Rare Disease Day Policy Event

The Human Rights of Persons living with Rare Diseases

Keynote remarks by ASG Andrew Gilmour OHCHR

Thursday, 21 February 2019, Conference Room 8, United Nations, New York

Your Excellencies,
Ladies and Gentlemen,

I'm happy to be with you today to talk about the human rights of persons living with rare diseases.

While figures vary, some estimates indicate that between 300 and 400 million people worldwide live with rare diseases,[1] half of whom are children[2] - more than people living with AIDS and cancer combined.

Information on how people living with rare diseases are affected is often very poor, and the services provided both inadequate and insufficiently adapted to their specific needs.

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[1] International Federation of Pharmaceutical Manufacturers and Associations, Rare Diseases: Shaping a Future with No one Left Behind, Geneva, 2017, p. 7, available at: https://www.ifpma.org/wp-content/uploads/2017/02/IFPMA_Rare_Diseases_Brochure_28Feb2017_FINAL.pdf. See also Global Genes, "Facts & Stats about Rare Disease", available at https://globalgenes.org/wp-content/uploads/2015/12/2016-WRDD-Fact-Sheet.pdf and Submission received from NGO Committee for Rare Diseases (2017).

[2] Global Genes, "Facts & Stats about Rare Disease", available at https://globalgenes.org/wp-content/uploads/2015/12/2016-WRDD-Fact-Sheet.pdf.

To break with the past and ensure that all persons living with a rare disease live in dignity, a human rights framework should be the standard against which results are measured.

The right of everyone to enjoy the right to health, without discrimination, was affirmed as early as 1946 in the WHO Constitution, and guaranteed in the Universal Declaration of Human Rights and other international agreements.

Complementing the human rights framework, the political commitments made in the 2030 Agenda for Sustainable Development provide an important additional framework for advancing the rights of persons with a rare disease.

“Leave no one behind” is a united entry point for human rights in a wide range of areas. Its transformative vision envisages a world with equitable and universal access to health care and social protection.

Addressing rare diseases is not just a social, medical or scientific issue. It is – at base – a question of human rights.

The right to health framework calls for States to ensure that health facilities, goods and services are sufficiently *available*, *accessible* and *affordable,* without discrimination.

It means that health facilities, goods and services must be gender-sensitive and culturally *acceptable*, scientifically and medically appropriate, of good quality, and respectful of medical ethics.

Other human rights, including the rights to privacy, information, education, work and participation, are integral aspects of the right to health.

While the right to health has been broadly accepted at a normative and a rhetorical level, it has rarely been integrated into public health practice. This has been a challenge in regard to mental health and certain sexual and reproductive rights – but it is also true of rare diseases.

A key factor which drives the marginalisation of rare diseases is the paucity of medical and scientific knowledge, with the result that many diseases remain undiagnosed and therapies are difficult to develop.[3] I have some personal experience, a young nephew has a serious condition so rare that there is no diagnosis, and therefore he doesn't qualify for much of the help he needs.

Rare diseases attract stigma and discrimination. Many people living with a rare disease are excluded from participation in employment and from integrating into society.

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[3] Rare Diseases Europe, Position Paper: WHY Research on rare Diseases?, 2010, p5, available at: https://www.eurordis.org/sites/default/files/publications/why_rare_disease_research.pdf

Exclusion from the job market arises from a lack of accessible facilities and flexible working arrangements. The absence of reasonable accommodations in schools also has an impact on the ability of children with rare diseases to access education, limiting opportunities for jobs in the future.

So what needs to be done to ensure respect for the human rights of persons living with a rare disease?

First, recognise and elevate the issue: As long as rare diseases are not identified as a policy priority, efforts to address gaps in treatment will remain marginal. As part of their obligation to ensure the right to health, States should develop national health strategies through a participatory and transparent process. Special attention should be paid to all vulnerable or marginalised groups, including people living with rare diseases.[4]

"Leaving no one behind" means – in practice – the prioritization of rare diseases along with other health concerns. This of course means ***dedicating resource***s – which we know is easier said than done. And States must also enable civil society actors to play a proper role in setting priorities and planning strategies.

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[4] Committee on Economic, Social and Cultural Rights, General Comment No. 14 (2000) on the right to health, para. 43(f).

Second, universal health coverage is indispensable for ensuring the availability and accessibility of medicines, therapies and services for people living with rare diseases.

The exclusion of rare diseases and conditions from medical coverage schemes is a violation of the right to health. As Member States move to seek consensus on a Political Declaration on Universal Health Coverage later this year, we need to ensure that rare diseases are covered and that people living with them benefit from this protection.

So, changing the situation for people living with rare diseases calls for the transformation of public health policy in a number of ways, including persuading health policy planners to embrace a human rights approach that takes into account the specific needs of people living with rare diseases.

The role played by civil society – including all of you in this room – is absolutely critical in this regard. Your efforts are deeply appreciated.

Thank you.