SPECIAL ISSUE: **PAYING FOR CANCER CARE** | FEATURING CONFERENCE COVERAGE: **FLASCO | ISPOR**

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PRECISION FINANCING TOOLS

MIT Group Brings Together Stakeholders to Brainstorm How to Pay for Curative Therapies Over Time

Mary Caffrey

PATIENTS TREATED WITH CHIMERIC antigen receptor (CAR) T-cell therapy describe a process that is a miracle. After all else has failed, these engineered cells made with a patient's own T cells are let loose in the bloodstream to attack the cancer. For many patients who have lost hope, the treatment brings complete remission.

But the miracle comes at a cost. There's the price of the treatment itself—either $373,000 or $495,000, depending on the indication—and the total cost rises above $1 million,[1] including administration and treating adverse effects once called "the worst flu you've ever had."[2]

Right now, major academic medical centers say they are losing money every time a Medicare patient receives CAR T-cell therapy, as a reimbursement solution remains on hold.[2] But with more lifesaving and life-changing durable, curative therapies in the pipeline, the question of how to pay for CAR T-cell treatment will hardly be the last logjam of its kind.

A Novartis company, AveXis, recently said it would offer payment-over-time options for a $2.1 million single-treatment gene therapy for pediatric spinal muscular atrophy. A multistakeholder group at Massachusetts Institute of Technology has spent years exploring new payment options of this type for life-saving durable and curative therapies.

Credit: Novartis photo

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ONCOLOGY CARE MODEL

Two-Sided Risk in the Oncology Care Model

Kashyap Patel, MD, ABOIM, BCMAS; Maharshi Patel, MBA; Taylor Lavender, BS, PA; Dhwani Mehta, MS, RD; Asutosh Gor, MD; Sashi Naidu, MD; and Chuck Newton, BS

THE US HEALTHCARE SYSTEM remains one of the most inefficient healthcare systems in the world. The Bloomberg Health-Care Efficiency Index ranked the United States 54th among 56 countries in 2018, tied with Azerbaijan and only ahead of Bulgaria.[1] This occurs even though the United States spends $10,244 per capita annually on healthcare, a figure representing 17% of the gross domestic product.[2]

Our expensive yet inefficient healthcare system has been blamed on a fragmented, disorganized, and uncoordinated delivery system, with silos and redundancies that create inefficiency.[3] Despite rapid advancements in treatment, the discovery of new drugs, and new technology aimed at improving patient outcomes, the overall performance of the US healthcare system in aligning incentives has not met expectations

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GAINING THE PAYER PERSPECTIVE

NCCN's Putnam Serving as Point of Contact for Payers, Employers to Keep Cancer Care "Accessible"

Mary Caffrey

PUTNAM

A YEAR AGO, the National Comprehensive Cancer Network (NCCN) added the word "accessible" to its mission statement, stating that the group is "dedicated to improving and facilitating quality, effective, efficient, and accessible cancer care so that patients can live better lives."[1]

But innovative therapies won't reach patients unless payers and, increasingly, employers are willing to include them in benefit plans. So, in March, NCCN named Duane Putnam, BBA, as its director of Payer

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MIT Group Brings Together Stakeholders to Brainstorm How to Pay for Curative Therapies Over Time

Mary Caffrey

TRUSHEIM

Mark Trusheim, MS, BS, is strategic director at NEWDIGS and a visiting scientist at MIT.

GLASSPOOL

John Glasspool is CEO at Anthos and an unpaid senior advisor at NEWDIGS.

BARLOW

Jane F. Barlow, MD, MPH, MBA, is executive vice president and chief clinical officer for Real Endpoints.

CONTINUED FROM COVER

More and more, stakeholders across the healthcare system—providers, commercial payers, pharmaceutical companies, large employers, state Medicaid officials, and even state budget officers—are grappling with the fact that the old pay-as-you-go way of covering medicines, even cancer drugs, was not built for these revolutionary therapies. Things like co-payments and coinsurance, created to force patients to make financially sound choices, make little sense for life-saving therapies that require preauthorization and have no competition, experts say; they only create additional barriers for very sick people or caregivers who are already missing work.

