

Emerging Challenges in Health Policy: Multifaceted Case of Rare and Neurological Disorders in Pakistan

Health indicators of Pakistan are improving like increase in life expectancy, decrease in maternal and child mortality. However, the country still faces the double burden of disease along with disasters. More than 60% of this is due to non-communicable diseases which includes rare diseases.¹

A rare disease is found in 1 in 2000 people and has certain characteristics, e.g., 50% of them are genetic or childhood diseases, are usually severe, chronic, progressive, degenerative, and life-threatening. Most cases lead to disability with compromised quality of life. Such diseases have high psychosocial burden both for patients and caregivers and stigmatisation. Lack of access to correct diagnosis is one of the biggest challenges in treating these diseases. There are usually delays in diagnosis and sometimes misdiagnosis. Referral paths and treatment guidelines are also not available in countries, like Pakistan. Treatment requires a multidisciplinary approach that includes physiotherapy, nutritional assessment, and psychological care. Cost of treatment can, therefore, be high.

At the moment, around 300 million people are living with 6000 rare diseases all over the world (RDI 2019). While every disease has its own symptoms, treatment, challenges and issues, there are some common factors and implications, which requires that each country should have a 'Rare Disease Policy' in place.

A resolution adopted by the UN General Assembly on 16 December 2021 on 'challenges for persons living with rare diseases' focuses on 'recognizing the need to promote and protect the human rights of all the persons, including estimated 300 million living with rare diseases worldwide.' The Resolution represents a major shift in the global policy landscape, 'encouraging greater integration of rare diseases in the agenda and priorities of the UN system.' It calls upon Member States to 'strengthen health systems, notably in terms of primary healthcare, in order to provide universal access to a wide range of healthcare services that are safe, of quality, accessible, available and affordable, timely, and clinically and financially integrated.'

Pakistan is also working towards Universal Health Coverage and trying to improve its Universal Health Coverage Index ranking from 50 to 65 by 2030 under the Sustainable Development Goal (SDG) target (MoNHSRC 2021). It has also implemented Universal Health Insurance in two provinces and in the Federal Capital, Islamabad. However, there is no specific policy/ strategy or treatment guidelines for standard of care for rare and neurological² diseases in the country. Furthermore, no data or disease registry is available to correctly gauge the magnitude of these diseases which is a barrier to evidence-based policymaking and planning.

In this regard, the session will discuss the following issues to come up with policy recommendations to address the challenges in dealing with rare and neurological diseases in Pakistan, with a particular focus on Hemophilia and debilitating spinal diseases for which supportive treatment can improve quality of life:

¹ Rare diseases include, but are not limited to, rare genetic diseases. They can also be rare cancers, rare infectious diseases, rare poisonings, rare immune-related diseases, and rare undetermined conditions.

² 'Neurological disorders are diseases of the central and peripheral nervous system. In other words, the brain, spinal cord, cranial nerves, peripheral nerves, nerve roots, autonomic nervous system, neuromuscular junction, and muscles. These disorders include epilepsy, Alzheimer disease and other dementias, cerebrovascular diseases including stroke, migraine and other headache disorders, multiple sclerosis, Parkinson's disease, neuroinfections, brain tumours, traumatic disorders of the nervous system due to head trauma, and neurological disorders as a result of malnutrition, etc.' (WHO 2016).

- What is the status of disease awareness in Pakistan in context of neurological diseases and how can it be improved?
- How can Pakistan move forward in the establishment of a disease registry?
- What should be the mechanism for development of a national policy/ strategy/ action plan for rare and neurological diseases?
- What should be the effective mechanism to develop treatment guidelines and patient pathways? How can those be implemented?
- What can be different ways and innovative financing solutions for rare and neurological diseases to cover prevention, screening, diagnostic and treatment services?

References

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