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# Executive summary

In today's era of rapid scientific progress, public- and private-sector researchers are seeking to leverage their strengths in collaborative ways to accelerate innovation in patient treatment and care.

Biopharmaceutical companies increasingly are partnering with other stakeholders to address scientific and technological challenges, create greater efficiencies in research and development (R&D), and accelerate the discovery, production, and delivery of new treatments for patients.

Relationships in the R&D ecosystem have evolved beyond concentrating primarily on advancing individual assets (i.e., a potential new drug candidate) to focus more prominently on understanding the science behind and behavior of particular diseases and conditions.

In recent years, collaborations have become more diverse in terms of the stakeholders involved, contributing new perspectives, capabilities, and resources to drive scientific advances. Innovative biopharmaceutical companies serve not just as partners, but often as integrators in this ecosystem, bringing together diverse players and providing scientific, regulatory, and delivery system insights, operational capabilities, and financial resources to help deliver critical new therapies to patients in need.

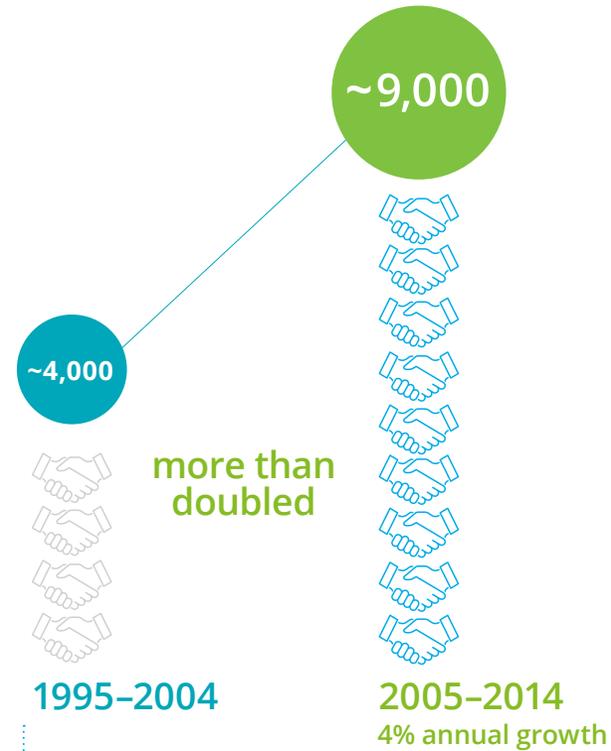
Deloitte was contracted by the Pharmaceutical Research and Manufacturers of America (PhRMA) to analyze the various types and number of biopharmaceutical partnerships created over the past several decades, which resulted in a comprehensive database of partnerships formed between 1980 and 2014. We found that R&D-focused partnerships—most notably, non-asset-based models (those whose primary objectives do not necessarily center on a specific drug candidate) which often take the form of joint ventures (JVs) and consortia—have grown substantially over the last decade. JVs involve two or more partners that contribute to R&D-related activities to achieve a specific task, and consortia typically have three or more parties which participate openly and pool resources to pursue a common goal.



## Executive summary

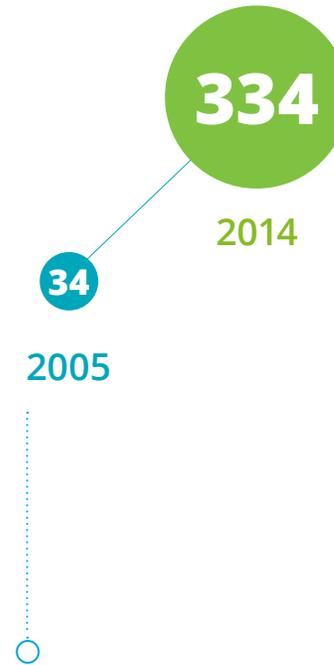
Recent increases in JV, consortia, and other non-asset-based partnerships highlight the growing role and importance of more open, collaborative approaches to R&D innovation.

### New R&D partnerships



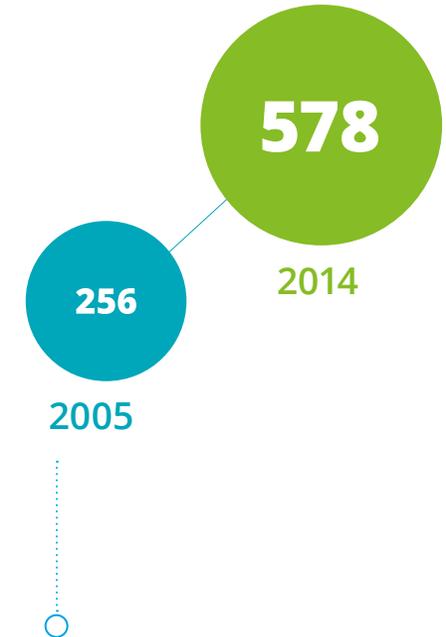
Approximately 9,000 new biopharmaceutical R&D partnerships were formed between 2005 and 2014 at an annual growth rate of four percent during that 10-year period. The 9,000 new biopharmaceutical R&D partnerships formed between 2005 and 2014 are more than double the number formed (approximately 4,000) in the preceding decade (1995-2004).<sup>1</sup>

### Consortia increased 9x



334 new R&D consortia: ~9 times the number formed during the prior decade.

