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EDITORIAL COMMENTARY

Economic Evaluation of Rare Disease Therapies in India — The Time has Come!

Neerja Gupta[1] · **Mounika Endrakanti**[1]

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The introduction of Enzyme replacement therapy (ERT) for Gaucher disease (GD) in 1994 heralded the beginning of the era of therapeutics for rare genetic disorders, particularly lysosomal storage disorders (LSDs). Although many more LSDs have been approved for ERT subsequently, ERT in GD patients remains far more effective in alleviating disease manifestations, and improving quality of life, thereby prolonging their life expectancy. Sustained benefits with symptom control and prevention of disease progression however mandates lifelong treatment. Currently, in India, the treatment for rare inherited conditions is either out of pocket expenditure or through reimbursement for government employees or provided through the National rare disease policy up to a limit of 50 Lakhs. To optimally utilize the available limited resources, it is important to perform an economic evaluation of existing therapies.

The economic evaluation of therapeutics in GD type 1 and 3 by Mhatre et al. emphasizes the necessity to extend such studies to various other rare inherited conditions with significant treatment costs [1]. The cost of disease-specific treatment [ERT/Substrate reduction therapy (SRT)] alone (₹1,80,69,091.36 per patient) constituted 99% of the total annual healthcare cost of all the patients with GD managed at the center. The cost spent on each patient receiving disease-specific treatment was about 180 times higher than that of ERT/SRT treatment-naïve patients (₹1,00,941.5 per patient). Although the ancillary costs involved in diagnosis, investigative work-up, hospitalization, and other supportive treatment were proportionately low when compared to ERT, these costs incur economic burdens in both treated and treatment-naïve groups and are mostly out-of-pocket expenses. The striking contrast in the expenses associated with therapy as compared to that without, underscores the substantial economic burden placed by disease-specific therapies in GD on the health-care system, in the Indian setting. The study emphasizes the relevance of economic evaluations in promoting efficient use of finances and resources low- and middle-income nations [1].

In health economics and cost-effectiveness analysis, Incremental Cost Effectiveness Ratio (ICER) represents the additional cost needed to achieve an additional unit of health benefit [measured in terms of Quality-Adjusted Life Years (QALYs) gained] when comparing two interventions. ICER values of a health intervention that exceed the willingness-to-pay threshold suggest higher additional cost needed to achieve an additional unit of health benefit than what the society or policymakers are willing to pay suggesting poor cost-effectiveness. The systematic reviews by Connock et al. in 2006 (£380,000 per QALYs gained) and Katsigianni et al. in 2022 (€884,994 per QALYs gained) reveal a consistent pattern of high ICER values for ERT in LSDs, including GD [2, 3]. The evaluation of cost-effectiveness of ERT in a Dutch cohort of GD patients, revealed a 97% higher average cost of living in patients on ERT as compared to treatment-naive patients [4]. These observations suggest that the cost-effectiveness of ERT, measured in terms of the cost spent per additional QALY gained, is notably unfavorable in economic terms despite the treatment being effective. It also typically exceeds the willingness-to-pay thresholds for cost-effectiveness in healthcare.

Improved awareness about the diagnosis of treatable conditions necessitate attempts to enhance patient accessibility and affordability of these expensive, but effective treatments. Although the National Rare Disease Policy is a major endeavour by the Government of India that seeks potential solutions to treat patients with rare diseases by providing financial assistance, it is pertinent to have stringent selection criteria for the treatment eligibility for proper utilization of available resources and larger health gains [5]. It is the time to focus on innovative approaches aiming at an actual reduction in the spiralling cost of ERT/SRT to

✉ Neerja Gupta
neerja17aiims@gmail.com

[1] Division of Genetics, Department of Pediatrics, All India Institute of Medical Sciences (AIIMS), New Delhi, India

yield higher benefits to ICER. To make sure that all eligible patients receive treatment, proactive policy decisions must be made, including concerted attempts to negotiate drug pricing, advocacy to promote local manufacturing, and the implementation of health technology assessments to ensure that the most cost-effective therapies are prioritized for rare disorders.

Declarations

Conflict of Interest None.

References

1. Mhatre SP, Muranjan M, Gogtay NJ. Economic burden of Gaucher disease at a tertiary care public hospital in Mumbai. Indian J Pediatr. 2023. https://doi.org/10.1007/s12098-023-04740-4.
2. Connock M, Burls A, Frew E, et al. The clinical effectiveness and cost-effectiveness of enzyme replacement therapy for Gaucher's disease: a systematic review. Health Technol Assess. 2006;10:iii–iv, ix–136.
3. Katsigianni EI, Petrou P. A systematic review of economic evaluations of enzyme replacement therapy in lysosomal storage diseases. Cost Eff Resour Alloc. 2022;20:51.
4. van Dussen L, Biegstraaten M, Hollak CEM, Dijkgraaf MGW. Cost-effectiveness of enzyme replacement therapy for type 1 Gaucher disease. Orphanet J Rare Dis. 2014;9:51.
5. National Policy for Rare Diseases 2021 | Ministry of Health and Family Welfare | GOI [Internet]. Available at: https://main.mohfw.gov.in/?q=newshighlights-45. Accessed on 18 Dec 2023.