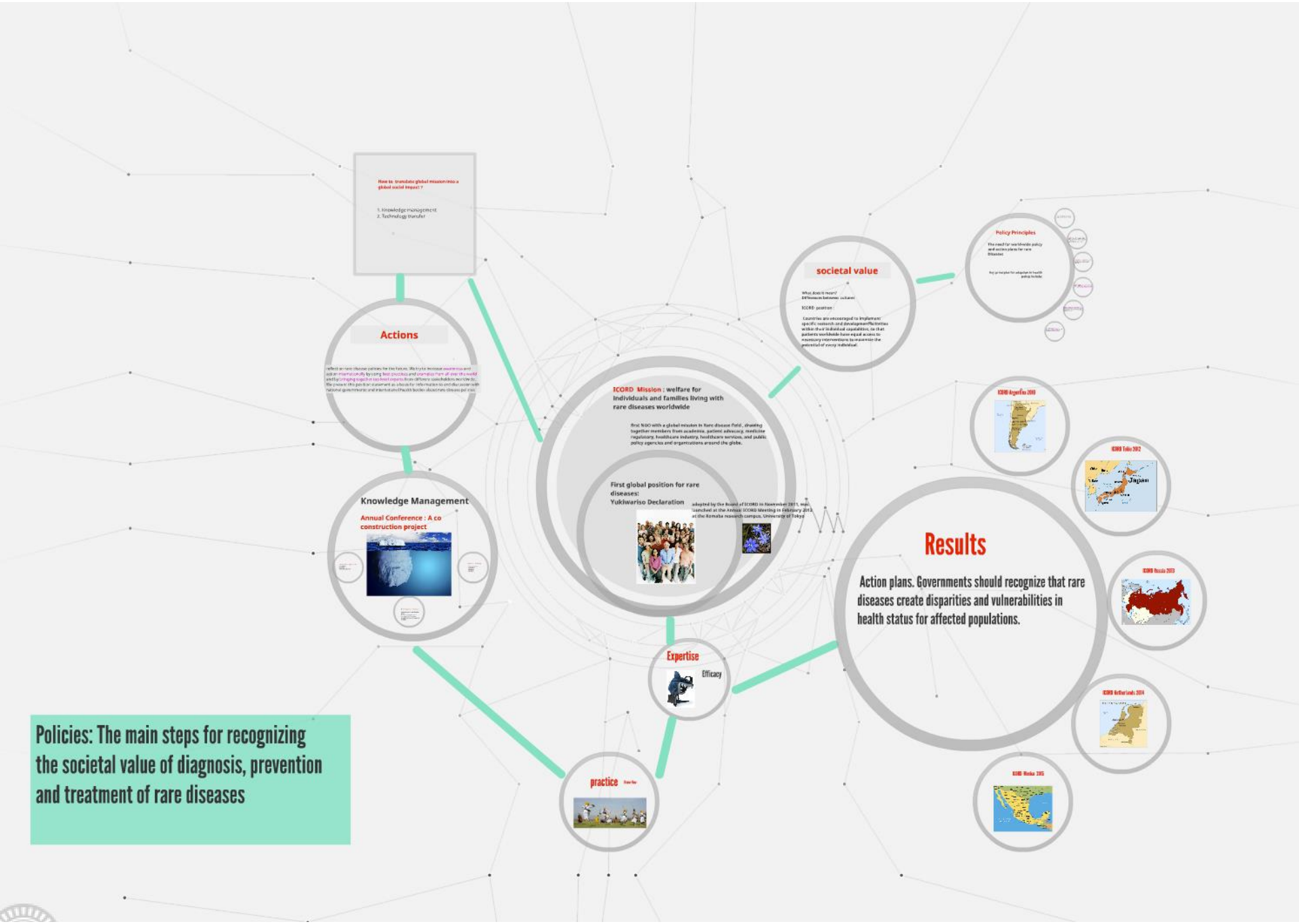
The challenging process from a global vision to a global social value impact

Virginia A. Llera, MD

IX ICORD – session I, plenary lectures

Ede, October 7, 2014



Policies: The main steps for recognizing the societal value of diagnosis, prevention and treatment of rare diseases

ICORD Mission : welfare for individuals and families living with rare diseases worldwide

first NGO with a global mission in Rare disease field , drawing together members from academia, patient advocacy, medicine regulatory, healthcare industry, healthcare services, and public policy agencies and organizations around the globe.

First global position for rare diseases: Yukiwariso Declaration

adopted by the Board of ICORD in November 2011, was launched at the Annual ICORD Meeting in February 2012 at the Komaba research campus, University of Tokyo

- First global position for rare diseases:
  Yukiwariso Declaration

adopted by the Board of ICORD in Nove
launched at the Annual ICORD Meeting
at the Komaba research campus, Unive

societal value

What does it mean?
Differences between cultures

ICORD position :

Countries are encouraged to implement specific research and development activities within their individual capabilities, so that patients worldwide have equal access to necessary interventions to maximize the potential of every individual.

Policy Principles

The need for world-wide policy and action plans for rare Diseases

Key principles for adoption in health policy include:

1
Rare diseases are a significant public health issue. Together they may affect up to 8% of the population, corresponding to a significant minority population.

Health care and treatment for rare diseases is a human rights issue. Non-discrimination, justice and equity of access to health care all require that specific policies are put in place to address the needs of people affected by rare diseases.

Every country is encouraged to have a rare diseases research development program, with emphasis adjusted to its existing capabilities.

