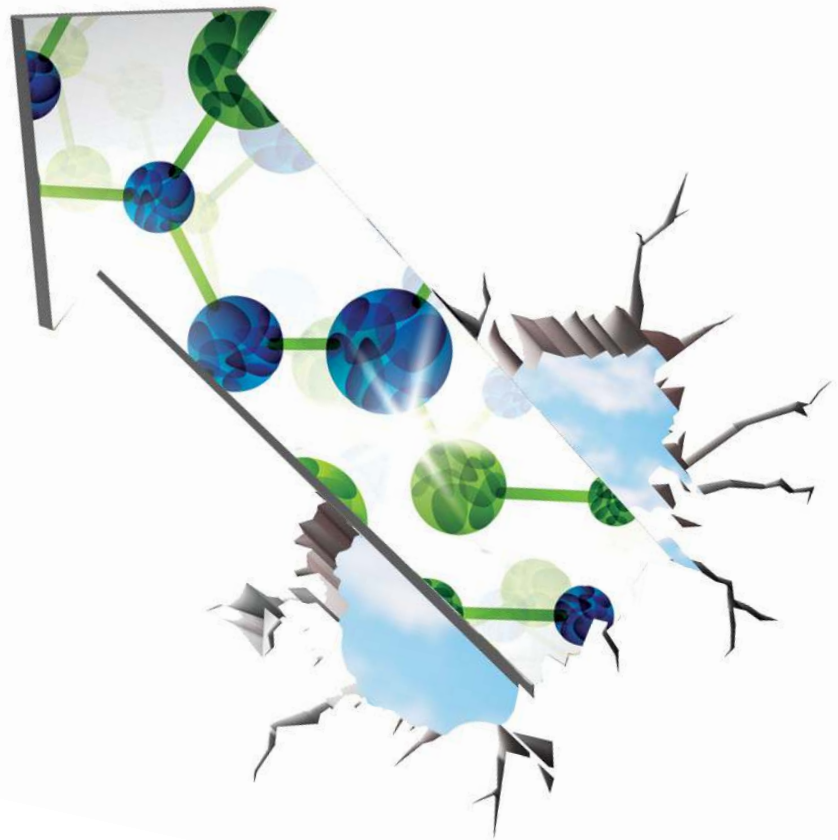


**MassBio<sup>®</sup>**

# ***Value of Health Series***

## **PART III**

***Potential Disruptors' Impact  
to Value, Access, and  
Reimbursement***



# Value of Health Series **Part III**



## *Potential Disruptors' Impact to Value, Access, and Reimbursement*

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# Potential Disruptors' Impact to Value, Access, and Reimbursement

## Introduction

**P**art I of MassBio's Value of Health series examines the value equation from the perspectives of the various healthcare stakeholders, the increased use of health technology assessments (HTAs) in the U.S. in determining value, the current state of innovative payment methods with private payers, and the importance of real-world evidence in demonstrating the long-term value of therapies. We looked at real-world case studies from companies who are leading the way in value-based agreements, and the increased use and impact of HTAs, like the Institute for Clinical and Economic Review (ICER), in determining the cost-effectiveness of new therapies.

While Part I focuses almost exclusively on how to best demonstrate value to private payers, the public insurance market in the U.S. is a critical piece to understand as it covers more than one-third of insured Americans. So, in Part II we explore this in detail, breaking down Medicare and Medicaid in the U.S., existing access and reimbursement issues for innovative therapies and cures, proposed changes to those programs, and barriers that exist to innovative payment models. We also considered those key international models of assessing value and determining access and what the U.S. may be able to learn from them. We found that successfully bringing new therapies and cures to patients, regard-

less of the market or country, shares a common theme: clearly demonstrating the drug's value to payers is critical to ensuring access and reasonable reimbursement. Public payers are no exception and while the need, methods, and opportunities to show that value differ, together Medicare, Medicaid, and international markets strive to provide the best value to the people they cover and the governments they work for.

This paper, Part III of the series, considers disruptions to the ever-evolving value, access, and reimbursement landscape as new players enter the arena and others consolidate. What impact will mergers between payers and pharmacy benefit managers (PBMs) and digital technologies have on drug pricing, patient access, delivery, and the ability to track and analyze data? What impact will one-time cures have on the system? What new financing methodologies are gaining traction and what else can we do to support their widespread adoption? This whitepaper attempts to answer those questions and more. But, because Part III is more forward-looking, we don't want you to take our word for it. So, we interviewed a variety of thought leaders in the healthcare sector, asking what they believe the value, access, and reimbursement landscape will look like in 5-10 years. ■



# Delivery

In the last few years, vertical integration through mergers and acquisitions among payers, PBMs, pharmacies, and even provider networks is disrupting the healthcare delivery model and the way we track patient data. CVS Health acquired Aetna in 2018, followed by Cigna's acquisition of Express Scripts a few months later. UnitedHealth created its own PBM in 1990 with OptumRx and most recently acquired DaVita Medical Group in an effort to expand the insurer's outpatient care services, in addition to several other smaller acquisitions of providers and pharmacies. Anthem, following on the trend of an integrated insurer/PBM model, announced that it will launch its own PBM, IngenioRx, in 2020. In nearly every announcement of the mergers and acquisitions, the promise was to better integrate care and lower costs. Some even acknowledged the ability to achieve greater cost savings as a combined entity by bringing pharmacy and hospital costs under one umbrella and gaining greater insight into the impact of prescription drugs on other healthcare costs. From the biopharma industry perspective, being able to measure costs avoided in the healthcare system from a prescription drug seems to be the most impactful way such mergers and acquisition could disrupt value, access, and reimbursement. Having this data could help validate the "value" of new therapies to the overall healthcare system, in addition to the health benefits accrued to an individual patient. On the flip side, critics also argue that bringing these entities under one umbrella could provide insurers with more clout to negotiate drug prices while also potentially steering patients to drugs that have larger rebates. Neither of these scenarios are yet to fully play out.

