



NGO COMMITTEE FOR
RARE DISEASES

Tackling Rare Diseases Internationally through Bottom-Up Care Strategies

The Case of Spina Bifida and Hydrocephalus.

Lieven Bauwens, Secretary General
International Federation for Spina Bifida and Hydrocephalus
11 November 2016

A Committee of **CoNGO**



CoNGO
The Conference of NGOs
in Consultative Relationship
with the United Nations





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Spina Bifida (NTDs)



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Hydrocephalus



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International Federation for Spina
Bifida and Hydrocephalus

Areas that we work in

Preconception
Prevention

Access to
health care

Life-long
follow-up

Right to Health

Right to life
DATA

Registration at
birth

Registry

International Federation for Spina Bifida and
Hydrocephalus



Perception



It i

MAKE DISSEAS



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Unfortunately

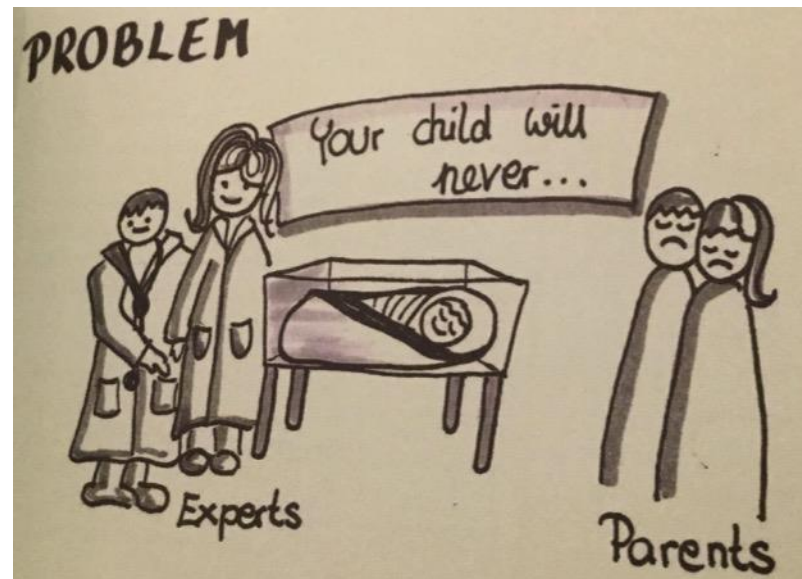
Negative Cycle

Loss of
hope

No care available –
no access to care

Institutionalization
Termination of pregnancy / life
Unmotivated professionals and parents

Negative
outcomes
→ negative
image



Positive Cycle

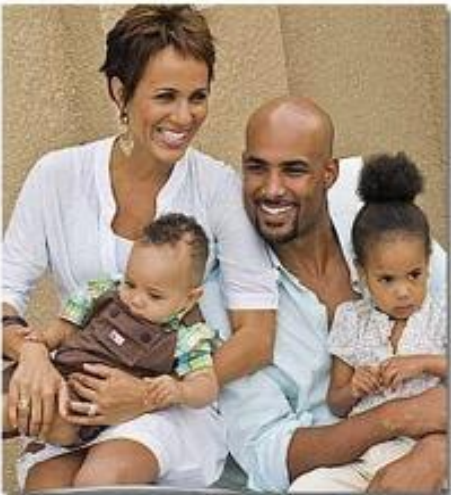
Hope

Quality care
available – access
to care



Motivated parents and professionals
Timely referral / improved outcome
Inclusion in society

