

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

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This is an interesting, relevant, and important article, which takes up a clearly insufficiently addressed issue: Rare diseases in Southern Africa. I think the authors do a good job in presenting the problem, including laying out how rare diseases affect, despite the rarity of the individual disease, affect the lives of many people. I also believe that the authors provide some interesting and feasible policy proposals. A few suggestions for improvements:

- It would be interesting to know whether the proposals are similar or dissimilar to those taken in other parts of the world. Especially in regions/countries with low wealth levels and limited healthcare access.
- I think the authors move a little too quickly in the section on cost-sharing and universal coverage. While it is, of course, true that these measures will make it cheaper for the individual who has a rare disease, treatment (if available) might still be very expensive, and thus take up resources also needed elsewhere. Of course one can accept that as a consequence and consider it fair, but this consequence should be acknowledged or addressed. There is an extensive literature on rare diseases and priority-setting, which might be relevant to mention here (Albertsen 2022; Juth 2017; Magalhaes 2022; McCabe, Claxton, and Tsuchiya 2005; Paulden 2017; Paulden et al. 2015)

A minor thing: The abstracts contrast NCD and rare diseases, but aren't many rare diseases NCDs? The authors must have a different contrast in mind.

Thank you for a well-developed and interesting article. I hope the above is useful.

## Literature

Albertsen, Andreas. 2022. "Rare Diseases in Healthcare Priority Setting: Should Rarity Matter?" *Journal of Medical Ethics* 48(9): 624–28.

Juth, Niklas. 2017. "For the Sake of Justice: Should We Prioritize Rare Diseases?" *Health Care Analysis* 25(1): 1–20.

Magalhaes, Monica. 2022. "Should Rare Diseases Get Special Treatment?" *Journal of Medical Ethics* 48(2): 86–92.

McCabe, Christopher, Karl Claxton, and Aki Tsuchiya. 2005. "Orphan Drugs and the NHS: Should We Value Rarity?" *BMJ* 331(7523): 1016–19.

Paulden, Mike. 2017. "Recent Amendments to NICE's Value-Based Assessment of Health Technologies: Implicitly Inequitable?" *Expert Review of Pharmacoeconomics & Outcomes Research* 17(3): 239–42.

Paulden, Mike, Tania Stafinski, Devidas Menon, and Christopher McCabe. 2015. "Value-Based Reimbursement Decisions for Orphan Drugs: A Scoping Review and Decision Framework." *PharmacoEconomics* 33(3): 255–69.