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# *Transforming Healthcare Data to Precision Medicine*

- Data and Outcomes
- Coordinated Healthcare Solutions
- RWD and Outcomes-Based Pricing
- Importance of Care Coordination

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# Turning Healthcare Data into Better Patient Outcomes

*Experts from around the world explore creative solutions—from improving the quality of medical information to working with patient advocacy groups—that enhance healthcare ecosystems.*

A modern healthcare ecosystem involves many participants—from physicians and pharmaceutical scientists to payers and patients—and many forms of complex data. Through collaborations and a range of advanced approaches, patients around the world will benefit from more uniform and complete healthcare, which will address acute and chronic conditions.

To explore ongoing advances in solutions that promise better patient outcomes, *Pharmaceutical Executive*—in collaboration with Conga and Salesforce—arranged a virtual roundtable of experts to discuss challenges and solutions. Conga and Salesforce concentrate on patient-centric digital solutions that improve the overall workflow of healthcare companies.

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## DEALING WITH THE DATA

To improve outcomes for patients, healthcare companies seek more efficient access to holistic data. It's an ongoing challenge as new technology and techniques enter the field. "When it comes to data to help improve patient-outcome initiatives, one of the top-of-mind concerns that comes up within our organization—and for me as a leader—is really the quality of the data and ensuring that we have the best quality data so we can get the best insights to drive that outcomes-based view that we're looking for," said Shetal Vyas, Vice President, Operations at Ferring Pharmaceuticals. "Although we have democratization of data in healthcare at this point, there are still areas where we see gaps."

Integration of data creates a continuing challenge. "There are definitely platform- and system-integration challenges that need to be overcome," said Julie Fargnoli, Vice President of Market Access and Patient Services, Leo Pharma US. She'd like to see seamless connectivity between, for example, devices and databases.

As companies strive for developing connectivity between the best healthcare data, the analysis could also improve. "We need to be able to integrate and apply a variety of approaches, including artificial intelligence (AI) and machine learning, to complex data. The use of algorithms is becoming a compulsory practice, rather a nice to have," explained Hisham Hamadeh, Vice President, Global Head of Data Science for Genmab.

## TREATING DATA FAIR-LY

Integrating data from healthcare providers and patients creates a complicated task. "The data that we receive provides us wonderful insights that hopefully help us better understand patient needs and how to potentially market our products, but does it help us drive patient outcome?" Fargnoli asked. To improve those outcomes, she pointed out the promise of electronic medical records, but she noted the complexity of using them, especially in the US. "We end up with a bunch of different data formats that doesn't talk to each other," she said.

ON-DEMAND WEBINAR

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One way to improve that integration lies in the FAIR principles, which were published in 2016. This acronym arises from the objective of making data Findable, Accessible, Interoperable, and able to be Reused. "This not only applies to data within a given organization, but increasingly important from a macro lens since not any one organization will be able to generate all of the data they need," Hamadeh said. "We are all obligated to adhere to the FAIR practices, making sure we're doing this in a very responsible manner across different organizations, and lining up incentivization models to make it a reality."

Although Boeing agreed on following the FAIR principles, he noted some key questions. "How do you look at the required information technology dimension across enterprise boundaries? For example, when it comes to federated learning?" he asked. "How do you incentivize FAIR data behavior across boundaries, invest in education of shared data assets and other dimensions of distributed data partnerships?" Answering such questions depends on teamwork. "FAIR needs enterprise industry vertical groups, but also requires governmental support to fuel our future quality data," Boeing explained.

## PROGRESS FROM PARTNERSHIPS

Effectively integrating and making use of healthcare data requires multi-stakeholder partnerships. "We do believe in creating an innovation ecosystem," said Hamadeh. To do that, Hamadeh and his colleagues have developed partnerships with other biotechnology, data-science, and pharmaceutical companies, as well as academic institutions. As he said, "We need to be open to multiple types of partnerships, and for those to be successful, we always strive for alignment of goals, alignment regarding how fast we need to run, and, of course, harmonization of data exchange."

Setting common goals among the partners is crucial. "The value proposition for each stakeholder needs to be very clear for all who are involved," Vyas explained. "When you have that end goal in mind for each stakeholder and align those goals together, the outcomes—from our experience—have been the best outcomes we can get." She added that "transparency and communication around those goals has been an important and best practice for us."

> ***"When you have that end goal in mind for each stakeholder and align those goals together, the outcomes have been the best outcomes we can get."***
>
> -Shetal Vyas

The history of the pharmaceutical industry could play a key role in advancing common goals. "Pharma was always a partnering business," Boeing noted. As an example, he said, "When you go from early research into clinical development, if you have missing capabilities, if you need companion diagnostics, you see partnerships between established companies, but also new startups that have digital biomarkers that are partnering with pharmaceutical companies to create more reliable and targeted evidence that allows for better stratification of patient groups or individualized treatment afterwards, depending on the data streams that have been captured." Based on that, he said, "I think the pharmaceutical industry is actually a great breeding ground for science-driven collaboration."