Positive
outcomes
→ realistic
image

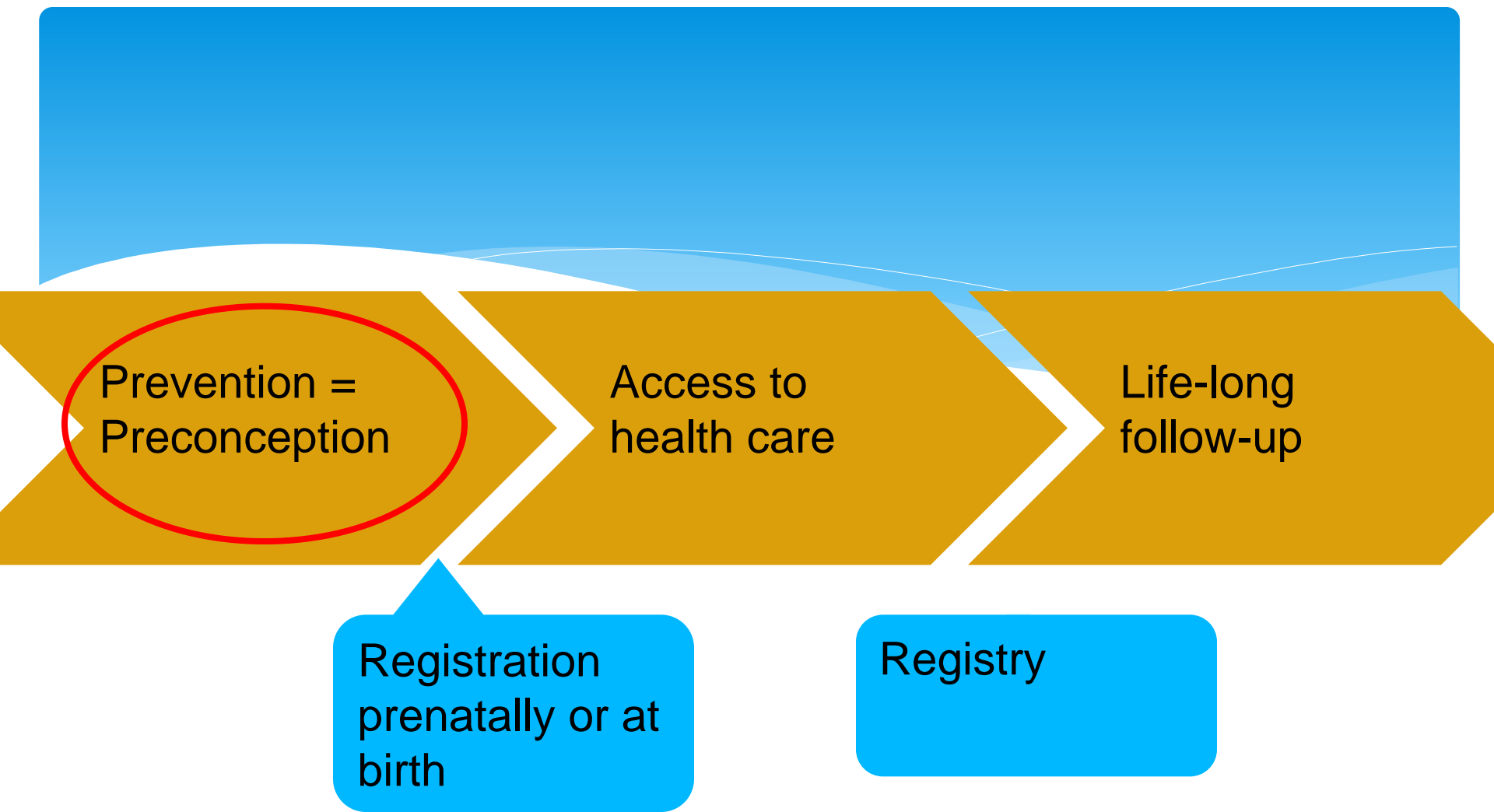




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PREVENTION

An Advocacy Example



Prevention

- ▶ Prevention of NTDs by taking Folic Acid (to 70%)
- ▶ (maybe) higher rate of prevention with other B-vitamins
- ▶ Recommendation: daily intake of 0.4 mg of folic acid
 - ▶ at least two months prior to the conception and the first months of pregnancy



