



# ICORD

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International Conference on  
Rare Diseases & Orphan Drugs

John Forman – ICORD President

Supporting countries emerging to rare diseases

Rare Diseases International – Edinburgh 25 May 2016

## About me

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With Judith, Timothy and Hollie  
Twins, age 41

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



## A 41 year journey that never ends

After 50 days in  
hospital and 42 days  
on IV antibiotics

An additional rare  
disease for Hollie

XGP kidney disease

No Orphanet or GARD  
entry, but treatable!!



# Introducing ICORD

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- 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](http://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

# The developing world

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- Our Declaration's 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 mean different approaches are needed 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

## ➤ 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,  
UNDP Administrator 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
- All of these mean we cannot just “transplant” models of rare disease policies and services from other advanced healthcare systems

# 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 reliance on 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