

Patient Groups - their connections and needs

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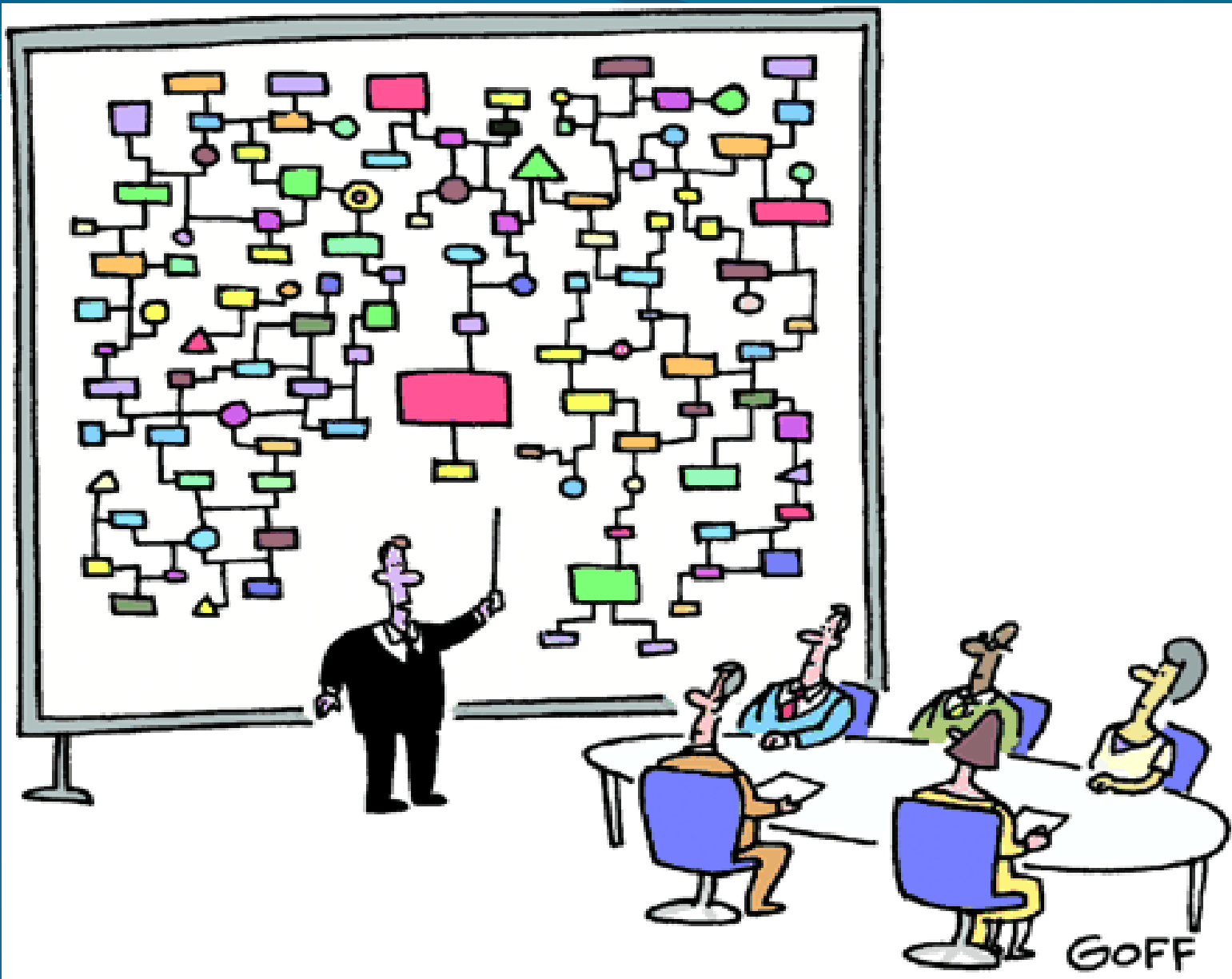
Declaration

- Paid full time by NZ Organisation for Rare Disorders
- NZORD receives 60% funding from Ministry of Health
– balance charitable fundraising
- No industry income for NZORD
- Two small grants from industry in 2002/03 for conference attendance – Human Genetics & Lysosomal diseases