- ▶ Parents at extra risk
should take daily 4 mg





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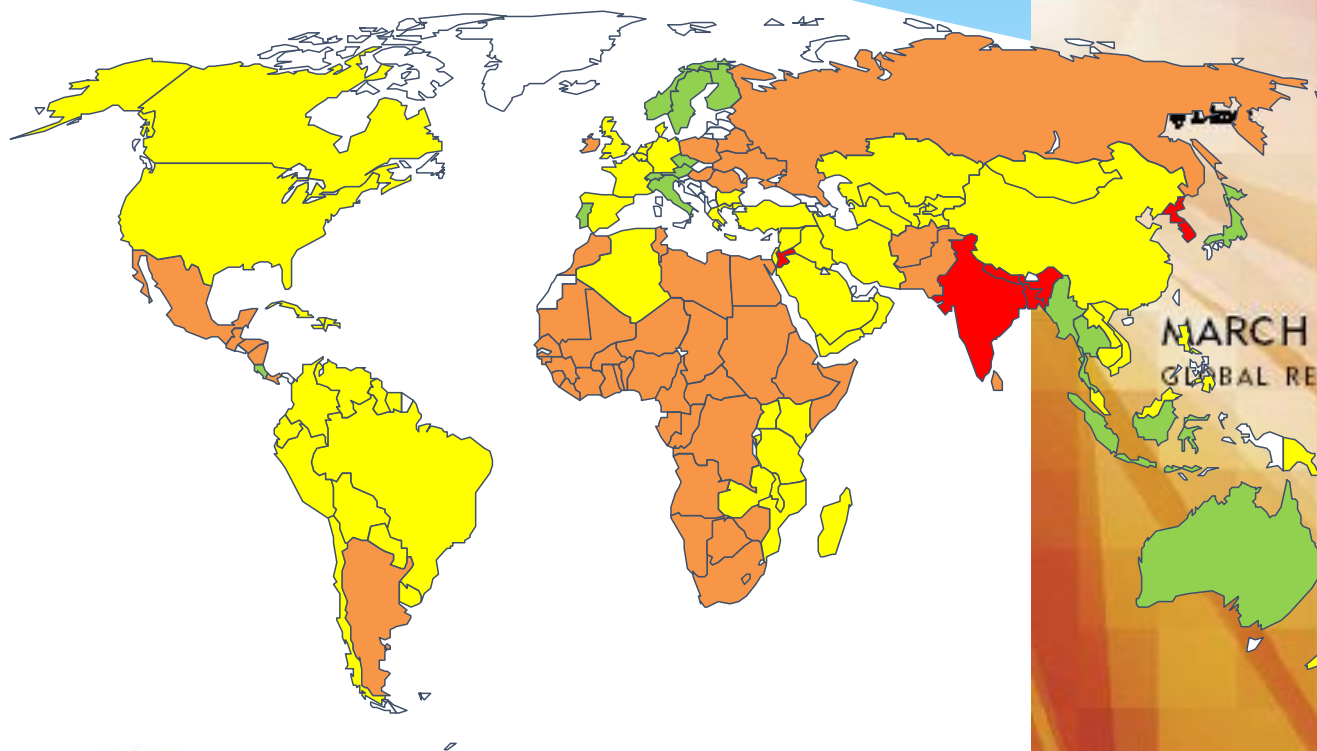
Data

CRPD Art. 18 - Liberty of movement and nationality

- * 1. States Parties shall recognize the rights of persons with disabilities to liberty of movement, to freedom to choose their residence and to a nationality, on an equal basis with others, including ...
- * 2. Children with disabilities shall be registered immediately after birth and shall have the right from birth to a name, the right to acquire a nationality and, as far as possible, the right to know and be cared for by their parents.



