

# 21<sup>st</sup> Century Pharmaceutical Collaboration: *The Value Convergence*

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## **At a glance**

Personal health information is the new currency of drug development and commercialization. Novel collaborations are helping pharmaceutical and life sciences companies maximize the value of new medicines.



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# The heart of the matter

*As market forces push pharmaceutical and life sciences companies closer to patients, new technologies and greater access to consumer data are catalyzing collaboration throughout the sector. And with prices rising again, forging a consensus about drug value will trump traditional business concerns in the New Health Economy.*

## Executive Summary

In the past, organizations working in different pockets of the health sector made decisions according to their own business priorities. But as pharmaceutical and life sciences pipelines rebound, consumer choice and provider cost-savings incentives are driving change in the industry's commercial model. As US specialty drug spending hits an all-time high, purchasers, prescribers and patients are considering price as a key component of a drug's expected health benefit.

In 2014, the US spent \$373.9 billion on medicine, a 13.1% increase over 2013.<sup>1</sup> Purchasers have taken notice, and are driving down drug costs using mandatory discounts and price protection clauses in formulary contracts.<sup>2</sup>

Patients are paying more for drugs as they switch to high-deductible health plans<sup>3</sup> and face larger out-of-pocket costs for specialty products. And physician groups and the government are becoming more concerned, and vocal, about the financial side effects of expensive new therapies. This is especially true as health systems shift to new payment models based on health outcomes, instead of volume of services provided.

Meanwhile patient advocacy groups are publicly assessing drug effectiveness, and providing money and data to help drug developers discover and develop tomorrow's new therapies. Both public and private purchasers are opening up their datasets for collaboration and research. Lawmakers are considering legislation that would allow commercial teams to tout the cost benefit of new products. And technology is facilitating the continuity of care outside of the clinic.

As a result, the divisions between biopharmaceutical R&D, Food and Drug Administration (FDA) approval and commercialization are blurring. Patients are deciding which drugs are valuable in the real world. Provider groups are considering the impact of treatment decisions on the total cost of care. And patient data, in aggregate, are being used by insurers to decide when, how and at which price points to make new drugs available.

Biopharmaceutical companies cannot afford to sit on the sidelines as patients and health plans negotiate access to their products. Putting drug costs into context requires access to patient data, and evidence connecting drug intervention with patient health outcomes. Collaboration is the key to demonstrating value and ultimately boosting revenues in a system that rewards outcomes and quality over volume: the New Health Economy.

To understand the landscape for collaboration, and to determine where the value lies, PwC's Health Research Institute (HRI) conducted dozens of interviews with insurer groups, health systems, new entrants, patient advocacy organizations and biopharmaceutical executives.

## Key findings:

- ◆ Purchaser groups are linking administrative claims data with electronic health records to conduct population research—with biopharma collaborators—to better understand important population segments, like patients with more than one chronic disease.
- ◆ Healthcare providers, pushed by new payment incentives, are collaborating with the drug industry to measure the effectiveness of therapies on the patients they treat.

Prescribing practices increasingly reflect sophisticated cost/benefit analyses.

- ◆ New entrants are bringing biosensor technology and digital tools to healthcare to help biopharmaceutical companies better understand the lives of patients, and how they change in response to drug intervention.
- ◆ Patient advocacy organizations are creating disease-specific registries for research, and consulting with industry players on clinical trial design and protocols.
- ◆ Proposed legislation such as the 21<sup>st</sup> Century Cures bill would make it easier for drug companies to promote cost effectiveness data as an additional product attribute.<sup>4</sup>



## What this means for your business:

As pressure builds to link patient health outcomes with the cost—and value—of new therapies, biopharmaceutical companies must transcend the traditional divide between drug R&D and commercialization. Evidence generation should continue after a drug receives FDA approval, as clinical safety and efficacy measures give way to real-world performance and demonstrated patient outcomes.

- ◆ *Place new bets with pilot programs.* New entrants<sup>5</sup> are flooding into healthcare. Advances in digital

monitoring and biometric sensor technology can shed light on patient experiences and identify remaining unmet need.

- ◆ *Get the purchaser perspective.* Collaborating with insurer groups and health systems provides access to the patient data used by these groups to make coverage decisions.
- ◆ *Embrace patients as partners.* Consumers are asserting themselves when it comes to data ownership, but will contribute health information if they understand the benefits. The National Institutes of Health (NIH) lists 39 disease-specific patient registries open for research.<sup>6</sup>

- ◆ *Anticipate regulatory change.* Regulators are exploring new ways to integrate patient experiences into drug review decisions. Working with patient data as regulations evolve will deliver a competitive advantage over companies that wait for new laws to take hold.

## An In-Depth Discussion

*New collaborations pairing traditional drug makers with insurers, health systems, patient groups and technology firms are reconfiguring three crucial business operations: drug R&D, regulatory submission and product commercialization.*

All of these collaborations have one thing in common: they aim to use newly available consumer health data to uncover the truth about drug value and its relationship to health outcomes. The need to collaborate also stems from a growing concern that drug development doesn't adequately address patient needs and medication adherence outside of the clinic.

As new drugs enter the market to compete with older drugs, differentiation and value is increasingly determined by patients and purchasers. Decisions are progressively made in response to outcomes data as they become available.

Understanding how patients respond to drugs over time requires new capabilities and new evidence. The ability to prescribe the right drug for an individual patient based on robust evidence is crucial, as patients face larger out-of-pocket costs.

Increases in specialty drug prices are also intensifying the cost-benefit calculation used by purchasers in deciding which drugs to cover, and for whom.<sup>7</sup>

New payment models such as Accountable Care Organizations and bundled payments are changing the way physicians think about their own finances. But physicians are also thinking about the financial health of their patients, and their practices.

In areas such as cancer, multiple sclerosis and arthritis, the cost burden for patients is rising. According to an HRI survey conducted in 2014, 92% of physicians said they consider cost when deciding whether or not to prescribe a drug.<sup>8</sup>

In March, Dr. Hagop Kantarjian, chair of the department of leukemia at M.D. Anderson Cancer Center, launched a Change.org online petition<sup>9</sup> asking

### New Health Economy

Driven by empowered consumers and connected technology, the New Health Economy is shifting business incentives from volume to value with a focus on health outcomes beyond the clinic.

