

ViewPoints: Building a sustainable health system for curative therapies

As gene therapies, cell therapies, immunotherapies, and other biopharmaceutical innovations advance in clinical trials, science is closer than ever to making the concept of curative therapies a reality. Yet the promise of a “cure” raises a number of questions about how healthcare leaders define and incentivize the development of curative therapies, evaluate their differential value, and structure regulatory and reimbursement pathways to ensure efficacy, safety, and access for patients.

The recent arrival of new curative therapies has already forced several of these questions to play out in current events, and not without controversy. The entrance of Gilead’s curative hepatitis C drug, Sovaldi, on the US market, and the first regulatory approval of a gene therapy in Europe for UniQure’s Glybera, priced at \$1.4 million, prompted considerable public debate over cost.¹ Both examples underscore the dilemma facing the healthcare community today: no party wants to discourage the development of cutting-edge technology that provides cures for devastating diseases – many of which are for rare indications – yet no one has agreed on appropriate payment solutions for assuring economic sustainability for all stakeholders – payers, patients, investors, and manufacturers alike. The timing is therefore ripe for relevant parties to come together and start driving at answers to the question, how can we shape a healthcare system that promotes and pays for cures?

The main challenge impeding easy uptake of curative therapies in the United States is that sustainability will require a departure from the status quo. The US healthcare system is currently designed to support and reimburse incremental management of chronic disease.² Curative therapies, on the other hand, promise significant clinical efficacy over time, but in the absence of disruptive thinking about reimbursement, they will require high up-front expenditures by payers for uncertain long-term outcomes.

As more manufacturers invest in curative and gene therapies, payers, policymakers, providers, and healthcare systems must start to think differently about managing the ensuing concentration of costs and accrual of benefits.³ In parallel, manufacturers must consider new models for realizing the value of their innovations and recouping investments. Fortunately, many stakeholders across the board are beginning to embrace new thinking and ideas.

“The larger [question] for everybody is, at what point do these therapies start to overburden an already costly healthcare system?”
- payer

¹ For information on the impact of Sovaldi on the US healthcare system, see US Senate Finance Committee, [“Wyden-Grassley Sovaldi Investigation Finds Revenue-Driven Pricing Strategy Behind \\$84,000 Hepatitis Drug,”](#) news release, December 1, 2015. For Glybera, see Makiko Kitamura, [“World’s Most Expensive Medicine: Is It Worth the Price?”](#) *Bloomberg*, May 21, 2015 and Michael Kooren, [“Exclusive: First Gene Therapy Sets Million-Euro Price Record,”](#) *Reuters*, November 26, 2014.

² Scott Gottlieb and Tanisha Carino, [“Establishing New Payment Provisions for the High Cost of Curing Disease](#) (Washington, DC: American Enterprise Institute Research, July 2014), 2.

³ *Ibid.*

In a series of discussions conducted in late 2015 and early 2016, over 50 individuals representing biopharmaceutical manufacturers, public and private payers, patient advocates, economists, financiers, and subject matter experts shared their views on the value of curative therapies and how best to manage their delivery in the United States.⁴ These stakeholders also offered feedback on potential policy solutions and innovative payment models. This work was underwritten by a consortium of biopharmaceutical companies comprising Baxalta, Biogen, Bluebird Bio, and GlaxoSmithKline. For a list of discussion participants, please see the appendix on page 19.

What follows is a summary of key insights from these conversations, including discussion of the impact of curative therapies, exploration of innovative payment models, and recommendations on productive paths forward and next steps. These sections are preceded by a two-part introductory discussion of the challenges in defining and assessing the value of curative therapies and the elements of the US healthcare system that are constraining their uptake.

Curative therapies: definition and value-assessment challenges

Academics and healthcare decision-makers share highly divergent views on how best to define curative therapies and assess their value. Participants discussed the challenges preventing easy consensus on which therapies can be deemed “curative” and why stakeholders reject common value-assessment frameworks for evaluating the costs and benefits of potential cures. While these debates can be seen as academic, they are instructive for introducing the unique challenges that curative therapies bring to the healthcare system.

Defining curative therapies

Many healthcare leaders argue that the term “curative” is misleading. During clinical trials, therapies may offer the promise of a cure, but real-world application and durability are highly uncertain at the time of regulatory approval. Furthermore, from a conceptual point of view, many existing interventions in healthcare – including surgeries, vaccines, and organ transplants – can fall under the “curative” heading.⁵ How then, many stakeholders ask, is this new class of so-called curative therapies distinct?

For the purposes of this effort, curative therapy was defined as

- an innovative one-time (or short-term) treatment,
- delivered via an irreversible process (or procedure or drug), and
- followed by a significant (multiyear) disease-free interval (i.e., long-term durable effect).

⁴ *ViewPoints* reflects Tapestry’s use of a modified version of the Chatham House Rule whereby comments are not attributed to individuals, corporations, or institutions. Discussion participants’ comments appear in italics in the margin and within the text.

⁵ For a detailed analysis comparing gene therapies with organ transplants, see Nicolas Touchot and Mathias Flume, “The Payers’ Perspective on Gene Therapies.” *Nature Biotechnology* 33 (September 2015), 902–904.

Gene therapy was provided as a concrete example of a curative therapy technology platform. While near-term gene therapies focus initially on rare populations, for the purposes of this effort, discussions explored the use of these technologies for broader specialty populations. Given the specific challenges of cancer (e.g., tumor resistance, multi-mutations, primary vs. secondary tumors, combination treatments, multiple lines of therapy) and reimbursement issues for oncology therapies (e.g., buy-and-bill considerations), oncology indications were specifically excluded in the scope of treatments considered. However, the points and approaches that emerged from this effort may have applicability for areas such as immuno-oncology.

When asked to share their own basis for defining curative therapies, healthcare leaders offered diverse opinions on the scope of a “cure.” Some payers embrace an inclusive view, suggesting that disease-modifying therapies that provide a patient with substantial quality of life for as little as one year could be considered curative. Others reject the term “curative” entirely, arguing that, in the words of one interviewee, *“you can’t prove it.”* Further, many question how these therapies differ at all from existing high-cost specialty treatments.

Assessing the value of curative therapies

A range of considerations comes into play when assessing the value of a new drug or therapy, including the durability of treatment, benefits for patients, and cost-effectiveness for the healthcare system. These considerations are not unique to curative therapies, but their impact is magnified when also considering a curative therapy’s degree of novelty, durability, irreversibility, and likely cost, especially for gene therapies. As such, curative and gene therapies complicate traditional approaches and pathways for assessing value.

