BREAKTHROUGH CURES, BLOCKBUSTER COSTS: FUTURE DIRECTIONS

Summary of a cross-sector dialogue sponsored by:

Blue Cross Blue Shield Association
Health, Medicine & Society Program of the Aspen Institute

BlueCross BlueShield Association

HEALTH, MEDICINE & SOCIETY PROGRAM
aspen institute

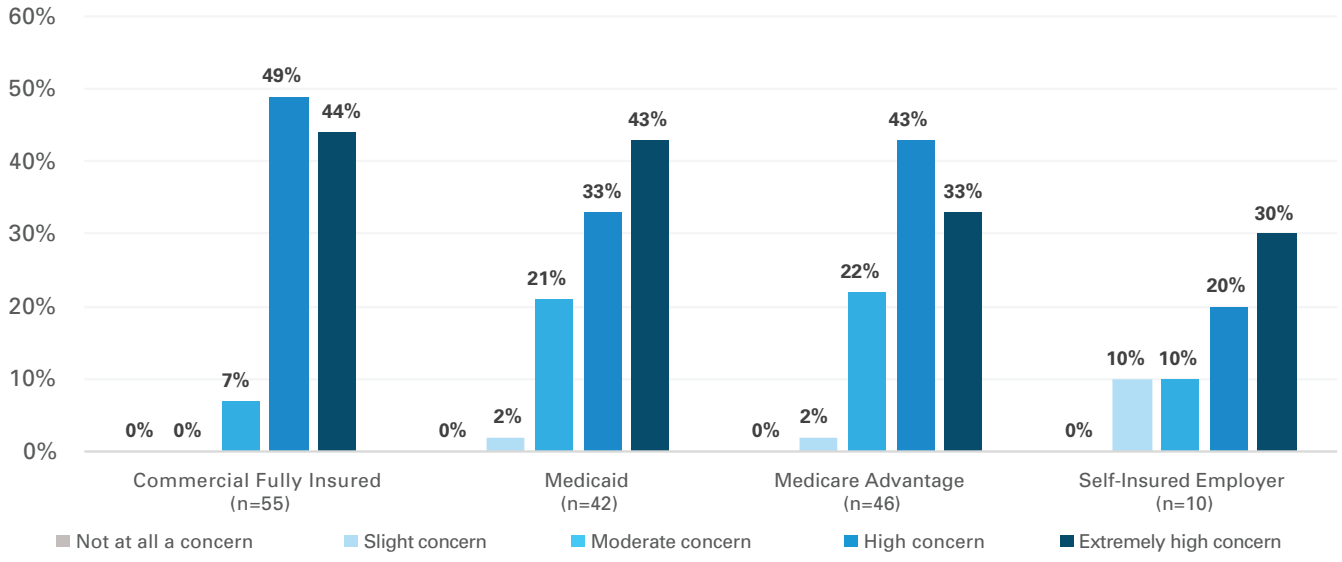
EXECUTIVE SUMMARY

Transformative pharmaceutical products that can cure or treat once-seemingly intractable medical conditions are coming onto the market in growing numbers, especially in the areas of genetic and cellular therapies and tissue engineering. Known as Advanced Therapies, these biomedical breakthroughs often treat rare indications and can be very costly to discover, develop, and produce. In part owing to high development costs and small markets, profits on these drugs are recouped over fewer patients and carry large price tags. Indeed, five such therapies approved from August 2017-May 2019 carry list prices that range from $373,000 to $2.125 million.[1] While follow-on innovation offers the potential to lower the expense of development and manufacturing and could translate into lower costs in a properly incentivized system, their prices nonetheless impact overall health care spending and could have the unintended consequence of crowding out other important services.

Even if the cost of developing and manufacturing these therapies drops, capturing their economic benefits remains a challenge. The current financing system was designed to consider actuarial risks on an annual basis, not to handle situations in which a short course of treatment is expected to have a very long-term

THE LIST PRICE FOR
5 APPROVED ADVANCED THERAPIES
FROM AUGUST 2017 TO MAY 2019 RANGED
FROM $373,000 TO $2.125 MILLION[1]

Figure 1. Level of concern regarding managing the financial risk and impact of high-cost durable therapies, by payer segment (n=153).[2]