President Obama, the Department of Health and Human Services (HHS) and members of Congress to protest cancer drug prices “so all patients with cancer have access to affordable drugs to save their lives.” Signed by 5,445 people in late May, the petition cites research showing that the average price of cancer drugs is increasing by roughly \$8,500 a year.

“Why treat the prices as immutable?” said Peter Bach, MD, director of Memorial Sloan Kettering’s Center for Health Policy and Outcomes, at the American Society of Clinical Oncology’s (ASCO) spring meeting. “Would we really pay an infinite amount for a microscopic benefit?”<sup>10</sup> In late June, ASCO published its own value framework to help physicians and patients compare clinical benefits, side effects, and costs of cancer treatment.<sup>11</sup>

Pharmaceutical and life sciences companies must look outward to anticipate, identify and act on new opportunities in drug development and commercialization. Many novel biopharma collaborations are already underway, with wearables firms<sup>12</sup> such as Fitbit and MC10 Inc.’s biostamp, or genetics companies such as 23andMe. Others are partnering with purchaser and patient groups.

“The pharma industry, providers and payers are all trying to improve

patient health,” Ruchin Kansal, executive director and head of business innovation at Boehringer Ingelheim Pharmaceuticals, Inc., told HRI. “All of us bring different capabilities and competencies to address patient needs, but now we can locate areas of common interest to help identify the right patient for the right intervention, or to inform a more effective use of therapies and techniques to help patients achieve better health outcomes.”

## I. Ones to watch: four key stakeholders

*Cost concerns, regulatory trends, new technology and data are changing the competitive dynamic among health industry groups, and opening the door to new models for collaboration.*

A number of factors are converging to catalyze collaboration across the health sector. They include rising drug costs; increasing competition in key therapeutic areas such as oncology, diabetes, multiple sclerosis and rheumatoid arthritis; inefficient and outdated clinical trial models; shifting provider regulations and incentives; and a new emphasis on patient-reported outcomes. The sum

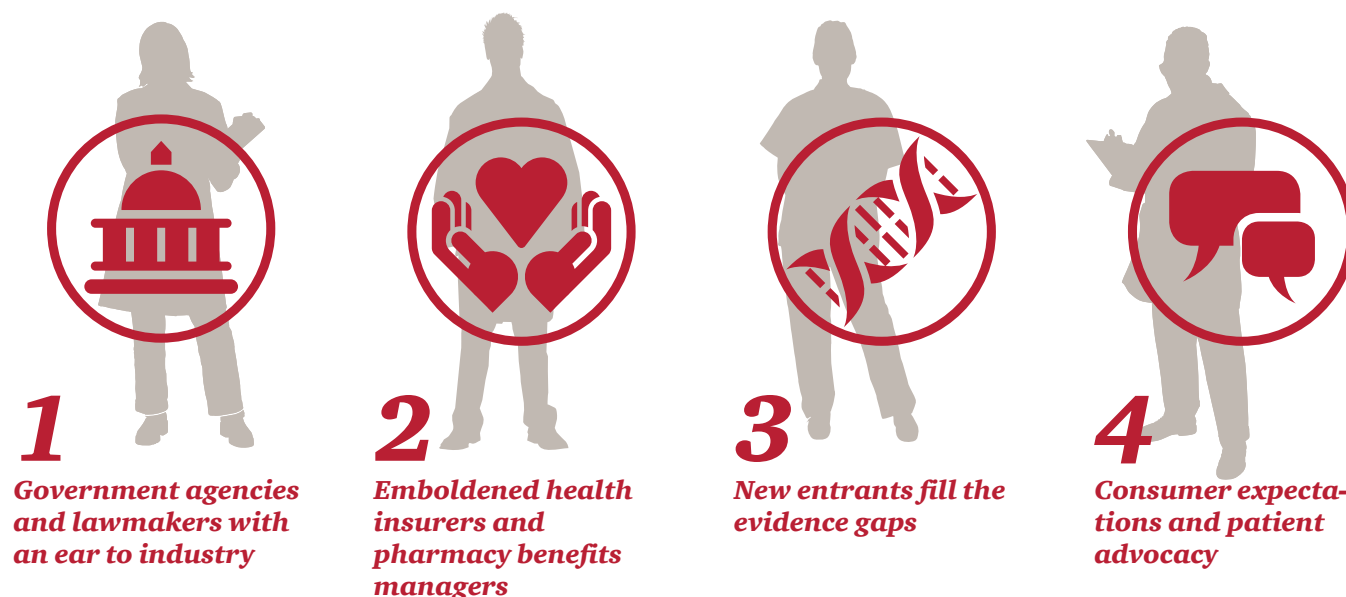
of these factors is beginning to exceed the number of remaining obstacles—such as a compliance issues and an unwillingness to share data—as health organizations prioritize optimal patient outcomes over conflicting business incentives.

In the New Health Economy,<sup>13</sup> the blurred lines of demarcation between traditional health industry business

models are giving way to a wide open marketplace in which data sharing, customer input and disease management is linked directly to consumer choice and payment. In this environment, HRI has identified four groups (see Figure 1) that are reconfiguring how biopharmaceutical companies develop and commercialize new medicines:

**Figure 1: Four key stakeholders**

Four groups are reconfiguring how biopharmaceutical companies develop and commercialize new medicines





## **#1: Government agencies and lawmakers with an ear to industry**

The formation of several federally funded public-private partnerships in recent years demonstrates the government's commitment to industry collaboration as a means for improving the nation's health. And a roadmap<sup>14</sup> produced by the US Department of Health and Human Services' Office of the National Coordinator for Health Information Technology defines how the government will work with the private sector to improve health information sharing and interoperability.

From a regulatory perspective however, biopharmaceutical companies are challenged by regulations that restrict the easy sharing of information beyond what's included on the FDA-approved label. For example, most comparative effectiveness studies and cost effectiveness data can only be shared if an insurer or health system asks a

pharmaceutical company to provide them, according to current regulations.

But the proposed 21<sup>st</sup> Century Cures legislation could alleviate this challenge by allowing drug companies to proactively share and promote such information. As competition for market share increases, the ability to produce and communicate research showing that one product works better than another, or saves money while providing a desired health outcome, is a powerful message to insurers and providers, and a benefit to patients. FDA leaders, however, are concerned that financial incentives cloud industry research and promotional messaging.<sup>15</sup>

Going further, pharmaceutical companies<sup>16</sup> are arguing for the right to promote unapproved uses for their products to physicians and consumers under the First Amendment. In 2012, the FDA lost an off-label promotion case on free speech grounds.<sup>17</sup> In May, Amarin Pharma filed a new lawsuit against the FDA, arguing for a constitutional right to share a study it

considers truthful and not misleading, but was rejected by the FDA for inclusion in the drug label.

