



To 'Build Back Better', do not leave behind people living with a rare disease in COVID-19 response and recovery

What this global pandemic has shown is that "the virus does not discriminate, but its impacts do"

Antonio Guterres, United Nations Secretary General

Rare Diseases International (RDI) wishes to bring to the urgent attention of policy makers and authorities the increased vulnerability that the 300 million people living with a rare disease (PLWRD) worldwide are facing during the COVID-19 pandemic. The impact and recommendations outlined in this statement can be extended to the undiagnosed population, a group within the rare disease community that is experiencing paralleled vulnerabilities, in addition to facing a diagnostic odyssey.

RDI commends the commitment and actions of health and social care professionals, as well as policy makers from all over the world during the crisis. In addition, **RDI calls for continued and reinforced efforts to: 1. Eliminate the risk of exacerbating pre-existing inequalities; and 2. Protect people living with a rare disease from being discriminated and becoming even more vulnerable in COVID-19 response and recovery strategies, as well as in long-term health system restructuring plans.**

The onset of the COVID-19 crisis has unveiled pre-existing health, social and economic inequalities that prevail between and also within countries of the world and has demonstrated the need to urgently address them. **PLWRD are a vulnerable population that is disproportionately impacted considering the chronic, highly complex, progressive and severely disabling nature of their diseases, which generate specific care needs. As such, PLWRD need to be considered a high-priority high-risk population during COVID-19 response and recovery.**

RDI welcomes the efforts led to protect people living with non-communicable diseases (NCDs)¹ and people living with all kinds of disabilities² during the COVID-19 pandemic, and wishes to highlight that PLWRD are often marginalised, underserved, and forgotten even within these populations. **With this statement, RDI urges authorities, NCDs and disability advocates to acknowledge that PLWRD are an identifiable vulnerable population having specific needs and concerns linked to rarity and requiring a targeted approach during COVID-19 response and recovery.**

Member States have acknowledged the need to protect populations with pre-existing conditions from COVID-19³. **In order to 'build back better' it is essential to remind them of their universal health coverage (UHC) commitments, which go beyond COVID-19 itself and include specific efforts to address rare diseases.** Indeed, this reflects the UN Political Declaration on UHC⁴ and is in line with the guiding principle to 'leave no one behind' of the Sustainable Development Goals (SDGs) and earlier UN Resolutions, such as WHA63.17 on Birth Defects⁵.

1. The impact of the COVID-19 pandemic on the global rare disease community

RDI and its members have collected the experiences of persons living with a rare disease through surveys at the national and global levels. Despite differences in the extent of impact depending on the country of residence - related to differing regulations, infrastructures and organisation of care, level of access to information and services, organisation of civil society, and stage in the pandemic - **results have demonstrated a similar experience for this vulnerable community across the world.**

The common global experience of the rare disease community is one of **overall disruption in access to care.** During this crisis, people living with a rare disease have experienced limitations, delays - and at times denial - in access to testing, health services, surgeries, rehabilitation therapy, supportive care, and medicines.

It is important to note that if the collected data reveals such a negative impact, in countries where there is practically no support and infrastructures for PLWRD and where such data collection is not possible, the impact of the pandemic is surely even worse.

¹ Efforts led by this community have ensued in the creation of a WHO Working Group on COVID-19 and NCDs which releases guidance and collects data, among other actions. Info [here](#).

² The [International Disability Alliance \(IDA\)](#) leads strong advocacy on COVID-19 strategies that take into account the needs of persons with disabilities. In addition, the UN and WHO have released guidance on disability-inclusive response ([here](#) and [here](#)).

³ World Health Assembly Resolution 73.1 on COVID-19 response, available [here](#).

⁴ [Political Declaration on Universal Health Coverage](#), reference to rare diseases under par. 34

⁵ Sixty-third World Health Assembly WHA63.17 on Birth Defects, May 2010, available [here](#).

Overall:

- **PLWRD have experienced interruption in the care they usually receive** either by not being able to meet with their healthcare professionals, or by their care being put on hold (9 out of 10 people according to a global survey⁶, 85% in Colombia⁷, 74% in the USA⁸, 50% of respondents in Canada⁹)

In addition, a testimony from a rare disease organisation in the Asia Pacific region¹⁰ reads *“the usual centres for rare diseases have been embargoed for COVID-19 response and rare disease patients have had to turn to overcrowded public hospitals, which leads to greater danger of infection”*.

- **People have not been able to access appropriate testing in relation to their disease**, which could prevent them from getting accurately diagnosed (6 in 10 people according to the global survey).