## EVOLVING TOWARD OUTCOME-BASED CONTRACTS

The healthcare industry is transitioning to contracts based on outcomes. For example, the cost of a drug is based on its positive impact on a patient. Nonetheless, defining an outcome varies, even in general ways.

For instance, Boeing described episodic scenarios—the treatment of one health problem. Here, the outcome can often be easily measured because it involves solving a one-time health issue and often being performed by a single healthcare provider organization. "When you then get into longitudinal care, the primary outcome is not just survival in a certain episode, rather, it is quality of life," Boeing said. "You look at other secondary cost effects." As an example, he mentioned that failure to manage Parkinson's disease could increase a patient's risk of falls, which impacts the outcome and cost.

To make the most impact on cost, Fargnoli noted opportunities in chronic conditions, such as diabetes and cardiovascular disease. "You've got large populations of patients and a multitude of outcomes," she said. "How do you measure those outcomes over such a long period of time, particularly when patients are not necessarily under one health plan for more than a year at a time?" As a solution, she suggested integrating data across multiple stakeholders. "In order to really make a difference in bending the cost curve, you need scale, and you need changes in legislation that allow you to partner in the right ways," she explained. To her, that means finding solutions that provide incentives for everyone involved.

In addition, Fargnoli explained that weight-loss medications provide an opportunity to quickly measure outcomes. With existing medications, she said, "you're seeing significant weight loss in under a year, which can obviously result in significant healthcare benefits." However, she added that these medications can be expensive. Nonetheless, quick results can motivate patients to take these medications and physicians to keep prescribing the drugs. As a result, she said, "It could be a great way to bring these partnerships together, because of the aligned incentives and the timeframe for which you can measure outcomes."

Part of that partnership building, Hamadeh suggested, could involve patient advocacy groups. "They know patients," he said. "They lobby for patients." Consequently, he indicated that these groups could play a key role in solving data-integration challenges. As he put it, "They could be an integrator on many levels."

## PUSHING TOWARD PATIENT-CENTRIC CARE

Making healthcare more patient-centric depends on many factors. For one thing, Boeing brought up digital biomarkers. "They're easy to deploy and are not very costly," he said. "There's a common belief that digital biomarkers have a significant role

to play to enable personalized treatments, but in terms of penetration, in terms of medical relevance at scale, they're still far behind in terms of potential."

Some of the use of digital biomarkers could improve through using them as companion diagnostics for new drugs. To do that, said Hamadeh, "It cannot be an afterthought, but rather an important consideration that is incorporated early on in the creation of some of these medicines."

Beyond new drugs and digital biomarkers, though, patients also play a role in improving their care. Nonetheless, patients are not always as motivated as physicians would like, sometimes due to a lack of understanding. For outcomes-based systems to work, though, all stakeholders must be motivated. If the physicians and patients are motivated, Fargnoli said, then payers and pharmaceutical companies should be more inclined to work with them. "It should be easier to find a win-win," she explained.

To accelerate the pathway to better outcomes for patients, some of the experts mentioned digital therapeutics, which are software-based tools for treating a healthcare condition. "There is an emergence of new companies that are focused on digital therapeutics," Turcotte said. "I think pharmaceutical companies need to pay closer attention to how digital therapies can augment traditional ones."

## THE FUTURE FOCUS

In concluding remarks, the experts participating in this virtual roundtable suggested a variety of next steps. For example, Vyas said, "I think we're actually at a tipping point, and there are multiple things that are adding to this." First, she pointed out that the COVID-19 pandemic forced "all stakeholders across the healthcare ecosystem to deliver the best quality care in the fastest and most appropriate way, meeting the patient where the patient is versus bringing the patient into a system." Second, she said that the quality and interoperability of data are adding to the tipping point. Third, she mentioned that improvements in technology and infrastructure allow healthcare experts "to bring these large sets of data together, and the skillsets from an AI-machine learning standpoint have really amplified what we can do with these data sets and help build these smart systems." Last, she noted increasing attention to the role of patients in their own healthcare. As she said, "All of these things, combined, are going to help tip this to the next level."

***"How much more efficient would it be if a provider was able to have one patient portal and every single product could be integrated into that?"***

- Julie Fargnoli

Part of that next level, Fargnoli explained, could come from brand-agnostic healthcare apps. Although she mentioned existing apps for specific healthcare problems, she envisioned "one application across multiple different therapeutic areas or multiple different drugs within a therapeutic area." Currently, healthcare apps consist of company-specific ones and a collection of patient portals. So she asked: "How much more efficient would it be if a provider was able to have one patient portal and every single product could be integrated into that?"