SOURCE: MIT NEWDIGS. (October 11, 2019). FoCUS White Paper. Survey results–Payer perspectives on financing and reimbursement of one-time high-cost durable therapies. http://newdigs.mit.edu/sites/default/files/MIT%20FoCUS%20Payer%20Perspectives%202019F210v044.pdf

therapeutic effect, as in the case of Advanced Therapies. Virtually all payers (Medicare, Medicaid, commercial insurers, self-insured large employers, and others) have expressed significant concerns about the potential burden of covering their cost, especially as the number of these products grows.[2]

At the same time, developers have at times turned away from promising Advanced Therapy research for commercial, not clinical, reasons and fear that the growth of genetic therapies and related advances could be stunted without supportive pricing, financing, and coverage policies.[3] For their part, patients are concerned that the industry may lack incentives to pursue some promising products, especially for very rare diseases, or that payers will fail to cover their costs.

While the federal government has made various policy accommodations to encourage and expedite the research and development of breakthrough drugs, there have been no corresponding adaptations in how such products are paid for, once approved. The stakeholder consensus is that without innovative pricing and payment strategies, corporate, state, and national budgets could eventually be overwhelmed, industry could reduce its pursuit of some long-shot research into rare diseases and debilitating illnesses, and patients could be denied access to medical advances with extraordinary promise.

In early 2020, the Blue Cross Blue Shield Association and the Health, Medicine & Society Program of the Aspen Institute convened a working group of 15 leaders from industry, academia, government, and patient advocacy groups to accelerate discussions about Advanced Therapies and consider alternative financing frameworks to enhance access to these high-priced and potentially life-transforming products. Two former FDA commissioners—Scott Gottlieb (2017 to 2019) and Margaret Hamburg (2009 to 2015)—co-chaired the group. Alan Weil, editor-in-chief of *Health Affairs*, moderated the discussions. (See Appendix for list of participants.)

Over a day-and-a-half, participants explored the pricing, financing, access, decision-making, ethical considerations, and data-collection dimensions of the growing wave of Advanced Therapies expected to provide enormous benefits to patients who currently lack medications that could save their lives or improve them dramatically. The conversation touched on various aspects of the broad ecosystem for biomedical product innovation, which involves investors, pharmaceutical companies, regulators, payers, patients, and the taxpaying public.

While the dialogue was wide-ranging and thoughtful, it came with no expectation of identifying definitive solutions to an evolving and complex set of issues. The meeting was instead designed to lay out consensus themes that merit deeper exploration, recognizing that Advanced Therapies can move efficiently through the development and distribution pipeline only when the relevant players, policies, and programs are aligned. Ultimately, of course, the goal was to identify strategies that will sustain the health care system while making Advanced Therapies available and affordable to those who need them.

KEY FINDINGS

These are the group's key findings:

- It is imperative that anyone who can benefit from lifesaving Advanced Therapies be able to access them. Improving the health of people in need is the overarching goal of developing novel financing mechanisms to pay for these therapies.
- Current mechanisms to pay for Advanced Therapies are inadequate to ensure long-term sustainability. Society needs to consider the broader health and social system opportunity costs of different pricing and payment models and develop innovative new strategies for at least some of the therapies with blockbuster costs.
- Price and value must be part of any dialogue on payment strategies. The launch prices for Advanced Therapies are not necessarily a given and merit scrutiny, especially since the initial discoveries leading to their development are often funded, at least in part, by taxpayers. Understanding the risks and costs throughout the drug production pipeline, as well as the benefits they deliver to patients, is needed to assess price and value.
- The incentives that motivate product developers to pursue pioneering cures and treatment for rare diseases should be recognized and appreciated in order to ensure continued innovation.
- Outcomes or value-based payment models hold promise, particularly for treatments that offer immediate benefits or reduce otherwise needed care. To adapt these and other current financing approaches to emerging Advanced Therapies, better ways to assess clinical performance over time must be created.
- Although Advanced Therapies are sometimes called "cures," their long-term utility has not generally been established at the time of market entry owing to their novelty, and their durability is not fully established. Collecting rigorous, longitudinal data while minimizing the burden of patient data collection through registries that track outcomes is essential to inform experiments with novel financing.
- Society must create the right tools and venues to give patients meaningful input as value equations are considered. Beyond sharing anecdotes of clinical experience and emotional impact, patient feedback must be considered as part of regulatory, pricing, and access decisions.
- Existing regulatory policies should be thoroughly reviewed both to identify possible barriers to access, innovation, and competition and also to foster continued innovation and access to Advanced Therapies. Such review should include policies related to the financing, pricing, and purchasing of Advanced Therapies, such as Medicaid best-price requirements and the Orphan Drug Act.
- Risk pooling or stop-loss mechanisms may be necessary to distribute the costs of advancing treatments and cures equitably in order to share benefits widely.