Linked to the issue of diagnosis, a testimony from an international federation for a rare disease shows the impact that the COVID-19 can have on awareness of rare diseases at the clinical level: *“Opportunities for patients and clinicians to network has been greatly affected, leaving patients less informed and empowered and fewer clinicians educated about a group of conditions that few clinicians know about”¹¹*.

- **People have seen their appointments for rehabilitation therapies, such as speech and physical therapies, postponed or cancelled** – even when these therapies represent the only available treatments (8 in 10 according to the global survey, 50% in Canada).
- Not having access to infrastructures, healthcare workers and support services has increased the caregiving burden on families who have also lost their support from extended family and friends due to confinement measures. As a consequence, **carers have had to stop working or significantly reduce their working hours since the pandemic started** (3 in 10, according to the global survey), leading to increased pauperisation of families.

⁶ [Rare Barometer Survey](#) (June 2020) based on 8551 respondents (people living with a rare disease or their carers) from all EU countries and beyond

⁷ According to the breakdown results of the Rare Barometer survey the impact was also high in other Latin American countries: 95% in Uruguay, 94% in Ecuador, 89% in Peru, 85% in Mexico, 76% in Chile, and 75% in Argentina

⁸ <https://rarediseases.org/infographic-illustrates-impact-the-covid-19-pandemic-is-having-on-the-rare-disease-community/>

⁹ <http://www.raredisorders.ca/content/uploads/Applying-Lessons-from-COVID-19-3.pdf>

^{10 11} Collected through a survey carried out by RDI with all its member organisations on COVID-19 impact and response

- **PLWRD have also faced difficulties in accessing their medicines and treatments as a number of countries have encountered supply difficulties** (40% in Canada). For example, in patients with haemoglobinopathies, *“the pandemic has secondary consequences, such as blood shortages”*¹² that may have long-term effects on health status.

Also, a key feature of the rare disease community is the lack of effective treatments, and therefore greater need to continue fostering research and innovation for rare diseases. **The COVID-19 pandemic poses a threat to the continuation of clinical trials and research on these diseases.**

In addition, both confinement and deconfinement measures can have a severe psychological impact on PLWRD and their carers/families due to increased isolation and uncertainty, and to disruptions in access to mental health services (almost 6 in 10 have seen their psychiatry follow-up interrupted according to the global survey). Under normal circumstances, being affected by a rare disease has a strong impact on mental health, and the COVID-19 crisis has exacerbated this.

Furthermore, **PLWRD are particularly vulnerable to negative economic consequences** ensuing from the crisis’ impact on the labour market and the economy overall, such as lay-offs and reduction in income, loss of benefits due to loss of employment, etc. A rare disease organisation from South Africa declared that *“Some people living with a rare disease have lost their medical insurance cover due to the COVID-19 pandemic”*¹³. This could result in further difficulties to access the care and services they need for their health and a life in dignity.

PLWRD are concerned that worsening finances in individual countries will lead to long-term consequences in terms of withdrawal or non-provision of health and social care support for them.

2. The added value of organisations of PLWRD to the global response to COVID-19

Civil society has proven to play an essential role in identifying and meeting the needs arising from the COVID-19 pandemic. **Organisations of PLWRD worldwide have been no less in these efforts and should be seen as key partners in the global response to the crisis.**

It is important to understand that organisations of PLWRD emerged as a result of experience gained by patients and their families from being excluded from health care systems and having to manage their own disease themselves due to lack of knowledge

¹² Farmakis, D., Giakoumis, A., Cannon, L., Angastiniotis, M. and Eleftheriou, A., 2020. COVID-19 And Thalassaemia: A Position Statement of The Thalassaemia International Federation.

¹³ Collected through a survey led by RDI with all its member organisations on COVID-19 impact and response

and attention by the scientific community and competent authorities¹⁴. They also stem from the need to feel connected to other individuals and families experiencing the same issues. These organisations often gather, produce and disseminate limited existing information on a disease, they provide support services to PLWRD and they advocate on their behalf for better lives.

This supportive role has become essential during the COVID-19 crisis. RDI member organisations around the world have taken different actions, including:

- Developing surveys to identify the impacts of COVID-19 and the needs of their constituents;
- Releasing public statements to raise awareness on the situation of their members and reaching out to national authorities about the specific needs of PLWRD;
- Developing information resources catered and adapted to the needs of their constituents and offering forums for discussion where PLWRD can voice their concerns and exchange good practices;
- Assisting patients to continue to access treatment and care, for example through the creation of Special Emergency Funds;
- Offering psychosocial support to patients and families;
- Ensuring PLWRD have access to protective measures (masks, hand sanitizer, etc);
- Helping local, national or regional authorities develop a response to the crisis that takes into account the specific needs of PLWRD.