My other interests



- Early & accurate diagnosis.
- Best clinical care.
- Best treatments.
- Access to specialist services.
- More investment in research.
- Good information.
- Good access to community support services.
- Faster development of new therapies.
- Respect for privacy and autonomy.
- Improved disability support services.
- Informed consent.
- Safe medicines.
- Good prevention options.
- Culturally safe services.
- Involvement in research design and approvals.
- Participation in advisory committees and policy setting.
- Better training for health professionals.
- Incorporation of genetic and other technologies into healthcare.
- Improved palliative care services.
- Revision of screening criteria for newborns.
- More registries and biobanking options.
- Open access to health information and research results.
- Improved access to novel therapies.
- More affordable healthcare.
- Review of risk assessment for orphan drugs.
- Prevention of unsafe medical treatments and practices.
- Improved patient care pathways in healthcare.
- Reduced waiting lists.
- Respect as a valued partner in healthcare (personal or health system).
- Nurturing and support of patient groups.
- Reduced inefficiencies in health systems.
- Wider health literacy.
- Better information resources for patients and families.
- Improved health coverage portability.
- Unblocking of silos and barriers in health systems.
- Acceptance of patient and family expertise.
- Resources for sustainable group operations.
- Just a few of the challenges we face

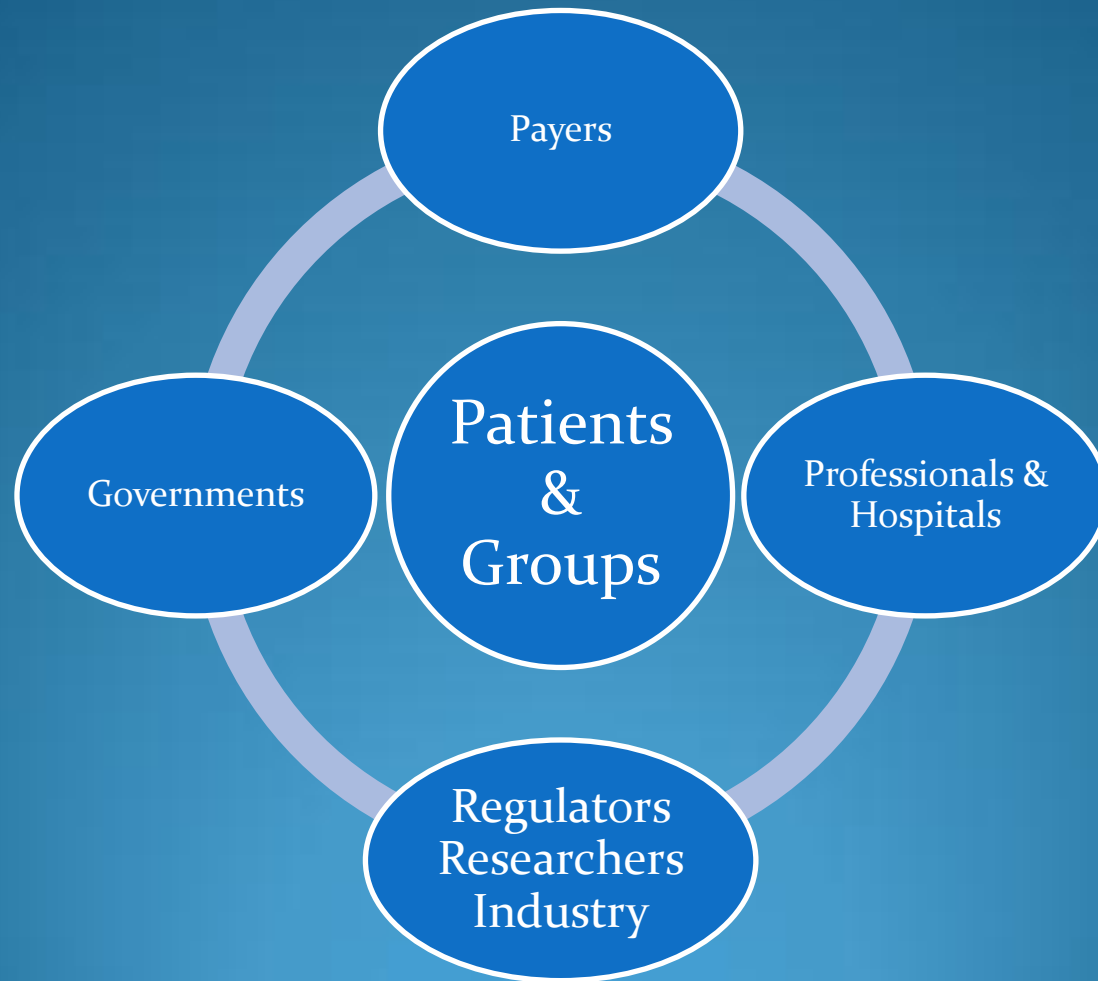


"And that's why we need a computer."