Lack of data



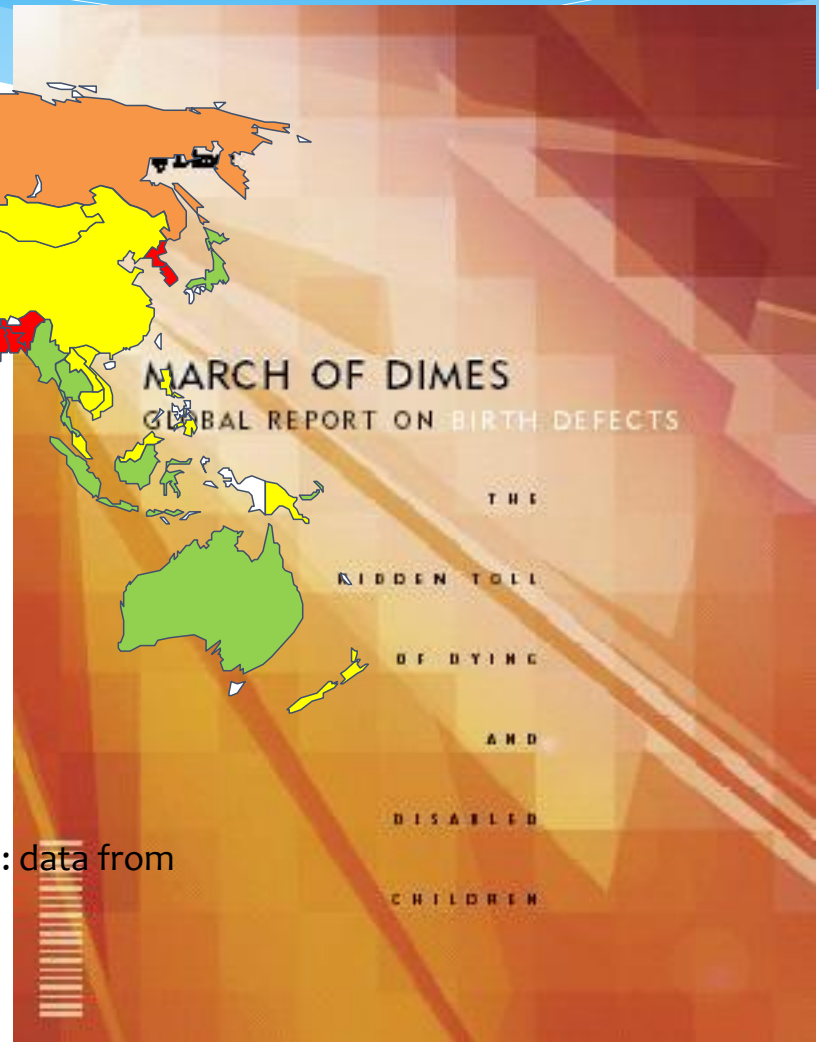
≤ 1.0

2.1-3.0

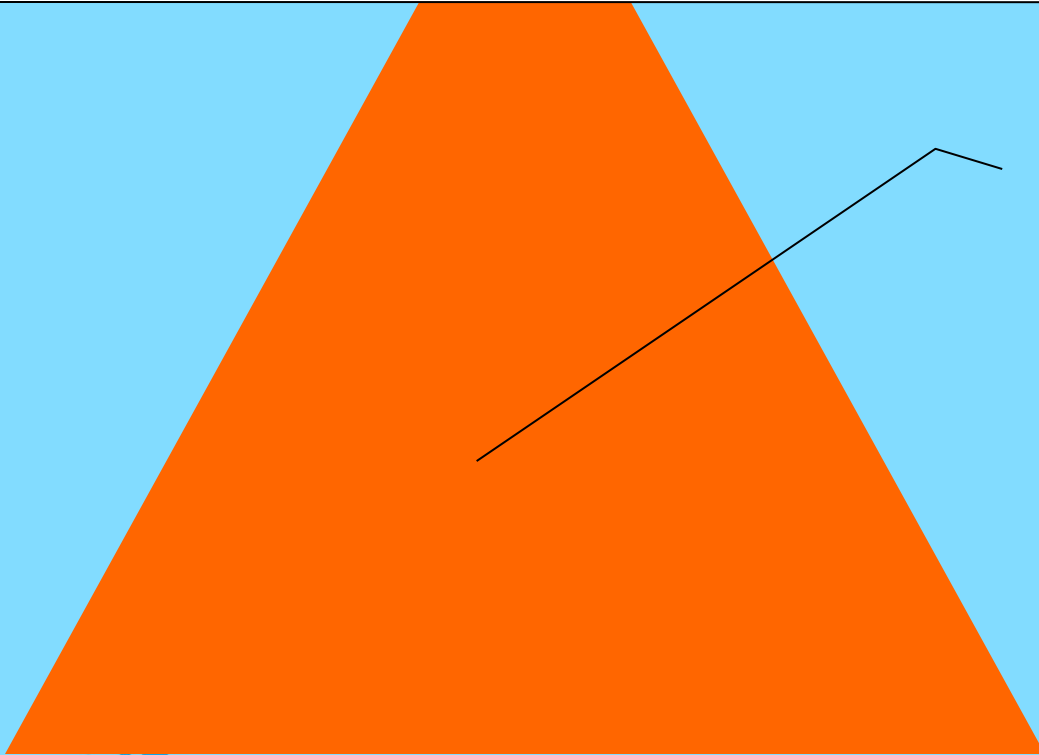
1.1-2.0

> 3.0

Rates per 1000 births: data from
March of Dimes



Ignoring NTDs is not prevention



Other health outcomes:

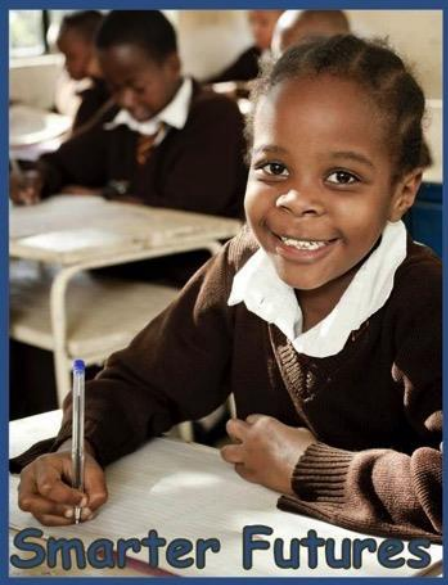
Certainly: FA deficiency and related anemia

Probably: stroke

Possibly: Low birth weight, pre-term birth, cancer, other birth defects, ...

Recurrence?

Focus on surveillance



Data drives efforts in prevention
Data drives efforts in care provision



World Health
Organization



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GUIDELINE:

OPTIMAL SERUM AND
RED BLOOD CELL FOLATE
CONCENTRATIONS IN WOMEN
OF REPRODUCTIVE AGE
FOR PREVENTION OF
NEURAL TUBE DEFECTS



IF Supports Fortification



INTERNATIONAL FEDERATION
FOR SPINA BIFIDA AND HYDROCEPHALUS

Consultative status special category with Economic and Social Council of the United Nations
Participatory status, Council of Europe

IF POLICY STATEMENT ON PREVENTION OF NEURAL TUBE DEFECTS AND MANDATORY FOOD FORTIFICATION

**Adopted by the IF Annual General Meeting on 28
June 2005 in Minneapolis**

IF calls for action to:

1. Promote the health benefits of the vitamin folic acid.
2. Ratify a policy calling on all countries to fortify staple food with the vitamin folic acid to reduce the incidence of neural tube defects (NTDs).
3. Encourage further research into the prevention of neural tube defects (including spina bifida).