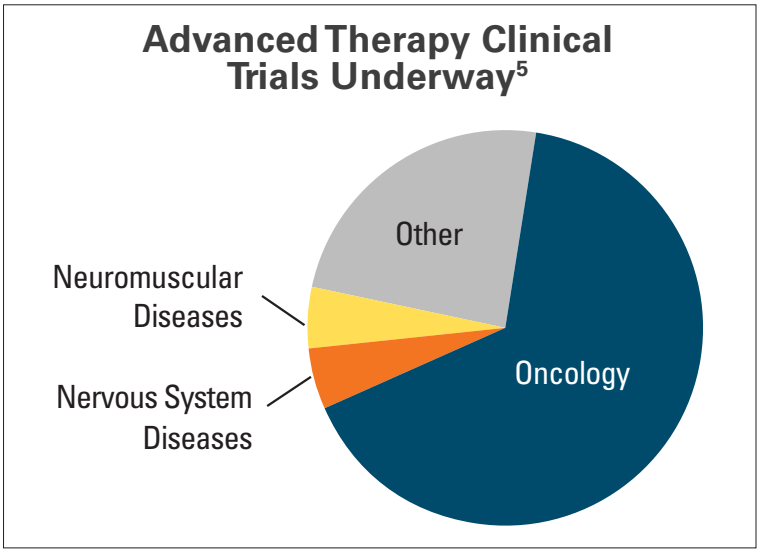
FRAMING THE PROBLEM

The remainder of this paper captures highlights of the discussion that generated these findings. Additional background was provided in a paper circulated prior to the meeting, which placed the issues in their larger context.[4] That paper reviewed the state of the science, the mechanisms currently in place to pay for approved pharmaceutical products, and the options that have been proposed to cover the high costs of Advanced Therapies.

Due to the COVID-19 pandemic, the world changed considerably in the year that followed the meeting. Pressures on health care delivery systems, constraints on spending, new thinking about how to approach R&D given the rapid-fire development of coronavirus vaccines, political shifts, and a deepened appreciation for the critical role that science and technology play in advancing health have all shifted the environment in various ways. These developments, and others sure to follow, underscore the imperative of designing responsive, dynamic systems that can accommodate the evolving science, technology, and policy framework for Advanced Therapies.

> "No one is admitting that the system is failing. It's not just that we have inequity, it's that what we have right now cannot correct the inequity."
>
> –Brian Wallach, I AM ALS

The potential to cure or transform the lives of patients with rare diseases, and ultimately to apply research advances to conditions that affect larger numbers of people, such as those with cancer or Alzheimer's disease, is at an inflection point, generating tremendous excitement within the patient, scientific, and medical communities. More than 1,220 clinical trials of Advanced Therapies are underway, according to the Alliance for Regenerative Medicine.[5] Approximately two-thirds of the trials are focused on oncology; the next most popular categories are neuromuscular and central nervous system disease groups, at five percent apiece.



As growing numbers of novel treatments and cures complete the US Food and Drug Administration (FDA) approval process and enter the marketplace, prices will remain high. Estimates from the Massachusetts Institute of Technology indicate that up to 90 gene and cellular therapies are likely to be approved by 2031 and used by a total of 550,000 patients. Based on the pricing of past products, annual acquisition costs for 90 new Advanced Therapies will equal $30B.[6] (Note that this does not account for offsets in the cost of usual care and improvements in productivity and quality of

life resulting from better outcomes or for the added expenses associated with the ancillary medical services that accompany treatment.)

The escalating financing challenges reflect innovations within the complex system that guides their research, development, and marketing. While most Advanced Therapies that have been approved recently, or are in the research pipeline, are for rare diseases with very limited treatment options, indications for their use are likely to grow over time. That expands hope for the 10 percent of the US population that is dealing with some type of rare condition,[7] it further complicates the pricing equation.

While it can be easy to talk in abstractions about access to medical breakthroughs, and the merits of making therapy available to all who might benefit, a patient's story adds another layer of meaning to the conversation. Aliya Rinaldi, a 35-year-old patient advocate with beta-thalassemia, a rare form of anemia, clarified the stakes for the group. When she was an infant, doctors told her parents that she was unlikely to reach her 20s and would require chronic blood transfusions for the rest of her life.