These mergers and acquisitions have also resulted in entirely new delivery systems for care. As a result of the CVS-Aetna merger, CVS is now rolling out Health-HUBs across the country in an effort to transform the consumer health experience and offer a mix of in-store healthcare services and digital and on-demand tools.

In a recent announcement on the expansion of Health-HUBs, Alan Lotvin, M.D., Chief Transformation Officer for CVS Health said, "Through physical and virtual interactions, we provide convenient, personalized and integrated access to health care support and services." Amazon is currently piloting a similar model for its employees in select cities, combining virtual care with in-person visits to the home or office, and prescribing and delivering prescription drugs right to consumers' homes. With its acquisition of PillPack in 2018, Amazon is now competing with the largest PBMs and pharmacies but going a step beyond by delivering prescriptions in convenient packets to remind patients which ones to take and when, helping to improve adherence. In both cases, there is incredible potential to track patient outcomes, and change the access and reimbursement model for prescriptions, along with the delivery of basic healthcare services. The potential is especially beneficial for patients with chronic conditions or comorbidities. ■

“As health care is constantly being disrupted, health systems are required to adapt and deliver services and products that fit their consumers' needs. The traditional model of ownership has now given way to systems that strategically partner with vendors/suppliers (think Uber). The recently announced mergers between CVS Health-Aetna, Optum-DaVita are case in point. Organizations that deliver integrated care at the right time, right place and right price are disrupting the industry today.”

—Lesley Solomon, Chief Innovation Officer,  
Dana-Farber Cancer Institute



# Technology

## Digital Health & Big Tech

**D**igital health and big tech players like Apple, Google, Microsoft, and Amazon are undoubtedly disrupting the value, access, and reimbursement landscape, providing consumers and providers with better tools to track patient adherence and measure outcomes. Apple provides apps to clinicians to allow them to access electronic health records (EHRs) and other patient data where and when they need it, also offering patients a means to better communicate with their medical team in a continuous fashion. Microsoft has teamed up with Walgreens Boots Alliance to develop solutions that lower healthcare costs. The partnership will combine Microsoft Azure, Microsoft's cloud and AI platform, with Walgreens' outpatient healthcare and retail footprint to compete with rivals like CVS Health.

While Amazon is certainly disrupting the delivery in healthcare, as explored above, it's also changing the way we track patient data and even diagnose disease. Amazon has a secret lab, what they're calling 1492, to push and pull patient data from legacy electronic medical record systems to make it more easily accessible for physicians and consumers. It's also exploring how to position Alexa for at-home medical diagnosis and care. Alexa can already give advice on some matters, like breastfeeding and first aid, but has potential for even greater impact around disease management for things like diabetes and is partnering with healthcare providers to build these skills. With over 100 million Prime users, Amazon already has massive amounts of consumer data, and by combining it with health data, can create a more holistic view of the patient experience, and therefore better track outcomes that impact patient care and access.

Google is studying the use of artificial intelligence (AI) to diagnose cancer, predict patient outcomes, and even prevent things like blindness. Its life sciences arm, Verily, has partnered with several biopharmas to create new efficiencies in the clinical trials space, reach patients in new ways, and aggregate data across a variety of sour-

es, including EHRs and wearables. And Google just announced a new partnership with Ascension, the U.S.'s second-largest health system covering around 50 million patients, to aggregate all the disparate datasets from electronic health records and other patient data. The project, dubbed "Project Nightingale," "appears to be the biggest effort yet by a Silicon Valley giant to gain a toehold in the health-care industry through the handling of patients' medical data," according to a recent Wall Street Journal article. Google said its goal is to improve outcomes and reduce costs for patients.

That seems to be the general tune among all of the tech giants, with various healthcare stakeholders leveraging the latest technologies to reach patients in a more targeted way and better track their outcomes. This will have indirect impacts on the value, access, and reimbursement landscape by potentially identifying waste in the system and making patient data more accessible and actionable. The latter will be critical for the shift to value-based care or value-based arrangements where reimbursement for a therapy is based on measurable patient outcomes. ■

“Where we see AI having the most impact is in predicting individual patient trajectory and optimizing interventions (both therapeutic and non-therapeutic) at any given point in time. The application of causal AI can be applied to uncover biomarkers of response, predictors of disease exacerbation and evaluation of efficacy for all therapeutics options available at the individual patient level. This leads to a reduction in administration of ineffective therapy and associated costs, while improving patient outcomes.”

