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Examining New Payment Ideas for Curative Therapies

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How can the health industry ensure that cutting-edge gene therapies and other curative treatments get to the patients that need them, without leaving payers financially exposed? Representatives of payers and biotechnology companies discussed some of the novel discussions that are taking place as they work through issues of expense and access during “Paying for Cures: Ensuring patient access and system sustainability," a 1-day event in Washington, DC.

The conference was hosted by the FoCUS (Financing and Reimbursement of Cure in the US) Project at MIT, and an afternoon session, “Putting Theory into Practice,” described the payment pilots FoCUS is facilitating, including plans for a Massachusetts pilot on performance-based annuities with commercial payers, and milestone-based contracts with public payers.

Jane F. Barlow, MD, MPH, MBA, with Real Endpoints, a data, analytics, an advisory company for payers and biopharmaceutical companies, said the “accessibility issue lies largely with payers.” All payers are different, and “a lot has to do with how they manage risk in their population as well as the regulatory constraints,” she said.

“Size does matter a lot, and the actuarial risk for a small payer is huge,” she said. Projecting numbers on what amounts to just a very tiny sliver of patients for a small insurer makes it difficult to price the treatments in their premiums, she said.

“You have to cover what needs to be covered in order to be competitive and attractive, but also, you don’t want to cover things that others aren’t covering so you don’t get adverse selection,” Barlow said.

“The small player has to charge competive premiums,” in order to have a level playing field, and that explains some of the story behind the impetus for new payment pilots, she said.

As an example, Lovena Chaput**,** vice president of managed markets and reimbursement at AveXis, a biotechnology company that was bought by Novartis last year, discussed spinal muscular atrophy (SMA), which she said was a leading cause of infant deaths caused by genetic disease. SMA affects 1 in 10,000 live births. There are 3 types of the genetic disease, with type 1 making up 60% of SMA cases. In type 1, the disease is present at birth or even before. Most children with type 1 die before age 2, having lost all neuromuscular function.

In December 2018, the FDA granted priority review for AVXS-101, a 1-time gene therapy for the disease. The treatment would be sold under the name Zolgensma.

All of the patients treated with the therapy are still alive, she said. All are speaking and most are feeding orally. All but 1 have met the milestone of sitting, while 2 are walking and 2 are standing, she said.

Michael Sherman, MD, MBA, MS, chief medical officer and senior vice president of Harvard Pilgrim Health Care, discussed how his company is exploring the idea of performance-based annuity pilots, which would be portable, multiyear agreements crafted through agreements with biotech companies, payers, providers, and patients, in order to pay for treatments like Zolgensma.

“We realize there’s a benefit to paying over time, given the budget impact, given unknowns about the durability, and given a desire to align with true outcomes,” he said.

He noted that most think about “churn” as it applies to switching insurance companies, but what about when a patient leaves the state? Such an idea would require patient tracking to address portability and mobility issues, and would also need federal approval to work around issues relating to antikickback statutes, Medicaid best price rules, Medicaid price reporting rules, and average sales price. He said that is currently hard to do without addressing portability and mobility.

In the example currently being envisioned, a high value gene therapy would be paid for over 4 years, split into payments of 20% each. Payment to providers would be plan-specific.

There would be common elements, such as performance metrics and payment structure; elements specific to plans would stay specific to those plans, such as network and reimbursement issues or internal processes.

This idea would not come without implementation challenges, he said, such as price reporting for average manufacturer price and Medicaid best price.

Harvard Pilgrim, which is talking to AveXis, is also keeping in mind that it wants to create a scalable approach that anticipates future therapies. AveXis is expecting a decision from the FDA by May of this year, so they are working on a tight timeline to come up with a process that works, he said.