A comprehensive approach to rare diseases is needed, including education, prevention, diagnosis, care and treatment, social support and inclusion as well as support of both basic and clinical research.

Quality information, informed consent and autonomous decision-making are critical for upholding the rights and protection of patients and their families. Combining genetic knowledge with screening to identify risks should be actively pursued to provide choices about prevention, balanced with careful attention to informed consent and autonomous decision-making.

1

Rare diseases are a significant public health issue. Together they may affect up to 8% of the population, corresponding to a significant minority population.

Health care and treatment for rare diseases is a human rights issue. Non-discrimination, justice and equity of access to health care all require that specific policies are put in place to address the needs of people affected by rare diseases.

Every country is encouraged to have a rare diseases research development program, with emphasis adjusted to its existing capabilities.

A comprehensive approach to rare diseases is needed, including education, prevention, diagnosis, care and treatment, social support and inclusion as well as support of both basic and clinical research.

Quality information, informed consent and autonomous decision-making are critical for upholding the rights and protection of patients and their families. Combining genetic knowledge with screening to identify risks should be actively pursued to provide choices about prevention, balanced with careful attention to informed consent and autonomous decision-making.

Patient groups play an important role in the development of knowledge about rare diseases and are suggested to be **included** at all levels in the development of their **policies and services.**

How to translate global mission into a global social impact ?

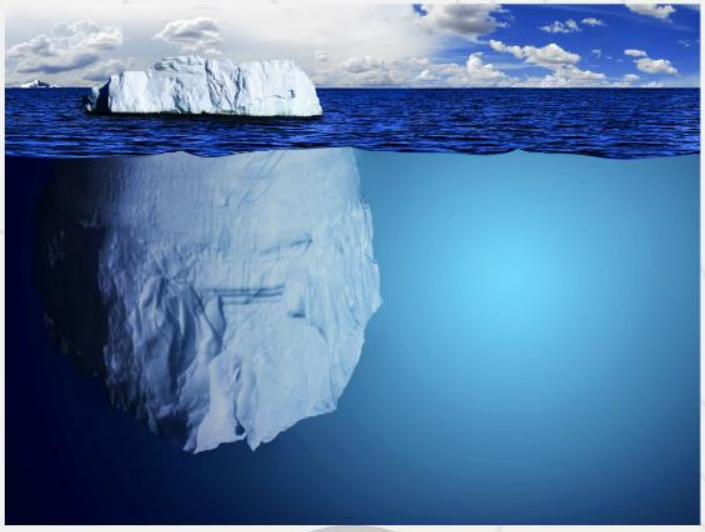
1. Knowledge management
2. Technology transfer

Actions

reflect on rare disease policies for the future. We try to increase awareness and action internationally by using best practices and examples from all over the world and by bringing together top level experts from different stakeholders worldwide. We present this position statement as a basis for information to and discussion with national governments and international health bodies about rare disease policies

Knowledge Management

Annual Conference : A co construction project



selection of local organizer: data
commitment
pro active
follow the guidelines

Experience : Knowledge

Defining objectives
facing risks
Experience
evaluation

Maturation process : Information

a) attending an Annual Meeting before
b) accompanying the process checking its understanding
c) organizing their own needs into a project

selection of local organizer: data
commitment
pro active
follow the guidelines

Maturation process : Information

a) attending an Annual Meeting before
b) accompanying the process checking its understanding
c) organizing their own needs into a project

Experience : Knowledge

Defining objectives
facing risks
Experience
evaluation

practice

Know-How

Expertise

Efficacy

er 2011, was
February 2012
y of Tokyo

Results

Action plans. Governments should recognize that rare diseases create disparities and vulnerabilities in health status for affected populations.

ICORD Ru

ICORD Netherlands 2014

ICORD Argentina 2010

ICORD Tokio 2012

ICORD Russia 2013

ICORD Netherlands 2014

ICORD Mexico 2015

How to translate global mission into a global social impact ?

1. Knowledge management
2. Technology transfer

Actions

reflect on rare disease policies for the future. We try to increase awareness and action internationally by using best practices and examples from all over the world, and by bringing major or top-level experts from different stakeholders review to the present this position statement as a basis for information to and discussion with national governments and international health bodies about rare diseases policies

Knowledge Management

Annual Conference : A co construction project

ICORD Mission : welfare for individuals and families living with rare diseases worldwide

first NGO with a global mission in Rare disease field , drawing together members from academia, patient advocacy, medicine regulatory, healthcare industry, healthcare services, and public policy agencies and organizations around the globe.

First global position for rare diseases:
Yukiwariso Declaration

adopted by the Board of ICORD in November 2015, was launched at the Annual ICORD Meeting in February 2016 at the Kamaka research campus, University of Tokyo

societal value

What does it mean?
Difference between cultures

ICORD position :

Countries are encouraged to implement specific research and development/Activities within their individual capabilities, so that patients worldwide have equal access to necessary interventions to maximize the potential of every individual.

Policy Principles

The need for worldwide policy and action plans for rare Diseases

Key principles for adoption in health policy include

ICORD Argentina 2008

ICORD Tokio 2012

ICORD Russia 2013

ICORD Netherlands 2014

ICORD Mexico 2015

Results

Action plans. Governments should recognize that rare diseases create disparities and vulnerabilities in health status for affected populations.

Expertise

Efficacy

practice

Know-How

Policies: The main steps for recognizing the societal value of diagnosis, prevention and treatment of rare diseases