The outcome of the Amarin case, like the passage of the 21<sup>st</sup> Century Cures bill, is uncertain. But either could dramatically change the way drug companies market their products and the types of economic and patient outcomes data they could bring to customers.

## **#2: Emboldened health insurers and pharmacy benefits managers**

With the return of rising drug prices, insurers and pharmacy benefit managers (PBMs) are acting swiftly to protect their drug margins.

Consolidation among health insurers<sup>18</sup> and PBMs—and the resulting size of the US patient population they've come to represent—has enabled the aggressive contracting and drug rebates achieved most recently in the hepatitis C category of drugs.<sup>19</sup> Products dropped from the Express

## **Public Private Partnerships**

### **Accelerating Medicines:**



The Accelerating Medicines Partnership (AMP) brings together the National Institutes of Health (NIH), the Food and Drug Administration (FDA), 10 pharmaceutical companies and 12 patient organizations to investigate and develop new drugs for critical diseases. The AMP has committed \$233 million over five years to expedite drug discovery and development in Alzheimer's disease, type 2 diabetes, rheumatoid arthritis and lupus.

### **Precision Medicine Initiative:**



The Precision Medicine Initiative (PMI) intends to “pioneer a new model of patient-powered research to...accelerate biomedical discoveries and provide clinicians with new tools, knowledge, and therapies to select which treatments will work best for which patients.” Individualized genetic research supported by a \$200 million proposed budget in 2016 will compliment studies on consumer lifestyles and environment. The PMI will fund research efforts in collaboration with private industry, patient groups and academic centers, and plans to establish a one-million person registry including genetic and other health data.

### **ClinGen:**



ClinGen is dedicated to building a central resource that defines the clinical relevance of genomic variants for use in precision medicine and research. Over 350 institutions, private companies and hospitals have contributed data to the program. The project, based on sharing genomic and phenotypic data, aims to improve drug development and evidence generation, and integrate genetic data into electronic health records systems.

Scripts national preferred formulary, for example, lose covered access to a population of at least 25 million US consumers.<sup>20</sup>

Mark Cziraky, vice president of research at HealthCore, a clinical outcomes research subsidiary of Anthem, Inc. said biopharma collaborators have graduated from using aggregate data to generate hypotheses about drug interventions, to “testing outcome and effect...we’re getting to pragmatic trial designs where you are randomizing [patients] and then stepping back to watch it happen in the real world, which is an ideal prospective observational design” for making coverage decisions.

Randomized controlled clinical trials remain the gold standard for evidence generation in drug development, but companies spend “seven to ten years and \$1 billion generating evidence on 7,000 people, on average,” said Marcus Wilson, president of HealthCore. “You can’t extrapolate that out, but you can get 7,000 people using the drug in our population the first month it launches.”

Reconciling a drug’s performance in clinical trials with how it performs in the real world, where patients and treatment circumstances may differ dramatically, helps to inform decisions about drug coverage, and also what happens after an insurer makes those decisions, he said. HealthCore announced two new five-year collaborations late last year, with Eli Lilly and Boehringer Ingelheim, focused on generating real world evidence to support health economic and outcomes research in disease areas of mutual interest.

Aetna’s Healthagen is also attracting pharma collaborators. Van Crocker, president of Healthagen Outcomes, said recently that the “return on investment is unbelievable if pharma can think in terms of population health management, and use data to build the necessary story to demonstrate effectiveness.”<sup>21</sup>

### **#3: New entrants fill the evidence gaps**

If data warehouses were actual warehouses, there wouldn’t be enough undeveloped real estate on the planet to break new ground. In the era of big data, organizations are realizing that bigger isn’t always better; being able to access and communicate relevant data as they relate to individual business objectives or specific audiences is the key to demonstrating value. New entrants are disrupting healthcare delivery by using consumer technology as a point of entry.

“What matters now is data of a certain kind,” said Dolors Terricabras, director, neurology, new solutions development at UCB, a global biopharmaceutical company. “If you can start collecting the same type of data in a structured manner from a very specific patient population, that’s where you can really start comparing and generating quality insights.”

Last July, UCB announced a partnership with MC10 Inc. to develop the latter’s Biostamp device—a clinically-focused, flexible biometric sensor patch with the potential for a temporary tattoo—like form factor—for use with UCB’s drugs to treat movement disorders such as Parkinson’s disease and restless leg syndrome. The UCB/MC10 Biostamp research collaboration will test the Biostamp with patients, to track movements in the home and around the clock. UCB hopes analysis of the Biostamp data can optimize treatment for patients with movement disorders.

“We also hope that a better understanding of the patient condition could lead to the discovery of unmet need that even patients or physicians haven’t realized were there,” Terricabras told HRI.

Organizations such as Teva Pharmaceutical Industries Ltd. are collaborating with new entrants on implantable drug delivery microchips



What matters now is data of a certain kind.”

—Dolores Terricabras, director, neurology, new solutions development at UCB

for patients<sup>22</sup> and telehealth services.<sup>23</sup> Teva also formed a research incubator with Philips Healthcare aimed at developing new medical technologies to support drug therapy. And Google’s life sciences group—Google X—launched a wearable device designed specifically for patients participating in clinical trials.<sup>24</sup>

New entrants bring not just technology, but new business models to bear on care delivery and consumer health. Biopharmaceutical companies, through collaboration, can leverage the speed and innovation of emerging technology, device and diagnostic firms to merge the consumer experience into the health ecosystem.<sup>25</sup>

### **#4: Consumer expectations and patient advocacy**

Consumer expectations in healthcare have shifted in the last five to 10 years, due in part to experiences in other industries.<sup>26</sup> Easy access to health information, increasing costs, and a desire to understand—at a physical and biological level—what’s happening in their bodies has led to stronger opinions about how, where and when to deal with illness.