— Iya Khalil, Ph.D., Chief Commercial Officer and Co-Founder, GNS Healthcare



## Impact of One-Time Cures & Personalized Medicine

When we think about technology, we're not just considering big tech and digital health tools, but also new innovations that promise to cure life-threatening disease. One of the most disruptive new technologies in this sense are gene and cell therapies that are administered as one-time cures. According to Evaluate, the gross sales of gene therapies is expected to reach more than \$16 billion in the U.S. by 2024. The first two gene therapies approved in the U.S. as one-time cures come with price tags of near or over \$1 million, both of which were agreed to be of good value by most economists and stakeholders, but still may present budgetary problems. This is in large part because the U.S. payer system is not set up to absorb the upfront cost of one-time cures, as payers operate on annual budget cycles, look at yearly snapshots of patient outcomes, and have to contend with portability issues, as patients stay with one payer on average for less than two years. There are several groups focused on solving the financing challenges for these one-time cures, as the current payer model undoubtedly needs to change, and also the portability issue, as patient bounce from plan to plan.

Related to cell and gene therapies, but more relevant to a wider swath of personalized medicines, is the impact of genetic tests, biomarkers, and predictive analytics to diagnose and treat illness. If a clinician knows with some degree of confidence that a specific therapy will work for someone with a particular genetic makeup and has the diagnostic tool to prove that to the payer,

then payers can feel confident they're reimbursing for an effective medication—meaning more access for patients. We'll also likely see variations in reimbursement for therapies that have different effects in different indications. If we move to a value-based reimbursement model for more therapies, the indication could also help determine the value, and therefore what price manufacturers can charge. ■

“ We've always known that drugs have different benefits in different patients, but we're finally at an era where we know much more about how patients with different indications respond to a drug. This creates a scenario where manufacturers can charge different prices depending on the indication and the patient, which can lower the price for less effective settings. This expands access because drugs would be reimbursed based only on the value and effectiveness they offer. It can increase innovation too, because of capturing more value in more effective settings. ”

— Amitabh Chandra, Professor of Business Administration and Public Policy, Harvard University



# Financing

**T**here are several academic organizations around the country that are piloting new solutions to pay for therapies and/or policy changes that need to take place to allow for more innovative payment models. Duke’s Margolis Center for Health Policy was established in 2016 with the mission to “improve health and the value of health care through practical, innovative, and evidence-based policy solutions.” Through a range of initiatives, the Margolis Center seeks to improve access and affordability for patients and support the shift to value-based health-care. It formed a Value-Based Payment Consortium to address the challenges involved with paying for value to better align policies that support wider adoption of value-based arrangements (VBAs). As part of this, it will address operational challenges associated with tracking patient outcome data. University of Michigan’s Institute for Healthcare Policy & Innovation has a similar focus, and is “committed to improving the quality, safety, equity, and affordability of healthcare services.” It’s evaluating Medicaid policy and healthcare reform, promoting greater value in healthcare, innovating in IT and health care delivery, and more, piloting such projects to inform state and national policy decisions and to demonstrate of the value of healthcare.

While there are undoubtedly other academic institutions that seek to solve the financing challenges associated with paying for breakthrough therapies, the Massachusetts Institute of Technology’s (MIT) NEW Drug Development ParadIGmS (NEWDIGS) takes a particularly collaborative approach. NEWDIGS is a “unique collaborative “think and do” tank focused on enhancing the capacity of the global biomedical innovation system to reliably and sustainably deliver new, better, affordable therapeutics to the right patients faster.” It touts its ability to bring together diverse stakeholders within a neutral setting, taking advantage of MIT’s expertise in systems engineering as a differentiator in enabling meaningful change. NEWDIGS has unveiled various pilots and projects, but one in particular seeks

to address the challenges around financing and reimbursement of cures, the FoCUS Project. Although their work is ongoing, the FoCUS research project analyzes precision financing solutions that include milestone-based contracts and performance-based annuity payments. Based on that research, FoCUS has determined that addressing the below themes would help make multi-year payment models a reality:

- *Determining payer/reimbursement metrics;*
- *Accounting for patient mobility issues;*
- *Organizing relationships and contracts to effectively address HIPAA, anti-trust challenges and anti-kickback laws;*
- *Obtaining exceptions from Medicaid best price rules.*

To support the implementation of a pilot to test new payment structures, FoCUS and its stakeholders have presented their research to policymakers, industry associations, and healthcare leaders. These efforts target the challenges of Medicaid best price and Average Selling Price (ASP) reporting—two of the major hurdles to widespread adoption of VBAs and payment-over-time models. The ultimate goal is to help facilitate fair contracting among payers, manufacturers, and providers as well as improve patient access to important therapies. ■