In this context, RDI calls on policy makers to engage with and support organisations of PLWRD. These organisations are connected to their grass-roots communities and can back the healthcare and social services by ensuring information flow, good communication and peer support. 'Build back better' will require solutions that are co-created with patient organisations.

3. Recommendations to protect the rare disease community during COVID-19 response and recovery plans

COVID-19 has reinforced the call for equity embedded within recent international UHC agreements and has emphasized the need for explicit strategies to reach and protect vulnerable groups¹⁵. Ensuring equity in access to care directly contributes to the fulfilment of everyone's right to health, including that of PLWRD, while it helps prevent the spread of the infection, ultimately resulting in better health outcomes for all and

¹⁴ Aymé, Ségolène & Kole, Anna & Groft, Stephen. (2008). Empowerment of patients: Lessons from the rare diseases community. *Lancet*. 371. 2048-51. 10.1016/S0140-6736(08)60875-2.

¹⁵ UHC 2030 Discussion paper on health emergencies and UHC (May 2020), available [here](#)

economic prosperity and security¹⁶. It is essential to integrate a human rights-based approach in the response to COVID-19¹⁷.

For equity to work in practice and protect members of the rare disease community during COVID-19 response and recovery plans, RDI calls on policy makers to implement the following measures in accordance to the stage of the pandemic in each country:

- **Recognise PLWRD as a high-priority high-risk population** in need of targeted policies in the monitoring of and response to the COVID-19 pandemic.
- **Safeguard the continuity of care and access to medical consultation for PLWRD.** A key action is to direct funds and efforts towards reinforcing medical workforce and equipment. Having strong health care systems, with sufficient capacity to address the needs of all, would prevent discrimination and the need to resort to triage in clinical practice.
- **Guarantee access to testing and personal protective equipment (PPE) for PLWRD** as part of the vulnerable high-risk population group.
- **Ensure the adoption of concrete measures/protocols warranted by the complex needs of rare disease patients in the provision of emergency healthcare during the COVID-19 crisis.** This includes contacting the patient's permanent medical practitioner and/or expert centre, involving patients and carers in decision-making, and considering the creation of temporary special hospital wards for particularly vulnerable rare disease patients.
- **Vulnerable populations, including PLWRD, should be granted priority access to vaccine or other prophylactic treatment** if their health condition allows for such vaccination.
- **Access to medication to treat COVID-19 should also be ensured for vulnerable populations, including PLWRD.**
- **Governments should actively take measures to protect the most vulnerable groups in the population,** and not only leave measures to the discretion of individual service providers and employers. Clear non-discriminatory guidelines¹⁸ that prioritise access to healthcare, social care, opening of schools and educational facilities and resource centres are needed both during confinement periods and as these measures are progressively lifted.

¹⁶ UHC2030 Statement on COVID-19 and UHC (26th March 2020), available [here](#)

¹⁷ WHO Publication 21 April 2020: Addressing Human Rights as Key to the COVID-19 Response, available [here](#)

¹⁸ Refer to WHO Disability considerations during the COVID-19 outbreak, published 26 March 2020, available [here](#)

- **Schools, educational facilities and resource centres should be prioritised when relaxing confinement measures, as they are essential for both PLWRD and their family and carers.** These service providers should be supported to ensure that they have the necessary flexibility to assess individual situations and adopt adequate measures for PLWRD. The guiding principle in all policies put in place should be “assisting those who need intensive support to live independently”¹⁹.
- **Parents and caregivers of PLWRD (adult or child) should be waived from requirements to return to the office if/when their jobs allow for remote working - as exposure to the virus puts them and their loved ones at greater unnecessary risk.** When the nature of a job does not allow for home working, adjustments to the workplace or flexible working arrangements should be made to prevent people putting themselves and their families at risk. These guidelines should be clearly communicated to the public.
- **Facilitate the use of online consultations when people are not able to physically access healthcare facilities** because of logistical/practical reasons or fear. Access to an internet connection / other digital resources is not universal, therefore alternatives or support to access them need to be ensured. Necessary clinical assurance and data protection safeguards should also be guaranteed.
- **Ensure and facilitate the continuation of research on rare diseases** by, for example, disseminating and following-up on the guidance issued on clinical trials during the COVID-19 pandemic.

RDI and its members call upon the whole society to uphold the principles of solidarity, equity and cooperation in efforts to ‘build back better’.

All citizens must play their role in protecting the general population and the most vulnerable ones, in particular by adhering to the guidelines and minimising the risk of infection.

It is also the time to invest in people and prioritise strong, well-funded, public health and welfare systems that also address the needs of the most vulnerable, and to coordinate and collaborate in public health policy across borders.

The rare disease community stands ready to co-create this brighter future.

¹⁹ In line with article 19 (Living independently and being included in the community) of the Convention on the Rights of Persons with Disabilities.