Patients are pushing for further inclusion into the FDA drug review process, to ensure that new drugs are truly addressing their needs in meaningful ways. FDA officials have suggested that the next Prescription Drug User Fee Act (PDUFA) reauthorization, in 2017, will codify patient input as part of FDA’s review process.<sup>27</sup>

Consumers are also pursuing an agenda that can sometimes run counter to conventional methods of drug development and approval. Demonstrating that a tumor shrunk, or that a biomarker was successfully targeted by a drug during clinical trials doesn't necessarily mean that a patient will feel any better. In chronic obstructive pulmonary disease, for example, the ability to accomplish

daily tasks—such as lifting children, or using a hairbrush—without succumbing to breathlessness, can be more important to patients than a clinical efficacy number.

Collaboration with patient groups has paid off, for patients and pharmaceutical companies. The Cystic Fibrosis Foundation's (CFF) collaboration with Vertex

Pharmaceuticals, for example, continues to bear fruit. Kalydeco, a blockbuster drug for cystic fibrosis patients first approved in 2012, was discovered at the CFF and developed by Vertex. At the same time, Kalydeco's price of about \$300,000 a year has fueled broader concerns about the rising cost of medications.<sup>28</sup>

## ***Case Study: Collaborating with the Chordoma Foundation***

Rare diseases, particularly illnesses with no known treatment, are challenging for drug developers. A small patient population is often spread across the country or globe, making clinical trial recruitment difficult. The symptoms patients experience as the disease progresses are often poorly understood by individual companies or regulatory agencies, complicating clinical trial design and the assessment of results. But a collaborative model for R&D is emerging, one that leverages the resources of patient groups and drug developers.

Chordoma is an exceedingly rare form of bone cancer, diagnosed in about 300 people each year in the US. The standard of care for chordoma involves surgery, followed by radiation. But patients typically die seven to nine years after a diagnosis.<sup>29</sup> The Chordoma Foundation, an advocacy group located in Durham, North Carolina, was founded in 2007 by Josh Sommer, a chordoma patient, and his mother. In less than a decade, the foundation has dramatically improved the scientific understanding of the disease and helped to evaluate new therapies in development.<sup>30</sup>

Sommer dropped out of Duke University after his diagnosis and immersed himself in the science of brachyury mutation, a protein active in many malignant cancers. The same mutation eventually led

Tim Rodell, CEO at GlobeImmune, a biopharmaceutical company, to the Chordoma Foundation. GlobeImmune had already planned a clinical trial testing its experimental immunotherapy product in other metastatic cancers, but had learned that brachyury was a key driver of metastasis in chordoma tumors as well. The foundation invited GlobeImmune and its research partner, the National Cancer Institute (NCI), to present their findings at its scientific meeting.

"As a result of that meeting, we collectively made the decision to amend our phase 1 trial protocol to allow for the enrollment of patients with chordoma," said Rodell. "We put the study notification up on the Chordoma Foundation website, and within a very short time we had more volunteers for the study than we had places remaining."

Data from the phase 1 study was positive, but limited by the trial size and design. Since no other drugs have been shown to work for chordoma patients, there weren't established endpoints for clinical trials. Clinical endpoints are the only way to assess how well a drug works.

Last year, the Chordoma Foundation held a meeting at ASCO, which included key opinion leaders from the few hospitals that treat chordoma patients, leading researchers in the

field, GlobeImmune and Celgene scientists and FDA officials.

"We spent all afternoon going through the data and talking about what the realistic endpoints might be" for the next stage of clinical development," said Rodell. "Based on that input from everyone, we got to a final protocol for phase 2." That study began enrolling chordoma patients in March.

Rodell urges biopharma colleagues to ask patient groups for help, to come in with an open mind and to do it very early in the development process. The days of a pharma company identifying a pathway, identifying a molecule that intervenes in that pathway, doing some toxicology studies and then writing a big protocol to hand over to investigators, are over," Rodell told HRI. "It has to be an iterative, collaborative process, and the patient perspective must be integrated into that process."

The ongoing collaboration between the NCI, GlobeImmune and the Chordoma Foundation has expedited the clinical trial process by helping recruit the right population, without delay, said Rodell. "We could see which patients to potentially recruit into a trial to demonstrate a meaningful difference" in response to therapy, but not a patient population "so homogeneous that it would take 20 years to enroll the trial," said Rodell.



## II. Partnering to forge a working consensus on the value of new medicines.

*Value in healthcare resides in the eye of the stakeholder. A shared vision is needed to accomplish the triple aim of improving quality, access and lowering overall costs.*

It's no secret that a degree of skepticism often creeps into the room alongside pharma marketers toting sheaths of data meant to demonstrate the superiority of a given drug. According to a 2012 HRI survey, just 5% of insurer respondents (see Figure 2) indicated that they are very confident in the economic data provided by the drug industry when making coverage and formulary placement decisions.

However, 60% strongly agreed that pharma must demonstrate a significant clinical benefit compared to other available treatments to be considered for formulary placement, and 45% agreed that a clear cost savings argument was necessary.<sup>31</sup>

What evidence is needed to address the credibility gap? Part of the issue has to do with getting the right information into the right hands. Key account managers and biopharma sales reps with clinical expertise were the two most effective elements of the new commercial model for biopharmaceutical companies,

according to a 2014 survey conducted by PwC's Strategy& group.<sup>32</sup>

Collaborative projects and publications can be an effective way to promote consensus, especially when research is customized to the members of a particular insurer. Last July, Cigna entered into a "first-of-its-kind" contract with AstraZeneca to provide customers at an increased risk for atherosclerosis with access to Crestor, a brand name statin used to control cholesterol.<sup>33</sup>

Crestor's retail price tops \$200 for a month's supply, compared with as little as \$4 a month for a generic statin. Cigna and AstraZeneca used medical and pharmacy information to create a predictive model that identified patients at a higher risk for atherosclerosis. Patients meeting the high-risk criteria were given access to Crestor as a preferred brand-name statin, with a lower co-pay and without a requirement for prior authorization.

Emerging real world evidence studies examining comparative effectiveness

and costs between competing medications—such as AstraZeneca's study<sup>34</sup> comparing Symbicort to Boehringer Ingelheim's Spiriva, in COPD patients—have impressed purchasers, said Roger Longman, CEO of Real Endpoints, a company that advises biopharmaceutical organizations on evidence generation from the perspective of health insurers. The AstraZeneca study was conducted in partnership with HealthCore.

Another large pharmaceutical company has improved formulary access to its type 2 diabetes medications by maintaining an outcomes registry of patients using those products.