# Evaluation

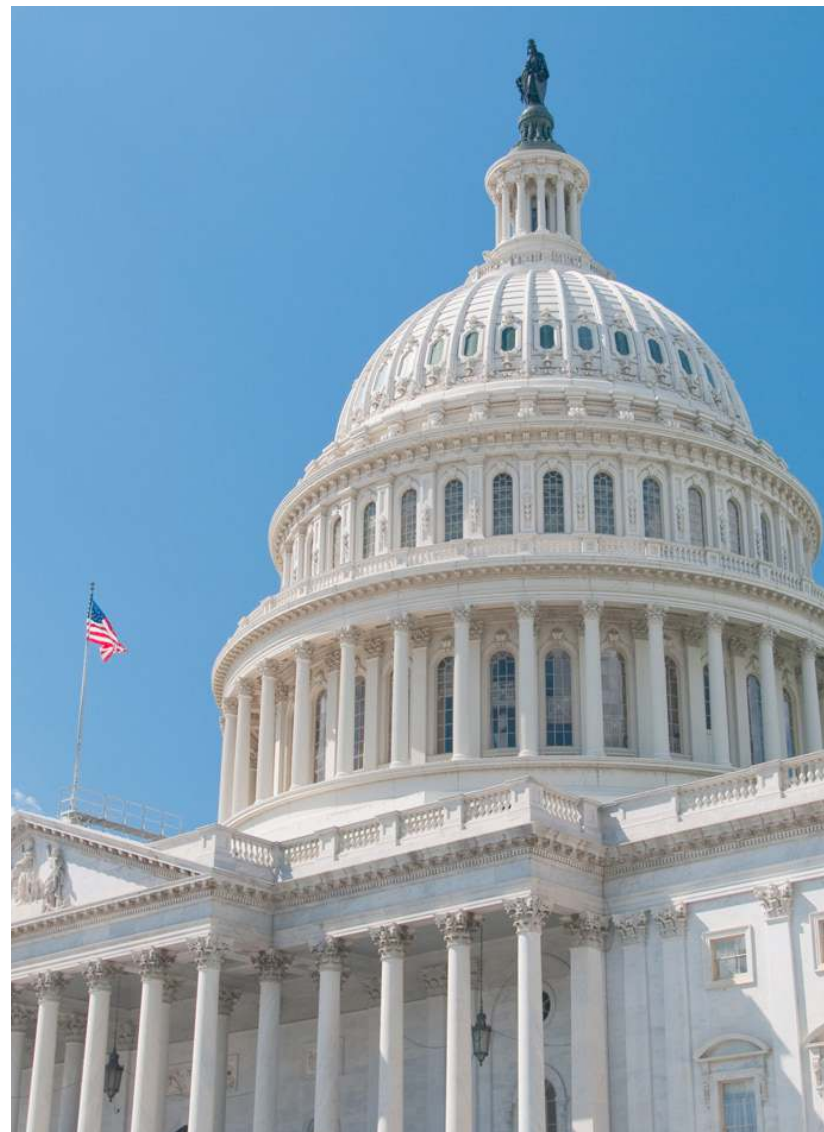
## Government Price Setting

**I**n the last year or two, state and federal legislators are increasingly willing to consider policies that would effectively let government set the price for certain prescription drugs. This is a marked shift and one we expect to remain for years to come. As we write in November 2019, the U.S. House of Representatives is on the cusp of passing legislation that would allow Medicare to directly negotiate the price of drugs with manufacturers, using the average price for the same drug in six foreign countries (Australia, Canada, France, Germany, Japan, and the United Kingdom) as the upper limit on the negotiated price. If manufacturers refuse to negotiate, they would be penalized up to a 95% tax on that drug's revenue. A "negotiation" where the upper limit is set and where severe penalties exist for those unwilling to negotiate is really just government price setting under a different name. Various states have passed or are seriously considering similar policies—legislation like setting an "upper price limit" on what payers can reimburse for a drug or giving a state agency authority to assess the value of a drug and seek to force manufacturers to lower the drug's price if it's found to be excessive or unreasonable. With growing agreement among both leading political parties that drug pricing must be addressed, a serious debate on this issue will continue regardless of which party is controlling government, in states and federally.

## Institute for Clinical and Economic Review (ICER)

When it comes to valuation and drug pricing decisions, the disruptive impact of ICER cannot be overlooked or underestimated. ICER's assessments of pharmaceutical drug prices, primarily using Quality Adjusted Life Years (QALYs) as a measurement of cost-effectiveness, appears to be making a real impact both to payers when they are making coverage and reimbursement decisions, but also to manufacturers when they are determining value and ultimately price. ICER's methodologies continue to evolve, however, as it recently

recognized that "single or short-term transformative therapies" must be reviewed in a different manner than chronic therapies. We expect ICER to continue to expand their influence in the coming years, as ICER's annual budget has more than tripled in the last five years, and the Arnold Foundation continues to provide a constant and growing source of funding. Although legitimate questions about whether QALYs are discriminatory, the transparency and inclusivity of ICER's review process, and the lack of competing entities like ICER to offer competing analysis remain, we must assume that ICER is here to stay. ■





# Industry Insights

**W**e asked a diverse range of thought leaders in the healthcare sector to answer the question, what will the value, access, and reimbursement landscape look like in five to 10 years? Here is what they said.

**Raolat Abdulai, M.D.,**  
*Clinical Research Director, Sanofi*

“In 10 years, the value of a drug is going to be based more on the whole patient—not just one endpoint. We’ll likely see more personalized therapies with companion diagnostics to ensure effectiveness and coverage. With these therapies, the future reimbursement model could look at complex endpoints, and include a digital tool to measure some other determinant of outcomes. Value will have to be seen in the entire health-care system, not just a 30-day reduction in re-admissions to the hospital but the overall system.”

**Amitabh Chandra,**  
*Professor of Business Administration and Public Policy, Harvard University*

“This future landscape very much depends on if the U.S. becomes a single payer system where the government is the sole payer. If this is our reality in 5-10 years, then the government will be the only negotiator with manufacturers and we’ll see a long-term decline in innovation because there won’t be competition—government will just be setting prices. If we remain in a world where manufacturers are negotiating with private health plans then we’ll see more protection of innovation because private health plans will lose enrollees if they limit their coverage too much.”

