John Forman – ICORD President

Global Strategies for Rare Diseases

ICORD’s vision and the Yukiwariso Declaration
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
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 international conferences
- Sweden, Spain, Belgium, USA, Italy, Argentina, Japan, Russia, the Netherlands
- 10th annual meeting now in Mexico, October 2015
- 2016 – Cape Town, South Africa.
- 2017 – China?
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
The developing world

- 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

- 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

- 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

- 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

➢ 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

- 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
The take home message

- Do parallel development to ensure fair and equitable provision for all sections of the community
- Encourage governments, health planners and health agencies like WHO to think more creatively about health needs in the developing world
- We have a right and they have a duty
- It may be challenging, but it is not “too hard” to do
Thank you

➢ 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