their rare disease
- Without access to basic healthcare, rare disease patients have higher mortality, greater morbidity and compounded social disadvantage
- Establishing the basics improves outcomes for all, including those with rare diseases
- The basics are a necessary platform for diagnosis, care, prevention and treatment of rare diseases

Models to consider

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- WHO/WAOPBD, 1999. Services for the Prevention and Management of Genetic Disorders and Birth Defects in Developing Countries
- A formula for parallel development of specialist services
- Offers good insight into what can be done

Models to consider

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- Mexico's efforts from 2004 through *Seguro Popular*, a government-funded program.
- 52.6 million previously uninsured Mexicans enrolled in healthcare in less than a decade.
- Achieved universal healthcare by 2012.
- World Bank 2013 – "Nine countries from across Latin America and Caribbean highlighted for their healthcare successes."

Models to consider

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- Philippines - Kalusugan Pangkalahatan, Universal Healthcare
- Since 2012, boosted healthcare expenditure by close to 100% through a Sin Tax on tobacco and alcohol
- Healthcare coverage extended to 82% of the population - an additional 45 million people
- 1,000 more doctors, 10,000 more nurses, 6,000 more midwives
- A solid foundation - necessary for the survival and identification of rare disease patients

Combine the models

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- Build the basis and develop the specialist services at the same time
- Avoid the unfairness of a linear approach
- Comprehensive primary and public health services need specialties like pathology, clinical genetics and laboratories to support them
- These specialist services also provide essential basics to identify and care for rare diseases
- Recognise the different stages of development that means different approaches are need in the developing world

“Top down and bottom up”

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- UN, WHO, World Bank support to influence governments
- Partnerships with research and medical allies
- Build grassroots patient advocacy

- Work on all 3 levels in parallel. One or two only will be very difficult

An ideal opportunity

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- Rare disease day 2013

"Rare diseases must become a public health priority around the world. Improved access to diagnosis, accurate information…. Access to healthcare and social services, and a focus on research that will lead to treatments or even a cure for people living with a rare disorder."

Helen Clark, Former Prime Minister of NZ and Candidate for UN Secretary-General

Beware the pitfalls

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- Template models will not work.
  - Challenges of cross-border healthcare
  - Absence of genetic services
  - Criteria for Orphanet
  - Language and political/cultural barriers
  - Very limited patient advocacy networks

The take home message

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- Promote parallel development to ensure fair and equitable provision for all sections of the community
- Encourage governments, health planners and health agencies, while also building patient advocacy and alliances with other rare disease allies
- Adapt policies to stages of development
- Avoid template solutions

Thank you

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- To my family for supporting me in rare disease advocacy
- To my mother for instilling my values about caring for the disadvantaged
- To all of you, whose work and interests helps improve the lives of those with rare diseases