Aliya defied the odds, graduating college, marrying and raising a family, and pursuing a professional life—all the while receiving those transfusions, which involved a full-day hospital visit every few weeks. Various side effects developed along the way, including an iron overload that had to be treated daily with a self-administered injection and later oral therapy. Then, at age 30, she qualified for a clinical trial designed to modify the faulty gene that caused her beta-thalassemia. The rigorous regimen required strong doses of chemotherapy, stem cell transplantation, and many weeks of hospitalization, but the treatment was a success: Rinaldi has not required a blood transfusion in four years.

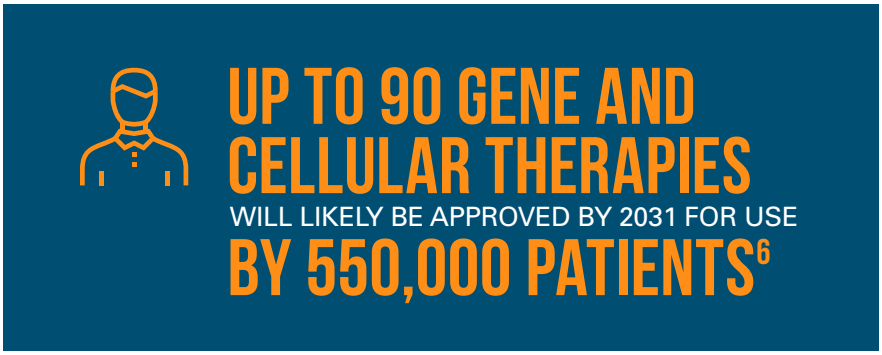
With Rinaldi's outcome as a kind of North Star, participants began building a framework for their discussions. Whether they were reflecting the concerns of self-insured employers considering blanket exclusions for certain costly drugs or insurers making product-by-product decisions about how to cover newly approved Advanced Therapies, representatives across sectors agreed that traditional reimbursement levers are by themselves no longer adequate to meet the therapeutic opportunities of the future while keeping health care spending sustainable.

Indeed, there was clear consensus about the need to test and evaluate alternative strategies before the system is further strained. Although many payers currently feel able to absorb the financial risk of a limited number of high-cost therapies, they anticipate growing challenges as the flow of products through the pipeline quickens, if future products follow the same development and pricing model that has been established for current therapies.

The heterogeneity of conditions and populations targeted by Advanced Therapies, their development costs, the mix of private and public payers involved, and the actuarial calculations that inform decision-making suggest the need for multiple, tailored financing approaches. Innovations that can substantially lower the development and manufacturing costs of Advanced Therapies are underway, but reimbursement mechanisms that can capture these savings for patients and use them to improve access to safe and effective therapies are still needed. Among the options in use or being considered are new insurance products, contracts that link payment to outcomes, milestones, other value determinations, risk-sharing mechanisms created through public/private partnerships, and structured loans. (Some of these are discussed further below; they are also detailed in the background paper).

> "They basically flipped on a light switch that had just been off my whole life."
>
> –Aliya Rinaldi, patient advocate

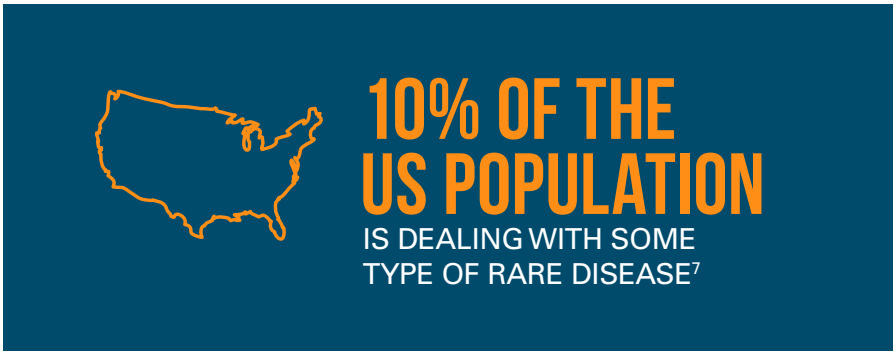
A dynamic "let a hundred flowers bloom" approach, with a strong data collection component, can foster experimentation and seed innovation and learning. In areas of unfolding practice, a structured yet fluid analytical and evaluation strategy is needed. Importantly, it should include steps for introducing new learnings back into the system so that as knowledge accumulates, it can be shared, analyzed comparatively to determine what works, and in what context, and replicated as appropriate.