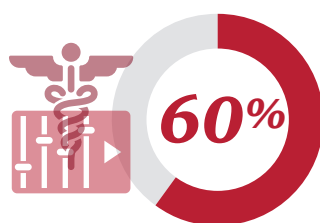
Many external collaborations remain in the exploratory phase and involve building not just a capability to work with new data, but also trust. Choosing the right partner requires an understanding of the information, technology and services available, as well as a clear strategy and set of objectives, and a means for measuring progress.

**Figure 2: Value in healthcare resides in the eye of the stakeholder**

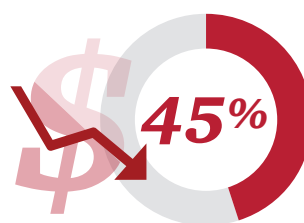
An HRI survey of 100 insurance company managers, directors and executives found that purchasers want pharma to demonstrate drug value, even if skepticism around the data persists



**Very confident in the economic data provided by the drug industry**



**Strongly agreed that pharma must demonstrate a significant clinical benefit**



**Agreed that a clear cost savings argument was necessary**

Source: HRI Unleashing the Value 2012

Roslyn Schneider, global patient affairs lead, Pfizer medical, said recently that new partnerships should be “built around a specific health outcome unique to a therapeutic area or product.”<sup>35</sup> Biopharmaceutical companies should consider pipeline and portfolio and prioritize a specific set of objectives—in R&D or commercialization—that can drive progress.

However, a drug is only one part of the consumer health equation. Biopharmaceutical companies should approach collaboration as an integer, and not the sum of this equation.

Experimental pilot programs may prove to be illuminating. In April, Pfizer announced its participation in a collaboration that includes the AARP, UnitedHealthcare and the Georgia Tech Research Institute. The goal of

the program is to better understand how Americans over the age of 50 use technology for health.

The first study will provide 80 consumers aged 50 and older with five different sleep and activity tracking wearables for use over a six week period, and then report on how participants used the devices, and the barriers they encountered.<sup>36</sup> The biometric trackers may serve as “innovative solutions to challenges such as medication management and adherence for older adults,” Wendy Mayer, vice president of worldwide innovation at Pfizer, in a statement.

Other kinds of collaboration present a greater degree of risk to biopharma, but that may be necessary to produce compelling, actionable results. In order to forge a true consensus on value, all partners must have skin in the game.

“Very few companies do what most payers want them to do, in terms of late-stage clinical trials, which is a head-to-head comparison against the standard of care” in a given disease category, said Longman. “One key issue is, how much are companies willing to spend, in terms of additional research, for a marketed product?”

Biopharmaceutical companies must be “willing to be part of a study design that’s more risky to a brand than what has been done in the past, in the post-market space,” said Healthcore’s Cziraky. “Companies are starting to bring trial designs that support marketing messages, based on randomized clinical trial development and drug indication, into the post-market world. Those kinds of designs are more risky to the business, but I think that nowadays, it’s more risky not to do them.”

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### ***III. Leveraging consumer and patient health information***

*An increasingly comprehensive understanding of the patient experience and journey is necessary to define health outcomes and to measure the impact of medicines on peoples’ lives over time.*

While there’s nothing close to a one-stop-shop for patient data and information—yet—a variety of organizations have succeeded in piecing together disparate data. The results are building progressively vivid representational models of people as they manage their health, access care and continue living, or die.

The critical first step in considering a data-sharing collaboration is understanding which data, and what kind of access, are unique to a potential partner organization.

Insurer subsidiary groups, such as UnitedHealth’s OptumLabs, Anthem’s HealthCore, Humana’s Comprehensive Health Insights and Aetna’s Healthgen, offer varying quantities and degrees of administrative medical and pharmacy claims data, linked

with provider-side clinical data originating from EHRs and paper-based medical records.

Medical records housed with provider groups and health systems are not the black boxes they once were; the growing quantity of EHR data now accessible for research has enabled new analytical capabilities and data linkages for constructing a more detailed portrait of patients and disease progression.

The HHS Office of the National Coordinator for Health IT is committed to achieving a “learning health system” by overcoming barriers to interoperability. Collaboration between government, consumers and the private sector is crucial to the National Coordinator’s 10-year plan.<sup>37</sup>

Other data sources, including patient registries created by public and private groups, are organized primarily around specific disease areas. The data housed in these registries vary dramatically according to patient population size, data type and accessibility. A clear strategy and set of objectives is needed to determine which data are most valuable to an individual organization.

A biopharmaceutical company’s product portfolio, pipeline and therapeutic areas of interest will narrow the field of traditional and non-traditional partner options. But the business models for collaboration across different organizations, and the results that can be achieved, are as varied as the kinds of data available.

At Anthem's HealthCore, for example, partners are not given direct access to the data, but must collaborate with HealthCore's team of researchers to conduct studies. From HealthCore's perspective, this prevents biopharma companies from being led astray by natural biases intrinsic to insurer data sets, and puts to use a hard won institutional knowledge built over a decade of conducting research.

Conversely, OptumLabs, through its Humedica technology, offers companies the option of direct, hands-off access to its database.

Aetna's Healhagen database lets organizations "go from retrospective patient data to prospective data, to do sequential analysis of the same patients over time," said Crocker. This methodology can determine whether

adherence directly improves outcomes, for example. What biopharma should ask of its would-be collaborators is, "what basic question can a new data source answer, that saves extra work?" said Crocker.

## ***Case Study: OptumLabs pieces it together***

Formed in 2013 as a partnership between Optum and Mayo Clinic, OptumLabs has become collaboration central. The open center for research and innovation has enlisted more than 20 partners ranging from professional associations, hospital systems and schools of public health, to medical device firms and pharmaceutical companies. OptumLabs' objective is to address the biggest problems in healthcare through a combination of diverse perspectives, massive data and analytic tools.

Few if any public or private health organizations have access to as much personal health data as OptumLabs. The organization gives partners access to its de-identified dataset, which contains claims data for around 150 million lives, clinical data on about 50 million lives, and linked claims and clinical data on upwards of 15 million lives.

"We can use our data to answer questions more meaningfully with larger sample sizes than most researchers can access," Paul Bleicher, MD, PhD and CEO of OptumLabs, told HRI. "Sometimes this results in developing samples of tens of thousands, or even 100,000 lives or more. That amount of linked claims and clinical data in a specific population area can lead to big insights into populations and groups of people within them."