**Bob Darin,**  
*Senior Vice President & Chief Data Officer, CVS Health, Chief Analytics Officer at CVS Retail*

“CVS Health is making significant investments in data analytics and member-friendly technology to more efficiently connect patients to the right services and tools—from retail pharmacy to nurse care management to HealthHubs. Our innovations allow us to collect more

robust data to determine the best ways to improve care and lower costs. In the coming years, we will have access to more clinical data and outcomes data from real-world settings. This will enable a broader set of capabilities to further drive health care access and quality improvement, while ensuring reimbursement is tied to outcomes. More connected patient data will influence trial design and real-world evidence application, and impact how we structure value-based agreements with pharmaceutical manufacturers and providers. We’ll also see increased adoption of consumer tech and increased interoperability, which will help put us in a better position to work with biotechs and payer clients to really address value. Players like CVS will help pull together a complete picture of patient interaction—from the point of diagnosis through the care continuum to outcomes—enabling us to connect dots that have historically been difficult to connect.”

**Jay Desai,**  
*CEO & Co-Founder, PatientPing*

“Value-based care is going to continue to play a leading role across all of these areas. Now more than ever, providers are recognizing the need to deliver higher-quality, patient-centric care. Over the next five years we’ll continue to see value-based care initiatives and regulations expand and evolve.”

**Iya Khalil, Ph.D.,**  
*Chief Commercial Officer and Co-Founder, GNS Healthcare*

“It is clear for most that existing frameworks are not satisfactory enough to ensure unobstructed patient access to therapeutics while maintaining sustainable pharmacy spend budgets for employers, and government run programs. So called “value based” arrangements have begun to make a modest impact in a few therapy areas but are still not widely adopted. Absent of major government regulatory changes, we see pharma, providers, payors and employers working together towards more transparent models of assessing efficacy and paying for value. This means less reliance on re-



bates and greater use of technology and algorithms. Approaches adopted by ICER and NICE are a start, but they are blunt instruments in a world where personalized medicine is becoming more prevalent. Models built using causal AI that can both optimize treatment selection and predict response will provide the access patients and providers desire, while ensuring the budget stability and payment for value that payors and employers are demanding.”

**Leigh Anne Lease,**  
*Vice President and North America Head,  
 Public Policy, Novartis*

“In 2017, the government paid for over half of all drugs dispensed in the US. Changes under discussion in government programs, such as passing through of rebates and limits on pricing, will have a ripple effect into the commercial sector. These changes could limit access to newer drugs, which may provide fewer discounts.

“By 2025, the FDA expects to approve 10 to 20 new cell and gene therapies each year. Evaluating the benefit created by these transformative treatments, which sometimes cure, is a challenge we must meet head on. Only after appropriate determination of value that accumulates over a lifetime can we address appropriate reimbursement and payment mechanisms.

“Medicaid and Medicare can present barriers to the use of new payment models needed to appropriately reimburse transformative therapies. Flexibility is needed to ensure patient access to novel therapies that can save costs over the long-run.”

**Chris Leibman,**  
*SVP of Value and Access, Biogen;  
 MassBio Board member*

“In 10 years, patient access to healthcare will directly be linked to the benefits anticipated from intervention and then proven through direct measurement of the patient over time. The reimbursement of healthcare will be driven by these measurements of value to ensure payment is linked to value-based delivery. This will require holistic healthcare collaborations which will:

- Improve incentives that reward data collection and consideration of evidence generation as a continuum, not limited to only data available at approval of new intervention;
- Transition from system where access is considered a hurdle or barrier to treatment availability to one where access is a conduit to demonstrating value and payment linked to this value;
- Evolve the system from focusing on the value of single-point interventions to one which is focused on bringing value through a total healthcare solution to a challenge.”

**Lizabeth Leveille,**  
*Associate Vice President and Head, Boston  
 Innovation Hub of BD&L Merck Research  
 Laboratories; MassBio Board member;  
 Member of MassBio Value of Health  
 Advisory Group*

“It is widely recognized that change is coming but it is important we gauge value and improve access without negatively impacting innovation. We are already witnessing the potential for digital technologies to positively disrupt healthcare allowing the capture and analysis of data that will improve efficiencies and enhance the patient experience.”

**Erin Mistry,**  
*Vice President of Market Access,  
 Intarcia Therapeutics; Member of  
 MassBio Value of Health Advisory Group*

“Sophisticated Providers that are part of robust healthcare systems and analytics will base clinical decision making on predictive analytics to determine the best treatment options for subsets of populations. We eventually want to understand how genetic variations influence individual responses to medications. Genetic tests for guiding treatment decisions are becoming increasingly available across diverse areas of medical care. These tests get more-effective drugs to patients earlier in their treatment and with fewer negative side effects, and some even reduce costs. Now, a physician



can select a treatment based on a patient's genetic profile that may not only minimize harmful side effects and guarantee a more successful result, but can be more cost-effective compared with a 'trial-and-error' approach to disease treatment.

"Could the above then enable more personalized health plans for subpopulations of patients based on their genetic variations. For example, one genotype could respond a certain way to a therapy and therefore may be served better by one medication vs another and could they have a different formulary option. This could pose potential ethical issues but perhaps a flexible formulary option?"

"The above could also drive conditional reimbursement—only products that are working as intended (or as during trials) will be reimbursed. Having a more defined patient response will be essential to proving the VALUE of the product. Increased collaboration will happen between manufacturers and providers and also payers to then prove the value. These partnerships with large academic medical institutions and payers, those that are progressive and willing to take risks will engage more in two-sided risk agreements and additional financial schemes will arise.