The need for non-traditional financing is apparent because these therapies are exceptionally expensive. In many instances, research costs contribute to the high prices associated with novel science, but how drugs are priced does not explicitly reflect the extent of any federal investments. The prices may, however, reflect a product's curative intent, which means that product developers will seek to recoup their investments on initial prescriptions, given that future cases will be limited in number. The entire treatment may be administered just once, requiring that direct and indirect costs plus profits be recouped through that single course of therapy. Moreover, in some cases, competition is unlikely to arise because the need may be largely addressed by a first-to-market therapy. Pricing is further influenced by the high costs of goods and manufacturing required for the product, the comparative costs of standard care, the possibility that therapeutic indications could expand, and the type of payer involved.

Whatever the reasons, high prices become an even greater pressure point on the system as Advanced Therapies are approved to target broader populations. This is already apparent with treatments to cure hepatitis C and will likely intensify with continued advances, such as therapeutics to prevent or delay Alzheimer's disease.

The regulatory environment is another area ripe for attention so that policies are in place to support experimentation with new payment models. In particular, the requirement that product developers charge Medicaid the lowest price they offer to any other purchaser ("best price") was identified by the group as a potential barrier to innovation in pricing models. The best-price policy would come into relief if a drug developer agreed to charge nothing should a patient fail to achieve a therapeutic response within a certain time frame. The obligatory "best price" in that situation would fall to zero. (A regulatory change in December 2020 that provides greater pricing flexibility under value-based purchasing agreements somewhat eases this impediment.[8])



"It's not the payment processes that are resulting in the problem. It's the fact that a high-cost drug is resulting in payment mechanisms that don't work any longer."

–Sarah Marché,
Highmark, Inc.

DEFINING THE USE CASE

To narrow the conversation while providing a springboard into the broader universe of Advanced Therapies, participants defined the characteristics of a therapy that would most likely resist market forces. Typically, a novel financing strategy might have utility for a condition prevalent in a small population, where a cure is possible for those currently affected but the rarity of the disease would not support a secondary market. A further criterion would be that the disease is so debilitating that patients would opt to use the new therapy immediately, rather than turn to the standard of care or wait until competing products are approved.

> "I think the answer to who bears the cost is everybody."
>
> –Anita Wagner, Harvard Pilgrim Health Care Institute

A prototype of a high-cost, high-impact Advanced Therapy, for example, could be a first-to-market gene therapy for a very limited population. Administered either once or in a short sequential series, it would cure the prevalent population and then be administered much less often to newly diagnosed individuals. The developer would either need to expand the indication or branch out to other indications to support ongoing commercialization. Moreover, the ability of a first-to-market product to cure the prevalent population may reduce the incentive for second-to-market innovation, since the existing pool of patients might have already received definitive therapy. Given the high cost of developing and manufacturing these products, the population of people who are newly diagnosed with a rare condition each year, once existing patients are treated, may not be large enough to support the cost of a second entrant. This could further reduce competition that could otherwise lower prices and broaden access. In effect, there may be a natural monopoly for a first-to-market product in these settings.

The discussion question: How should such a therapy be paid for?

Payment strategies for this kind of high-cost drug can be considered along a continuum. On one end, the entire burden of covering a blockbuster therapy could be placed on each individual; on the other, the full cost could be socialized, with no out-of-pocket expenses for patients. Between these poles, the determination of how far to aggregate and share risks has a range of implications for who gets leverage over price, whether decision-making is centralized or dispersed, and who provides the resources.

In reality, any viable model will likely require that costs be borne by some combination of individuals, insurers, investors, pharmaceutical companies, and taxpayers. While most of those players are assumed to have a role in refining solutions, the patient voice is too often left out of the equation. Integrating that perspective is essential.

CONSIDERING VALUE

Relative value can be part of the equation used to calculate payments for Advanced Therapies. While economists define value in many ways, the term at its most basic can be thought of as what one gets for what one pays, a framing that factors in the component parts of outcome and cost. No uniform methodology exists for determining value, although the Institute for Clinical and Economic Review (ICER) establishes what it calls a "value-based price benchmark" for drugs, which is based on its analyses of clinical and economic value and used by many payers as a guide.