Some experts assert, for example, that applying quality of life-years (QALY) frameworks to assess the value of a curative therapy – wherein economists assign a quantitative measurement to the value of remaining life-years based on an individual’s state of health – may be a helpful place to start. QALY frameworks, which are used by some health technology assessment bodies in Europe to make coverage decisions, can help decision-makers understand if they are *“in the ballpark”* for calculating value.

Other subject matter experts and health economists argue that using QALY frameworks may not be practical, since for curative therapies they may justify high up-front costs for treatment that do not take into account total cost implications for the health system. The issue for curative therapies is not the QALY framework per se, these experts argue, but rather the lack of an agreed-upon annual threshold per QALY in the United States reflecting the system’s capacity to pay. Other factors, such as societal benefits associated with a curative therapy (e.g., productivity gains from patients able to return to work), are not reflected through QALY frameworks, which focus on medical offsets.

Many stakeholders also take issue with assessments wherein a curative therapy is valued as a multiple of the annual cost of existing standards of care. For example, if a physician “cures” a 25-year-old of a rare disease by treating him or her with a gene therapy that replaces an existing standard of care costing \$250,000 per year, then a million-dollar

“A strong ‘yes’ that QALY frameworks can be applied to curative therapies like gene therapies ... It allows you to put patient gains at the forefront; the thresholds are what differ.”
- subject matter expert

price tag for the gene therapy may be proposed.⁶ But the challenges with this approach are twofold: (1) payers cannot be certain of long-term outcomes, so the risk to the payer associated with the up-front cost increases substantially, and (2) the million-dollar cost still needs to be absorbed, which raises questions about patient affordability and system sustainability.

These examples show that healthcare stakeholders do not share a common view on the definition of curative therapies nor the best approach to assess their value. Rather, leaders call for more data and analysis on curative therapies in the pipeline as a starting point to begin considering the best strategies for calculating value. Others recommend developing a new value-assessment framework – one more flexible than either a QALY or a multiple of the existing standard of care – that takes into account clinical uncertainty, sustainability over time, societal benefits, and other factors unique to curative therapies’ application and reimbursement. Still others emphasize that focusing on value-assessment frameworks is less useful than tactical planning for sustainable payment and reimbursement models, as discussed below.

Elements in the US healthcare system constraining uptake of curative therapies

If the FDA approved multiple high-cost⁷ curative and gene therapies for the US market tomorrow in the absence of regulatory or policy changes, several existing factors would inhibit solutions to mitigate the budget impact of these therapies for public and private payers. These constraints include the following elements.

Health system fragmentation

For a payer to recoup its investment in a hypothetical \$1 million gene therapy example, a patient would need to remain with that same payer for years in order for it to attain the accrued economic benefit. In the fragmented US multipayer system, patients switch payers frequently,⁸ so payers are not incentivized to make up-front, high-risk investments.⁹ Patient portability across multiple payers also hinders opportunity for amortizing or annuitizing costs (i.e., paying in installments over time), a concept to be further discussed below.

In addition, many key healthcare stakeholders – manufacturers, payers, patients, and clinicians – view each other with cynicism and have historically distrusted each other’s

“The challenge [with QALYs] is that any price assumes a cost-effectiveness threshold, which we do not have here in the United States.”
- subject matter expert

⁶ Touchot and Flume, “The Payers’ Perspective on Gene Therapies,” 902.

⁷ The use of the term “high cost” is relative to other treatments. Scenarios considered during interviews assumed that curative and gene therapies would likely be similar to the cost of existing biologics and rare-disease therapies such as enzyme replacement treatments and/or known public prices of therapies such as Sovaldi or, for gene therapy, Glybera. Interviewees also raised organ transplants as another prospective cost benchmark.

⁸ See [National Association of Medicaid Directors to members of the US Senate and House](#), October 28, 2014 and Benjamin D. Sommers, “Insurance Cancellations in Context: Stability of Coverage in the Nongroup Market Prior to Health Reform,” *Health Affairs* 33, no. 5 (2014), 887-894.

⁹ J.D. Kleinke and Nancy McGee, “Breaking the Bank: Three Financing Models for Addressing the Drug Innovation Cost Crisis,” *American Health and Drug Benefits*, 2015 8, no. 3 (2015), 120-121.

interests and motives. Within this context, building the trust and credibility required for new solutions may prove challenging.

Policy barriers to managing healthcare costs

Stakeholders cited several existing policies that they believe would hinder payers' ability to manage the cost of new curative and gene therapies. Some of these apply to managing specialty drug and treatment costs broadly, but stakeholders underscored that these barriers will likely have serious implications for curative and gene therapies as more are approved:

- **Best-price requirement.** Many stakeholders perceive that the Medicaid best-price requirement inhibits manufacturers from engaging in innovative performance-based contracting or other creative risk-sharing payment models.¹⁰ The best-price requirement stipulates that a manufacturer must extend to Medicaid any discounts it negotiates with any other payer, wholesaler, or provider.¹¹
- **Prohibition on direct negotiation.** Laws that prevent the Centers for Medicare and Medicaid Services (CMS) from negotiating directly with manufacturers are often pointed to by patient associations, provider organizations and advocacy groups as the bottleneck preventing deeper discounts. However, some thought leaders contend that direct negotiation alone may not be sufficient to bring about a more cost-effective approach to reimbursement in the United States.¹²
- **Mandatory Medicare and Medicaid coverage of FDA-approved therapies.** Many payer representatives, health economists and subject matter experts share concerns that in most cases Medicare and Medicaid cannot refuse to cover a new therapy approved by the FDA. These stakeholders assert that policymakers must address how the United States will pay for curative and gene therapies now, rather than later, in order to avoid high-cost precedents being approved without a strategy in place to mitigate costs to Medicare and Medicaid. In addition, they stress that Medicaid in particular is uniquely affected by therapies with high costs and/or budget impact, given that Medicaid is a payer of last resort with limited ways to spread costs across its risk pool and subject to budget rules at the state and federal levels. And while state Medicaid programs can negotiate supplemental rebates beyond federally mandated minimums, in the words of one public payer, *"just chasing higher rebate levels doesn't cut it"* when it comes to long-term sustainability.
- **Regulation of private-payer premium increases.** Recent regulation prevents payers from increasing their premium prices too rapidly without public explanation.¹³ While this measure aims to protect patients, some interviewees anticipate that the

"There are two ways forward: the price needs to change and/or the way we pay needs to change."
- private payer

"When we think of more curative therapies, you can pretty much see the lawsuits coming [on limits-to-access controls]."
- public payer

¹⁰ Kleinke and McGee, *"Breaking the Bank: Three Financing Models for Addressing the Drug Innovation Cost Crisis,"* 122.