"There will be an increased (already seeing this) expectation of real-world evidence (RWE) prior to approval not just through post marketing data or Phase IV studies. The likely result will be manufacturers engaging in more head to head clinical trials. Manufacturers, providers, health systems and payers will begin to take a more holistic view of a patient's healthcare utilization using both the medical and pharmacy claims data. This again will truly identify the value that a therapy brings not only around surrogate data points but around cost-avoidance or reduction in co-morbidities using longitudinal data.

"Most importantly: As we move into a healthcare landscape increasingly customized for some patients or patient subgroups—both in treatment and service—we must also make sure that we're expanding access so

that more patients and providers have the option and therefore can benefit from a therapy's advantages."

**Peter Neumann, Director, Center for the Evaluation of Value and Risk in Health, Tufts Medical Center; Member of MassBio Value of Health Advisory Group**

"In the next five to 10 years, I think we can expect to see much more engagement of public and private payers around value assessment for new drugs, along with more experimentation with outcomes-based agreements."

**Joy Russell, Vice President, Genentech**

"As long-awaited breakthrough medicines and even potential cures have become available, our reimbursement systems have not been able to keep pace with the rate of discovery. We must be just as innovative in the way we think about the reimbursement of medicines as we do about the science. Of note, we must ensure a modernized reimbursement system that ensures both access to life-saving medicines for patients and the innovation ecosystem.

"Many stakeholders in industry, including Genentech, are experimenting with ways to balance these two priorities, access and innovation. We have piloted several value-based agreements, aligned internal processes to accelerate our R&D timelines, and have engaged constructively with policymakers on ways to effectively modernize our reimbursement system.

"However, to effectively transition from pilots to large-scale structural changes, partnership and collaboration are critical, especially as the healthcare landscape is becoming more complex.

"Stakeholders—manufacturers, insurers, providers, patients, technology developers, data aggregators, and policy makers, just to name a few—must come together to find a way to modernize our healthcare system to ensure better outcomes for patients at a lower cost to the system."



**Lesley Solomon,**  
*SVP Innovation, Chief Innovation Officer, and*  
**Lalitha Ramachandran, Project Manager,**  
*Business Initiatives and Alliances,*  
**Dana-Farber Cancer Institute**

“Providers are slow to catch up to the move to value-based care. Over 75% of a provider’s revenue still comes from fee-for-service reimbursement. Government payers will continue to implement new regulatory reforms to drive value-based care among providers. However, market forces, increasing mergers & acquisitions, and rise of value-based reimbursement models will force providers to adopt value-based care. There will be a continued rise in community-based care, market consolidations, and alternate payment models. Providers that utilize data analytics to track patient utilization and outcomes will rise to the top. The use of big data analytics to track patient utilization and outcomes will continue to drive value in the industry.”

**Mark Trusheim,**  
*Strategic Partner, NEWDIGS,*  
**FoCUS Project – Paying for Cures**

“The FoCUS consortium includes patients, payers, providers, policy makers, developers and academics jointly working to address the challenges of durable, potentially curative, therapies. While these therapies share issues such as concentrated upfront costs for long term benefits and uncertainties regarding their durability and universal effectiveness, the conditions they treat and the technologies they employ differ significantly. Similarly, U.S. payer segments will likely remain varied regarding the populations they serve, the sources of their funding and the regulations they must follow. FoCUS envisions a continued fragmented landscape in which multiple precision financing solutions can be tailored to meet the distinct needs of each patient and payer in ways that work for providers and developers and comply with policy. We envision a core of precision financing solutions such as short-term and multi-year performance contracts; as well as carve-out risk pools such as “orphan reinsurer and benefit managers”. Through innovative reimbursement patients can receive appropriate access while the other stakehold-

ers receive appropriate value for their roles. Achieving this outcome will require continued adaptation of our data systems, regulatory environment and operational capabilities.”

**Anna Turetsky,**  
*Principal, Venture Investments, The Mark*  
**Foundation for Cancer Research; Member of**  
**MassBio Value of Health Advisory Group**

“Disruptive biotechnological advancements, including the first approvals of one-time, curative treatments such as cell and gene therapies, have forced a reckoning in how VCs are thinking about reimbursement for drugs and biologics. FDA clearance can no longer be thought of as the final existential hurdle for a new drug. Payors are becoming more proactive and empowered, with the help of independent third parties like ICER. Other forces include a political push to expand Medicare and government-led price negotiation, a backlash against middlemen like PBMs, telemedicine, overall fear of rising costs, and new pharmacy entrants. By five years from now, it’s likely that today’s largely intellectual emphasis on intricately linking reimbursement to value will start to become a reality. This will, in turn, fuel the value of real-world data and prediction models, and precision medicine in areas beyond oncology. It will also put additional burden on a new generation of providers to keep up: these things are not easy to measure and physicians have traditionally not taken price into account. I’m optimistic that over the next 10 years the general theme will be that innovation in a variety of realms will be rewarded: innovation to prevent, diagnose and treat disease; innovation in data collection, digital tools, and how we find appropriate care; and innovation in payment structures to ensure access—for the right patients at the right time—to state-of-the-art diagnostics and therapeutics. It will be challenging for the innovations that control total costs to keep up with innovations that increase cost, and it remains to be seen whether the more successful push is from public policy or private players.”