One financing model builds on outcomes-based contracts to inform decisions about what products are used, by whom, at what price, and with what therapeutic goal. Already in limited use, they represent a move from the standard of simply paying a set price for the amount of medicine purchased. The outcomes measured differ markedly across plans and therapies, as reflected in the use of criteria that include reduced hospitalizations or mortality, period of remission, patient-identified benefits, or validated surrogate markers. From a patient's perspective, length of survival and quality of life may be the outcome indicators of greatest interest, but other factors tailored to the distinctive qualities of the therapy being assessed and the stakeholders involved can also be relevant.

> "When people say value-based payment, what does that mean? Value for whom? Defined by whom? Over what timeframe and in what context? How do we define it and how do we get there?"
>
> – Margaret Hamburg, former FDA Commissioner

Creating a payment system built on outcomes is complicated by the recent vintage of Advanced Therapies, their many clinical unknowns, and the relatively small patient population in which they can be administered. The high price of Advanced Therapies raises the stakes here, especially given the lack of data on durability. While the language of "cure" is used in many discussions, including in this paper, their true utility has in most cases not yet been established. The reason is simple: these products have not been around long enough to measure the long-term durability and risks associated with their use, and there have been too few eligible patients to take their full measure.

Moreover, the relative value of a particular outcome, and how it should influence price, is subject to debate. For example, in certain situations, the developer of CAR-T therapy for

children with leukemia may receive or retain payment only if the patient responds to treatment within 30 days;[9] correlation between that early metric of success and long-term survival is still being established. The promise and limitation of that outcome hint at the intricacies of deciding what to measure and how much weight to assign each metric. Likewise, the perceived benefit of Luxturna, a genetic therapy to treat blindness, is highly personalized, given that the level of benefit will vary by individual; in some cases the therapy restores the ability to see grays, or to make out shapes, a potentially transformative improvement to many patients previously living in a world of total blackness. A drug under development to curb progression of amyotrophic lateral sclerosis (ALS) would likely extend the lives of patients with already significant and costly comorbidities. Measuring not only the value of the benefits to patients, but also the other health care costs that these treatments can offset, is another example of the complications inherent in attempting to ascribe objective standards of value.

Ultimately, it may not be practical to fully calculate the package of downstream benefits and costs as part of a value determination, but more rigorous tools for collecting and using post-marketing patient data would nonetheless add a layer of accountability to these assessments.

As Advanced Therapies evolve from development to approval and marketing, a series of questions will need to be considered. How does value pricing impact a company's pricing considerations or its ability to attract venture capital? How do shifts in patient populations or treatment response influence value pricing over time? How sustainable is an initial price if the target population grows? A well-developed framework does not yet exist for collecting and assessing the evidence that can lead to the answers. In addition, broader concerns have been raised about how such reimbursement questions might, over time, foster or stymie investments, innovation, and new product development.

"You want the initial value to be somehow negotiated appropriately and you want it to be confirmed with real-world evidence over time and adjusted."

–Mark Trusheim, MIT NEWDIGS

"The outcomes conversation has to include the voice of patients and caregivers, making sure that at least part of the definition of outcomes is linked to data and information and insights about what's important to them."

–Alan Balch, National Patient Advocate Foundation

SHARING RISK

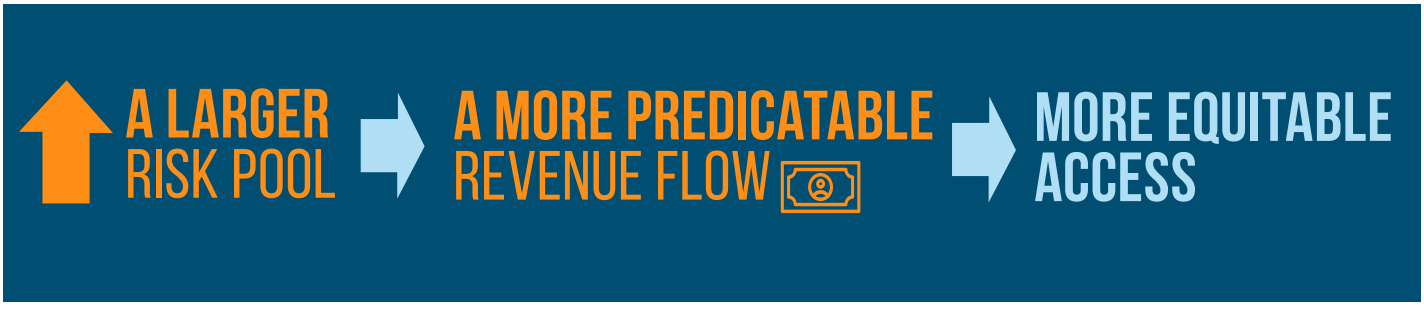
As equations to determine value are developed, patients and their caregivers need a platform from which to provide information and insight about what they think matters most. At present, no such infrastructure exists to integrate patient-reported outcomes and perspectives into the decision-making of product developers, the FDA, or public and private payers.