Americans are united in their belief that something must be done to fix the way we pay for drugs that save people's lives—especially cancer drugs. Since 2015, the idea that drugs cost too much and that those on Medicare should have a cap on out-of-pocket spending has enjoyed bipartisan support, according to the Kaiser Health Tracking Poll.[3]

Slowly, answers to the question, "How do we pay for it?" are emerging. Some new models are at work already. In January 2018, Spark Therapeutics announced an outcomes-based rebate arrangement with Harvard Pilgrim for its gene therapy, voretigene neparvovec-rzyl (Luxturna) to treat inherited retinal disease.[4] In May 2019, AveXis, a Novartis company, said it would offer payment-over-time options for onasemnogene abeparvovec-xioi (Zolgensma), a $2.1 million single-treatment gene therapy for pediatric spinal muscular atrophy (SMA).[5]

These payment models are not coming out of the blue. Elements of the reinsurance, financial, and pharmacy benefits infrastructure—some familiar with specialty pharmacy, some less so—are coming together in new ways to develop mechanisms to take up the reimbursement challenge, drawing on insights from a group at Massachusetts Institute of Technology's (MIT) Center for Biomedical Innovation (CBI) that has been working on a solution to this problem for years, the **NEW D**rug **D**evelopment Parad**IG**m**S (NEWDIGS)** Initative**.**[6]

NEWDIGS' FoCUS project, for Financing and Reimbursement of Cures in the US, convenes stakeholders from across healthcare to develop ideas that become pilot financing projects. In January the project issued a white paper, "Precision Financing Solutions for Durable/Potentially Curative Therapies," which was the product of 3 years of work and featured 4 payment models that FoCUS leaders say are at various stages of development.[7]

The idea is simple: If an innovative, 1-time therapy is a cure that will last for years—perhaps for the rest of a person's life—then a 6-figure price tag, while expensive in the near term, may make sense in the long term. The first challenge, then, is to find a financing solution that spreads the cost over a period of years, or at least long enough to confirm that the treatment has worked.

"There is a sense that we are bringing some new tools that we commonly use in other fields of our lives, in the form of car payments and mortgages and other things, and adapting them into healthcare, where we have not been accustomed to paying for things over time," Mark Trusheim, MS, BS, strategic director at NEWDIGS and visiting scientist at MIT, told *Evidence-Based Oncology*™ (*EBO*) in an interview.

John Glasspool, who is the chief executive officer (CEO) at Anthos and an unpaid senior advisor at FoCUS, told *EBO* that the moves toward outcomes-based payment, as seen in the Spark Therapeutics and Novartis contracts, "are happening not just because the therapies are expensive, we should understand this cost is due to the accrual of benefit versus an acute administration. Given the value is dependent on an estimate of durability—people are recognizing that this justifies a new model."

The second challenge, Trusheim said, is to make this process invisible to the patient. "Even if the payer chooses to pay for things over time, there's a concern that the patient not get hit for a deductible year after year."

While new financing mechanisms could take different forms, 1 constant is that payers want to know that high-cost therapies are going to work, said Jane F. Barlow, MD, MPH, MBA, executive vice president and chief clinical officer at the market access company Real Endpoints. Barlow, who is also a senior adviser to the MIT NEWDIGS' FoCUS project, told *EBO*, "We did a survey of payers, and the payers overwhelmingly said if they are going to pay these high prices, they need to ensure the results are there to support them. They are very interested in the idea of performance-based payments."

"How that plays out is going to vary between each treatment and each biotech company, and each payer and payer type," she said.

Different Models for Different Circumstances

The concept of using reinsurance or payment-over-time structures to deal with the escalating cost of novel therapies has received attention in journals in both the United States and Europe in recent years, especially as some treatments are not just drugs but processes.[8-10] Novartis' CEO Vas Narasimhan told *The Financial Times* in December 2018 that the company was exploring relationships with reinsurers to develop a payment model in anticipation of FDA approval of its SMA treatment.[11] But the work at NEWDIGS has received the most attention, both for its multistakeholder approach and its ability to delve into the details.

Glasspool said there is great value in bringing the various stakeholders together over an extended period because the work has become "granular." The FoCUS project moved past the development of the models to show how they might be implemented. This could only occur by allowing different stakeholders to slowly build relationships and not simply try to optimize their positions. "There is always a degree of mistrust," he said. "Over time, that mistrust is reduced."

The FoCUS white paper outlines 4 specific precision-financing solutions,[7] which are at various stages of development and designed to address specific reimbursement challenges. They are:

One-Year Milestone-Based Contracts. These have been seen already, in the Spark Therapeutics deal with Harvard Pilgrim and in the original agreement that Novartis had with CMS for CAR T-cell therapy reimbursement, although that agreement ended when UnitedHealthcare successfully demanded a National Coverage Analysis.[12] These agreements call for a full or partial refund when patients do not achieve prespecified minimum outcome levels. Barlow, Glasspool, and Trusheim each said this model has the strongest near-term potential for implementation.

Five-Year Performance-Based Annuity. Providers are paid right away, in exchange for agreeing to certain tracking requirements.

Drug developers receive an initial payment at treatment, followed by 5 annual payments that are tied to predetermined performance metrics. On the plus side, the annuity structure can incorporate data collection and tracking costs; it can address unexpected patient surge. On the downside, this solution depends on finding ways to give patients incentives to participate in follow-up care, and it must address patient mobility.