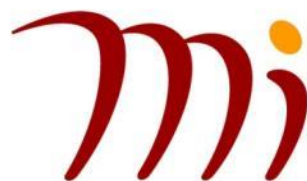


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Consensus for Fortification



INTERNATIONAL
FEDERATION
for
SPINA BIFIDA
and
HYDROCEPHALUS



**Micronutrient
Initiative**



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**World Health
Organization**

Recommendations on Wheat and Maize Flour Fortification Meeting Report: Interim Consensus Statement

PURPOSE

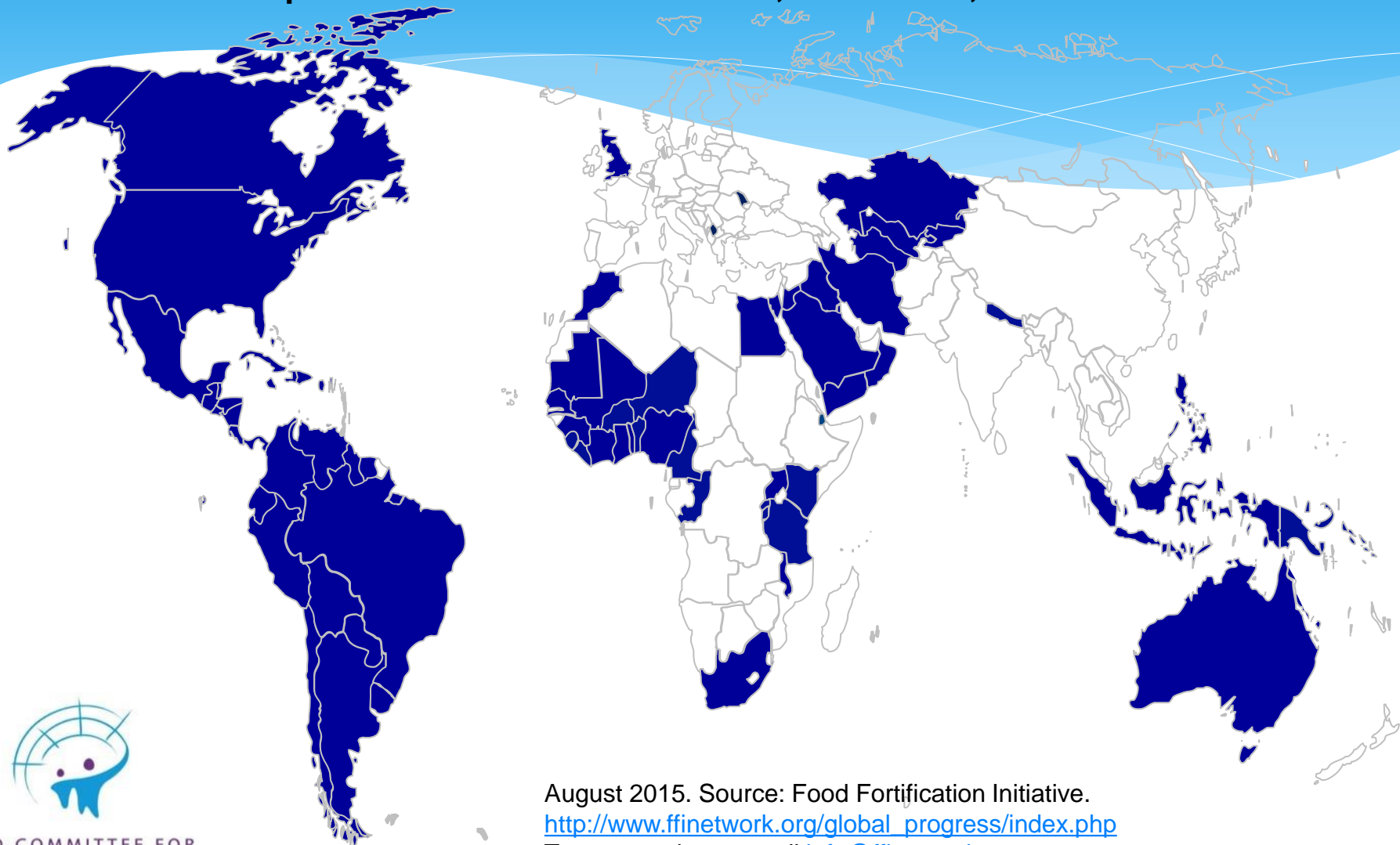
This statement is based on scientific reviews prepared for a Flour Fortification Initiative (FFI) technical workshop held in Stone Mountain, GA, USA in 2008 where various organizations actively engaged in the prevention and control of vitamin and mineral deficiencies and various other relevant stakeholders met and discussed specific practical recommendations to guide flour fortification efforts being implemented in various countries by the public, private and civil