### Early-stage partnerships more than doubled



More partnerships are forming in earlier stages of the R&D process (i.e., prior to a potential new therapy entering clinical trials), with the average number of new early-stage (discovery, basic research, and pre-clinical) partnerships more than doubling between 2005 (256) and 2014 (578).<sup>1</sup>

## Executive summary



“It would be hard to imagine five years ago that the industry would be sharing resources and information about drug targets as openly as it does now. Many companies are essentially working on the same targets; we all share the need to achieve a better understanding of what underlies them.”

**David Wholley, MPhil, Director, Research Partnerships, Foundation for the National Institutes of Health<sup>2</sup>**



“The knowledge derived from precompetitive collaboration is the source of future competition itself. Well-validated targets are good for the industry. We’ll compete in other ways—over how good our chemists are, how quickly we can generate effective new drugs, and how efficiently we then can bring them to market.”

**Adam Keeney, PhD, Global Head, External Innovation Strategy and Business Development, Sanofi<sup>3</sup>**

Successful non-asset-based partnerships and consortia align incentives and share both risk and reward across multiple stakeholders to pre-competitively pursue scientific discovery. Over recent years, consortia have made progress in increasing understanding of some of the most challenging diseases and conditions at the molecular level; quickly and effectively diagnosing disease progression; using biomarkers to monitor patients; developing novel ways to conduct clinical trials; and exploring clinical benefits of combination treatments. Notable examples include the Alzheimer’s Disease Neuroimaging Initiative (ADNI), which is developing standard methods to improve clinical trial efficiency in Alzheimer’s, and the Lung Master Protocol (Lung-MAP) consortium, which has developed a novel, multi-drug clinical trial design that leverages genetic profiling to allow for more personalized therapeutic approaches for lung cancer patients.

Non-asset-based partnerships are expected to continue to increase in number and participation and become more collaborative, diverse, and open with respect to business arrangements. Technology companies with products that enable collection of patient-generated data are likely to play a larger role in these and more traditional arrangements. Biopharmaceutical companies perform a critical function, particularly as integrators of diverse stakeholders and capabilities, throughout all stages of discovery, development, and delivery. Based on the research findings, it is clear that other members of the R&D ecosystem are increasingly working with biopharmaceutical companies through pre-competitive, multi-party collaborations and shared-incentive partnerships to manage growing scientific complexities and to address some of society’s most pressing unmet patient needs, including treatments for various cancers, Alzheimer’s disease, and rare diseases such as amyotrophic lateral sclerosis (ALS).<sup>1</sup>

# 1. Navigating the biopharmaceutical innovation process: Opportunities and challenges

## R&D activity increasing to address unmet patient needs

The last decade has seen a steady rise in Food and Drug Administration (FDA) drug approval rates, demonstrating progress in addressing patient unmet needs despite growing R&D and regulatory challenges. Between 2011 and 2016, 204 new molecular entities (NMEs) were approved by the FDA through the Center for Drug Evaluation and Research (CDER), compared to just 131 NMEs approved between 2005 and 2010.<sup>4</sup> Further, the FDA's CBER (Center for Biologics Evaluation and Research) approved 11 new medicines in 2015 in addition to several biological and blood products not formally classified as drugs. There is also significant current R&D activity, with more than 7,000 new medicines in development across the globe.<sup>5</sup>

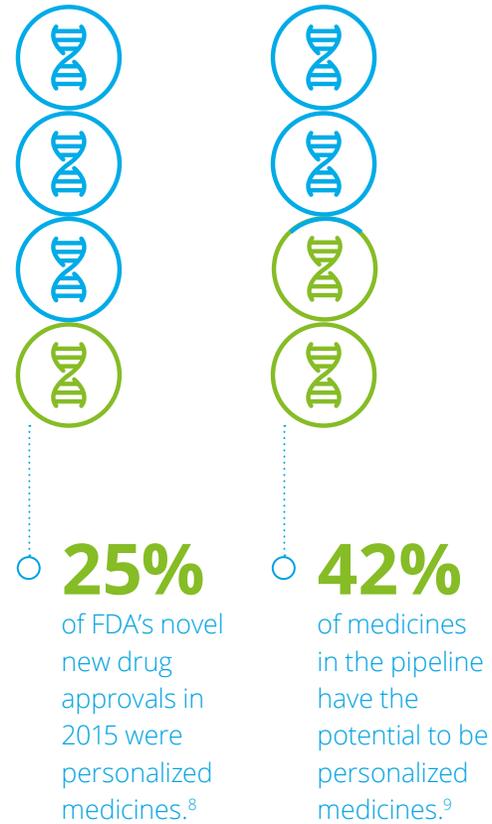
## The R&D process

It takes an average of 10–15 years for a new medicine to advance from discovery through preclinical development, three phases of rigorous clinical trials, regulatory review and, ultimately, delivery to patients. Undertaking this long and complex process is incredibly risky, with less than 12 percent of molecules that enter clinical development (Phase I) ever receiving approval.<sup>6</sup>

