

An IPWSO guide to the world of rare diseases, November 2020

A rare disease affects only a small percentage of the population, but collectively rare diseases are not rare, affecting over 300 million people worldwide.

Rare Diseases International ([RDI](#)) highlights that *'rare disease patients face common challenges derived from the rarity of their conditions'*. These common challenges must be tackled internationally, the World Health Organization ([WHO](#)) states that *'while a domestic focus is not particularly problematic for many health issues, for rare diseases it is disastrous'*. This is because due to the low prevalence of rare diseases individually, no country or region has the resources to tackle rare diseases alone.

Disparities in healthcare systems and rare disease management between countries and regions is a significant problem. RDI states that, *'addressing rare diseases on an international level is critical to reduce health inequalities between populations worldwide and ensure that people living with a rare disease have access to the same resources as any other population'*.

So a global effort on rare diseases is vital not only to make progress on diagnosis, treatments and care but also to ensure those living with a rare disease do not suffer from the disparities in healthcare systems between countries.

Who are the key players?

The United Nations ([UN](#)) is a highly important body for impacting the rare disease world as it takes meaningful actions to achieve its goals that can align with the global effort on tackling rare diseases.

With regards to global health its overarching goal is Universal Health Coverage (UHC,) that is *'all individuals and communities receive the health services they need without suffering financial hardship'*. The UN's efforts in global health are led by the World Health Organization, a specialized agency of the UN. WHO's primary role is to *'direct and coordinate international health within the United Nations.'* The decision-making body of WHO is the [World Health Assembly](#), which is held annually in Geneva, Switzerland and it is structured around a specific health agenda. One of WHO's top priorities is ensuring Universal Health Coverage (UHC,) including access to diagnosis and treatment for people with rare diseases.

IPWSO relies on donations to support people with PWS and their families around the world. To find out more about our work and donate please visit us at www.ipwso.org/donate

What can IPWSO do to influence the UN and WHO?

IPWSO can work to influence the UN and WHO and as a result also influence international health policy through being a part of the larger rare disease organisations such as RDI, [EURORDIS](#) and the [NGO Committee for Rare Diseases](#). For example, RDI and WHO are collaborating on the development of a *'global network of centres of excellence for rare diseases'*. IPWSO is a member of RDI and is very engaged in their activities and will seek to join EURORDIS and the NGO Committee for Rare Diseases in the near future.

We have recently written a report looking at our role in more depth. If you would like a copy of *'Putting Prader-Willi syndrome on the map: navigating the world of rare diseases'* do let us know.

Non-Rare Disease movements and organisations

Our interviews with various specialists and advocates in the field of rare diseases and Prader-Willi syndrome, has shown the need and benefit of also connecting with non-rare disease communities and organisations. RDI told us that it was incredibly important for the rare disease community to collaborate with other relevant groups. As well as rare diseases the needs of people with PWS can sit within the human rights movement, disability movement and beyond.

How can national PWS associations get involved?

How to be engaged in the rare disease world:

- Look out for international activities centred around rare diseases
 - For example, participating in [Rare Disease Day](#).
- Form a strong relationship with your national rare disease alliances.
 - National rare disease alliances will be very influential in the future, for example in RDI's work with WHO.
- Where there is no rare disease alliance, reach out to a national disability alliance, they may know someone who might have or has Prader-Willi syndrome.
- Link with non-rare disease organisations and groups that are part of the PWS identity:
 - Our voices are stronger and more impactful together.
- Advocate for the development of rare disease plans and organisations in your country if they do not already exist.

Keep us informed!

We are really excited to be working more closely with colleagues in the rare diseases world and would love to hear about how you work with other organisations in your country and what the impact of that has been. Do get in touch with us with your comments or to request our report on office@ipwso.org.