ISSN(O): 2320-5407 | ISSN(P): 3107-4928

ISSN NO. 2320-5407

# International Journal of Advanced Research

**Publisher's Name: Jana Publication and Research LLP**

*www.journalijar.com*

## REVIEWER'S REPORT

**Manuscript No.: IJAR-59508**

**Manuscript title: Who Gets Treated? Global Access to High-Cost Therapies for Duchenne Muscular Dystrophy.**

**Recommendation:**
Accept as it is .....
Accept after minor revision.....
Accept after major revision **YES**
Do not accept (*Reasons below*) .....

| Rating | Excel. | Good | Fair | Poor |
|---|---|---|---|---|
| Originality | | YES | | |
| Techn. Quality | | | YES | |
| Clarity | | | YES | |
| Significance | | YES | | |

Reviewer's ID: JPR- 148

## *Detailed Reviewer's Report*

Overall assessment: The manuscript addresses an important issue in global health equity: unequal access to high-cost treatments for Duchenne muscular dystrophy (DMD). It combines regulatory decisions, medicine pricing, reimbursement policies, and access barriers across countries. Its three-filter framework—biological eligibility, regulatory availability, and affordability—is useful and provides a clear structure for the discussion.

However, several methodological, numerical, and evidentiary issues need to be addressed before the manuscript is suitable for publication.

# Major comments

1. Review methodology: Although the authors identify the work as a narrative review, the search methods are insufficiently reproducible. Specify search terms, search dates for each source, and criteria for selecting and verifying information.

2. Accuracy of regulatory claims: The manuscript makes broad claims about international approvals and access. The statement that no relevant therapy is authorised in the European Union appears inconsistent with the manuscript's own report of conditional EU authorisation for givinostat. Clarify the distinction between individual products, marketing authorisation, and reimbursement.

3. Numerical consistency: Recheck the affordability calculations and ensure that the abstract, tables, figures, and discussion report consistent values. For example, the abstract states that the gene therapy costs more than 1,200 years of average income in India, whereas Table 2 reports 1,208 years at list price and 966 years after a 20% discount. Explain which estimate is used in each conclusion.

ISSN(O): 2320-5407 | ISSN(P): 3107-4928
International Journal of Advanced Research
Publisher's Name: Jana Publication and Research LLP
*www.journalijar.com*

**REVIEWER'S REPORT**

4. Evidence quality and balance: Some conclusions about pricing, commercial incentives, and the superiority of standard care are stronger than the narrative review design can establish. Distinguish documented findings from the authors' interpretations and acknowledge uncertainty in treatment effectiveness and cost comparisons.

5. Data sources and references: Several important claims rely on company announcements, trade publications, and secondary reports. Prioritise primary regulatory documents, peer-reviewed studies, and official reimbursement decisions wherever possible. Verify that every reference supports the associated claim and complete any missing bibliographic information.

6. Affordability measure: Comparing US list prices with national gross national income per capita illustrates price disparities, but it does not directly measure affordability for patients or health systems. Explain this limitation more prominently and avoid interpreting the ratio as the actual amount a household would need to pay.

7. Scope and interpretation: The manuscript includes six high-cost therapies but also discusses comparison treatments such as vamorolone, deflazacort, and ataluren. Define the scope consistently and explain how these comparison cases contribute to the research questions.

# Recommendation

Major Revision. The manuscript addresses a relevant and potentially valuable research question, and its comparative framework could make a useful contribution. However, publication should be considered only after the authors resolve inconsistencies in regulatory claims and numerical reporting, strengthen source verification, clarify the methodology, and ensure that conclusions are proportionate to the available evidence.