

**Abstract Title:** Challenges in ERT for treatment of LSDs in India

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**Abstract:** Enzyme replacement therapy (ERT) is the standard of care in the treatment of selected lysosomal storage disorders (LSDs). Recombinant ERT is not manufactured in India and the challenges of treating Indian patients are many.

The most important of these is the cost. Given lifelong, as weekly or fortnightly intravenous infusion, the average annual cost in a 10kilogram child ranges from 30-50 lakh rupees. Till the announcement and implementation of the National Policy for Rare Diseases India, in 2021, the cost of treating the affected relied on charitable access programmes, crowd-funding or rarely self-funding. Beyond the expense, other challenges include lack of prevalence data about individual LSDs, non-availability of ERT for Mucopolysaccharidoses Types IVA and VI in India, limited response of neuronopathic and cardiac variants of Gaucher disease to ERT, lack of specialized centres with expertise to monitor therapy, limited public awareness about these disorders delaying early diagnosis, as well as lobbying for customs' duty and tax exemption for these therapies.

I will discuss the steps taken nationally to bring many more affected individuals into a continuously monitored programme of ERT and the new challenges that prevail.

**Area of expertise:** Clinical Genetics, Lysosomal Storage Disorders, Clinical Dysmorphology