¹¹ *Ibid.*

¹² Chuck Shih, Jordan Schwartz, and Allan Coukell, *"How Would Government Negotiation of Medicare Part D Drug Prices Work?"* *Health Affairs Blog*, February 1, 2016.

¹³ *"Rate Review and the 80/20 Rule,"* Healthcare.gov, accessed April 5, 2016.

cost of paying for high-priced gene and curative therapies will ultimately be absorbed by payer margins, which may then shrink to an unsustainable degree.¹⁴

- **Legal pathways to challenge access controls.** Public payers like Medicaid and its contractors can use tools such as prior authorizations to limit access to a high-cost therapy in order to manage costs until the next budget approval, as Medicaid has done with Sovaldi.¹⁵ However, prior authorizations can be overturned in court, as discussed further below.
- **Unclear ramifications for preapproval discussions.** FDA regulations largely prohibit manufacturers from sharing information about a product's clinical efficacy prior to approval, other than publicly available information on clinical trials.¹⁶ The intent of this regulation is to protect patients and payers from potentially misleading information. However, the regulation has a concomitant "*chilling effect*" on industry's willingness to share any preapproval information, including information that might be helpful to payers, such as pharmacoeconomic data, in order to avoid potential legal ramifications.¹⁷ Several stakeholders emphasized that the lack of preapproval data in turn impedes payers' ability to accurately predict and manage the cost implications of new therapies.

Dynamic political climate and pending legislative and legal decisions

There is rising pressure to address the high cost of specialty drugs broadly. Policymakers are calling for greater transparency in how manufacturers determine price, as seen in proposed bills in various states including California, Massachusetts, Virginia, and Washington. Clinicians are also joining the fray, as evidenced by the American College of Physicians' recent pricing-transparency policy recommendations.¹⁸ In parallel, the Senate Finance Committee has actively investigated pricing practices by Turing Pharmaceuticals and recently completed an in-depth investigation of Gilead's launch of Sovaldi.¹⁹ However, the extent to which these efforts will drive the systemic changes required to better manage costs remains to be seen, especially in an election year.

Court decisions may help spur urgency among policymakers, as evidenced by pending class-action lawsuits that could reverse Medicaid's prior-authorization controls for Sovaldi.²⁰ Some stakeholders anticipate that these court decisions may create a precedent for unrestricted patient access to curative therapies, which would pose a crisis-level burden on budgets.

¹⁴ For a detailed discussion on this topic, see Richard A. Young and Jennifer E. DeVoe, "[Who Will Have Health Insurance in the Future? An Updated Projection.](#)" *Annals of Family Medicine* 10, no. 2 (2012), 156–162.

¹⁵ [National Association of Medicaid Directors to members of the US Senate and House](#), October 28, 2014.

¹⁶ Eli Lilly and Company and Anthem, "[Facilitating Open Communication About Emerging Therapies](#)" (Eli Lilly and Company and Anthem, January 29, 2016).

¹⁷ [Ibid.](#)

¹⁸ Hilary Daniel, "[Stemming the Escalating Cost of Prescription Drugs: A Position Paper of the American College of Physicians.](#)" *Annals of Internal Medicine*, published online March 29, 2016.

¹⁹ Sarah N. Lynch and Bill Berkrot, "[Senate Panel Focuses on Toll of Valeant, Turing Drug Price Spikes.](#)" *Reuters*, December 9, 2015; Senate Finance Committee, "[Wyden-Grassley Sovaldi Investigation Finds Revenue-Driven Pricing Strategy Behind \\$84,000 Hepatitis Drug.](#)"

²⁰ Jake Harper, "[States Deny Pricey Hepatitis C Drugs to Most Medicaid Patients.](#)" *NPR*, December 27, 2015.

The impact of curative therapies: challenges and gaps

As discussions aimed to shed light on how healthcare leaders are beginning to think about the impact of curative therapies in the United States, participants identified several challenges and gaps that could inform next steps and priority setting.

Cross-cutting challenges

While a diverse cross-section of stakeholders offered nuanced views on the challenges curative therapies pose to their organizations and the US healthcare system broadly, several themes consistently arose across interviewees:

- **The timing is ripe for proactive engagement on meaningful solutions before a crisis occurs; however, there is no single, easy solution.** Stakeholders expect that because curative therapies are unique, complex policy changes will be needed to address and manage them.²¹ There is, in short, no low-hanging fruit, several leaders cautioned. Many fear that only a crisis will compel change; others stress the importance of getting out ahead of a potential crisis now.
- **The degree of acceptance of high-cost curative therapies varies.** Some stakeholders reject the presumption that a cure needs to be costly. Others embrace the premise that curative therapies, if effective, offer high value, and they suggest focusing instead on better predictability and cost management. Any efforts to address curative therapies will need to harmonize and respond to these diverse viewpoints around value.
- **Uncertainty of outcomes and long-term efficacy for patients are major barriers to forward progress.** Payers, patient organizations, and subject matter experts universally emphasize that any approach to reimbursing curative therapies will rely on understanding and mitigating risks associated with clinical uncertainty. Stakeholders agreed that the more convincing the clinical trial data is vis-à-vis the current standard of care, the easier solutions can be planned for, paid for, structured, and potentially financed.
- **Price concentration and total cost to the system are serious concerns.** The major challenge, most stakeholders agree, is not with a new, ground-breaking gene therapy for a rare disease or two, but the cumulative concentration of cost if a number of such therapies are approved at the same time. Additionally, leaders fear the onset of more curative therapies for diseases with a larger patient population – or, in other words, another Sovaldi-like situation. As such, many stakeholders assert that solutions for curative therapies must involve policy-level conversations about long-term health financing in the United States.

“There is no magic bullet.”
- payer, on potential solutions

“The first question is, does it [curative/gene therapy] work?”
- subject matter expert

“We are heading in an out-of-control direction because soon we may have curative or specialized therapies for everyone for every condition. It’s clear that it’s unsustainable.”
- payer

²¹ Policy changes would include public-sector rules and regulations as well as rules, practices, and operating procedures for both the public and private sectors across stakeholders (e.g., manufacturers, payers).