Boeing also noted the importance of simplification. "If we want to scale, we need to also look at the downstream implications of complexity," he said. "Different partners have to work together, but not only once, multiple times and the back-office processes and systems have to be ready for that." To address that issue, Boeing explained, the industry needs "to think about back-office solutions that use AI and other technologies to make collaborations easier."

All of these future improvements will depend on partnerships. As Hamadeh concluded, "I think the challenge, especially with multi-stakeholder partnerships, is to stay focused on the end goal and work with the same urgency." ■

Visit www.pharmexec.com/creating-solutions to watch videos featuring roundtable participants as they talk more about Creating Solutions within Healthcare Ecosystems to Improve Patient Outcomes.


# *Multi-Stakeholder Partnerships and Patient-Centric Healthcare Solutions*

## *Digital healthcare and multi-stakeholder partnerships an accelerator for patient-centric healthcare solutions*

Q&A WITH:

**Werner Böing**
CEO & Co-founder,
evisory GmbH

**Q: Is partnering for healthcare and pharmaceutical companies a new trend?**
**A:** No, absolutely not. In fact, two-thirds of drugs in the pipeline were born outside BioPharma companies in 2021. And looking forward, this number will likely increase with the growing focus to address rare diseases.

**Q: What is changing, regarding importance and type of partnerships in healthcare?**
**A:** First and foremost, the internal research and development of new drugs within pharmaceutical companies is evolving and requiring partnerships at various levels—from collaborating with healthcare data and analytics partners to identify drug targets to partnering with MedTech and BioMarker players to

provide companion diagnostics to the well-established partnerships with CROs that perform the execution of clinical trials.

For diseases such as diabetes, there is a shift away from just treating patients with therapeutics. Rather, patients are now being equipped with more information and education about their disease and are being empowered with a combination of drugs, diagnostic devices, and adjacent health services. This requires partnership across the healthcare stakeholder landscape.

Digitalization is offering new opportunities to help providers deliver better care to patients. Telemedicine and the use of artificial intelligence can support difficult decision making and coordinate collaboration between various stakeholders to deliver an orchestrated solution of physical and virtual services.

***New business models are emerging, requiring a shift from a pure activity-based incentive system to a more outcomes-based incentive system. This requires all stakeholders involved to partner together to generate reliable and meaningful outcomes data.***

**Q: What examples have you seen of multi-stakeholder partnerships?**
**A:** Let's start with examples of partnerships of equals, to gain relevance and market power and generate efficiencies. An example is the partnership of multiple hospital groups combining their data and creating a larger data pool and adjacent services. We recently heard about two large pharmaceutical companies establishing a collaborative effort to pair cutting-edge digital methods to improve interpretation of tissue-based assays.

In addition, players along the healthcare value chain are teaming up to combine different capabilities and access to channels, markets, etcetera. It is common to see the partnering of a drug, diagnostics, digital platforms, and adjacent health services to provide a single solution hub for patients with chronic diseases like Asthma.

**Q: What has worked well, in terms of multi-stakeholder partnerships?**
**A:** Partnerships where all participants work at a scientific eyes-height level typically work well. At molecule level, partnerships for the licensing of a new molecule have proven successful for a very long time.

Collaboration between companion diagnostics and pharmaceutical companies has become a well-established partnering area. In fact, a partnership to deploy a new AI-based biomarker test for advanced bladder cancer in a clinical setting was just recently announced.

Partnerships between the research area of pharma and R&D of startups,

scale-ups and tech companies seems to generally work, given a similar mindset (i.e., entrepreneurial, risk-taking, deep domain knowledge, etc.). Collaboration seen during the COVID-19 pandemic in the space of vaccine development is one example. Another recent example is the announcement of the launch of a multi-year strategic collaboration to deploy a series of AI-powered software solutions to detect hidden cardiovascular conditions. A multi-year collaboration agreement to drive the use of AI-powered pathology in translation research and clinical trials was extended, further demonstrating the value of these types of partnerships.

**Q: In forming multi-stakeholder partnerships, what has not worked well?**
**A:** In general, startups find it difficult to partner with pharmaceutical companies. It is difficult to find and engage with the right part of "Big Pharma". Also, timeframes are very long for startups. Many times, they are too long given Venture Capitalists expectations.

Bringing different domain expertise together (i.e., tech players, data players, service providers, etc.) versus core life science experience seems rather difficult, in spite of progress seen. Pharmaceutical companies have significantly invested in their own expertise, connecting digital competences, to address that, but cross-functional capabilities remain hard to master within an enterprise, and especially across company borders.