THE FFI SECOND TECHNICAL WORKSHOP ON WHEAT FLOUR FORTIFICATION

Nearly 100 leading nutrition, pharmaceutical and cereal scientists and milling experts from the public and private sectors from around the world met on March 30 to April 3, 2008 in Stone Mountain, GA, USA to provide advice for countries considering national wheat and/or maize flour fortification. This Second Technical Workshop on Wheat Flour Fortification: Practical Recommendations for Maize and Amaranth was a follow-up to the FFI 1st UK Country for Maize

Grain Fortification Legislation

84 countries require fortification of wheat flour, maize flour, and/or rice



August 2015. Source: Food Fortification Initiative.

http://www.ffinetwork.org/global_progress/index.php

To request data, e-mail info@ffinetwork.org



5 CONDITIONS TO DESTROY THROUGH FOOD FORTIFICATION

SPINA BIFIDA

Each year
1,500 babies

in the US are born with spina bifida, a birth defect causing paralysis or brain damage.

Spina bifida can be prevented by boosting **folic acid** intake before and during pregnancy.

More governments in countries across the globe are making folic acid **flour fortification** mandatory.



GOITER

Enlarged thyroid gland due to low **iodine** in the diet. Iodine deficiency early in life can cause brain damage in young children.

Adding iodine to **salt** can fight goiter and other iodine deficiencies. The World Bank reports that global salt iodization would cost just

US\$0.05 per child per year.



ANAEMIA

Anaemia, a condition where the number of red blood cells in the body is too low, is a major cause of maternal death.

Iron, vitamin B12 and folic acid fight anaemia and its symptoms like exhaustion.

Fortifying **everyday foods** (e.g. bread in Egypt) provides **up to 40%** of women's daily iron needs.



RICKETS

Not enough **vitamin D** and **calcium** weakens bones in babies and children – a condition called rickets.

In many countries, **fortifying milk** with vitamin D is mandatory.



NIGHT BLINDNESS

An estimated **250,000–500,000 vitamin A** deficient children become blind every year, half dying within 12 months of losing their sight.

Large scale fortification programs add **vitamin A** to **vegetable oil, maize and wheat flour**.

