

CAN WE BE FAIR

When We Consider Value for "Rare"?

Drugs that treat rare diseases have historically remained protected from the access barriers imposed on very high-priced treatments for more common conditions. But that protected status is beginning to erode.



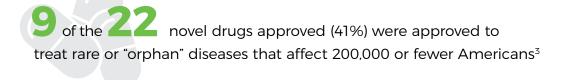
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Open access to these drugs was granted by most payers because:

...it was widely recognized by insurers that even very high prices, when multiplied by small patient numbers, would produce a limited impact on budgets and insurance premiums.¹

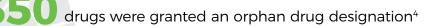
The National Organization for Rare Disorders (NORD) estimates that about 30 million Americans are affected by one of approximately 7000 rare diseases.² Hundreds of these diseases are now considered treatable. Drugs that treat rare diseases are no longer rare, and they are more frequently approved by both the US Food and Drug Administration (FDA) and its European Union counterpart, the European Medicines Agency (EMA).

In 2016



More drug manufacturers are seeking an "orphan indication," even in situations where a rare disease was not the initial approved indication or original therapeutic target of the drug.

In 2015



That's because the FDA's orphan drug designation carries with it commercial benefits created in 1983 when Congress first passed the Orphan Drug Act. It provided the industry with incentives to dedicate more resources to finding cures for rare diseases, including a 7-year period of market exclusivity that does not begin until FDA approval (independent of the drug's current patent status), a 50% tax credit on R&D, access to certain federally funded research grants, and Fast Track FDA approval.

Worldwide sales of orphan drugs first reached \$100 billion in 2015 and are predicted to more than double by 2022, which, if accurate, would comprise nearly one-fifth of global Rx sales⁵

Advancement in clinical innovation where few or no treatment options existed before also sets up a confrontation with society's—or more specifically, society's health insurance systems'—willingness to pay.



ICER, the Institute for Clinical and Economic Review, convened a stakeholder summit in May 2017 to examine how the US, Canadian, and various European healthcare systems are currently addressing the complex challenges of assessing value and making coverage decisions regarding treatments for rare

diseases. ICER planned to use lessons from the summit to adapt the current version of its value framework evidencebased assessment tool to reflect the special circumstances of drugs and therapies developed to treat rare diseases.

Following the meeting, ICER decided to narrow its focus only to ultra-rare conditions. In July 2017, ICER published (for comment) "Proposed Adaptation of the ICER Value Framework for the Assessment of Treatments for Ultra-Rare Conditions."⁶ In it, ICER defines an ultra-rare condition as one with a patient population of <10,000 in the United States.



ICER proposes applying the adapted value framework for ultra-rare conditions if:

- The treatment offers a major gain in improved quality of life and/or length of life, and
- There is little chance of future expansion of the indication or population that would extend the size of the treatment population above 20,000 individuals

ICER proposes using the same evidence-rating method as it uses in its current value framework, but suggests that the specific context of the challenges of generating evidence for ultra-orphan treatments would be taken into account.⁶

ICER proposal⁶

Create a cost-effectiveness model for every new treatment

Adapt its analyses to accommodate a broader willingness-to-pay threshold

Create a value-based benchmark that will provide information on how decision makers in the United States and internationally apply special weighting and contextual considerations to these types of treatments Account for other benefits and disadvantages and contextual considerations that would take into account positive effects on family, school, and community

Attempt to quantify value in its overall assessment but with a different approach in considering the ultra-orphan drug cost, including not assigning a long-term-valuefor-the-money appraisal of treatments for ultra-rare conditions if the base case costeffectiveness ratio is above \$175,000 per quality-adjusted life-year



Opportunity for manufacturer input on R&D costs

This value framework for ultra-orphan disease treatments also attempts to create a template through a collaborative process that includes manufacturers to provide information in its reports on "the research, development and other relevant costs related to new treatments for serious, ultra-rare conditions."⁶

ICER is accepting comments on its proposed "Adapted Value Framework for Ultra-Rare Conditions" until Monday, September 25, 2017

While ICER is becoming one of the more prominent voices in value assessment in healthcare, its assessment tools are among several developed by a number of healthcare policy organizations and arms of medical societies and institutions, including the American Society of Clinical Oncology.⁷ These frameworks are increasingly exerting sway on payers and providers thinking about cost and coverage. Almost half of payer respondents cited ICER as a credible and reliable source of cost effectiveness (CE) information in independent payer market research conducted by Entrée Health in August 2017. Payers noted that they consult ICER reports, when available, in situations where they lack internal expertise or resources to develop CE models for select high-cost therapies, or when they'd like to confirm conclusions drawn from internal CE analyses.

Ensuring manufacturer share of voice in the debate over value in rare disease

While some drug developers and manufacturers objected to the notion of applying "value frameworks" and "evidence blocks" to treatments that often take dozens of years and hundreds of millions of dollars to develop, most accept that value-based approaches to healthcare are here to stay and will continue to evolve; thus, it is critical that manufacturers:

- Assert their share of voice and do it in ways that are both credible and convincing to other stakeholders
- Understand how they might influence the development of these value assessment tools, and thereby shape healthcare coverage and reimbursement decision-making
- Anticipate how these assessment tools could shape future markets for new treatments and cures that come at an exceptionally high cost



To learn more about how Entrée Health can help, contact Andrew Gottfried at agottfried@entreehealth.com or 212-896-8026.

References: 1. Ollendorf D, Chapman R, Pearson S. Assessing the Effectiveness and Value of Drugs for Rare Conditions, A Technical Brief for the ICER Orphan Drug Assessment & Pricing Summit, May 2017. https://icer-review.org/wp-content/uploads/2017/02/ICER_Assessing-the-Value-of-Drugs-for-Rare-Conditions_051017.pdf. Accessed September 7, 2017. 2. Genetic and Rare Diseases Information Center; US Department of Health & Human Services. FAQs about rare diseases. August 11, 2016. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases. Accessed September 7, 2017. 3. US Food and Drug Administration. 2016 Novel Drugs Summary. Silver Spring, MD: US Food and Drug Administration; January 2017. https:// www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DrugInnovation/UCM536693.pdf. Accessed September 7, 2017. 4. Kesselheim AS, Treasure CL, Joffe S. Biomarker-defined subsets of common diseases: policy and economic implications of orphan drug act coverage. *PLoS Medicine*. 2017;14(1):e1002190. 5. Evaluate™. EvaluatePharma Orphan Drug 2017 Report. http://www.evaluategroup.com/public/Reports/EvaluatePharma-Orphan-Drug-Report-2017.aspx. Accessed September 7, 2017. 6. ICER, Proposed Adaptation of the ICER Value Framework for the Assessment of Treatments for Ultra-Rare Conditions, July 2017. https://icer-review.org/wp-content/uploads/2017/05/ICER_Proposed_VAF_Adaptations_Orphan_ Drugs_072517.pdf. Accessed September 2017. 7. American Society of Clinical Oncology. Value in Cancer Care. http://www.asco.org/practice-guidelines/ cancer-care-initiatives/value-cancer-care. Accessed September 7, 2017.