***Large-scale partnerships to build a united health vision have failed given the huge size, complexity, different cultures, and consequent risks involved. In addition, hyped efforts regarding big tech players have failed because of overplaying the role of technology, among other reasons.***

**Q: What are some combinations of partnerships that you see that are either emerging or you would like to see emerge?**
**A:** This is a fast, emerging field. What I sense is that the first wave of digital healthcare partnering with a focus on getting access to pure digital and data technology is declining in importance. Pharmaceutical players aren't getting excited when a new healthcare platform player comes along.

Instead, partnering increases with players that have mastered the technology and scientific/medical application in a particular high value use case. Examples of that are capabilities to perform synthetic clinical trials and solutions for drug target identification. In fact, there was the announcement of the identification of multiple, new potential therapeutic targets for amyotrophic lateral sclerosis (ALS).

What I would like to see is the partnering of players and various business models to solve some very difficult and important healthcare issues. I'd like to see solutions

that strengthen preventive efforts, as well as increase access to healthcare and empower patients.

As a multi-industry veteran, myself, I have learned that it takes a village to solve complex problems. Healthcare is the biggest and one of the most complex targets you could choose. Hence, I hope that there will be a second wave of partnerships with nontraditional healthcare players, be it tech, consumer goods, etcetera. However, given the current economic climate, I am not so sure that this will happen soon. Therefore, I invest my own energy in supporting start-ups that want to create the initial energy for change.

**Q: We are seeing more requests for manufacturers to offer outcomes-based pricing. What factors are causing the underlying request to move to outcomes- or value-based healthcare?**
**A:** Today, the reality is that most healthcare systems still have incentive systems in place that are activity-based: "You do this, and I pay you." The beauty of activity-based systems is they are simple, and you can directly measure input and reward.

Changing something that is easy and successful, and that people are used to is one of the hardest change management efforts you can choose. Why governments, payers, and some providers add outcomes-based systems into the mix is a fair question.

Health, not treatment, is the essential outcome from medicine, as highlighted by Harvard's Michael E. Porter over 10 years ago. Providers should be paid on the basis of the patient and health system outcomes they achieve, rather than for the number of treatments they provide. This is a very intuitive ask, but it was an almost impossible task to implement without the progress with digitalization and data in healthcare over the last decade.

VIDEO RESOURCE

**Creating Solutions within Healthcare Ecosystems to Improve Patient Outcomes**

Given the challenging situation of rising healthcare costs and debates over drug pricing, outcomes-based pricing may be an effective means for rewarding providers for innovation. Effectively managing a devastating disease or even curing the disease can be measured, versus the current state of care, in spite of the complexity of the steps from diagnosis to treatment, to reaching the desired health outcomes. However, we still have a very long way to go, in terms of reaching value-based healthcare, and this is not limited to drugs.

**Q: Who are the various partners that must participate for outcomes-based pricing to be successful?**
**A:** All stakeholders in healthcare need to participate to create the much-needed

transparency and insights. Ultimately, this requires the establishment of trusted outcomes data at the national or geographic level.

**Q: What are the challenges associated with outcomes-based pricing?**
**A:** Optimizing the delivery of a desired patient outcome requires multiple interventions and services to come together. When they are delivered within an intervention episode and all services are covered by a single organization, like a hospital, a payer can agree to outcome prices. These are the easier cases, involving the payer and provider in a contractual relationship that is typically governed by some governmental policies.

BLOG

**How the Healthcare Industry is Evolving**

When the delivered intervention is happening over a longer period of time, however, or even in a chronical setting, the outcome needs to be measured longitudinally. This is far more complex, and requires clear outcomes data and multiple players to combine their capabilities into a solution. In the real world, the longer the course of treatment is the more likely it is that the patient changes payers and/or providers, which can add another level of complexity. The availability of validated and affordable biomarkers for ongoing patient monitoring can be a critical enabler for these types of scenarios. Digitalization and trusted data platforms can be critical components for outcomes-based pricing.

On a practical level, with the growing importance, complexity, and number of partnerships, we need to be able to effectively develop a legal framework for outcomes-based contracts and operationalize processes across the partnering companies through the entire contract lifetime. Conga, for example, provides a flexible framework that can be used to create an outcomes-based agreement that allows customers to design payer-specific plans that incorporate performance tiers, measurements, outcomes, payouts, etcetera. It is then the job of an emerging class of trusted platforms in various regions across the globe to calculate performance. However, without the growth of digitalization, outcomes-based models will not become successful.