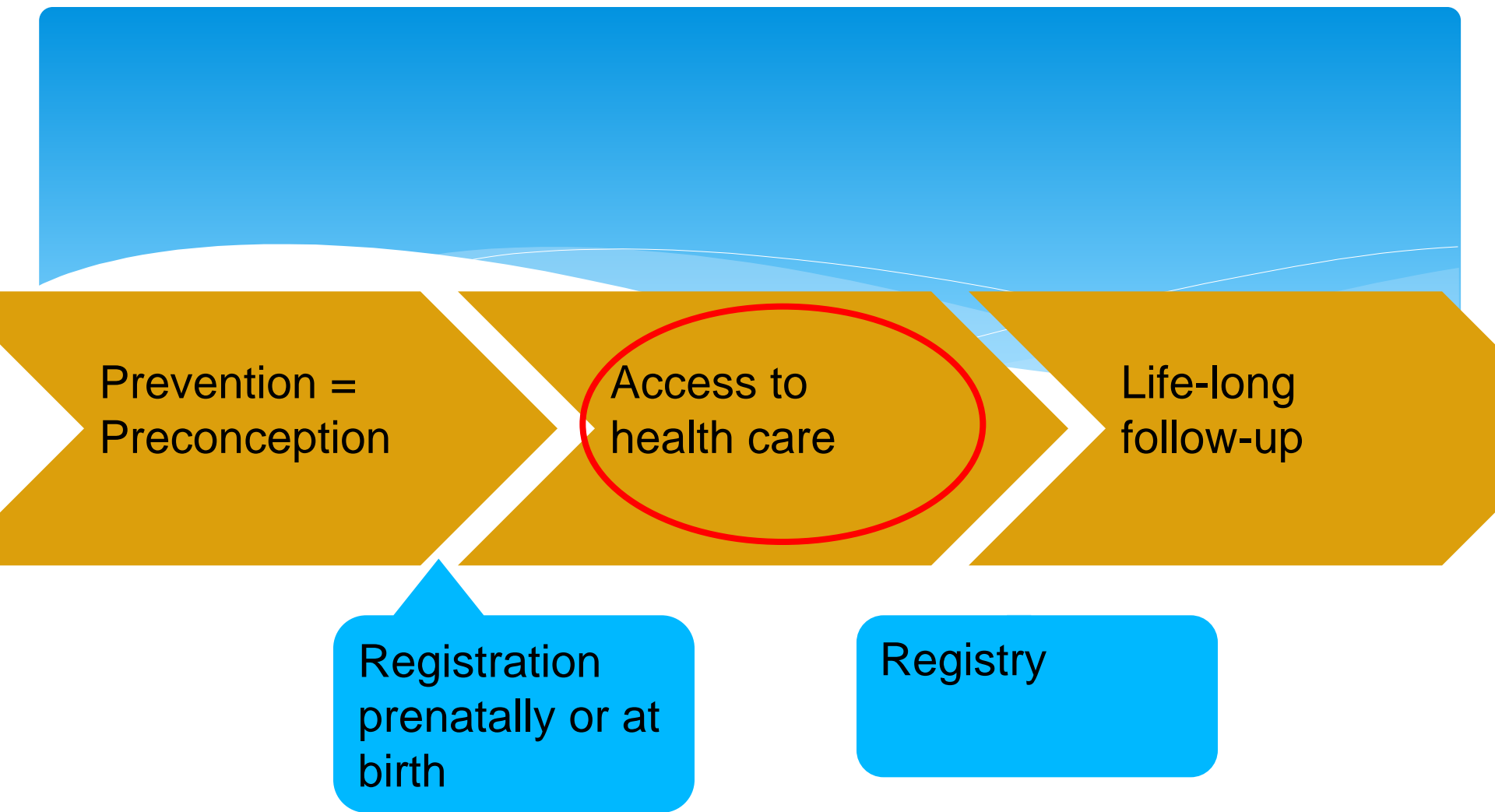




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Right to Care

Advocacy example 2:



Life long care

Survival

- Life-saving surgeries
- Shunts
- Counseling to new parents

LIC

Early years

- Early intervention and (re-)habilitation
- First mobility equipment
- Accessible childcare

School years

- Inclusive education and teaching assistance
- Accessibility of schools and other activities
- Interaction with peers

Transition

- New health needs
- Beginning of independent life
- Gaining life skills

MIC

Adulthood

- Work and social support
- Family life
- Sexual and reproductive health

Ageing

- Prolonging independent life
- Age-related health care needs
- Dignified retirement

HIC

Barriers to treatment

- Lack of neurosurgical manpower / available care
- Poverty
- Corruption
- Lack of information / training
- Negative stereotypes on SB (referrals)
- Lack / cost of transport
- Poor infrastructure
- Regions of insecurity

* → Resulting in extremely high mortality or secondary disabilities



On the ground, access to health



Distributed 40.000+ shunts so far

EQUIPPING, ENABLING AND EMPOWERING

Priority Assistive Products List



Thank you!

Lieven Bauwens

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