**Robert Urban,**  
*Former/Retired Global Head, Johnson & Johnson Innovation, LLC; Member of MassBio Value of Health Advisory Group*

“Within five years, the emerging market leaders will have coupled transformative products to innovative contracting. They will be the few who have looked well past delivering what is required for “approval” to also robustly invest in what is required to “win in commercialization.” To capture market share through risk sharing with validated tools that demonstrate the value they have delivered.”

**Jim Clement and Harry Vargo,**  
*Partners, Coeus Consulting Group*

“Keeping in mind the regulatory environment could change drastically depending on who takes (or stays) in office and controls the house and senate we believe the following areas are what the future holds for the reimbursement landscape in the coming years.

- Access to innovative therapies and those newer to market may be dependent upon greater risk being transferred to the patient under both successful and negative treatment outcomes. Patient’s may be forced to weigh their financial resources against probabilities to cure or side-effect profiles. As a result, health care financing could become similar to student-loans. However, we feel that alternative payment models will come to market to address the financial risk in the form of mechanisms such as market backed securities, etc.
- Outcomes-based payment methodologies will proliferate through all channels within healthcare as payers seek to only reimburse therapies when there is a proven clinical and durable benefit.
- Budgetary strain—driven by highly personalized medicine and cellular and genetic therapies, will make reimbursement a highly dynamic event and dependent upon additional delivery system variables such as provider decision-making, diagnostic choice/selection, and percentage of pathway adherence.

- Gene & cell therapies will continue to grow as AI and other information technology platforms allow for rapid analysis of national and global episodes of patient subsets pushing their development along at a more rapid rate.”

**Colin Wight,**  
*CEO, GalbraithWight; Member of MassBio Value of Health Advisory Group*

“1. We will no longer pay for the medicines, but rather pay a license-for-access fee removing the chemical volume-based and patient-weight-based old fashioned payment methods. Methods for paying for medicines over time will eventually become the norm, as we gain the legal framework (doing away with Medicaid Best Price and 340B Drug Pricing Program laws) to enable these to become mainstream, together with appropriate data allowing the measurement of clinically meaningful outcomes over time.

“2. Early Scientific Advice with Payers will be the norm for all innovative therapies in development and be conducted in parallel with Regulatory Scientific Advice early in development, resulting in one single evidence development program which meets the needs of both. ICER will undertake formal Early Scientific Advice for the USA, working in collaboration with CADTH in Canada, NICE in England and a wider range of HTA agencies around the world, including China & Japan.

“3. Problems of data with patients changing plans will disappear, as data will be freely exchanged between all insurers and become a legal requirement, with good data security, so opening up the ability to both measure and pay for outcomes over sustained periods of time. As Elon Musk can land a rocket on a beer mat, insurers can work out how to exchange data securely—and by making it a legal requirement, they’d fix it tomorrow.

“4. ‘Cures’ will become the norm, not the exception for many diseases. As we develop better and better versions of cell and gene therapies, which deal with issues like loss of efficacy due to antigen escape, toxicity issues such as cytokine release syndrome (CRS),



and graft versus host disease for allogeneic forms, cell and gene therapies will be developed for a much wider range of conditions, (not just cancer), including diabetes and immunological disorders. Consequently, prices for these types of therapies will fall dramatically over time, as will the costs of treatment (such as the need for hospitalization), and time to administration, meaning much wider access to these regenerative therapies.

“5. Cancer will become, at worst, a chronic disease as we learn how to detect it very early via routine blood tests (such as the Grail test), enabled by much faster and cheaper genetic testing from companies such as Illumina, and develop cell therapies with greater efficacy and durability, alone or in combination with low-cost, patent-expired check-point inhibitors. This will cause issues within the healthcare system as more people live longer and have chronic conditions for longer. This will cause further downward pressure on the overall budget impact of health care and social care costs.”

**Terry Wilcox,**  
*Co-Founder & Executive Director, Patients Rising and Patients Rising NOW; Member of MassBio Value of Health Advisory Group*

“Right now, we are seeing the state of the industry being disrupted by better data and insights—this is sort of the evolution of “big data” from a few years back. We will see this shift from an emphasis on data aggregation to a renewed emphasis on privacy, data quality, transparency, and the question of who owns patient

data and who owns practice/physician data.

“Coming on the back of this, I think there will be major changes brought by digital data asset-backed financial instruments (e.g. complex reinsurance mechanisms or ability to invest in “outcomes funds” built on blockchain-based technologies) and a new realization that we will be able to more transparently follow not only spending, but how that spending translates into tangible health outcomes.

“Health spending per person is unlikely to decrease in the short term unless we invest heavily in the things that impact social determinants of health. We need to emphasize reduction of diabetes and obesity, for example, if we want to reduce disease burden, and thus spend, and also “free up” pressure around spending on rare disease. As another example, to reduce lung cancer spending, the only path that moves the needle is to get people to stop smoking and reduce air pollution, not cut off access to lung cancer drugs. These are often issues that are politically at different sides of the spectrum, which makes it difficult to have a unified voice - we need to emphasize prevention if we want to also be able to spend on innovation.