**Q: Value-based healthcare takes the perspective of a patient. What other elements need to be reflected for a successful implementation of patient centricity?**
**A:** Given that healthcare is highly fragmented, at the provider, payer, and administration side, it is clearly not designed with an empowering patient journey in mind. To me, patient centric

means turning healthcare from a product centric world into a solution and service world that looks at the whole human, and not only his or her diagnosis.

We are seeing digital health companies evolve that enable patient centricity for patients with diseases like asthma and COPD. By combining connected devices with data and science, a consumer business-like design is established to focus on patient experiences, integrating many relevant dimensions beyond drug usage and adding adjacent services to help deliver better outcomes. It comes to establishing a partnership for which the ecosystem involves various players from Pharma, MedTech, hospitals, and Health Systems to make the patient experience as seamless as possible.

Another example I'd like to point out is that we underestimate the burden and role of caregiving in supporting patients, be it children, partners or parents, with chronic diseases like diabetes, asthma or Parkinson disease. They are charged with becoming informed and educated about the patient's disease, navigating/orchestrating parallel tasks, ensuring reimbursement of services, setting up appointments with HCPs and hospitals, ensuring treatment compliance, adjusting treatment plans according to HCP guidelines, etcetera. This patient and caregiver journey needs to be at the center of design briefs of healthcare solution companies.

***What I see is that healthcare players are aware and are adding less sophisticated (e.g., effective ways for patient-reported outcomes) but useful features to their products and are partnering with other adjacent providers (e.g., remote medical education) to enable a more integrated experience.***

**Q: What challenges impact the implementation of patient-centric healthcare?**

**A:** Patient-centric solutions can help navigate the complex healthcare system more effectively, but only to a certain point. Unless healthcare systems are being transformed more systematically, there is a limit, and patients' frustrations (e.g., long waiting times to have access to a HCP) will remain.

Behavioral interventions are essential to improve healthcare outcomes in many areas. However, the use of digital therapeutics is still limited. In order to have individualized, effective and affordable interventions for patients at scale, we need to find ways to add digital therapeutics into the mix. We need to move from sick care to healthcare. We have to start intervening when people are people (i.e., through prevention efforts) and not patients. Getting this solved is at the heart of designing patient-centric care—seeing us as humans and not a disease.

**Q: What are the successful outcomes?**
**A:** To me, when people are confident with a statement declaring they own and control their health we have reached the nirvana of patient-centric health. However, even I can't say that with confidence, living in one of the wealthiest countries in the world with access to top level healthcare providers, etcetera.

So, to me, it means that we are, individually, but healthcare players, as well, addressing these issues, one by one. We are taking chances and hoping that digitalization can accelerate healthcare. All people deserve better healthcare, and creating ecosystems and multi-stakeholder partnerships are essential keys to delivering better care and matching expectations and ambitions.


# *Outcomes-Based Pricing Using Real-World Data for Novel Cell and Gene Therapies*

*Mutually beneficial, outcomes-based agreements between manufacturers and payers promote adoption of expensive, novel therapeutics while delineating their value using real-world evidence.*

**FEATURING INSIGHTS BY:**

**Thomas Cowen**
Head of Vertical Strategy – Healthcare & Life Sciences, *Conga*

**Eric Kalabacos**
Associate Partner/Principal, *ZS Associates*

## INTRODUCTION

Recent advances in cell and gene therapy (CGT) are yielding life-changing treatments for patients with chronic or fatal illnesses, producing outcomes that were previously unattainable by traditional small molecule or protein-based drugs. Market growth is expected to be exponential, with over twenty CGTs currently approved by the United States Food and Drug Administration (FDA) in areas such as hemophilia, oncology, ophthalmology, and neurology (*1*), and hundreds more in clinical trials for these and other therapeutic areas (*2*). However,

these advanced therapies come with a high price tag: several cost $1 million or more for a single treatment (*3*). Payers therefore seek clear evidence of value to justify reimbursement costs for these products. Real-world data, including traditional and/or digital biomarkers, are key to demonstrating the value of these therapies for individual patients. Thomas Cowen, Head of Vertical Strategy, Healthcare & Life Sciences at revenue lifecycle management company Conga, and Eric Kalabacos, Associate Partner at consulting firm ZS Associates, recently discussed the current state of the CGT market and the role real-world data play in the development of outcome-based pricing contracts, which payers and manufacturers are increasingly negotiating to balance risk-benefit ratios and control healthcare costs.

## CELL AND GENE THERAPY AT A GLANCE

The goal of CGT is to address the underlying source of a disease by introducing a new or corrected gene, either to isolated cells *ex vivo* for infusion (cell-based therapy) or directly *in vivo* by drug injection or infusion (gene-based therapy). Where successful, some of these therapies could essentially represent a single-dose cure. These results are highly desirable clinically, compared to simply managing symptoms with ongoing drug therapy, and could transform health care for diseases in many therapeutic areas.