Risk Pooling. This concept could be used by both commercial payers and state Medicaid funds to handle actuarial risk. The white paper envisions the use of reinsurance and stop-loss policies to address the needs of commercial insurers and self-insured employers. The downside of these vehicles, Trusheim said, is that reinsurers could exclude some therapies; use of risk pools alongside multiyear annuities would likely need to include tools to handle durable therapies to avoid disrupting insurance markets.

Orphan Reinsurer Benefit Manager (ORBM). These entities would combine the functions of a reinsurer and a pharmacy benefits manager to absorb patients who need treatment with high-cost gene therapies that require medical management. An ORBM could serve several payers or self-insured employers with value-based agreements, creating the scale to gain operational expertise. Trusheim said that the ORBM would carve out gene therapy patients much like ehavioral health management firms carve out mental health patients to provide them specialized care and to provide payers with predictable costs.

Barriers and Challenges

The recognition that old payment models are failing does not make new ones materialize overnight.

Moving Targets and Data. More than a year ago, CMS abandoned its early value-based agreement with Novartis to pay for the first CAR T-cell therapy, tisangenlecleucel (Kymriah), after UnitedHealthcare objected.[1] In the interim, leaders at academic medical centers that offer CAR T-cell therapy and treat Medicare patients have been doing so at a loss while CMS has worked on a reimbursement solution, one that would require extensive data collection, including patient-reported outcomes. The proposal would also ask Medicare patients to enroll in clinical trials or registries for institutions to be paid.[12]

Institutions said in their comments that CMS' data collection requirements would offer valuable insights, but they asked who would pay for this process.[1,13] During a panel discussion in March at the National Comprehensive Cancer Network's annual meeting, UnitedHealth Group's Jennifer Malin, MD, PhD, suggested that the manufacturers might pay for this process and that payers feared that the original value-based agreement allowed no room for new indications that would emerge.[1] On May 17, 2019, the day the payment proposal was to take effect, CMS announced that its plan was on hold.[14]

Medicaid Best Price (MBP). The white paper and FoCUS members said the most pressing barrier to its financing ideas, especially the 5-year annuity, is the need to reform MBP rules. Created to ensure that Medicaid always received the lowest price for a medicine, the rules now create chaos when applied against value-based agreements that call for refunding payment if a therapy doesn't work. It's quite possible that a gene therapy for a rare condition could result in such a rebate—and then also set the pricing floor for all patients covered by Medicaid for whom the therapy would work perfectly. Fortunately, the MIT FoCUS members who spoke with *EBO* say members of Congress are keenly focused on this point, and Trusheim said bipartisan legislation to fix the problem is possible.

Patient Mobility. Payers, employers, risk managers, and the government have all historically budgeted around 1-year time frames, so figuring out how to break out of that mindset will be one of the biggest challenges of the new financing models that the MIT FoCUS leaders envision. Solving the puzzle of what happens when a patient is treated with an expensive therapy and then changes health plans won't be easy, but it must be addressed. Glasspool sees it this way: Say the former insurer entered into a 5-year annuity on behalf of a beneficiary. If the person's employer switches insurers, and the former insurer has made 3 payments, the new insurer must make the last 2 payments.

Just as the new insurer must take on the pre-existing medical condition, he said, "That new company is taking on the preexisting financial commitment." As these kinds of agreements become common, insurers will take on and offload each other's multiyear agreements, and things will even out.

Clarity on Payer Communication. Most payers take 3 to 6 months after a therapy is approved to evaluate treatments before making final coverage decisions, Barlow said; in the interim, most have a process for a "medical necessity review" for individual cases. Recently, the FDA has allowed conversations prior to approval between payers and drug makers about which populations will be affected by a new therapy, but there's still a need for more clarity on what is allowed and what is not. "Each company is trying to interpret that on their own," Barlow said. "The problem is, you don't know you're stepping over the line with your interpretation until you are."

Greater clarity from the FDA, more robust data sharing, and a better understanding by providers of what information they must have to gain coverage would all help, Barlow said.

Size of the Pipeline

Trusheim notes that data published by NEWDIGS show that the number of durable therapies expected before 2030 is not so large that the health system cannot absorb them. A separate paper just released in Value in Health projects that 350,000 patients would be treated by 30 to 60 products by 2030.[15]

Trusheim said that this translates into a $40 billion market. "So, that's real money, but compared to a $3 trillion total healthcare spend, it's not catastrophic to the whole system," he said. "But the sticker shock on any individual product is still certainly high."

From the perspective of a nonprofit payer or a self-insured employer, a therapy for 1 or 2 rare diseases in a single quarter could be difficult to manage but is unlikely a threat to their reserves, Trusheim said.

Coming up with solutions to smooth out payments, to avoid shocks to these smaller entities, gives these entities the tools to manage their way into the future. "I'm hopeful in all this," he said. ◆

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