The appeal of a social construct to spread risk and share benefits surfaces when the unique circumstances of expensive science, a limited patient population, and an enduring monopoly combine to generate significant direct costs from a treatment. Just how many products will need alternative payment models is uncertain, but growing numbers underscore the importance of establishing criteria for employing alternative financing mechanisms.

> "The risk pool is a mechanism that allows private and public stakeholders to go all in together for the greater good."
>
> –Diana Han, GE Appliances

Meeting participants did not contemplate a large-scale overhaul of the current reimbursement system in place for the great majority of approved therapeutics. Indeed, care needs to be taken not to disrupt parts of the market that already maintain a reasonable balance between providing incentives that promote bold investments and preserving access to the results. These conventional models can generate data to inform innovations in Advanced Therapies and help shape policies to accommodate these novel products. Complementary financing, most likely some form of risk pooling or a reinsurance mechanism such as stop-loss coverage, can then be woven into the system to share the cost of Advanced Therapies. Where such a paradigm is appropriate, broader stakeholder agreement and collaboration would, in essence, substitute for market determination of price and associated decisions related to outcome measures, access strategies, and reimbursement.

A pooled funding stream would likely include a mix of public and private contributions. Notably, the larger the shared risk pool, the more predictable the revenue flow and the easier it would be to generate equitable access. However, both product developers and payers stand to lose agency in any such structure because pricing oversight in some form is a necessary companion to risk pooling. By definition that requires all parties to relinquish some autonomy.



Pricing determinations under this type of funding vehicle should include consideration of budget impact, such as shifts in funding to health care from other sectors and premium increases. They could also be based on inputs that include health technology assessments or other calculations of a therapy's expected health benefits, product labelling, size of the indicated population, related treatment costs, return-on-investment measures, direct and indirect costs of development and manufacturing, and other benefits, such as reduced health care costs. By giving up their ability to determine price unilaterally or in the context of bilateral negotiations, product developers could gain secure funding, rapid entry into the marketplace, increased speed of uptake, reduced marketing and sales costs, and greater predictability.

Payers, in turn, would be bound to provide coverage at the determined price in exchange for protection from excessive costs.

When participating in such a risk-sharing arrangement, drug developers might be expected to clarify the basis of their proposed price. In addition to the timely completion of FDA-required confirmatory clinical trials, they would be expected to contribute to long-term surveillance and other data-collection efforts so that real-world evidence is available to validate predicted clinical outcomes. As strategies to pay for Advanced Therapies continue to evolve, capturing and sharing data and learnings are foundational to assessment, adaptation, and replication. Resources will be needed to create patient registries and other infrastructure that improve the flow of information among prescribers, hospitals, clinics, and payers, enabling ongoing monitoring, analysis, and reporting.

> "Anything we do to try to pool risk and offload some of the costs onto society is going to have to go hand in hand with scrutiny as to how these things are priced. I think that tradeoff is inevitable."
>
> –Scott Gottlieb, former FDA Commissioner

Given the interdependence of the health care ecosystem, the development of cost-sharing mechanisms will require a governance structure, guided by appropriate private and public policies and processes. Although the possibility exists that capping risk-adjusted returns could discourage corporate investments in therapeutic innovations, policy or political uncertainty about whether a product will be covered at all could be an equal or greater threat to investment and innovation. Fashioning a system that creates more predictability in payer coverage decisions would

likely be an inducement to developer participation. Further policy changes may be needed to address the Medicaid best-price issue.