Gene-based therapies initially focused on disorders caused by mutations in a single gene, such as *F9* in hemophilia B. These rare, monogenetic disorders have the advantage of a clear genetic target and relatively well-defined clinical outcomes. Their status as rare or orphan diseases can accelerate clinical trial pathways and regulatory review. However, the patient populations are generally small, access to point of care may be limited, and long-term efficacy and safety of new drugs may not be as well-established.

BLOG

**COVID-19 and the Future of Health and Life Sciences**

Recent technological advances have enabled an evolving focus of CGT in additional indications, of which oncology is the most clinically advanced. Cell-based gene therapy can be used to increase the targeting and activity of immune cells against native tumor-expressed antigens. On the other hand, tumor cells can be induced via targeted gene-based therapy to express a gene which, for example, enhances sensitivity to conventional therapies or stimulates an anti-tumor immune response [reviewed in (*4*)]. According to Cowen, many of these treatments are being pioneered at major cancer centers, such as the MD Anderson, Sloan Kettering, and Dana-Farber Institutes, where patient population and access to point of care is less of a challenge. “It will be interesting to see whether the [same] outcomes are delivered at the secondary and

tertiary hospitals," Cowen said. Consistency of institutional quality of care is one potential challenge. However, oncological CGTs have also generally not been as widely successful to date as those in monogenetic diseases due to polygenicity and high variability between individuals or even between tumors in a single patient (*4*).

***"Obviously, a $1 million price tag for most Americans is a staggering and unapproachable price. So, the responsibility falls on the payer."***

-Thomas Cowen

## KEY CHALLENGES TO ADOPTION

Challenges such as patient population size, barriers to point of care, and uncertainty of long-term outcomes are inherent in marketing any drug. One key difference particular to CGTs relates to their up-front cost, with several priced between $1 million and $4 million at launch (*3*). "Obviously, a $1 million price tag for most Americans is a staggering and unapproachable price," said Cowen. "So, the responsibility falls on the payer." While total cost of therapy can be high for long-term treatment of diseases using traditional small molecule or protein-based drugs, the payments are spread out over time. However, many CGTs, including all gene-based therapies, are given as a single treatment with a one-time bill. Even for diseases where CGT can be curative, potentially saving millions of dollars over the patient's lifetime, the budgets of insurers or governments are significantly impacted by these very large, one-time payments (*5-6*), particularly when a drug is first approved. This could become a critical issue as these drug modalities expand into therapeutic areas with larger patient populations.

A second key challenge for CGTs is a lack of long-term efficacy and safety data in real-world clinical use to demonstrate the value of these therapies. The accelerated clinical trials and regulatory approval that are often possible for diseases with high unmet need can also leave many unanswered questions. Vectors or delivery mechanisms currently believed to be safe must continue to be monitored for unpredictable delayed adverse effects (*7*). Lastly, CGT treatment modalities will need far longer time in-use than presently available to observe whether their effects are stable over patient lifetimes or will eventually begin to wane. Payers and manufacturers alike would therefore benefit from a fuller understanding of the potential impacts of these newer drugs, which requires collection and sharing of clinical data.

"Patient outcomes and real-world data are key to being able to demonstrate value," confirmed Kalabacos. It is important to note that this data collection timeframe may last years beyond the point of treatment. One example is in oncology, where Cowen noted that no evidence of disease (NED) over a five-year period is generally considered a cure. "The challenge falls then on fitting a business model that can handle that long time frame," he added.

## CELL AND GENE THERAPY AND PRICING STRUCTURES

For payers, each of these CGTs will have a different case and benefit for coverage that may not initially align with patient demand, particularly since many of the current therapies address conditions that only exist in a small population. "We all know about hemophilia, but only 6,000 people have hemophilia B. So, it is incumbent then on these populations to lobby their payers for support," said Cowen. "Adoption, in many cases, may turn into a grassroots effort," with patient advocacy groups bringing these diseases to the forefront. Payers must begin considering how they will handle this new wave of therapies, he added, "to make sure they are responsive and at the same time judicious to the patients." This could include programs such as per-member fees for participating employers to cover these therapies, he indicated, which would extend patient access while easing budgetary disruptions for the payer. Such programs may become particularly relevant for payers in countries such as the United States that lack a single-payer system, where an insurer could cover an expensive CGT only to realize none of the subsequent cost savings when the now-healthier patient changes insurers. Meanwhile, Kalabacos stated that many pharmaceutical companies have begun developing capabilities around real-world data capture. "ZS has provided support to many pharmaceutical manufacturers in this area as they build out this critical capability," said Kalabacos. "Such capabilities can be leveraged both to improve clinical outcomes over time, but also as a key data source for outcomes-based agreements." Realizing that the current buy-and-bill structure is untenable for many CGTs, insurer and government payers are exploring alternative payment models (*5*,*8*).


