

# Rare Diseases: Economic Evaluation and Policy Considerations

# Value in Health

Rare diseases are targets of an increasing fraction of new products. They have presented challenges for reimbursement approvals due to high pricing, limited evidence, and seemingly unattractive economic value. Should they be treated differently—and, if so, how? In this special themed section of *Value in Health*, the Editors seek broad-ranging papers addressing these issues.

Potential topics for this special issue include, *but are not limited to*:

### **Rarity, severity, unmet need, value, and equity**

- What distinguishes “rarity” from more prevalent conditions (eg, severity, unmet need, equity)?
- When is rarity per se an important factor to consider (eg, budget impact, quality of evidence expected) and how might this be addressed?
- Is there (as sometimes claimed) a societal preference for treating rare and severe diseases?
- How do rare diseases fit into the current focus on equity in HTA and ICERs?

### **Evidence in rare diseases: small and heterogeneous populations, uncertainty**

- Is there evidence that the nature of (clinical/quality of life and economic) evidential uncertainty in rare diseases is different from more prevalent conditions?
- How should HTAs accommodate the often more limited evidence available for rare diseases?
- Are existing economic approaches to dealing with uncertainty adequate for rare diseases?
- Is there evidence of more patient heterogeneity in rare diseases? What does this imply for HTA?
- What role can clinical experts, patients, and patient organizations play in helping us to understand rare disease epidemiology and heterogeneity and uncertainty?

### **Supplemental pricing and reimbursement processes for health technologies to diagnose and treat rare diseases**

- Are supplemental pricing and reimbursement processes for rare diseases needed?
- Should different elements of value be considered? Are some of these already being recognized by payers? Are there differences between private and public payers in this regard—and what can we expect from each of them in reaction to different levels of innovation/value?
- Should willingness to pay differ for rare (or severe) diseases? Non-economic views welcomed.
- Are there different issues with initial (versus no treatment, for example) compared to follow-on product (versus first product) comparators in HTA and pricing for rare diseases? What could solutions be to ensure appropriate evaluation of new drugs with high unmet need?
- Are there outcomes generally excluded from, or special considerations in, an economic analysis that need to be included for rare diseases?

### **Policies to guide and incentivize research in rare diseases**

- What types of incentives are needed to encourage R&D, commercialization, and patient access to orphan drugs? Are there examples of push-and pull-incentives to learn from?
- What might the impact of policy changes to existing orphan drug frameworks and incentives be on investment, R&D/commercialization, and patient access?
- How can we better incentivize R&D into areas with highest severity and unmet need? How should severity and unmet need be defined?
- What levels of orphan drug expenditure should be considered reasonable or affordable?

Please direct any content-related questions to the Guest Editors, **Brian Rittenhouse, PhD** ([brian.rittenhouse@mcphs.edu](mailto:brian.rittenhouse@mcphs.edu)) and **Elena Nicod, PhD** ([elena\\_nicod@dolon.com](mailto:elena_nicod@dolon.com)). Submissions received before **March 1, 2024** will have the best chance of being published in *Value in Health* in 2024. All submissions will undergo the journal's peer-review process before the Editors make final decisions about papers to be included in this themed section of *Value in Health*.

Authors should submit manuscripts through the journal's online submission system at <https://mc.manuscriptcentral.com/valueinhealth> and be sure to indicate in their cover letter that the paper is to be considered as part of the Rare Diseases theme.