



**At the Inauguration of the NGO Committee for RD, United Nations, New York, 11 Nov. 2016**

Thanks for the opportunity to talk to this important meeting on rare diseases at the UN today.

ICORD, the International Conference on Rare Diseases and Orphan Drugs, has been in existence since 2005 and has held 11 annual meetings so far. We are a multi-stakeholder society, and that's an important feature. We are a place when representatives from academia, medicine, industry, research, regulatory agencies, and patient advocates, can work together to share experiences and develop a common agenda to improve outcomes for those with rare diseases.

The multi-stakeholder aspect is a distinguishing feature of ICORD, one which sets it apart from other important groups such as RDI, IRDiRC, and various health professional bodies, yet enables an engagement with these complementary groups to form a strong coalition of rare disease interests. This approach is an important part of effective engagement with governments and health systems to improve knowledge and care for rare diseases.

ICORD's annual meetings generate debate and activity in the region in which it is held. In the early years our annual meetings helped facilitate some alignment of US and EU medicine regulatory requirements for rare diseases. Later on we have focused on taking our meetings away from the US and Europe to boost debate and activity in a variety of countries. In all countries we have succeeded in getting government officials and politicians to engage with ICORD and focus on how they address the issues in their country. Outcomes include a decision in Japan, after our meeting there, to add nearly 200 diseases to the programme they have for rapid access to social supports and medicines for rare diseases. After the recent meeting in Cape Town, South Africa, the meeting stimulated the formation of a regional network of rare disease groups for the southern half of the African continent.

Seven years ago ICORD developed our Yukiwariso declaration which sets out our rationale for rare disease policies and actions in all countries. This is an important document as it ties the needs of rare diseases to the fundamental principles of universal healthcare, and connects them to the development agenda, currently outlined in the Sustainable Development Goals. It draws in the human rights and moral factors that should guide decisions about rare diseases as a public health priority.

This declaration was written to influence governments and the international policy bodies such as the UN, the WHO and the World Bank, and it's a matter of great satisfaction to see this happening today at this inaugural meeting of the UN committee for rare diseases. Our engagement with the UN Development Agency has generated a very positive statement from agency administrator, Helen Clark, placing rare diseases squarely in the development agenda and linking them to universal healthcare and the SDGs. This is an outcome of the "long-game" approach ICORD has followed, of setting out the principles and the objectives, and working on them over time.

The next step is to extend the declaration to give advice on rare disease policy and action plans to the developing world. Template approaches will not provide solutions in other parts of the world, without careful consideration of the practical steps required to adapt ideal models to the reality faced in many different countries. Work has begun on that.

Outside of our annual meetings we have also engaged with the UN Secretary-General's high level panel on innovation and access, and it is very pleasing to see their recent report has included rare diseases among public health priorities, alongside non-communicable diseases, tropical diseases and infectious diseases. It was a great delight to us when in Cape Town to hear Ms Precious Matsoso the Director-General of health for South Africa, who was also on that panel, speak about the needs of rare diseases as a priority alongside these other issues, including anti-microbial resistance.

Today, it is clear to see that rare diseases are issues whose time has come. We are here at the centre of global debates on such topics, and it is very satisfying to have made it here. But we must keep the momentum going to ensure it becomes an unstoppable force. This requires cooperation across all the stakeholder interests, and though the likes of RDI, IRDiRC, Orphanet, and other stakeholder groups, along with ICORD, might make a slightly "lumpy" coalition, it is important we keep working together to achieve recognition of our needs.

We need to address many diverse issues including HTA, human rights, moral factors, research needs, screening criteria, better prevention, faster diagnosis, and improved clinical care, patient information and protection, social support and education needs. We neglect any of these at our peril. And we need to demonstrate to health and research decision makers how their traditional view of these topics may need to change to address the many specific needs of rare diseases that, currently, systems often struggle to deal with.

This important meeting today, to inaugurate the UN CfRD, has come about a bit faster than I anticipated and it is already clear that many different efforts have pushed and pulled us to this point. ICORD played its part while other groups such as Eurordis and Agrenska did much to get us here as well. This clearly demonstrates the effectiveness of multiple drivers and diverse interests working to a common objective. ICORD looks forward to working with you all to make even more progress on this in the future.

/John Forman, ICORD Past-President