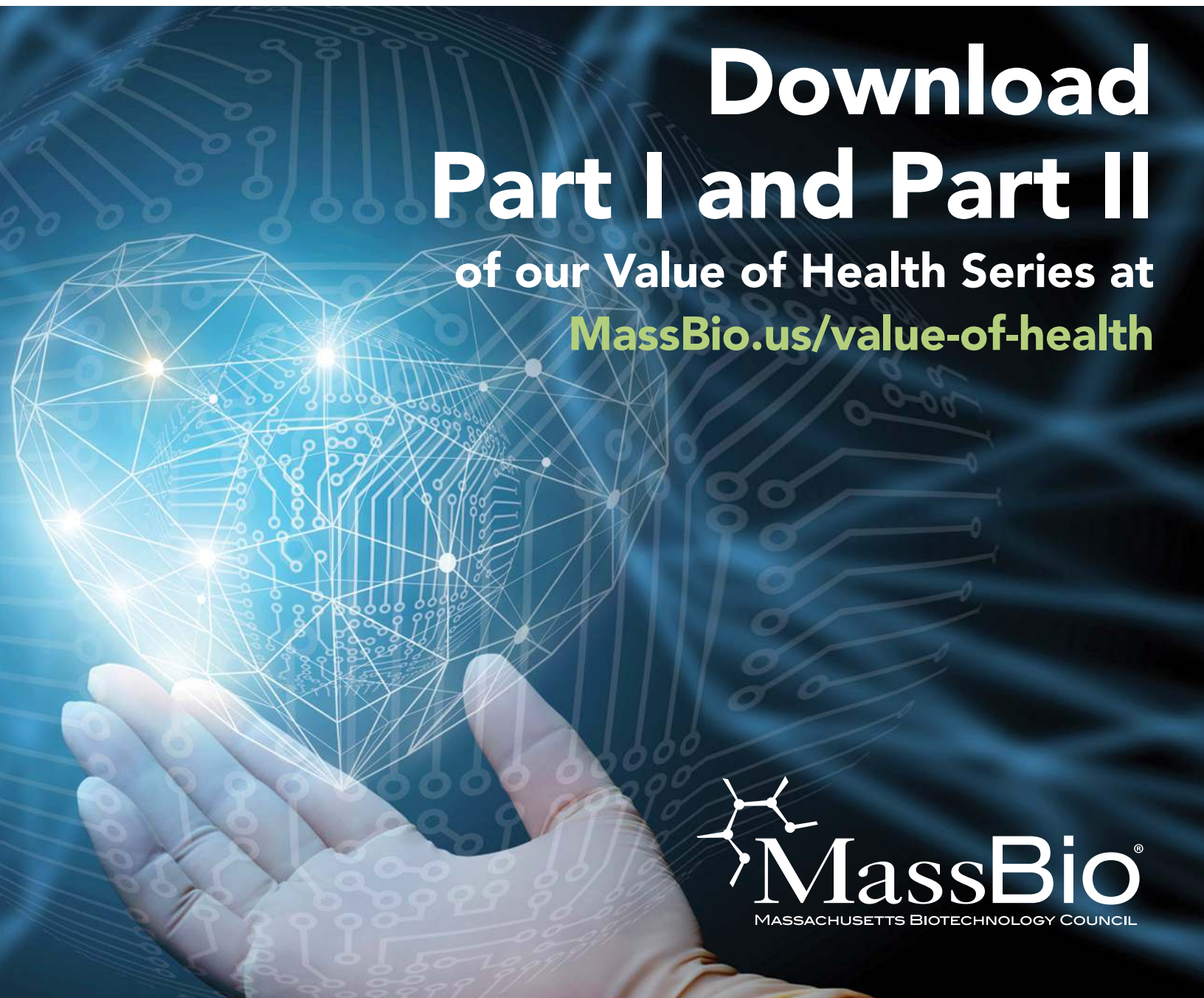
“The biggest risk to continued biotech investment and innovation is groups like ICER, in my view, particularly if they gain more of a political toehold.” ■



## Conclusion

**V**alue, access, and reimbursement for prescription drugs is a complex, ever-evolving landscape, as new players and technologies disrupt the status quo, offering both exciting advances for the ways in which we track patient outcomes to demonstrate value, and major challenges for access and reimbursement. It's up to the industry to continue to innovate not only on the scientific and technological

side, but also on the delivery, financing, and evaluation of therapies. The wave of incoming therapies and technologies are only impactful if patients have access, and that means addressing the value equation early in the development process and in a collaborative manner, considering the disparate needs of various stakeholders. The future for patients is brighter than ever, and it's up to us as an industry to keep it that way. ■



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# Appendix

## **MassBio Value of Health Advisory Group**

**Pat Cerundolo**, *Partner, Foley Hoag*

**Lizabeth Leveille**, *Associate Vice President and Head, Boston Innovation Hub of BD&L, Merck Research Laboratories*

**Erin Mistry**, *Vice President of Market Access, Intarcia Therapeutics*

**Peter Neumann**, *Director, Center for the Evaluation of Value and Risk in Health, Tufts Medical Center*

**Clark Paramore**, *Head of Value Demonstration, bluebird bio*

**Anna Turetsky**, *Principal, Venture Investments, The Mark Foundation for Cancer Research*

**Robert Urban**, *Former/Retired Global Head, Johnson & Johnson Innovation, LLC*

**Colin Wight**, *CEO, GalbraithWight*

**Terry Wilcox**, *Co-Founder & Executive Director, Patients Rising and Patients Rising NOW*

## **MassBio Authors**

**Susan Martin**, *Director of Government Affairs, MassBio*

**Jennifer Nason**, *Director of Communications, MassBio*

**Zach Stanley**, *Vice President of Public Affairs, MassBio*

**John Tagliamonte**, *Entrepreneur in Residence, MassBio*