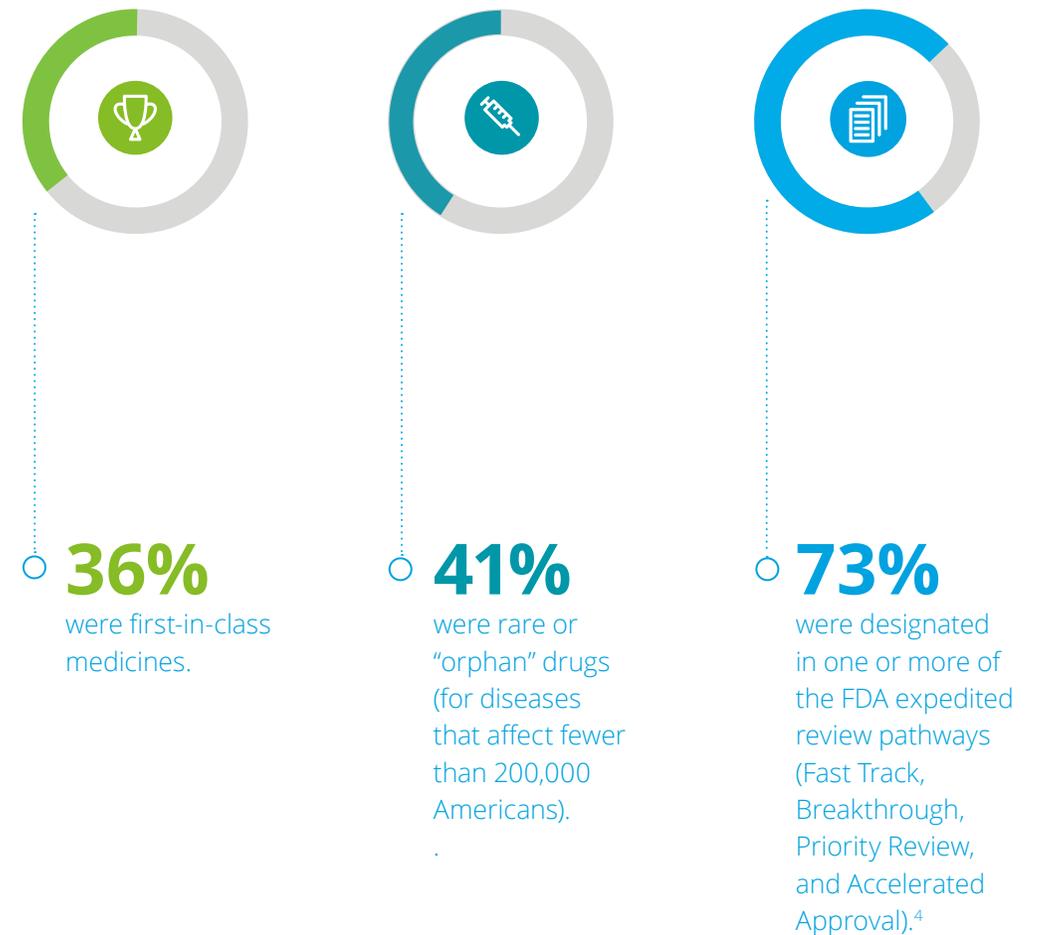
The costs of bringing a new medicine to market have never been higher—and they continue to rise. The Tufts Center for the Study of Drug Development calculates the cost of developing a medicine, including the cost of failures, at \$2.6 billion<sup>6</sup>—more than double the estimate of just a decade ago. Other cost estimates range both higher and lower, but the costs and complexity of drug development have increased, particularly as researchers focus on areas of unmet need. According to the 2015 Deloitte UK Centre for Health Solutions report *Measuring the Return from Pharmaceutical Innovation*, “[some] believe this increase is due to the cost of staffing and resourcing programs with low probabilities of success, the escalating costs of study execution in complex disease areas, and ongoing overhead and infrastructure costs.”<sup>7</sup>

Over the last 10 years, biopharmaceutical companies have increasingly sought to address previously unmet medical needs by building on scientific advances in genomic and molecular medicine. For example, the era of personalized medicine is rapidly changing the way diseases are identified, patients are diagnosed, and treatment decisions are made. Collaboration across the biopharmaceutical R&D ecosystem has been essential in driving important scientific breakthroughs in novel diagnostics technology and in identifying molecular targets for the development of personalized medicines. These advances are reshaping drug development. Biopharmaceutical companies are committed to advancing targeted therapies and medicines to treat serious conditions and unmet medical needs.