All data contributed to and used by OptumLabs is de-identified consistent with the requirements of HIPAA, before entering the OptumLabs Data Warehouse. As part of the de-identification process, the data from each individual is linked by assigning a unique individual identifier through a process that uses one-way cryptographic hashing and enables researchers to conduct studies at the individual patient level.

Understanding how large groups of individuals use medications, live with their diseases, and purchase healthcare services can identify new areas for drug intervention, identify optimal care pathways, and surface potential improvements in care delivery and new populations for research. The de-identification processes used at OptumLabs makes this possible while simultaneously protecting individual privacy.

OptumLabs provides researchers with controlled access to its database in virtual "sandboxes," providing access only to data directly related to their approved work. It also offers tools and opportunities to collaborate across partner organizations. Diverse partners can work collaboratively with organizations they were unable to work with before. For example, pharma partners that want to conduct a new study using OptumLabs data could invite any of OptumLabs 11

leading academic partners to serve as the principal investigator.

All studies conducted in partnership with OptumLabs are transparent and intended for publication, but there are limits to what can be done with the data. "We only do pre-competitive research that is designed to be of benefit to the health system at large," said Bleicher. "This means that partners can't come in and try to use our data to prove that their product or service is better than everyone else's, and then go out and market that."

That said, companies could, for instance, look at the optimal time to introduce statin therapy in a US male population with a history of high cholesterol.

Using data to optimize treatment decisions based on both outcomes and cost is desperately needed in healthcare. For example, a 2014 study published by the American Diabetes Association, based on research using OptumLabs data, found that patients with type 2 diabetes had the same health outcomes using an older, cheaper therapy. But the impact of the study on physician decision-making, or insurer coverage decisions, is a work in progress. It takes time to broadly influence clinical practice, but the ultimate goal of OptumLabs is to do just that.

New technology that captures biometric data aims to further integrate the patient experience into drug development and illuminate health outcomes research. Drug company partnerships with wearable technology firms—such as UCB’s collaboration with MC10, Inc., or Biogen Idec’s partnership with Fitbit and PatientsLikeMe in multiple sclerosis<sup>38</sup>—are in the pilot stage.

Pilot programs may transition to core product services as more companies focus on health outcomes as a key measure of product value. “Some of these technologies have been useful in assessing activity and function as markers of recovery after an illness or surgery, but they are still in the formative phase,” said Harlan Krumholz, MD, SM, a cardiologist, Yale professor and a board member at the Patient-Centered Outcomes Research Institute (PCORI) established by the Affordable Care Act to conduct comparative effectiveness studies.

Validated accelerometers that measure precise body movements,<sup>39</sup> for example, are producing reliable data in the clinical setting. But it’s an open question whether activity trackers will become meaningful tools to support and define health outcomes.

Top biopharma organizations are betting that connected medicine and biometric analyses can indeed impact R&D and treatment decisions, as evidenced by new collaboration with Qualcomm Life, a wireless technology firm focused on health. Novartis’s “Trials of the Future” collaboration uses Qualcomm Life’s 2net platform for collecting and aggregating medical device data during clinical trials, to improve the convenience and speed of capturing study participant data and test results, according to a company statement.

Novartis and Qualcomm Life launched an initial study to explore the use of mobile devices with chronic lung disease patients—the study is not drug specific.<sup>40</sup>

Qualcomm Life’s 2net platform and 2net cloud collects biometric data such as blood pressure, body weight and movement in the home—through a small unit that plugs into the wall, or through a smart phone. The data are transmitted to physicians, pharmacists, nurse caregivers or family members who can monitor the health of their patients or loved-ones remotely.

To facilitate this monitoring system, Qualcomm Life enables the capture, aggregation and transmission of vital health information from connected medical devices through a private and secure connectivity platform. For example, condition-specific device kits for chronic obstructive pulmonary disease might include a spirometer, a connected inhaler, a weight scale and an activity monitor, while a diabetes kit may include a connected glucose meter, a connected drug injector or connected pill bottle, said Brian Niznik, senior director, business development at Qualcomm Life. The data can be de-identified but kept intact with a global unique identifier, to facilitate medical-grade, scalable care coordination and data management.

In the R&D space, “through the use of wireless and smart technology, Qualcomm Life can help pharma compress the time of a clinical trial, reduce the cost and improve the quality and speed of trial data collection,” said Niznik.

Instead of the traditional trial process of asking patients to document and self-report their health information and behaviors periodically, connected devices “such as a blood pressure patch, activity monitor and weight scale, for example, can result in a more frequent, near real-time data exchange reducing the potential of patients’ transposing or modifying their data incorrectly, or making errors.”

Once a drug hits the market, biopharmaceutical companies “see the future coming with regard to

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Tomorrow [drug makers] may not get paid for the molecule, they may only get paid for the outcome.”

—Brian Niznik, senior director, business development at Qualcomm Life

outcomes-based payment, and they are being very aggressive and proactive about creating these connected therapies,” said Niznik. “Tomorrow they may not get paid for the molecule, they may only get paid for the outcome.”

In April, Qualcomm Life announced a remote home health monitoring partnership with Cerner, a large electronic health record provider. Doctors and hospitals using Cerner’s EHR system will be able to monitor chronically ill patients in “near real-time” to reduce hospitalizations.<sup>41</sup>

“The only way the drug industry is going to be able to continue to charge premium prices for specialty products—or any products—is by being able to demonstrate disproportionate value against the competition,” said Roger Longman at Real Endpoints. “Payers are demanding exactly that, and they will do those kinds of assessments without industry input, if the industry doesn’t start doing it for itself.”



## Case Study: 23andMe targets drug development

Almost one million people have swabbed their cheeks and mailed the samples in to consumer genetics firm 23andMe. Now the company is building a drug discovery and development business on top of that data. Drug companies are using 23andMe's genetics database to shape and speed clinical trials, and shave costs off of the process.

23andMe has “moved into joint development collaborations where there are milestones or the potential for royalties,” said Emily Drabant Conley, 23andMe's director of business development. “And that is out of a recognition that the data are valuable.” In March of 2015, 23andMe announced the creation of a therapeutics group, to be led by Richard Scheller, former EVP of research and early development at Genentech. In addition to collaborations, 23andMe will search for and pursue drug targets on its own.