Other core elements also require consideration. Although market-based reinsurance mechanisms are well-established, refinements are necessary if they are broadened to include multiple payers. Decisions likewise need to be made to refine the structure of high-risk pools, including how contributions and benefits are allocated, the sources of public money, if any, that go into them, the nature of incentives most likely to motivate broader participation, and the extent to which any approach should be voluntary or mandatory.

Numerous other factors complicate risk-sharing structures, such as group purchasing practices. Any strategy to pool risk among multiple payers could encourage discounts based on the number of lives covered by the group purchasing structure or generate resistance by the product developer if it is perceived as failing to offer a benefit consummate with the expense. And, if the price of Advanced Therapies continues to escalate at the same time more of these treatments come online, the costs to each payer could potentially become unsustainable under almost any scenario.

Additionally, the many ways in which Americans obtain their health insurance means they move across different payment settings over time. The result is that the payer who initially covered a costly therapy (e.g., a commercial insurer) may not be the one to gain from later savings (which could accrue, for example, to a government program such as Medicaid). If an Advanced Therapy offsets costs to the health care system as a whole, some kind of mechanism could be considered in the short term that would accrue to the benefit of the initial payer. Over time, as more such therapies are approved, risk-pooling arrangements that aggregate resources and redistribute them could protect most parties from that kind of adverse exposure.

CONCLUDING THOUGHTS

Where breakthroughs lead to treatments or a cure for what had previously been a devastating condition, product developers may feel justified in charging a premium to compensate for the high-risk nature of discovery and development, the high manufacturing costs, or the value that they are delivering to patients with that condition. In the absence of competition, the question then becomes what role, if any, other stakeholders in the system should play to rationalize pricing as a way to help broaden patient access, support future innovation, and ensure a sustainable health system.

The answers are neither simple nor static. The systemic challenges posed by Advanced Therapies require nimble, collaborative thinking among many stakeholders. Scientific advances that are now at the cutting edge may ultimately be deployed more widely, allowing their costs to be distributed over larger populations; development and manufacturing costs may be substantially lowered through new innovations; and the entry of more follow-on products could create additional price competition. These and other circumstances may minimize or eliminate the need to consider a socially constructed solution for a given product. While traditional, market-based financing will in many instances continue to work, the entry of new therapies will pose fresh tests for a business-as-usual model.

Yet the group that gathered in Washington in early 2020 was cautious about abandoning mechanisms that have worked relatively well for a long time, produced breakthrough innovations that have improved patients' lives, and made the US a leader in life science innovation. As science advances, the limitations of current models become more apparent, but so too do the political, financial, ethical, and logistical complications of overhauling the system. The optimal alternatives are uncertain and imposing untested and disruptive strategies could make the cure worse than the condition it purports to correct.

In this dynamic environment, the time to plan for the future is now. We need to continue the conversation, pilot-test new financing mechanisms, and ensure that patients can benefit from the best that science and medicine have to offer in a system that is equitable and sustainable.

ENDNOTES

1 IPD Analytics CodeSource Database.

2 MIT NEWDIGS. (October 11, 2019). FoCUS White Paper. Survey results–Payer perspectives on financing and reimbursement of one-time high-cost durable therapies. http://newdigs.mit.edu/sites/default/files/MIT%20FoCUS%20Payer%20Perspectives%202019F210v044.pdf

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7 National Institutes of Health Genetic and Rare Diseases Information Center: FAQs about Rare Diseases. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases

8 Center for Medicare & Medicaid Services. (December 21, 2020). CMS issues final rule to empower states, manufacturers, and private payers to create new payment methods for innovative new therapies based on patient outcomes. https://www.cms.gov/newsroom/press-releases/cms-issues-final-rule-empower-states-manufacturers-and-private-payers-create-new-payment-methods

9 Novartis.com, Highlights of the Outcome-Based Agreement and List of Participating Treatment Centers. https://www.hcp.novartis.com/products/kymriah-oba/outcome-based-agreement/

APPENDIX: BREAKTHROUGH CURES, BLOCKBUSTER COSTS WORKING GROUP*

Co-Chairs

Scott Gottlieb, MD

Resident Fellow, American Enterprise Institute; partner, New Enterprise Associates; 23rd Commissioner of the US Food and Drug Administration (2017 – 2019)

Margaret Hamburg, MD

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Participants[10]

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Mark Trusheim, MSc

Strategic Director, MIT NEWDIGS

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Senior Fellow, Project Hope

* Participant affiliations were current at the time of the February 2020 meeting.

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