Gaps in the ecosystem

Stakeholders are only beginning to anticipate the approval of more curative and gene therapies. Several gaps, however, continue to impede decision-making and action:

- **Knowledge gap.** The lack of long-term clinical data, understanding of target populations, and cost-benefit analysis of curative therapies hinders payers' ability to shape reimbursement strategies. Current value-assessment frameworks are perceived by some to be deficient in taking into account the specific dimensions of a curative therapy; rather, they are more focused on chronic treatments.
- **Information-sharing gap.** Currently, manufacturers and payers hold clinical and economic data, including costs, within “silos” – a practice perpetuated by a lack of precompetitive forums to exchange information on emerging technologies before they are approved.
- **Leadership gap.** Sustainable access to curative and gene therapies is too complex a challenge for any single stakeholder within the US healthcare system to take on alone. Champions are needed to be proactive in defining solutions benefitting multiple stakeholders. Given the strained resources and limited bandwidth of public agencies, many relevant federal and state government agencies have yet to seriously consider how to manage the onset of curative therapies despite acknowledging their high priority.²² Similarly, national patient organizations recognize the importance of curative therapies in the mid- to long term, but they have limited resources that need to be focused on day-to-day topics related to current standards of care. Others call for bold new thinking and approaches by industry, especially on cost and reimbursement. This view is shared by some industry representatives themselves; however, many stakeholders perceive that manufacturers are unlikely to meaningfully self-regulate on their own.

“Industry self-regulation [of cost] is wishful thinking.”
- subject matter expert

Exploration of innovative payment models

Several academics and healthcare leaders have proposed innovative payment models for curative therapies in order to mitigate the high up-front budget impact and risks associated with long-term clinical uncertainty. These models, if implemented, could significantly change how reimbursement works in the United States. Table 1 presents a summary of the payment models discussed.

²² At the state level, curative therapies are of utmost concern, and some states are creating new policies and procedures in reaction to the Sovaldi crisis. One example is the new provision in Texas that requires any new drug estimated to cost the Texas Medicaid system over \$500,000 to be approved by the Legislative Budget Board. See Senate Committee on Finance, *The Price of Sovaldi and Its Impact on the US Health Care System*, S. Prt. 114–20 at 86 (2015).

Table 1: Innovative payment models explored with stakeholders²³

Model	Key attributes	Caveats
Consumer mortgage²⁴	<ul style="list-style-type: none"> Healthcare loans managed by financiers – true consumer model would entail debt financing for 100% of cost of therapy (i.e., for therapies fully denied by insurers) Costs amortized over time plus interest by patient, who borrows from a special-purpose entity financed by market/investors Payment linked to clinical efficacy over time Risks to bondholders can be reduced by guarantees on principal supported by third parties (e.g., philanthropy, developers, pension funds) No collateral required (most likely) – similar to student loans 	<ul style="list-style-type: none"> High interest rates Patient “double paying” (loan + healthcare premium) Likely to trigger regulatory oversight/consumer-protection vehicles For financiers, default risk
In-house	<ul style="list-style-type: none"> Healthcare loans managed by industry In-house customer financing function similar to auto loan business or GE Capital wherein pharma companies takes risk on their books 	<ul style="list-style-type: none"> Requires additional investment and risk by pharma and significant corporate restructuring to add new function Same as above (high rates, regulation, default risk)
Hybrid model²⁵	<ul style="list-style-type: none"> Partial coverage from plans and remainder from consumer – example modeled by Montazerhodjat et al²⁶ includes 50% cost shared by payer Akin to financing cost of large copays Terms for Sovaldi price range would be nine years at 9.1% See consumer mortgage model above for other principles 	<ul style="list-style-type: none"> Incentivizing payer coverage if they will not fully recoup clinical benefit return on investment Patient “double paying” (loan + premium) Near impossible to apply for gene therapies – best for Sovaldi price range
Rebates²⁷	<ul style="list-style-type: none"> Patients provide initial copays for expensive drugs based on formulary tier, but copayments decrease over time based on adherence and outcomes 	<ul style="list-style-type: none"> More relevant to existing specialty drugs (chronic-care model) Systems changes required to link clinical outcomes with claim payments May not support size/scope of needs
Amortization²⁸	<ul style="list-style-type: none"> Payer pays developer over time based on list price of therapy and achievement of clinical milestones (could also be structured as “debt” to developer) Payer presumably could negotiate lower interest rates and prices Model would require clear clinical milestones over time, which could follow the patient from payer to payer 	<ul style="list-style-type: none"> Patient portability in US Assumes development of clear clinical milestones and payer acceptance of value Management of clinical milestones a likely challenge (e.g., triggering/stopping payments as needed) Longer return on investment for developers/investors Requires changes to accounting practices Only spreads risk, does not solve long-term sustainability issues

²³ This analysis integrated payment models that have been discussed in separate academic papers, discussed publicly, and/or utilized in other geographic contexts outside of the United States, as well as hybrid models suggested in discussions with stakeholders and experts.

²⁴ Vihad Montazerhodjat, David Weinstock, and Andrew Lo, “Buying Cures versus Renting Health: Financing Healthcare via Consumer Healthcare Loans,” *Science Translational Medicine* 8, no. 327 (2016). Note that what Montazerhodjat et al propose is in fact a hybrid model. We include a true 100% consumer mortgage option to represent the full spectrum of healthcare financing models in the event that curative therapies are denied entirely by payers.

²⁵ *Ibid.* Note that the paper offers this model as a short-term fix in the event that traditional insurance coverage is delayed or uncertain. The authors underscore that they foresee the costs eventually shifting to payers.

²⁶ *Ibid.*

²⁷ J.D. Kleinke and Nancy McGee, “Breaking the Bank: Three Financing Models for Addressing the Drug Innovation Cost Crisis,” 123.

²⁸ Gottlieb and Carino, *Establishing New Payment Provisions for the High Cost of Curing Disease*; Touchot and Flume, “The Payers’ Perspective on Gene Therapies,” 902–904; and Kleinke and McGee, “Breaking the Bank: Three Financing Models for Addressing the Drug Innovation Cost Crisis,” 118–126.