Consumers who purchase 23andMe's genotyping kit for \$99 and send in their saliva samples get access to an online account to see their ancestry results and uninterpreted raw data. Inside that account, consumers are presented with a consent form and asked if they want to share their data for research. “More than 80% of our consumers opt-in to participate,” said Conley.

Those who opt in are prompted to answer survey questions about prior diagnoses, family history and medication use. A collaboration with Genentech announced at the start of 2015 is focused on identifying drug targets for Parkinson's disease. The majority of the 11,000 genotyped Parkinson's patients in 23andMe's database also provided information about their symptoms and how they've progressed over time, said Conley.

Working with Genentech, 23andMe is narrowing the population of 11,000 Parkinson's patients down to “a couple of thousand” who are particularly interesting from a drug discovery perspective. That group, which may include people with very early onset of the disease, very fast progression of symptoms, or people with known genetic risk factors will undergo a full genome sequencing and a more thorough genetic exploration as part of a clinical trial.

A collaboration with Pfizer in inflammatory bowel disease (IBD) turns the 23andMe kit into a patient recruitment tool for research. Individuals with an IBD diagnosis – such as Crohn's disease or ulcerative colitis – are eligible for a free kit in exchange for consenting to share the data with Pfizer. They also must fill out short surveys online.

The goal of the Pfizer study, which hopes to enroll 10,000 IBD patients, is to “use genetics to identify segments of the population that fit a certain profile,” said Conley. For example, patients that didn't respond to specific drugs to treat their disease.

“You can think about that from the insurance level, but also in how you structure your clinical trial,” said Conley. Another Pfizer collaboration, in lupus, asks participants to consent to have 23andMe “track down their medical records and use that data as part of the research.”

Asked about the next therapeutic frontier for genetics, Conley said the treatment of psychiatric disorders such as depression and bipolar disorder could be improved by genetics. The number of products available, and the wide variation in efficacy and response rates, leaves psychiatrists “throwing darts at a board,” said Conley. “That's an area where I do think we will be able to use genetics” to make better treatment decisions for individual patients.

“

The only way the drug industry is going to be able to continue to charge premium prices for specialty products – or any products – is by being able to demonstrate disproportionate value against the competition.”

—Roger Longman at Real Endpoints



## IV. Implications and opportunities for pharmaceutical and life sciences companies

*Collaborating strategically can maximize the dollars spent in drug development, fill evidence gaps in specific patient populations and demonstrate a drug's cost and comparative effectiveness.*

### Value in R&D

#### Know what you don't know

There is no shortage of data for sale, with or without entering a collaboration. Companies need to first understand what information they already have, what information is needed and what services a partner can offer to complement internal knowledge and capabilities. This understanding is the first step in deciding how best to deliver evidence to purchasers and providers.

Leveraging the strengths of patient advocacy organizations and networks can expedite recruitment and promote retention in clinical studies. Patient organizations formed around specific disease areas have a keen understanding of the issues that matter most, and can offer valuable insights to inform clinical trial design and protocols. Biopharmaceutical companies should also consider the various roles new entrants are playing to support patient organizations and to drive consumer engagement.

Prior to beginning a phase III trial, drug makers should also make certain they understand the competitive landscape from the perspective of health insurers. Collaboration and knowledge-sharing can ensure that trials are collecting the data and evidence needed to create a meaningful advantage over competing products already on the market, or entering late-stages of pipeline development.

#### Anticipate change in regulatory science

New regulations covering the use of genetic information, clinical trial

endpoints and biomarker identification and development will shape the way companies develop drugs and go to market. At the same time, regulatory science is trending toward patient-reported outcomes as another key criteria of drug evaluation. "We've been pushing PCORI to be more interested in the outcomes people experience, rather than [evaluating] biomarkers" as the measure of success in product development, said Krumholz.

The FDA roadmap<sup>42</sup> for patient-focused drug development encourages the use of clinical outcomes assessment measures to better understand patient experiences. In May, clinicaltrials.gov listed 343 open clinical studies that included at least one patient-reported outcome measure, according to an HRI analysis.

Between the 21<sup>st</sup> Century Cures legislation and PDUFA VI negotiations already underway, it's evident that patients are becoming a more powerful stakeholder group. Nineteen states have passed "Right to Try" laws<sup>43</sup> in reaction to patient demands for access to new, experimental therapies, before the FDA declares them safe for general use.

Consumers are also taking a more proactive approach to research participation, as demonstrated by 23andMe customers' willingness to contribute genetic data for research. Apple's ResearchKit application is already being used by top institutions such as the Dana-Farber Cancer Institute and Massachusetts General Hospital, and patient advocacy organizations such as the American Heart Association and The Michael J. Fox Foundation for Parkinson's Research.<sup>44</sup>

Collaborations that deliver patient data and insights to inform clinical development programs can reduce the need to generate such information once a drug receives FDA approval. It can also provide a basis for additional outcomes research and evidence generation after launch.

#### Put cost into context

Biopharmaceutical companies that sell products outside of the US are accustomed to regulatory assessment that examines price alongside safety and efficacy. In Europe, all drugs must present compelling data to support the price of a new product, or be subject to large price cuts. Although FDA does not consider pharmacoeconomics as a criteria for drug approval, a surprising 43% of pharmaceutical and life sciences executives supported the idea, according to a recent HRI survey.<sup>45</sup>

The FDA's clinical outcomes assessment tools don't consider drug costs, but any patient or insurer can tell you that price matters. Organizations that can successfully anticipate—in dollars—the value of cost effectiveness or comparative effectiveness studies, can save money when a drug comes to market by baking those studies in to clinical development. Exploring the impact of a therapy on the total cost of care represents an important opportunity to disintermediate a portion of the healthcare costs unrelated to drug prices.

PBMs in particular have taken an increasingly aggressive stance toward drug prices in recent months, so understanding the value drivers unique to a specific therapeutic area improve the odds of favorable reimbursement decisions. Collaboration with health systems,

insurer organizations, patient groups and new entrants can bring the patient perspective to bear on payment decisions.

### **Impact of New entrants**

Companies that can recognize, evaluate and collaborate with innovative technology providers can improve the quality of evidence collected during product R&D. The emergence of point-of-care diagnostics companies such as Therasys, clinical-grade monitoring technologies such as AliveCor's mobile electrocardiogram, and other consumer-facing diagnostics have the potential to disrupt the biopharmaceutical R&D process.<sup>46</sup> They can also enhance the commercial models needed to demonstrate outcomes, post approval.

