

## Health Minister expedites process of rare disease policy formation

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With the new Government taking the chair, rare disease patients in India see a new ray of hope. Last week, Union Health Minister, Dr. Harshvardhan directed ministry officials to speed up the process of formulating the National Policy for Treatment of Rare Diseases (NPTRD), in consultation with all stakeholders. Patient associations have lauded this recent move and are looking forward to a policy which is patients friendly.

Rare diseases are severe and chronic illnesses and are often life-threatening. Patients suffering from rare diseases, especially Lysosomal Storage Disorders (LSDs), often lead a very incapacitating life. There are about 50 LSDs out of which only 5 have approved treatment available in India. About 180 diagnosed patients across India have submitted their application to the Govt. for treatment support for such treatable LSDs.

Elaborating on the condition Dr. Ratna Puri, Senior Consultant and Chairperson, Institute of Medical Genetics and Genomics, Sir Ganga Ram Hospital, Delhi said, “Lysosomal Storage Disorders (LSDs) are a group of around 50 inherited disorders which occur due to defects in the lysosomal function. Lysosomes are enzymes performing a critical function in the body. Even if one of these enzymes are dysfunctional, it will cause one of the LSDs. Till now treatment is available only for a few of the LSDs- Pompe, Fabry, Gaucher, MPS (Type I, II, IV A, VI) and has proven to be effective. If treatment isn’t provided on time, these conditions can affect the quality of life of a patient dramatically and they are unable to do even simple daily tasks. Timely treatment is, thus, crucial for improving the quality of lives of patients.”

Lauding Dr. Harshvardhan’s efforts Manjit Singh, President, LSDSS, “It has been a long and painful journey for rare disease patients and caregivers to reach this stage. 50 patients have lost their lives in this arduous journey of over 3 years. These patients could have been saved had they been provided timely treatment. In India, there are only about 400 patients who are suffering from treatable LSDs. It’s a commendable move by Dr. Harshvardhan to get NPTRD back on track. It has given a wave of optimism for rare disease patients. In the meanwhile, it would be great if the Government can provide interim treatment support to those rare disease patients who had applied to the Rare disease cell, till the time the policy gets finalized

and implemented.”

Prasanna Shirol, Co-Founder, Organization for Rare Diseases India (ORDI) said, “Dr. Harshvardhan’s move has directed the Central Government to play its part in finalizing NPTRD. We are hopeful that this time Government and ministry will treat every life as equal and precious and provide treatment. At the same time, health being a state subject, it is equally important for states to understand the need of the hour and create rare disease patient-friendly environment. The State Government should create rare disease center of excellence in every state to address the needs of rare disease patients with timely and effective intervention and also their own state policy based on the state priorities.”

The onus now lies with the Government to act on their words and provide relief of rare disease patients in the country.