## Personalized medicine growth



## Of the 45 novel drugs approved by FDA's CDER in 2016:



## Navigating the biopharmaceutical innovation process

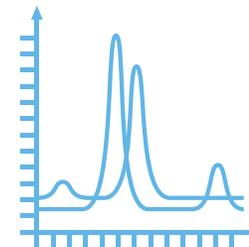
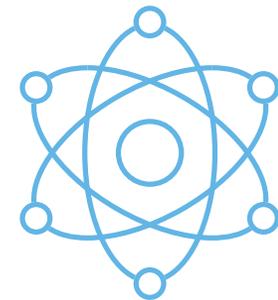
### An increasingly challenging biopharmaceutical R&D environment

As researchers learn more about the molecular underpinnings of complex diseases, traditional methods for assessing the clinical safety and efficacy of a medicine in development create myriad innovation challenges. Some of the scientific, regulatory, payment, operational, and financial hurdles that complicate the biopharmaceutical R&D process and can increase the cost, time, and risk of drug development include:

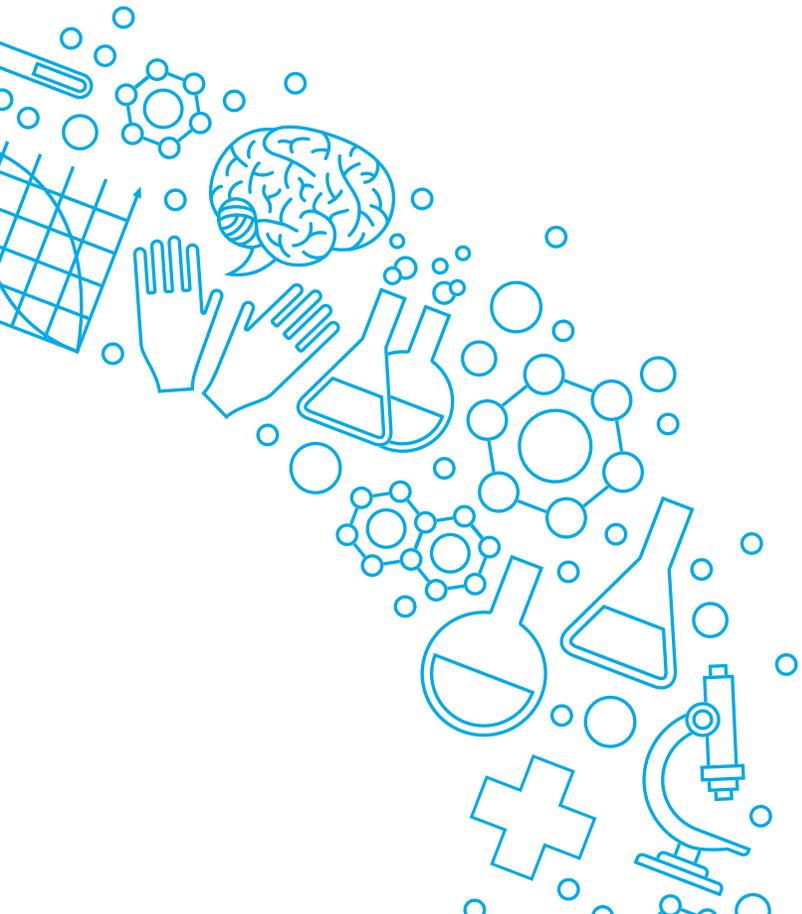
- **Scientific complexity:** Drug developers are pursuing more complex disease areas (e.g., rare cancers, neurological conditions, etc.) and are using new approaches to fight these diseases, often at the molecular and genetic levels.
- **Regulatory requirements:** Regulatory requirements have continued to proliferate. The rapid increase in new sources of evidence, including patient-reported

outcomes, patient-generated data, and real-world evidence (RWE) present new challenges related to data collection, analysis, storage, and confidentiality. Further, there remains uncertainty around regulatory acceptance of some of the more novel clinical trial designs and endpoints that companies are currently pursuing.

- **“Burden of proof” for coverage and payment:** Health plans, health care providers, and patients often have differing views of the value of new medicines. Restrictions on biopharmaceutical companies’ ability to proactively engage with health plans to discuss types of evidence prior to product approval may make it more difficult to determine what data would be most useful for coverage and payment decisions.