## SHIFTING TO OUTCOMES-BASED HEALTHCARE

In the standard contracting model, "manufacturers provide rebates to payers in exchange for advantageous formulary position and the payer reimburses accordingly, no matter the actual health outcome for the patient," Kalabacos said. "In an outcomes-based model, the parties agree to use some form of outcomes-based data to determine if a desirable outcome is achieved for the patient, and reimbursement is based on [that] outcome." An outcomes-based agreement therefore distributes the risk of adopting new, expensive therapies between

payer and manufacturer (*5*), with the added benefit of accruing real-world evidence for both parties regarding the potential value of these novel drugs in clinical practice.

***"In an outcomes-based model, the parties agree to use some form of outcomes-based data to determine if a desirable outcome is achieved for the patient, and reimbursement is based on [that] outcome."***

-Eric Kalabacos

"There are three main forms of outcomes-based agreements," Kalabacos explained. Each offers a reduced price or rebate if certain predetermined outcomes are not met following treatment. In a Patient Warranty or Outcomes-Based Rebate agreement, the payer pays the full amount up front, and then if the therapy does not meet defined outcomes in a patient, the manufacturer reimburses the payer in part or in full. The rebate may be based on short-term as well as long-term measures of efficacy (*5*,*9*). In a Pay for Response or Outcomes-Based Payment agreement, the payer pays a partial payment up front, and the balance is due only if the therapy yields a specific outcome. Closely related to this category is an Outcomes-Based Annuity agreement, in which the payer pays a fixed price in installments, but only for as long as specified outcomes are met (*5*,*9*). For each of these types of outcomes-based agreements, "the task then falls to the pharmaceutical companies and the payers to determine what is the desired outcome and how it is measured in a way that protects both parties," said Cowen.

## BUILDING OUT AN EFFECTIVE OUTCOMES-BASED PROGRAM

An effective outcomes-based agreement must first clearly define what specific outcomes to track. "An explicit agreement between the manufacturer and the payer on what data will be used as evidence of outcomes for such agreements is critical," confirmed Kalabacos. This requires that manufacturers and payers agree on definitions of 'positive outcome' in complex diseases that may vary in patient presentation. "NED either is or it isn't," Cowen said. "Other outcomes may be tracked on a scale – take cystic fibrosis where lung capacity is a vital metric. So here we may have a range of lung capacity on a spirometer." Other surrogates for clinical outcome could include biomarkers or even passive monitoring by smartwatches or other digital health technologies (i.e., digital biomarkers) (*10*). "Sometimes, financial data may be used as a proxy for explicit clinical outcomes data," added Kalabacos. "Cost of subsequent treatment is an example."

Once the desired outcomes and relevant data have been defined, it is important to specify who is responsible for obtaining those data, and how. "The greatest challenge in the adoption of these alternative payment models has been the availability and costs of obtaining the evidential data to demonstrate outcomes," stated Kalabacos. "Both parties need to agree on the approach." Following

individual patients across different provider systems and payers can become particularly complicated in countries without centralized healthcare, such as the United States. "To date, there are only about 50 publicly disclosed outcomes-based agreements in the U.S., largely due to difficulties in obtaining the required data," Kalabacos added. Successful outcomes-based agreements may therefore require the involvement of third-party data aggregators as well.

Pharmaceutical companies should consider discussing outcomes-based agreements for promising new CGTs with payers as early as possible, to enable more rapid adoption of these therapies upon approval (*9*). Contract lifecycle management companies such as Conga, together with expert consultants like those at ZS Associates, can help companies develop the most appropriate framework, including metrics, descriptions, and payouts, to ease creation of these complex contracts. "Market Access teams can certainly be as creative in deal structuring as possible, but there need to be constraints to this. At Conga, we believe that we have the key components in our solutions that complement contract lifecycle management and allow our customers to develop these flexible frameworks and prevent creation of programs that can't be supported," Cowen explained. "Conga's focus is on structuring the program and bringing it to life in the contract. This involves a flexible configuration that can evolve with the programs through our Conga Grid technology. From there, dynamic contracts are assembled through our Contract Lifecycle Management solution that introduces the relevant clauses for that program." Use of this technology can then in turn increase contract visibility, processing, accuracy, and compliance. "The Conga Configure, Price, Quote (CPQ) solution can also be leveraged to streamline the internal review and approval of the complex pricing structures associated with value-based agreements," said Kalabacos.

