



The NZ Organisation for Rare Disorders

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Is a Rare Disease Policy an Optional Decision for governments?

- Good rare disease policy and budgets in US and EU
- Absent from most of the rest of the world
- Is it just good lobbying, luck or wealth?
- Are there obligations on states to make provision for rare disorders?
- Or will many of us stay in the “too hard” basket?

Universal Declaration of Human Rights

- Not a binding legal document
- The aspirational rights in the UDHR include Article 25.1, which says:

“Everyone has the right to a standard of living adequate for the health and well-being of himself and of his family, including ... medical care and necessary social services, and the right to security in the event [of] ... sickness, disability, or other lack of livelihood in circumstances beyond his control.”

International Covenant of Economic, Social and Cultural Rights

- The ICESCR is a binding legal instrument, but no effective enforcement mechanism.
- The rights include “the right of everyone to the highest attainable standard of physical and mental health” (Article 12.1).
- Article 12.2 provides - to realise these rights, steps must be taken by the state to reduce infant mortality, to improve hygiene, to prevent, treat and control diseases and assure all medical service and medical attention in the event of sickness.

Limitations

- Implementation of the Declaration and the Covenant in each country is subject to laws, politics, policies, priorities and budget provisions

National Legislation

- Generally do not establish a right to health care (beyond emergency care) for any condition – rare or not

National Policies on Healthcare

- Most do make aspirational statements about goals of health care systems and intention to provide care when needed
- Most provide safety net basic health care
- These are often consistent with the Declarations but limited by budgets and priorities

Ethics and Moral Philosophy

- Every country has its unique culture, values and ethics that guide what is “right” to do
- In common law systems, what is “right” can become “duty”, even for governments
- Sensitivity to moral philosophy and ethics is important in political decision-making

Utilitarian Analysis

- Bentham's principle of utilitarianism, as developed by J S Mill
- Many political arguments claim the greatest good for the greatest number as the driving moral force in healthcare
- Often used as justification of existing rationing and prioritisation processes
- But this is a "crude" utilitarian analysis

Utilitarian Analysis

- There should be a “sophisticated” utilitarian analysis
- This will provide:
 - Consistency with Declaration and Covenant
 - Priority for serious and urgent
 - Attention to underserved communities
 - Reduction of health disparities in populations
- And:
 - Provision for rare disorders

Getting a Rare Disease Policy

- Not just luck in lobbying
- Comes from a mix of legal, political, moral
- Moral arguments have more power than we often recognise
- In common law systems they are a major influence on the common law

Where to from here?

- Patient groups in countries without a rare disease policy
 - Keep lobbying politicians
 - Keep pursuing legal arguments
 - Above all – get the moral philosophers on side to work with you.
- Success will come faster when all angles are covered

Parallel work needed

-with or without rare disease policy

- Work on service design and delivery issues
- Diagnostics
- Genetic services
- Specialist clinics for treatment
- Newborn screening

- Emotional, social and psychological support has limited value without basic services and care

Example – Newborn Screening

- Decisions usually made by professionals and health planners
- Patient/family input often limited
- Wilson & Jungner criteria need revision
 - Focused on treating the child
 - Need to include family impacts of inherited but untreatable disease
- Validated interventions to save lives and reduce morbidity are being missed
 - Because family voices are excluded

Recent Initiatives

- US Newborn Screening Act 2008
- Australasian Health Ministers' Advisory Council
- Excellent opportunity for input and influence
- Need to ensure planned and organised patient/family contributions

Newborn Screening

- Decision making needs to be more democratic
 - too closely held by professionals & officials
- Families are key stakeholders
 - not passive recipients of decisions
- The best information systems, and social, emotional & psychological supports have limited value if an avoidable problem is not avoided

Conclusion

- Patients and families as active participants in the information, clinical care and research issues.

“These examples demonstrate the changing face of support groups. They are staking claims for a role in research by providing epistemological information about their diseases, and becoming active partners in debates and practices about them.”

V. Rabeharisoa et M. Callon. Histoire et Sciences Sociales, Medicines/Sciences 2000; 16: 945-9