

Review of: "The Plight of Rare Diseases in Southern Africa: Health and Social Services Policy Recommendations"

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Potential competing interests: No potential competing interests to declare.

The authors have done a commendable job of raising awareness about rare diseases and suggesting ways to address the challenges and needs of the affected population. The manuscript is well-written and informative, but it could benefit from some revisions and enhancements.

1) The manuscript provides an overview of various rare diseases. However, it might be helpful to include some epidemiological background information for these conditions before suggesting possible solutions. This would help the readers to understand the prevalence, distribution and determinants of these diseases and their impact on public health.

2) In addition to describing the epidemiology and etiology of the disease, authors can also mention the existing therapeutic regimes available in the region. This information is relevant for assessing the feasibility and applicability of the proposed intervention. Moreover, authors should discuss whether these therapeutic regimes are accessible and affordable to all patients, especially those from marginalized or vulnerable groups. This would help to identify the gaps and challenges in the current health system and to justify the need for alternative or complementary solutions.

3) One of the challenges of developing and delivering treatments for rare diseases is ensuring their financial viability. Rare diseases affect a small number of patients, which limits the market potential and the return on investment for pharmaceutical companies. Therefore, authors who are involved in researching or advocating for rare diseases should address the issue of how to sustain the funding and the access to these treatments in the long term. This could include proposing innovative models of pricing, reimbursement, or collaboration among different stakeholders, such as governments, insurers, health care providers, and patient groups. By doing so, authors can demonstrate the value and the feasibility of their proposed solutions and contribute to the advancement of the field of rare diseases. I appreciate that the authors have discussed universal health coverage, but I wonder if the Governments in the region have resources to deliver this on the ground?

4) Centres of excellence are designed to foster high-quality research and innovation in specific domains. However, they also entail significant costs and risks, such as becoming obsolete or irrelevant over time. Therefore, it is essential to ensure that centres of excellence operate under a sustainable research model that aligns with the needs and priorities of the stakeholders, the funding sources, and the broader research community.

Thank you!

