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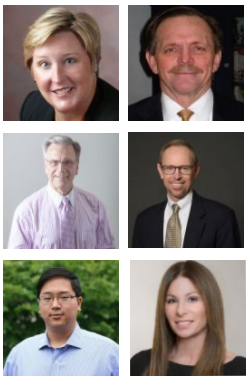
Pricing & reimbursement for rare diseases in the United States

World Orphan Drug Congress USA 2021 Brief



Moderated by Jane Barlow, Senior Advisor, MIT Center for BioMedical Innovation /NEWDIGS, the panel comprising Ron Bartek, President, Director, and Founder Friedreich's Ataxia Research Alliance; Peter Saltonstall, Chief Executive Officer, National Organization for Rare Disorders (NORD); Steven Pearson, President, ICER; Nanxin (Nick) Li, Senior Director and Head, Health Economics and Outcomes Research (HEOR), uniQure; and Lisa Deschamps, SVP & Chief Business Officer, Novartis Gene Therapies, discussed the current challenges in creating a sustainable pricing system for orphan drugs, how the new administration will impact pricing and access for the rare disease community, and updates on value-based reimbursement models.

Panelists were not consulted in the development of this summary

The call to action

The orphan drug designation processes for rare diseases have successfully incentivized development and commercialization of treatments for high unmet need conditions that, due to their small target populations, might otherwise not be commercially viable opportunities. But there are still major gaps in treatment for many rare diseases, as highlighted by the panel, which means the mission to bring new therapies is still very real. From the discussion though, the industry is committed to filling these gaps.

But with the advent of gene and cell therapies, the pricing and access landscape is changing, and those patients that are eligible for treatment might not have access to the transformational benefits they could. The reason? Numerous therapies with super-high costs, uncertain evidence, insufficient health system infrastructure, and a lack of coordinated collaboration between all stakeholders, including patients.

The panel picked up on this latter point. Patient voices are seldom actively sought in the development and especially in the pricing of drugs, but they are expected to foot the bill to receive them. With super-high-cost treatments like gene therapies, the first few of which have 1-time treatment costs of over $2 million, the out of pocket (OOP) costs to the patient could be off-putting.

From a health care system perspective, the cost of a few treatments per year may not be problematic for health insurers, though for smaller institutions it still could be. The real problem will come if the anticipated 20 or more gene therapies hit the market before 2025. It's the old adage 'rare diseases are not rare when combined.'

The panel's fear, accentuated by rumblings on Capitol Hill, is that if the industry does not sort out the issue of drug pricing themselves, the Government will take the ball into their hands. The predicted result is that the nuances of rare diseases could be lost in the rhetoric and politicization of drug pricing and rock-bottom prices would be sought for drugs, regardless of the value they bring to patients.

This disincentivizes drug developers from attempting to create treatments for diseases where they will not get any return on investment. The ultimate losers in this scenario, dubbed 'the circle of hell', are the patients who will continue to suffer with untreated rare diseases.

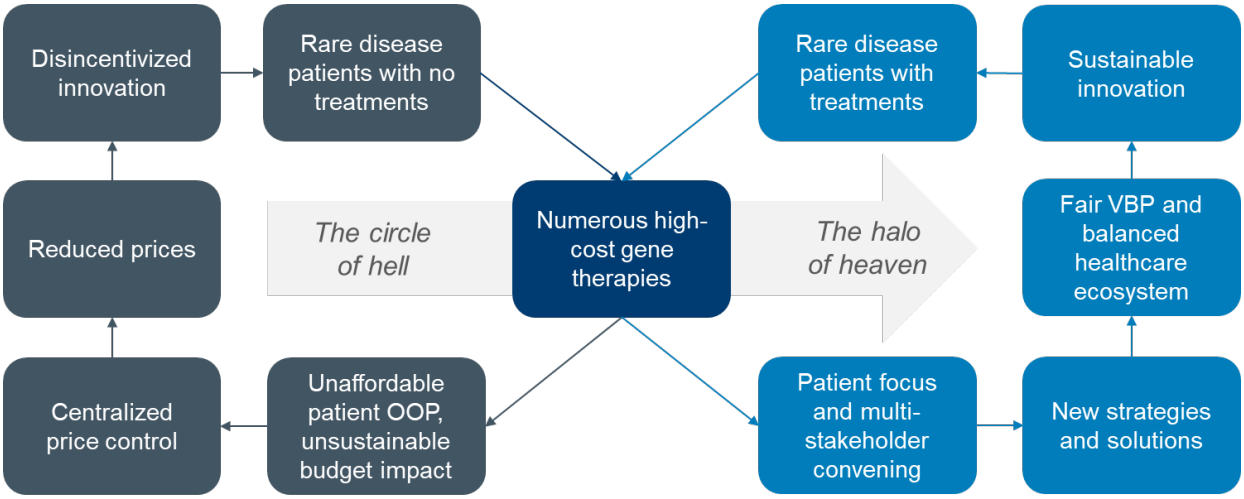


Figure 1. The sustainable innovation paradigm shift

The road to sustainable innovation

The panel considered the current situation unsustainable over even the short term. After all, time to access to some of these treatments is critical. The gene therapy Zolgensma for spinal muscular atrophy (SMA) is the exemplar here, as 90% of patients with the disease die within the first 2 years of life and the fast-progressing nature of SMA means that any delay in patient access could be fatal. Is there a better example of why the patients must come first in any solution?

No surprise then that involving patients, caregivers, and the wider patient advocacy community in a public conversation was recognized by the panel as a critical early step in the process. But it's key that all stakeholders have a seat at the table, so regulators, payers, providers, and manufacturers also need to be involved to develop a workable solution. It will certainly be a complex series of discussions, which is why one panelist suggested a single convenor will be needed to enable coordination and collaboration.

Transparency is important and will enable stakeholders to leverage learnings on what has gone well and what has not for these first gene therapy cases. This education may also help to overcome the initial

sticker shock that million-dollar drug costs cause, as patients, the public, and politicians will understand how these prices may be justified by considering both internal (product) and external (market) factors.

The panel took this further by calling for a more holistic assessment of the benefits these treatments can offer, to include not just survival extension and quality of life improvements on the product side but also the opportunity to offset the costs of other treatments on the market side. The transformative nature of some of these treatments necessitates inclusion of wider societal and economic benefits. This will help define a true value-based price.

However, what if the value-based price is still deemed unaffordable? Panelists discussed what was a fair price, since a new 1-time treatment for say hemophilia might be cost-effective at $20 million, but it might be uncertain whether this is a funding priority over other treatments that cannot offset so much external cost. Therefore, there needs to be a wider balancing of the health care ecosystem.

CAR-T cell therapies were called out as examples of where the system could not cope with either the cost or complexity of treatment delivery. On the first point, one issue was how much risk the system should bare when asked to pay hundreds of thousands of dollars for treatments with limited evidence.

The solution in some cases was performance-linked payment, but there are issues with these and similar annuity-based reimbursement models. Among others, these include lack of payer incentives and agreement on which outcomes are valuable, a conventional pay-as-you-go mindset, and the difficulty of administrating them across insurers. On the second point, the panel discussed the potential CMS carve-out of funding for CAR-Ts or utilizing public-private partnerships.

To address these difficulties, the panel calls for the 'halo of heaven' that is sustainable innovation. Ultimately, this is about securing the future of health care by fairly rewarding manufacturers based on the benefits their drugs provide to patients and society. You cannot have one without the other.

Figure 2. Key themes in sustainable innovation

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