

MALADIES RARES ET DROITS DE L'ENFANT



Fondation
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**FONDATION
BROCHER**

ProRaris et les HUG, ensemble pour les 10 ans de ProRaris



UN ETAT DES LIEUX SUR LA
PRISE EN CHARGE DES ENFANTS
ATTEINTS D'UNE MALADIE RARE
ET/OU COMPLEXE
UN REGARD SUR LA SITUATION AUX HUG
ET DANS LE CANTON DE GENEVE

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Sophie Inglin, MA, MPH, cheffe de projet
Lucia Volpato, MSc, assistante de recherche
Consultante externe : Christine de Kalbermatten

A new paradigm: Personalized Precision Medicine

Personalized Medicine deals with how specific genes (or environmental circumstances) affect the health of an individual person. Just like Rare Diseases.

«There is no better way to advance the proper practice of medicine than to give our minds to the discovery of the usual law of nature by the careful investigation of cases of rarer forms of disease». William Harvey (1578–1657)

Rare diseases are unfortunate experiments of nature that will play a determining role to make the promises of Personalized Precision Medicine come true.

Precision and Personalized = positive



Rare = negative

Categorizing diseases as Rare, and therefore of lower priority, is probably unethical. And certainly unwarranted, not only with respect to their patients but to the future of medicine in general.

Values, such as Human Rights,
can be embodied in laws, treaties, conventions

When it comes to health...

The WHO Constitution (1946) envisions: «... the highest attainable standard of health as a fundamental right of every human being without distinction of race, religion, political belief, economic or social condition».

Universal Declaration of Human Rights (1948, art 25): «Everyone has the right to a standard of living adequate for the health and well-being of himself and of his family, ...».

and concerning children

United Nations Convention on the Rights of the Child (CRC) (1989)

The world's most ratified human rights treaty in history. Ratified by 196 states, the latest being South Sudan and Somalia. Not by the USA...

This Convention goes beyond the Universal Declaration of Human Rights. It stresses that childhood, lasting until the age of 18, is a special, protected time.

The CRC guarantees every child's right to health, to grow and develop to full potential. It requires States to take measures to achieve the health and well-being of every child.

United Nations Convention on the Rights of the Child

Article 24: Right to health

*1. States Parties recognize the right of the child to the enjoyment of the **highest attainable standard of health** and to facilities for the treatment of illness and rehabilitation of health. States Parties shall strive to ensure that no child is deprived of his or her right of access to such health care services.*

A remarkable feature of the CRC is the following:

The Right Of The Child To Have His Or Her Best Interests Taken As A Primary Consideration

(Not the interests of the health system, the economic sector, society at large...)

No child is deprived... this implies: **without distinction** of race, religion, political belief, economic or social condition.

The implementation of the CRC is under the supervision of the **UN Committee on the Rights of the Child** (18 independent experts)

The Committee elaborates **General Comments** regarding the CRC, including on issues Related To Child Health

In this context, it has underlined:

- the need for increased understanding of the factors that contribute to death, disease and disability in children;
- the new opportunities and challenges to achieve children's right to health created by advances in technologies;
- the increasingly important role of the private sector in the development of technology, drugs, equipment, interventions and processes that can contribute to significant advances in children's health.

The Committee performs **regular evaluations** on the States' implementation of the CRC

In 2014, important deficiencies were pointed out regarding how Rare Diseases are addressed in Switzerland.

This led the federal government to elaborate a «Concept national maladies rares» and to set up the «Coordination nationale maladies rares» (2017).

With all the knowledge that has accumulated in bio-medicine over the last 30 years, we are in a position to act so that being born with a genetic rare disease is not a life-long curse, for both patient and family.

«Il est inacceptable que des personnes gravement atteintes dans leur santé doivent recourir à la charité pour accéder à leur traitement»
(P. Strupler, directeur OFSP)

**Demanding the implementation of Childs Rights:
a possible approach to boost the issue of rare diseases?**

“States Parties recognize the right of the child to the enjoyment of the highest attainable standard of health”

« Therapy delayed is therapy denied »

And this is particularly true concerning children