Capturing evidence at an increasingly granular level, with respect to nuances in patient populations along the lines of gender, ethnic origin, environment and consumer habits, delivers new insights into how specific patient populations respond to therapy. Successfully leveraging such data will provide an edge over companies that hide behind historical frameworks for clinical research, or outdated perceptions of FDA regulations.

New entrants bring speed to the traditionally sluggish process of developing and marketing new products. Collaboration with technology and device companies can help biopharmaceutical companies overcome barriers to improving and expediting entrenched business operations.

### **Value in the Market**

#### **Demonstrate value in the real world**

An understanding of the importance of real world evidence has bubbled up to the C-suite, according to a 2014 survey conducted by Strategy&. Asked about the importance of real world data

sources, and the associated quality level of such data (see Figure 3), 61% of the pharmaceutical industry leaders surveyed attributed a high level of importance to such data. Regarding the quality of real world data sources, 40% of respondents said provider data quality was high and 33% said insurer data was high quality.<sup>47</sup>

While collaboration with insurers and providers to understand the impact of new therapies on patient populations over time is not new, these organizations deserve a second look. Health industry consolidation and the digitization of patient data has drastically increased the quantity and scope of research capabilities, and the quality of insights available through advanced analytics.

New entrants such as Qualcomm Life and MC10 Inc. may improve the generation, quality and relevance of post-market studies, as constant monitoring and real-time data replaces periodic data collection in the doctor's office or pharmacy.

Mobile apps prescribed as part of a treatment regimen and biometric data collection will help to paint a more detailed portrait of the patient experience, to further correlate specific medical interventions with health outcomes.

#### **As regulations shift, adapt compliance practices**

Collaboration with insurers and especially health systems requires a careful evaluation of compliance issues. But the compliance and legal function shouldn't unnecessarily block access to valuable consumer and patient information or data-sharing.

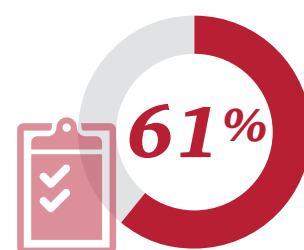
Confronted with escalating up-front costs for specialty drugs and new products for rare diseases, public and private insurers need pharmaceutical companies to tell an evidence-based story of value that goes beyond what's included on the FDA label.

An ongoing assessment of FDA's thinking, in light of pending legislation, lawsuits and government rulemaking, can turn the compliance department into a strategic partner for marketing and promotion, while controlling risks.

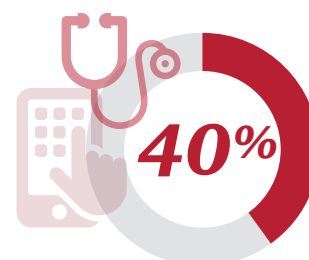
Understanding and responding quickly to the shifting regulatory environment—particularly for companies working in competitive therapeutic areas—may substantially

**Figure 3: Real world value**

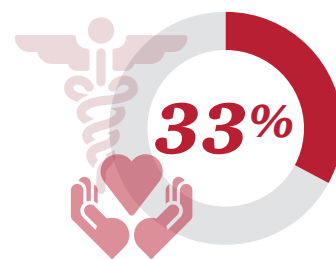
A survey of pharmaceutical executives points to a recognition of value in real world evidence, despite quality concerns.



**Attributed a high level of importance to real world data**



**Said provider data quality was high**



**Said insurer data was high quality**

Source: PwC's Strategy& - Revitalizing pharmaceutical R&D: The value of real world evidence 2015

impact the success of a launch, or the lifecycle management of products in the middle and late years of patent protection. Having a process to respond quickly and effectively to inquiries on cost effectiveness, patient segmentation, health outcomes and new drug research will become even more important as new and expensive therapies enter the market.

### ***Embrace patients as consumers***

Consumers are asserting themselves when it comes to data ownership, but will freely contribute health information if the benefit of sharing is clear. Patient engagement must go beyond product promotion and one-sided conversations about the benefits of a particular product.

Building trust and an authentic relationship between drug makers and patients requires sharing the bad with the good; for example, when real world evidence points to adverse events, or diminished efficacy or response rates in a particular patient

population, this information should be communicated publicly and without delay.

Patients taking maintenance medications for chronic diseases understand the benefits, or they wouldn't continue to take them. The opportunity to create a lifelong and mutually beneficial relationship with patients depends on trust and transparency, which means actively communicating new risks, not just new benefits.

### ***Pilot new services and technologies to innovate***

The shift to outcomes-based payments for drugs won't happen overnight, but companies that prepare for this eventuality are poised to have a competitive advantage over those that wait and must play catch up.

Reimbursement trends are shifting toward care management beyond the physician's office. Beginning in January of 2015, new Medicare

billing codes were released that allow physicians to charge for chronic condition management and transitional care management, or care for patients transitioning out of an institutional setting and back into the home.<sup>48</sup> The HHS goal of tying 85 percent of Medicare payments to value by 2016<sup>49</sup> will push this trend further.

Pharmaceutical companies have an opportunity to improve health outcomes, especially with chronic disease patients, by collaborating with clinicians and tech companies to improve care management, promote medication adherence and lower the total cost of care. Data collection and monitoring with wearables and other connected devices, present an opportunity to provide a valuable service to both patients and physicians.

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## ***Conclusion***

*Collaboration is critical to accessing and analyzing the data needed for an increasingly personalized product offering and the price tag such a product commands.*

Biopharmaceutical companies have dabbled in external collaborations for years. What's different now is the accessibility and quality of the consumer data that underlies new partnerships to help biopharma companies capture and explain the value of products. New technology is accelerating the pace of innovation in biopharmaceuticals by democratizing access to data and empowering consumers to manage their health.

As a result, new definitions of innovation and value are needed to

remain relevant in a rapidly changing healthcare system.

Pricing pressure will only increase as more specialty drugs enter the market. An ability to demonstrate value based on real patient experiences, in the real world, can serve as a release valve. But individual companies are incapable of gathering and producing enough patient data on their own.

The science of drug discovery and development is also changing. Oncology drug development is

leading the way for clinical trial models leveraging shared data across separate organizations. In the New Health Economy, the value of scientific discoveries, and the new drugs they become, increasingly will be determined by consumers over time. Collaboration is critical to accessing and analyzing the data needed for an increasingly personalized product offering and the price tag such a product commands.



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