

Statement by Mr Philippe Douste-Blazy, Under-Secretary General of the United Nations, Candidate to the Position of WHO Director-General



All human beings should be equal as regards health. In this sense, the third Sustainable Development Goal reminds us of everyone's absolute right to live in good health. And yet still today, too many people remain left behind, with no access to quality care. Particular attention should be paid to rare diseases, as it is our responsibility to provide everyone with equal access to health systems.

As a candidate to be the next Director-General of WHO, work in the area of rare diseases is of central importance to me, and I would like to commend the members of the European Organisation for Rare Diseases (EURORDIS) for their commitment and determination.

On the occasion of the inauguration of the NGO Committee for Rare Diseases, I would also like to recall the vital importance of health-related research and development. Innovation is key in order to meet the challenge of rare diseases, particularly in areas where the market does not allow treatments to be developed.

Philippe Douste-Blazy

Under Secretary General of the United Nations

To which extent do you personally consider that rare diseases can contribute to supporting and advancing the UN Sustainable Development Goals?

With more than 300 million people affected, and more than 7000 different types of diseases, rare diseases represent a huge challenge in public health that has not been addressed at all for many years. Most of them are genetically related issues and 50% of them appear among children. I believe in scientific, evidence based intervention in public health, and that access to innovative simple and better tests and medicines is a great part of the effective access to care. The progress on the research on the human genome and the progress to come in simplification of diagnostics, and potentially new and innovative therapeutics, including promising gene therapies will be part of the future of medical science and will represent important challenges in equity and access to care. In that sense, investing in R&D and thinking about access to fight rare diseases is also a way to address many challenges of the future of care for non-communicable diseases and also infectious diseases.

I also believe that the implication of civil society and NGOs is a key success factor for public health, and I know that this is the case in the fight against rare diseases to promote awareness, research and access to funding.

In her written statement to be delivered at the 11th Annual International Conference on Rare Diseases and Orphan Drugs (attached), UNDP Administrator Helen Clark expresses the view that "no country can claim to have achieved universal healthcare if it has not adequately and equitably met the needs of those with rare diseases". As a candidate for the leadership of the World Health Organization, what do you see as the role the WHO could play to that end?

I support the statement made by Helen Clark. Equity is one of the key values of the UN. WHO is the lead agency for public health of the UN system, and should enhance its global leadership. One of my priorities will be to improve access to affordable and better quality tests and medicines. This will include more work with member states, regulators and civil society and academia, to promote pharmaceutical R&D based on a public health agenda, including the need to fight rare diseases. The need for incentives for industry should be balanced with clear access programs and commitments in term of price policies and close monitoring.

The UN High-Level Panel on Access to Medicines stated in its Terms of Reference that "millions of people remain left behind when it comes to accessing medicines and health technologies that can ensure their health and well-being [and are] being denied access to lifesaving treatments for communicable diseases like HIV, TB, Malaria and Viral Hepatitis, non-communicable diseases (NCDs), NTDs and rare diseases". How can the WHO, but overall the UN system at large, have a more decisive impact in improving access to treatments, particularly innovative ones?

I have been following closely the work of the High level panel on access to medicines. A lot of examples and contributions have been provided from the work we have been doing with UNITAID, in particular with the development of pediatric formulations for HIV-AIDS, Malaria and TB. None of these formulations existed when UNITAID was created ten years ago, and that is why we set pediatric formulations as a core priority. This has been done with a clear strategy in working closely with key partners, WHO and Unicef as part of the UN family, but also Foundations, NGOs, civil society, pharmaceutical companies and regulators. This is a major achievement which has changed the reality of access to care for millions of children and their families, a difference between life or death in many cases. This is the kind of work that is needed to fight rare diseases, and that needs clear WHO leadership and Partnership.

I would add that, in my experience, the creation by UNITAID of the Medicine Patent Pool has been key in the success of the fight against HIV-AIDS. Access to innovation needs also innovative ways to deal with IP issues and clear incentives. MPP has paved the way towards more proactive and responsive access for the fight against HIV, and may play an important role in other fields. The high level panel also paid attention to the de-linkage between R&D costs and the price of the medicines when they reach the market. This was also clearly highlighted in the recent report on antibioresistance and the need to develop new antibiotics. This is the kind of options that needed also to be on the agenda for rare diseases.

The inauguration of the NGO Committee for Rare Diseases on Friday 11 November will mark the first time ever that rare diseases actually receive consideration as a global public health priority for action within the United Nations. Do you wish to extend a word of support and encouragement?

This NGO committee for rare diseases is an important step in the history of the fight against rare diseases, This is not only the recognition of the importance of the issues of rare diseases, and the need to address this challenge in terms of equity for hundreds of millions of people and their families around the world but also a clear example where NGO s and civil society will make a difference for the people in need and public health. Innovation and access to medicines and diagnostics tools are the two faces of the same issue: how to crack the code of the scientific challenge and link this discovery into a robust pipe of innovation? And how can these innovations reach the people that suffer from the diseases, taking into consideration the consequences for their family and their social life. I know that the progress made is due to the personal and collective commitments of members of the committee and the organizations they represent. Be sure that I will do my best to provide the support that you need to contribute to fight rare disease.