## 2. Characterizing the biopharmaceutical innovation ecosystem



An ecosystem model can be used as a lens through which to view the biopharmaceutical innovation landscape. Deloitte defines ecosystems as *“symbiotic, cooperatively evolving communities comprised of multiple diverse players.”*<sup>10</sup> The biopharmaceutical R&D ecosystem is comprised of a range of stakeholders including, but not limited to: biopharmaceutical companies (e.g., large and small biopharmaceutical companies, biotechnology companies, and start-ups/incubators), investors including venture capital firms, health care providers, federal research organizations (e.g., the National Institutes of Health [NIH]), academic institutions, non-profits (including patient advocacy groups and disease-focused communities), services, regulators (e.g., the FDA and the Patent and Trademark Office), health plans, and others (e.g., vendors providing health information, advanced engineering services, contract research and development execution, and others).



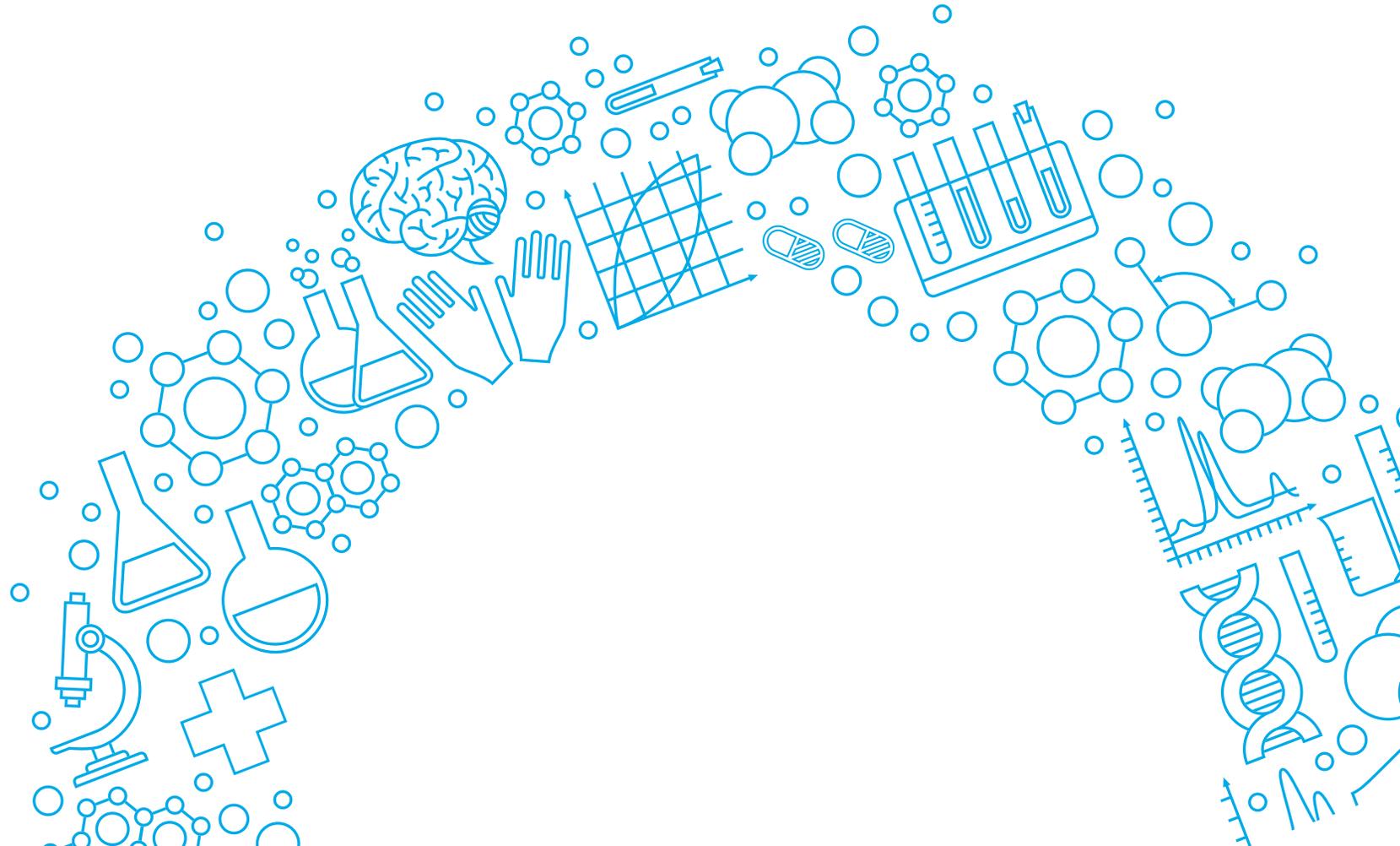
“Innovation stakeholders now all exist in one ecosystem—interdependence is being built; everyone supports each other in terms of medical progress. Only this way is the whole truly productive.”

