

## DRAFT

### Talking Points for the Rare Disease Day Policy Event at the UN, 21st February 2019

- The 2030 Agenda for Sustainable Development that all United Nations Members States have adopted commits governments to leave no one behind. The 2030 Agenda underscores that addressing inequality in all its dimensions is critical to making economic growth more inclusive and sustainable, and is essential for eradicating poverty, ensuring shared prosperity, advancing social progress and achieving sustainable development for all.
- While no specific target under any of the SDGs touches upon the situation of persons living with rare diseases<sup>1</sup>, about 300 million individuals worldwide are affected. Such magnitude resembles the figure of those living with chronic conditions such as diabetes.
- Persons living with rare diseases therefore are by no means a small group. Nonetheless, their needs are often not acknowledged, nor addressed which is contrary to the building principle and promise of the 2030 Agenda and the SDGs, namely leaving no one behind,
- More specifically, persons living with rare diseases must enjoy equal access to information, medical expertise, timely and quality diagnosis, as well as health care and treatments. In developing countries, accessing care and medicines can be even more difficult, as health services, specialists, and knowledge on rare diseases are often limited.

Rare diseases can also be cause of disabilities and SDG3 reminds us that

- “Ensure healthy lives and promote well-being for all at all ages” cannot be achieved until the rights and needs of persons living with rare diseases are not addressed and guaranteed. If we address the needs this has implications also on the family. In fact, families often have to deal with rare diseases on their own, spending large amount of time and economic resources to guarantee adequate support and care. When needed caring for a person with a severe disabling rare disease can imply a drop in family income, especially if one family member needs to reduce work hours or stop it altogether.
- All the above requires the development of strong social policy frameworks to be implemented at national level to overcome negative economic and social consequences for persons living with rare diseases and society. With proper treatment and medication, persons living with rare diseases are in the position to attain education, employment, leisure and full participation and inclusion in the communities they live in. This is a

---

<sup>1</sup> Defined as affecting <5/10 000 individuals in Europe, <200 000 people in the United States.

positive impactful way to achieve SDGs 1, 4, 8, 10, respectively “no poverty”, “quality education”, “decent employment and economic growth”, and “reduced inequalities

- Member States with the support of International Organizations need to make their social protection systems more inclusive to accommodate the needs of their citizens, that is also of persons living with rare diseases, guaranteeing that they and their families do not fall into poverty. Making social protection systems more inclusive also means that persons living with rare diseases enjoy rights and support on equal basis as their fellow citizens thus promoting inclusive growth.
- Furthermore, Member States have to further advance policy-making to address the needs of persons living with rare diseases, particularly, in the context of labour market policies. More specifically, these may entail, among others, enhancing accessibility to increase labour market participation of persons living with rare diseases and disability; ensuring that persons living with rare diseases are not discriminated in the labour market, as well as they are equally and adequately remunerated for their work allowing them to enjoy independent lives.
- Even if health-care systems cover treatment costs, the coverage might not be complete and the economic burden on the patients can still be catastrophic. Member States therefore have to progressively achieve full coverage to allow persons living with rare diseases to enjoy the highest standards of health, as well as to actively seek economic and social inclusion. More inclusive regulation must be developed to ensure that persons with rare diseases have access to adequate treatments and medicines. However, we have to implement the social model and not merely the medical one.
- To conclude, persons living with rare diseases face high risk of marginalization and exclusion due to the lack of a supportive environment.” The role of pressure groups and civil society is pivotal in enhancing the case of persons living with rare diseases, particularly with regards to treatments and medicines.
- We need to leverage the provisions enshrined in the 2030 Agenda on Sustainable Development and the Convention on the Rights of Persons with Disabilities to ensure that all persons living with rare diseases are not left behind.

#### **Additional inputs, for Q & A (as needed)**

- The UN Flagship Report on Disability and Development issued in December 2018 indicates that the employment-to population ratio of persons with disabilities aged 15 and older is almost half that of persons without disabilities. The report also highlights that 54% of persons with disabilities compared to 77% of persons without disabilities are literate,

while in some countries more than 50% of persons with disabilities have experienced discrimination.

- Due to the disabling nature of rare diseases, the implications behind these numbers are telling for persons living with them.
- Unpaid domestic and care work are more likely to be carried out by women who usually spend more time than men on these activities. The costs of family care for persons living with rare diseases are thus likely to reinforce gender dynamics that the Agenda 2030, and more specifically, SDG 5 pledges to overcome.
- The CRPD provides that States Parties recognize the right of persons with disabilities to enjoy the highest attainable standard of health without discrimination on the basis of disability. The CRPD, along with the United Nations Universal Declaration of Human Rights (Article 25.1) and the International Covenant on Economic, Social and Cultural Rights (Article 12.1), are instruments that need to be leveraged to guarantee that the right to health care is reality for all persons living with rare diseases.
- Member States need to recognize and value, as enshrined in SDG's target 5.4, unpaid care and domestic work through the provision of public services, infrastructure and social protection policies and the promotion of shared responsibility within the household and the family as nationally appropriate.
- The role of pressure groups and civil society is pivotal in enhancing the case of persons living with rare diseases, particularly with regards to treatments and medicines.
- International cooperation can also be leveraged to support developing countries in adopting strategies that enable them to work on a dual track. That is, to guarantee access to primary care while ensuring that the needs and rights of persons living with rare diseases are met.
- The role of partnerships will also be pivotal to create economies of scale, which can make research and development more appealing to academia and the private sectors. Countries may also benefit in working together on treatment of health diseases to reduce cost of production of the treatment per patient.
- International cooperation can play a crucial role to address specific bottlenecks related to rare diseases in developing nations and investigate ways in which screening, diagnosis, treatment and clinical training can be provided as part of official development cooperation.