RESOURCE

**Outcomes-Based Contracts in Healthcare: How To Automate Every Step**

## CONCLUSION

As more and more expensive, individualized treatments come to market, payers are increasingly looking for clear evidence of value to justify the high reimbursement costs for these products. To promote adoption and increase market access, some manufacturers are shifting towards mutually beneficial, outcomes-based agreements with payers, which balance the inherent financial risk of these novel therapeutics across multiple parties while demonstrating their value using real-world patient data to further delineate therapeutic safety and efficacy over time. Contract lifecycle management solutions and expert guidance can enable manufacturers to craft their outcomes-based programs as solidly and flexibly as

possible, increase efficiencies, and manage risk. These programs empower both payers and manufacturers, and grant patients with chronic and potentially fatal diseases increased access to these lifesaving and life-changing genetic therapies.

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# Care Coordination: An Important Asset to the Patient Experience

*Jennifer Turcotte, Senior Director of Pharma Strategy at Salesforce, identifies important areas for improvement in the collection and delivery of data and highlights how the information can be used to strengthen patient relationships and create personalized engagements that drive health outcomes.*

Q&A WITH:

**Jennifer Turcotte**
Senior Director, Pharma Advisory and Innovation, *Salesforce*

**Q: What is *personalized healthcare*? And how is digitalization impacting the way a patient's care is managed?**

**A:** I think the industry has been on a path to achieving personalized healthcare for more than a decade. And there's been some great progress made in just the last couple of years. To me, it's thinking about the whole patient throughout their care journey and accommodating their needs—whether it be financial, emotional, physical and more. It means ensuring that screening, diagnosis and even prevention of disease is more customized to an individual patient's needs. This also includes personalized medicine where a therapy is tailored to a specific patient or genotype. Together,

this is what truly holds the promise to transforming patients' lives more quickly and effectively.

**Q: From both the physician's and patient's perspectives, where do you see areas for improvement?**

**A:** Here's just one example: While most pharmaceutical companies offer patient support programs for their brand of therapy, patients are mostly unaware of their existence. Meanwhile, physicians might be aware of these programs but it's often difficult to enroll patients. There are various approaches being used to address these challenges. One is expanding enrollment channels to include text options or use of QR codes. In addition, some brands are using online forms to facilitate copay card reactivation and sign-ups. Brands need to offer multiple ways to sign up for and engage in these programs. Simple technology changes can go a long way in helping patients and physicians alike. For example, making it easier for patients to get both financial support and medication adherence support in a single interaction rather than through multiple channels.

CUSTOMER STORIES

**PatientPoint prescribes Conga to process contracts 9x to 10x faster**

*Simple technology changes can go a long way in helping patients and physicians alike. For example, making it easier for patients to get both financial support and medication adherence support in a single interaction rather than through multiple channels.*

**Q: In terms of data strategy, describe the rationale for developing a more holistic approach to accessing and managing patient data.**

**A:** Most healthcare companies, pharmaceutical companies included, are sitting on valuable internal patient data. Many also purchase third-party real-world patient data. This data provides a wealth of information that can only be properly mined and interpreted if connected together. For example, when a pharmaceutical company launches a new brand, companies should harness insights from previous trials run for that form of therapy to identify patient needs and mine patient-level data to determine how to more accurately target the correct cohort of patients. The right data exists, but the problem is it is siloed among different departments. Companies need to not only invest in capabilities and processes to consolidate and cleanse their data to put it to good use, but to ensure teams are connected to jointly use this data to benefit existing and future patients and their health outcomes as well.

**Q: How is Salesforce responding to challenges surrounding physician and patient engagement?**

**A:** Salesforce is continuing to invest in Health Cloud, our industry-specific platform that unites internal teams with their customers—physicians and patients—by providing a single, shared view of all customer data on an integrated platform. Employees can access the information they need to do their best work and, most importantly, provide the best experiences for their customers. That increases collaboration and alignment among all teams, leading to more connected, personalized experiences for customers. Physicians and patients can now receive the information they need, when they need it, and interact with you in the manner they prefer. Salesforce connects customer interactions across all groups—sales reps, med info and patient services—in a compliant way to better understand customer behavior and to continue to improve engagement and drive valuable insights. ■

**Jennifer Turcotte**
Senior Director,
Pharma Advisory and Innovation,
*Salesforce*

Jennifer brings over 20 years of experience in marketing and product strategy at life sciences and software technology companies. Before joining Salesforce, she served as Associate Director, Pharma Informatics, at Genentech. During her time at Genentech, Jennifer focused on strategic projects to build global solutions for scientific data management and data sharing. She also led a project to define the most patient-centric, ethical and legal approach to returning genomic data results to patients in trials. Prior to those roles, she was VP of Marketing at a variety of early-stage software companies and at an innovative genomics sequencing company. Jennifer holds a Bachelor of Engineering, Mechanical from Carleton University, Ottawa, Canada.