Annuity or “lifetime leasing”²⁹	<ul style="list-style-type: none"> ▪ Payer pays developer in defined installments (e.g., annual) over defined time span ▪ Not necessarily linked to list price nor structured as “debt” ▪ Cost and/or timeframe could be capped ▪ Payment based on maintaining clinical efficacy ▪ Opportunity for developer to make more than list price over time if efficacy is maintained (reward for performance) 	<ul style="list-style-type: none"> ▪ Same as above
Reinsurance³⁰	<ul style="list-style-type: none"> ▪ Insurance for insurers where multiple payers pool risk for very high-cost treatments (similar to catastrophe insurance model, transplants) ▪ Dedicated carve-out vehicle would come into effect at a lower threshold than currently exists ▪ Could annuitize payments, link payment to outcomes ▪ Would promote individual patient care management 	<ul style="list-style-type: none"> ▪ Unclear implications for patient premiums and cost sharing ▪ Requirements and specs from reinsurers are very narrow ▪ Only spreads risk; does not solve long-term sustainability issues
Managed entry, performance/ value-based³¹	<ul style="list-style-type: none"> ▪ Contracting solution between payers and developer in which reimbursement is linked to clinical outcomes in real world ▪ Many stakeholders assert that the Medicaid best-price requirement discourages these types of contracts 	<ul style="list-style-type: none"> ▪ Only works in easily identifiable and controllable patient populations ▪ Clinical data sharing highly sensitive ▪ Negotiations very difficult to finalize ▪ Costs/benefits over time for payers uncertain at contract start (i.e., if therapy is successful, will benefits of patient health outweigh costs of treatment?)
Bond mechanisms³²	<ul style="list-style-type: none"> ▪ Governments make legally binding, long-term financial commitments, which are made available immediately for short-term healthcare delivery by issuing bonds to market investors ▪ In the US, would need to apply this approach to a pool of payers (public and private) to achieve scale for market ▪ In US version, would be linked to clinical outcomes and likely targeted to high-cost gene therapies (\$1 million+) 	<ul style="list-style-type: none"> ▪ Not yet piloted in advanced-economy healthcare systems ▪ Effectiveness of health outcomes must be guaranteed ▪ Extensive policy changes and negotiations required to set up and govern pool of payers ▪ Requires legislation to mandate portability of debt across payers when patients transfer from one risk pool to another

Stakeholders reflected on the pros and cons of these new payment models in solving their main concerns and challenges with curative therapies, as highlighted below.

Public-payer views

Public payers share heightened concerns about the budget impact of more curative therapies in the wake of Sovaldi. They fear both an increasing “*stack*” of rare-disease therapies that will consume an ever-growing share of their budget to the detriment of critical public-health challenges and the prospect of gene therapies for non-rare diseases. However, many public-payer representatives recognize the unique potential of curative therapies and are eager to provide access to their patient populations, including through

²⁹ Touchot and Flume, 902.
³⁰ Kleinke and McGee, 122–123.
³¹ Don Husereau, “How Do We Value a Cure?” *Expert Review of Pharmacoeconomics & Outcomes Research* 15, no. 4 (2015), 551–555.
³² Husereau, 553–554. See also Montazerhodjat et al, “Buying Cures versus Renting Health: Financing Healthcare via Consumer Healthcare Loans,” in discussing payers replacing patients in healthcare loan model.

new payment models. Most public payers share the view that, because of the nature of their mandates, *“it’s not a matter of if we will cover it [a new curative treatment], but how.”*

Of top concern to payers is the transition from fee-for-service to value-based managed-care plans, which may constrain their ability to pay for new innovations, including curative therapies. Specialty and rare-disease populations, which may not be diffused equitably across risk pools, pose particular challenges for these types of plans. In the case of Sovaldi, states have responded by employing non-risk-based models where the state pays for treatment directly by carving out high-cost therapies from the capitated rates the state pays to managed-care organization contractors. Non-risk-based payment is a short-term, interim solution adopted by some states to manage coverage for specialty therapies under the constraints of set annual budgets. Some public payers argue that enhanced predictability on price would help state Medicaid departments better structure reimbursement for new innovations by ensuring that capitation rates for managed-care organizations are adequately set before a new therapy launches.³³

In addition to these short-term responses, what solutions are public payers considering for the long term? Broadly, several are eager to engage in innovative contracting arrangements linking payment to performance, which they deem a viable option for reducing risks associated with clinical uncertainty. Some caution, however, that curative therapies may not be the appropriate test case for such models. If a therapy is effective, then payers would still be *“on the hook”* financially, and such a therapy would bring high demand and further constraints on budgets. Performance-based contracts may be better suited, some argue, to traditional specialty drugs.

Other public payers are more bullish about performance-based models. While manufacturers stress that the Medicaid best-price requirement inhibits their ability to engage in performance-based contracts, many public payers perceive that manufacturers should be able to work around the lack of clarity in the current law when taking such a risk. The need for a waiver, they claim, is a matter of interpretation of the language.

See Table 2 for examples of pay-for-performance models.

Other public payers are interested in annuity and amortization models – and even briefly considered them for Sovaldi – but remain uncertain about how to remove operational barriers. One such barrier is portability of Medicaid patients. State systems are often unable to recoup the benefits of paying for a cure when patients’ income levels change or they transition from one state Medicaid program to another.

Given the myriad of constraints facing public systems, especially at the state level, some representatives assert that only a federal solution will be effective to manage high-cost cures. For curative therapies for large populations, this might include exceptional policy pathways such as direct procurement, as is currently the practice under the Vaccines for Children program, and/or the federal government serving as the payer of last resort.

³³ Others, however, feel that while better predictability can help manage initial budget impact, it will not mitigate total budget impact over time and therefore offers little with respect to sustainability.

Table 2: Pay-for-performance/value-based pricing examples

Therapy	Partners	Details ³⁴
Entresto	Novartis, Cigna, Aetna	<ul style="list-style-type: none"> Heart failure drug that helped keep patients out of the hospital during trials. Deal will link reimbursement for Entresto to results as measured by decreased hospitalization rates.
Repatha	Amgen, Harvard Pilgrim	<ul style="list-style-type: none"> Harvard Pilgrim agreed to include Repatha as the only PCSK9 (proprotein convertase subtilisin/kexin type 9) drug in its formulary. PCSK9 drugs help lower LDL cholesterol. Amgen offered a discounted price to Harvard Pilgrim and will provide rebates to the plan if the drug fails to lower cholesterol in plan members to the same degree that it did during clinical trials. Amgen will also provide additional discounts if patient utilization (and total cost to the plan for that utilization) exceeds predefined levels in order to target prescriptions to those patients who most need them.
Olysio	Janssen, NHS, National Institute for Health and Care Excellence	<ul style="list-style-type: none"> In England, Janssen agreed to a scheme wherein the National Health Service would only pay if the drug effectively cured hepatitis C in patients within 12 weeks of treatment. Janssen also agreed to refund any costs associated with failed treatments and is offering pretreatment blood tests to identify potential patients that may not respond to the therapy beforehand in order to limit potential exposure.