**Adam Keeney, PhD, Global Head, External Innovation Strategy and Business Development, Sanofi<sup>3</sup>**

This ecosystem and its highly specialized players enable the generation of new biopharmaceutical solutions that serve society’s medical and health needs. While both collaboration and competition are essential drivers of sustained ecosystem success, neither is solely sufficient. Ecosystem stakeholders may be motivated to collaborate by their shared ambitions, objectives, and commitments, while inherent competition may bolster ecosystem activity and contribute to a more efficient and productive R&D process.

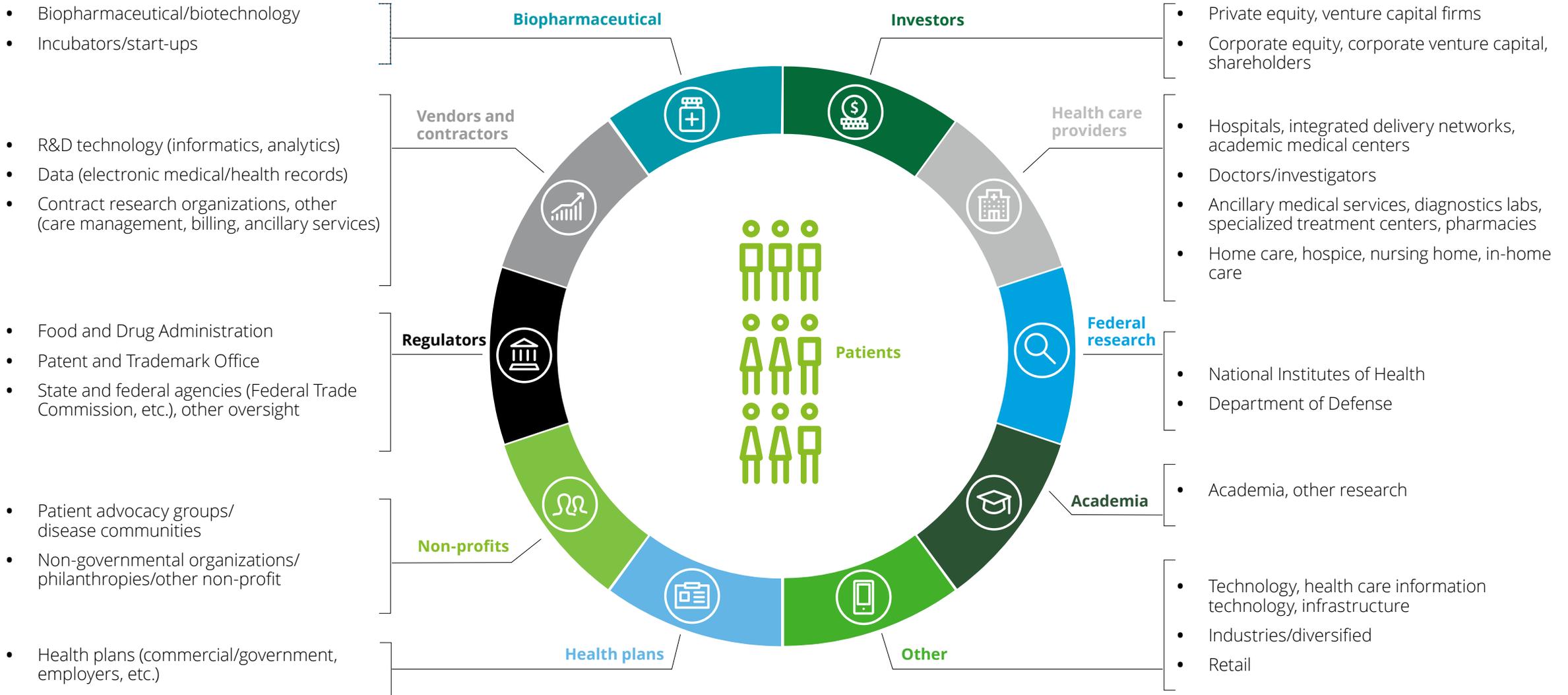
## Characterizing the biopharmaceutical innovation ecosystem

The biopharmaceutical R&D ecosystem enables a varied group of stakeholders to achieve together that which would be difficult for any individual participant. Biopharmaceutical companies serve as both contributors to and integrators of the R&D ecosystem, bringing together diverse stakeholders with distinct characteristics and contributions, with a common goal of improving patient health outcomes. As such, patients are positioned at the ecosystem's hub as both key participants in driving patient-centered innovation and as the recipients of the value the ecosystem collaboratively creates (Figure 1).



# Characterizing the biopharmaceutical innovation ecosystem

Figure 1: Illustrative biopharmaceutical R&D innovation ecosystem



## Characterizing the biopharmaceutical innovation ecosystem

### Overcoming scientific obstacles with collaboration

With multiple challenges impacting the biopharmaceutical R&D environment, collaborative relationships can help partners achieve scientific and technological advances in a more efficient and timely manner and bring new innovations to patients faster.