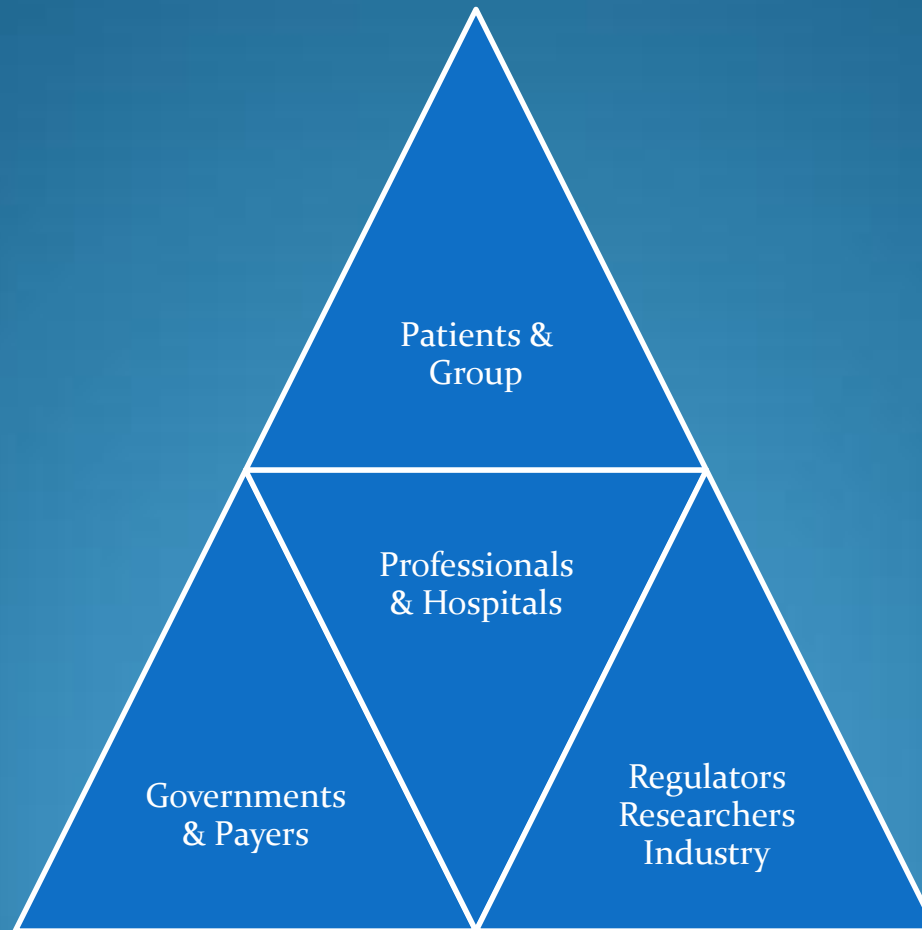
Group the issues by categories

- Patient care
- Patient information
- Research and therapy development
- Regulatory systems
- Government legislation
- Population health Vs personal health
- Common Vs rare
- Health systems
- Professional groups
- Government and insurance payers

Develop a context Patient-Centered Healthcare



However you shape it, patients should be central or top



Steps to solving problems for Rare Disorders

- What are the important current issues?
- What are the short-term achievable things?
- What is the long-term strategy?
- Who is on your side?
- Where are the battle lines?

- Then analyse the + and – of stakeholders

Health Professionals & Researchers

- Last century's battles

Autonomy

Consent

Safety

Respect

Privacy

- These issues are mostly settled
- Health professionals & researchers are our allies
- Leave behind the adversarial approach – work with them
- Focus on speed and accuracy of diagnosis

Regulators

- Provide important safety controls for drugs, medical devices, screening programs, etc
- BUT – have not adapted risk paradigms adequately to assess rare diseases appropriately but differently

Governments

- Some provide good recognition of patient group roles. They consult us and include us.
- Some do not.
- Most governments provide minimal support for groups despite increased expectations of us.
- Sustainability of groups is a challenge, especially in tight economic times.
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Payers (Government or Insurance)

- Many are tightening criteria for access to services and therapies
- Health Technology Assessment and health economics criteria MUST include equity and social values in decision-making

Patient Group Issues

- Last century's problem for groups was “Doctor knows best” - That issue is largely resolved but some groups are still waging that war
- This century's problem is “the health economist knows best” - There is no greater risk than a health economist without a moral compass
- Regulation of risk assessment (drugs and screening) needs reviewing– must be more inclusive of informed patient interests – must think differently about rare
- For most rare patient groups, early diagnosis is a bigger problem than lack of therapies

The challenge

Is not to adapt to the system as it is

It is to shape the system to respond better to our needs

Thank you