“Can’t we just imagine a world where these [therapies] would cost less?”
 - private payer

Private-payer views

Both public and private payers in the United States affirm they lack sufficient tools to manage the concentration of cost likely to occur if several new curative therapies are approved at the same time, but private payers in particular feel constrained by new regulations limiting the degree to which they can shift costs onto members through premium increases. While they recognize that such regulation is good for patients in the short term, they fear that shrinking margins will destabilize the insurance industry and/or cause payers to decrease benefits packages.

Within this context, some interviewees foreshadowed a *“tipping point”* where premiums will not be able to keep up with the costs of treatment.³⁵ Other payers are more receptive to the game-changing potential of curative and gene therapies but call for actuarial and economic models that better predict demand within their risk pools and the ensuing costs and benefits of a cure.

³⁴ Based on publicly available information. Tracy Staton, *“Surprise Proposal: Medicare Wants to Jump on the Value-Based Pricing Bandwagon.”* *FiercePharma* (blog), March 9, 2016; Caroline Humer, *“Novartis Sets Heart-Drug Price with Two Insurers Based on Health Outcome.”* *Reuters*, February 9, 2016; Carly Helfand, *“Novartis’ Pay-for-Performance Plan Has Its Critics – Express Scripts’ Miller Among Them.”* *FiercePharma* (blog), July 10, 2015; Adrienne Appel, *“Amgen Agrees to Pay-for-Performance Deal with Health Plan.”* *Bloomberg BNA*, November 16, 2015; Eric Palmer, *“Janssen Agrees to Rebate Cost of Olysio to England’s NHS If It Doesn’t Work.”* *FiercePharma* (blog), January 16, 2015; and *“Novel ‘Pay If You Clear’ Scheme Introduced in England for New Hepatitis C Treatment OLYSIO (simeprevir).”* *Medical News Today*, January 19, 2015.

³⁵ Also echoed in Touchot and Flume, *“The Payers’ Perspective on Gene Therapies.”* 904.

Private payers vary in their reactions to new payment models. Many are highly skeptical of models that pass on additional costs to patients, especially consumer-financing models. They also emphasize that amortization and annuitization of payments are difficult to implement in the real world. While several acknowledge the myriad challenges in negotiating performance-based contracts,³⁶ others suggest that curative therapies could be an excellent test case for “*making performance-based contracting work*” in the United States.

Patient organization views

For patient organizations, the value of a curative therapy depends largely on the availability and quality of existing standards of care. For gene therapy specifically, many argue that some patient groups would not be incentivized to try gene therapy if they had confidence in existing treatments, even if those existing treatments were costly. On the other hand, patients with diseases that have limited or no standards of care are more apt to try a gene therapy, even if long-term efficacy is unclear.

Patient representatives as a whole are concerned about any additional costs borne by patients. While most patient organizations are skeptical about consumer-loan models, deeming them a “*tough concept*,” a minority acknowledge that consumer financing could deliver immediate access for those who can afford it – but they stress that there are many patients that could never consider nor obtain such an option.

Subject matter expert, economist, and financier views

The primary concern among subject matter experts in and outside the United States is the lack of certainty around long-term patient outcomes, particularly challenges associated with patient monitoring, which is essential for tracking long-term efficacy and safety. While regulators can help enforce patient registries and other monitoring tools, data is not captured in a consistent or standardized fashion. Moreover, patient-monitoring tools may not capture other data points relevant to quality of life, societal benefits, economic data, or other information that may be useful for payers to manage risk.

In terms of new payment solutions, subject matter experts say consumer financing is a mixed bag: some stakeholders are confident that healthcare loans could be structured much like a home mortgage and provide patients with immediate access to high-value therapies if coverage by traditional payers is denied, uncertain, or delayed; others assert that financing models cannot work for healthcare and the application of these models will result in “*civil unrest*” if patients who do not meet credit criteria are denied access. Of particular concern are models in which patients “*double pay*” – pay for both healthcare premiums and loans – and models where industry is proposed to be part of the credit solution. These are perceived as industry being rewarded twice, both for the cost of the drug and interest on loan repayments. Some suggest, however, that

“You cannot forget that the patient desperately needs these treatments, but patients are often put between the payer and developers and often asked to play sides.”
- patient organization

“[The] challenge is that these therapies offer a substantive health gain that on normal grounds would justify a high price.”
- subject matter expert

³⁶ As underscored by the best-price requirement; see Kleinke and McGee, “[Breaking the Bank: Three Financing Models for Addressing the Drug Innovation Cost Crisis](#),” 122.

healthcare mortgages may be more palatable if patients are able to receive a rebate or reduction on their premiums.

Amortization and annuity models may be more feasible in Europe or the UK, where there is little to no patient portability, but most experts acknowledge that the implementation of these concepts in the United States would be difficult to realize in the short term.

Recommendations on productive paths forward

While healthcare leaders universally emphasized there is no low-hanging fruit in solving the question of how we pay for cures, they proposed several paths forward that could lay the groundwork for concrete solutions.

Better understand curative therapies and their economic implications

Healthcare decision-makers agree that more analysis on the practical application of gene and curative therapies is required before policy changes and new payment models can be seriously considered and adopted. Interviewees suggested the following:

- **Information-sharing forums.** Stakeholders should institute precompetitive forums or tools that allow payers, patient organizations, and others the opportunity to learn about new, potentially game-changing innovations as early as possible (i.e., prior to FDA approval), including economic scenarios (i.e., cost implications).
- **Modelling and data.** Manufacturers and payers should conduct cost-effectiveness analyses using more flexible value-assessment frameworks. New, forward-looking actuarial models could also be developed that allow payers to better accommodate new technologies and identify target populations. Analysts should also assess whether high-cost curative therapies can “*net out*” for payers across the US healthcare system – meaning that even if a payer loses the accrued benefits of covering a gene therapy because a patient transfers out of their risk pool, the cost is neutralized by new patients coming into the risk pool who were already covered by a previous payer.
- **Pilots.** Pilots could help assess the economic implications of gene therapies in the real world. Proposed ideas include tracking gene therapy recipients within a closed system (e.g., Kaiser) or a system with low turnover rates. After a certain time horizon, manufacturers and payers would be able to evaluate the degree of costs and benefits to the payer. Such an option, of course, would take time to yield meaningful data.