“Scientific discovery, particularly with the complexities of Alzheimer’s disease, doesn’t happen in isolation ... partnerships are key to us sharing knowledge and expertise, and it means we have the potential to help patients and their families at a faster pace than working alone.”

**Jim Summers, PhD, Vice President, Neuroscience Discovery Research, AbbVie<sup>11</sup>**

Each step in the R&D process requires a unique and increasingly differentiated set of capabilities to advance innovation. Collaborating across the R&D ecosystem can help biopharmaceutical companies of all sizes and their partners navigate increasingly complex scientific, technological, and regulatory hurdles. Various public and private stakeholders bring deep insights into patient needs and close relationships with patients through hospital systems and patient groups, unique basic research insights, innovative clinical development/technology capabilities, and more (Figure 2).



“Today, most important developments in medical science typically begin in laboratories, such as the discovery of specific new biological molecules, processes, or pathways, or innovative applications of existing knowledge. In most cases, these discoveries in and of themselves have limited effect beyond meeting a fairly narrow research goal. Their real impact for public health generally comes after several more significant steps—including further R&D, testing, approval by appropriate regulatory bodies (such as the FDA) ... and licensing these inventions to private entities to ensure use, commercialization, and public availability.”

**NIH Office of Technology Transfer<sup>12</sup>**

## Characterizing the biopharmaceutical innovation ecosystem

Figure 2: Overview of typical contributions of various stakeholders on innovation

Contributions to partnerships		Motivations to partner with other stakeholders
<b>Biopharmaceutical</b> 	Support and/or lead the overall drug development process, including basic research, discovery, chemical compound synthesis, pre-clinical and clinical development, regulatory submissions, commercialization (delivery), etc., with scientific, operational, and/or financial input	Advance platform technologies and basic research; pursue non-core therapy areas and challenging disease areas where specialized scientific expertise is required; optimize efficiency of portfolio programs in core/mature therapy areas (where, if partnered, they could be more cost-effective than in-house options)
<b>Academia</b> 	Perform basic research in areas of unmet patient need; advance scientific discovery in unsolved disease areas; develop new targets, drug technology platforms, compounds, etc., that can be tested in clinical settings	Integrate university labs with people, facilities, and equipment from biopharmaceutical companies, service providers, etc., to access resources and expertise, particularly in later stages of drug development (e.g., to run large clinical trials, engage with regulatory agencies, scale-up manufacturing, etc.)
<b>Federal research</b> 	Perform basic research, providing rationale and advancing scientific discovery to serve population health interests; develop new targets, drug technology platforms, and compounds that can be tested in clinical settings	Access resources and expertise, particularly in later stages of drug development (e.g., clinical research, manufacturing)
<b>Health care providers</b> 	Advise innovators during R&D, clarifying patient needs and preferences (including methods for drug delivery and treatment), particularly in basic research, clinical studies, and epidemiological research; execute clinical trials	Obtain funding, resources, and scientific expertise to support complex research approaches; communicate provider and patient needs and preferences as inputs to R&D
<b>Investors</b> 	Provide capital and strategic guidance for biopharmaceutical R&D innovation ecosystem players	Maximize investment returns; support the investigation of and solutions to unmet patient needs
<b>Non-profits</b> 	Promote disease- or condition-specific discovery and research (including regulatory processes, value assessments, etc.); foster relationships with patients and health care providers to ensure the “patient voice” is heard throughout the drug development process	Access capabilities necessary to execute scientific research and stimulate increased understanding of critical unsolved diseases; connect patients and communicate their needs, interests, and availability for potential participation in clinical trials; engage with other ecosystem stakeholders to promote innovation in areas of unmet need
<b>Regulators</b> 	Evaluate safety and efficacy to approve critical, needed new therapies; provide public funding resources and tax incentives to support R&D to address unmet needs; shape health system regulations, uphold laws, and protect the interests of the public	Facilitate expedited delivery of new therapies to patients in need; engage in open collaborations that promote greater understanding of diseases and human biology and those that aim to increase overall R&D productivity
<b>Vendors and contractors</b> 	Supplement in-house resources, often providing specialized services such as data management and analysis, laboratory analytical services, clinical trial recruitment and execution, etc.	Provide specialized expertise, capabilities, and capacity to support partnerships, individual companies, and broader ecosystem needs

### 3. Partnership models and trends

Deloitte analyzed the types of biopharmaceutical R&D partnerships created from 1980 through August 2015, building a comprehensive database of collaborations from EvaluatePharma Life Sciences partnership information, supplemented with non-duplicative data from FasterCures' Consortiopedia. To accommodate the diversity of partnership types and stakeholders investigated as part of this research, Deloitte conducted a manual scan of various PhRMA member company partnerships over the last 10 years (2005–2014) in October 2015, which resulted in the inclusion of additional unique, non-duplicative partnerships. Further, Deloitte interviewed 12 biopharmaceutical industry ecosystem executives, covering individual biopharmaceutical companies and consortia, academia, federal research, and other organizations. The database identified partnerships across five distinct types, described in Figure 3.

