

**Abstract Title:** Sustainable role of pharmaceutical companies in orphan drug development and drug repurposing for rare genetic disorders in India

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**Abstract:** Development of new drugs for orphan disease is challenging due to long timelines, very high cost of development and uncertainty in outcomes. Moreover, when the product reaches to market, it costs very high to patient.

Rare genetic disorders have very specific underlying pathophysiological derangements, which need drug specifically addressing the defect. While development of new drug has some possible accelerated development, it still has to follow all steps of conventional pathway.

Repurposed drug may not correct the underlying genetic defect; it can still be helpful in reliving symptoms and improving quality of life of patients and it is faster approach. The detailed pharmacological actions, safety profile and vast experience of use in population provides necessary helps in planning phase III studies directly. More importantly, it becomes available at a cost of generic drugs.

In recent times, Indian pharmaceutical industry has successfully developed NCEs, novel biologicals, biosimilar, antibody drug conjugates, indigenous COVID vaccines and most recently CAR-T therapy. Development capability along with comparatively lesser cost of development puts Indian pharmaceutical industry to an advantage for development of potential new drug as well as repurposing known drugs for orphan diseases. National Policy for rare disease and recent changes in drug regulations are encouraging development of drugs for orphan diseases.

However, for rare diseases, limited knowledge base, experience & understanding of the disease is a big challenge to the industry. To utilize the full potential and capability of Indian Pharmaceutical industry, close collaboration from academic institutes & clinicians sharing their guidance and experience in identification & clinical development of the drug is required. This could help develop drugs faster at lesser cost for rare genetic diseases.

**Area of expertise:** Clinical strategy, new drug development, repurposing of drugs