Consider new approaches to reimbursement, but be selective

Some stakeholders refute the utility of new payment models, focusing instead on cost containment through incentivizing or even mandating price reductions. However, for those who are more receptive to the concept of innovative payment options, models where manufacturers and payers take more risk vis-à-vis the patients are received more favorably. Reactions to the consumer-financing family of models – which includes consumer healthcare mortgages, hybrid models, and in-house loans – are mixed at best,

“[Consumer healthcare finance] looks in many ways like the residential mortgage market or the student loan market.”
- economist

as noted above. A limited group of stakeholders see consumer financing in healthcare as an innovative, market-based opportunity to rapidly close the access gap once new therapies are approved. However, in the course of this assessment, the vast majority of stakeholders, including industry, largely agreed that consumer-facing models were not a sustainable solution or even a practical short-term solution.

Amortization, annuities, reinsurance, and pay-for-performance contracts were all deemed as more favorable options. Some models face challenges in feasibility, such as managing patient portability, and others, such as reinsurance, fail to reduce long-term costs to the system. Yet some stakeholders opine that curative therapies, given their distinct potential and long-term uncertainty, will require new frameworks for reimbursement and therefore present a perfect opportunity to implement some of these new models.

Define and adopt postmarket strategies for assuring clinical outcomes

Curative and gene therapies will no doubt require a robust postmarket monitoring program. For rare diseases, some stakeholders assert, this should be a surmountable proposition given that rare-disease populations are already closely defined and monitored by patient groups. The challenge, most agree, lies in therapies that target larger populations. Stakeholders urge consideration for non-propriety registries while appreciating the difficulty in identifying appropriate financing sources to maintain such a registry. Germany offers an instructive example in arthritis, where a large registry for patients using a range of therapies is monitored by an independent academic center.

In terms of delivery, stakeholders agreed that for gene therapies in particular, manufacturers should establish centers of excellence. Payers will likely agree to limit coverage to procedures delivered at specific centers of excellence to minimize risk of mistakes and failures. Because of the unique and high-tech nature of gene therapies, payers and other key stakeholders will want to be assured of a “gold standard” when it comes to delivery. Delivery should not, however, be so restricted as to give only one or two leading service-delivery facilities a monopoly over the market.

Next steps

Manufacturers will not be well served by a scorched-earth approach of pricing curative and gene therapies at what the market can bear without a concerted multistakeholder conversation about value and reimbursement. A “winner-take-all” philosophy for curative therapies in the current political climate will limit the long-term market for all manufacturers and only prompt reactive legislation or price controls.

Most stakeholders interviewed as part of this assessment agreed that an influx of multiple gene therapies – even if initially for small populations – will result in an upfront cost that cannot be appropriately managed through current reimbursement regimes. Despite their divergent viewpoints on many issues, most also agreed that there was value in bringing all stakeholders to the table to start a conversation on how to shift the US healthcare

“Reinsurance is not a solution [for curative therapies]. It does not solve the issue of overall cost.”
- private payer

system from one that supports chronic treatments to one that enables and promotes cures.

Stakeholders already converged on a major point of consensus through the course of this initial landscaping effort, agreeing that consumer financing options where industry is either part of the credit solution or where patients are compelled to take out additional debt to finance access to cures is not a sustainable pathway. This outlook was shared not only by patient organizations and payers but also by industry. The benefits of “*emergency access*” that such pathways might bring for the few patients who can afford consumer loans pale in comparison to the caveats associated with an approach built on patient debt.

Based on discussions conducted as part of this analysis, there are two distinct yet related paths forward for ensuring sustainable access to curative treatments for patients. These collaborative paths would benefit all stakeholders across the US healthcare system and, with the appropriate mix of leadership and resources driving them, could be initiated in the short to mid-term.

1. Establish a multistakeholder initiative with a focus on value measurement that assesses the long-term costs and benefits of curative therapies

Academics and third parties can start developing more robust analyses of the value of curative therapies either in partnership with industry or through other resources. Forward-looking models that quantify the economic implications of therapies in the pipeline can provide a foundation for understanding how curative and gene therapies will work in the real world and their impact on quality of life for patients and their families. As a payer noted, *“This is a great opportunity and a really good time for the whole industry – all the manufacturers, the developers, as well as the payers – to come together and have some discussion about how we want to evaluate, manage, and track the patients who are being treated.”*

Questions such an initiative could seek to answer would include the following:

- How can stakeholders achieve consensus on frameworks or guiding principles that can inform value-assessment frameworks but also allow flexibility for different methodologies to be developed and applied?
- How can value-assessment frameworks appropriately reflect patient and/or societal benefits (e.g., increased economic productivity), and what is the path to achieve consensus on how these would be captured and evaluated over time?
- How can models reflect pathways for scalability of gene therapy and cost curves over time, including for different stakeholders?

2. Launch a multistakeholder working group on disruptive models for reimbursement better suited to realizing the value of curative therapies

Such an effort would focus on changing several underlying factors that currently inhibit innovative risk-sharing and payment models. These enablers include (1) formation of

“You can use investors’ expectations to [influence] a paradigm shift in the way healthcare provision has to be structured.”
- manufacturer, on the need for industry to rethink ROI for curative therapies

precompetitive forums and/or tools wherein manufacturers could openly talk about price and value before approval; (2) clarification under the Medicaid best-price provision, which would encourage more opportunities for performance/risk-based contracting; (3) establishment of appropriate information technology infrastructure, such as “big data” analytics, to capture not only patient medical information but also economic data relevant to long-term patient management and tracking of clinical benefits; and (4) changes around revenue recognition for manufacturers and patient portability for payers to enable amortization and annuity models.

In parallel, this effort would delve into specific prioritized payment models to better understand the costs, benefits, and feasibility of their implementation. Given the aforementioned conclusions on consumer financing, models to be further explored are likely to be reinsurance and amortization/annuities.

Questions to be resolved include the following:

- How could quantitative scenarios be developed using indicative pricing ranges for gene therapies that reflect the costs and benefits of reinsurance models without infringing on competition rules and regulations? Similarly, how could these scenarios be applied to amortization and annuity models? What is needed in the approach to differentiate the impact for public payers and private payers?
- Based on the above scenarios, what new payment models would be eliminated in order to focus future efforts?
- What pilots can be designed to test operationalization of models by using products currently undergoing clinical trials or by paving the way for a pilot for therapies once they are approved?

Undergirding these two paths would be a concerted, overlapping policy effort by industry, legislators, and payers to make large-scale adoption of the above pilots/concepts a reality. Best-price waivers and safe harbor for manufacturers to discuss their pipeline preapproval may be an appropriate place to start, given the existing momentum stakeholders and trade associations have undertaken on these efforts, such as the new partnership by Anthem and Eli Lilly. Portability-related legislation – wherein payers would absorb ongoing financial obligations to ensure that risk and benefit from investing in an upfront curative therapy continue to be evenly distributed across all payers – may be a longer road ahead. Finally, select industry players should offer some guarantee on advancing guidelines or frameworks for self-regulation if paths that do not foster long-term cost sustainability, such as reinsurance, are deemed to be the most viable for curative therapies in the short term.

The above questions and challenges are not easy to resolve, particularly in the context of ongoing pricing debates. But as several public payers underscored, healthcare stakeholders are at a point in history where they are *“trying to drive big changes to our healthcare system,”* and *“all stakeholders need to come to the table”* to sustainably deliver new, better therapies to more patients. Perhaps no other type of therapy stands to help realize this vision more than the prospect of a cure.

“Reinsurance will lead to premium increases. We couldn't go out there with reinsurance as a solution without having some skin in the game - responsible pricing practices, industry guidelines, etcetera.”
- manufacturer

About this document

The views expressed in this document represent consolidated views of those who participated in exploratory interviews for this Curative Therapy initiative and is integrated with broader landscape analysis. This document is not intended to represent the particular policies or positions of the individual participants or their affiliated organizations. This material is prepared and copyrighted by Tapestry Networks with all rights reserved. It may be reproduced and redistributed, but only in its entirety, including all copyright and trademark legends. Tapestry Networks and the associated logo are trademarks of Tapestry Networks, Inc.

Appendix: Participants

Payers

- Naomi Aronson, Executive Director, Clinical Evaluation, Innovation and Policy, Blue Cross and Blue Shield Association
- Mike Barlow, Vice President, Operations, Palmetto GBA
- Joseph Chin, Deputy Director, Coverage & Analysis Group, Center for Clinical Standards & Quality, Center for Medicare and Medicaid Services
- Kate Goodrich, Director, Center for Clinical Standards & Quality, Center for Medicare and Medicaid Services
- Paul Jeffrey, Director of Pharmacy, MassHealth, Office of Clinical Affairs
- Tamara Syrek Jensen, Director, Coverage & Analysis Group, Center for Clinical Standards & Quality, Center for Medicare and Medicaid Services
- Lee Newcomer, Senior Vice President, Oncology, Genetics and Women's Health, UnitedHealth
- Ed Pezalla, National Medical Director, Pharmacy Policy and Strategy, Aetna
- Jack Rollins, Policy Assistant, National Association of Medicaid Directors
- Alan Rosenberg, Vice President, Medical & Clinical Pharmacy Policy, Anthem, Inc.
- Deborah Smith, Managing Director for Medical Policy for the Federal Employees Program, Blue Cross and Blue Shield Association
- Matt Salo, Executive Director, National Association of Medicaid Directors
- Andy Vasquez, Deputy Director of the Medicaid/CHIP Vendor Drug Program, Texas Health and Human Services Commission
- John Yao, Vice President of Medical Policy, Anthem, Inc.

Providers/health systems

- Peter Bach, Director, Center for Health Policy & Outcomes, Memorial-Sloan Kettering
- Jo Carol Hiatt, Chair, National Product Council, Kaiser Permanente
- Joan Schottinger, Technology Assessment Lead for Southern California and Medical Oncologist, Kaiser Permanente

Subject matter experts/health economists/insurance/investors

- Sarah K. Emond, Chief Operating Officer, Institute for Clinical and Economic Review

- Andrew Lo, Professor of Finance, Massachusetts Institute of Technology Sloan School of Management
- Peter Neumann, Director of the Center for the Evaluation of Value and Risk in Health at the Institute for Clinical Research and Health Policy Studies at Tufts Medical Center
- Tom Rutledge, Head of Fixed Income Origination, Magnetar Capital

Professional societies, patient/policy advocates

- Val Bias, CEO, National Hemophilia Foundation
- Kimberley Haugstad, Executive Director, Hemophilia Federation of America
- Paul Melmeyer, Associate Director of Public Policy, National Organization for Rare Disorders
- Michelle Rice, VP, Public Policy and Stakeholder Relations, National Hemophilia Foundation
- Ellen Riker, Vice President, CRD Associates
- Martha Rinker, Vice President, Public Policy, National Organization for Rare Disorders
- Peter Saltonstall, President and CEO, National Organization for Rare Disorders

Selected European HTAs/health systems/subject matter experts

- Mandy Bodnár, Scientific Advisor, Pharmaceutical Department, G-BA, Germany
- Nick Crabb, Programme Director for Scientific Affairs, UK National Institute for Health and Care Excellence
- Jan Müeller-Berghaus, G-BA, Paul-Ehrlich-Institut (PEI), Germany
- Adrian Towse, Director of the Office of Health Economics, UK

Industry sponsors

- Martin Andrews, Senior Vice President, Global Rare Diseases, GlaxoSmithKline
- Mark Battaglini, Vice President, Global Government Affairs & Public Policy, BlueBird Bio
- Shannon Baumann, Director, Pricing & Reimbursement Strategy, Biogen
- Tanisha Carino, Vice President, US Public Policy, GlaxoSmithKline
- Paula Cobb, Senior Vice President, Rare Disease Group, Biogen
- Sarah Creviston, Vice President and Global Head of Patient and Innovation Policy, Baxalta

- Liam Cullen, Government Affairs Director, GlaxoSmithKline
- Doug Danison, Head of Global Pricing, Reimbursement, and Market Access, BlueBird Bio
- Alison Finger, Senior Vice President, Marketing, BlueBird Bio
- John Glasspool, Executive Vice President, Head of Corporate Strategy, and Customer Operations, Baxalta
- Heidi Higginson, Vice President of Pricing & Reimbursement Strategy, Biogen
- Mansi Hopps, Associate Director, Commercial Assessments, Biogen
- Charles Hsu, Chief of Staff to John Glasspool, Baxalta
- Andrea Hunt, Vice President of Franchise Head Blood Disorders, Baxalta
- Nick Leschly, Chief Executive Officer, BlueBird Bio
- Josh Mandel-Brehm, Strategy & Operations Director, Rare Disease Group, Biogen
- Josephine McLeod, Vice President of HEOR & Pricing, Baxalta
- David Miller, Senior Vice President, Global Market Access, Biogen
- Matt Rousculp, Senior Director, Government & Medical Policy, GlaxoSmithKline
- Claude Schmitt, Head of Market Access, Global Rare Disease Unit, GlaxoSmithKline
- Dee Simons, Senior Director, Public Policy & Government Affairs, Biogen
- Florian Turk, Vice President, Head Global Market Access & Healthcare Solutions, GlaxoSmithKline