These partnership types fall into one of two broad categories—asset-based and non-asset-based. Asset-based partnerships include acquisitions and licensing of compounds, products, or technology. Non-asset-based partnerships include JVs, consortia, and others, such as those focused on education and awareness.

Figure 3: Biopharmaceutical R&D partnership models

#### Asset-based



##### Acquisition

Entity purchases a company, division, portfolio, or one or more specific drugs, in-progress molecules, or products. Can be patient need- or science-driven channel(s) by which companies can enter a new therapeutic area (or increase depth of expertise and knowledge in an existing one), increase investment in new product R&D, move from generics to branded drugs, and/or explore new indications for existing therapies.



##### Licensing

**In-licensing:** Entity obtains licenses or rights for research, development, commercialization, or the marketing of a drug or portfolio of drugs from another entity.  
**Out-licensing:** Entity grants rights to research, develop, commercialize, or market a drug or portfolio of drugs to another entity.

#### Non-asset-based



##### Joint venture (JV)

Two or more entities enter a collaboration wherein all involved parties agree to jointly contribute to R&D-related activities to achieve a specific objective. Typically involve joint governance and decision-making, and sharing of accompanying risks and rewards.



##### Consortium

Three or more parties pool resources and work together to achieve a common goal, such as accelerating scientific discovery in a particular disease area or technology. Some consortia include “pre-competitive” arrangements where all players work together to solve problems and develop capabilities in areas where they would typically compete with each other.



##### Other

Parties provide financial resources and/or marketing, educational, and promotional programs (e.g., company support of broader disease awareness efforts).

## Partnership models and trends

Traditional asset-based partnerships typically involve two parties (such as a pair of biopharmaceutical companies) which are focused on a particular asset (i.e., investigational medicine), and use a structure (a “sponsor” and “partner” model) that distributes control, risks, and rewards. A common objective is to progress a single asset through the R&D process, obtain approval, and launch. Today’s non-asset-based partnerships diverge notably from that model—collaborative alliances may include three or more parties and are often comprised of a mix of ecosystem stakeholders including biopharmaceutical companies, academia, non-profits, and government entities. Importantly, these partnerships feature shared control and decision-making, thus spreading both the potential risks and rewards (Figure 4).

Non-asset-based partnerships, such as consortia, often aim to expand knowledge and understanding within and across one or more indications, therapeutic areas, or

even operational capabilities. An example, TransCelerate BioPharma Inc., formed in 2012, includes leaders from 18 biopharmaceutical organizations, other industry groups focused on clinical standards, and global regulatory agencies. The consortium has developed methodologies, processes, and systems to, for example, improve risk-based monitoring, clinical data standards, and comparator drug supply, and to create a centralized investigator platform ([see TransCelerate case study](#)).

Figure 4: Biopharmaceutical R&D ecosystem partnering trend



“We are funding consortium-based collaboration ‘pods’ where academic and biopharmaceutical industry researchers come together to work on a particular ALS-related target—they are operating in a way that would not be possible without industry involvement.”

**Chris Henderson, PhD, Chief Advisor, Target ALS Foundation<sup>13</sup>**

## Case study: TransCelerate BioPharma Inc.

### Overview

Ten biopharmaceutical companies originally formed a non-profit consortium in 2012 called TransCelerate BioPharma Inc., a pre-competitive collaboration designed to accelerate R&D by changing the methods and tools through which participating companies discover, develop, and deliver new medicines to patients.

*“Not everything [biopharmaceutical companies] do is competitive, some can be pre-competitive ... a rising tide raises all boats.”<sup>14</sup>*

**Dalvir Gill, PhD, Chief Executive Officer, TransCelerate BioPharma Inc.**

The consortium has leadership participation from 18 biopharmaceutical companies; partnerships with industry organizations responsible for standardizing clinical trials; and collaboration and insight from global regulatory authorities, including the FDA (US), EMA (European Medicines Agency, Europe), and PMDA (Pharmaceuticals and Medical Devices Agency, Japan). TransCelerate applies members' collective capabilities to improve the site investigator experience, facilitate information-sharing, enable clinical trial process harmonization, enhance sponsor efficiencies, and improve the patient experience.<sup>15</sup>

#### Examples of its initiatives include:

- **Clinical data standards:** Collaborating with the Clinical Data Interchange Standards Consortium (CDISC) to develop industry-wide standards for priority therapeutic areas that allow for cross-study analysis and data aggregation to enable greater efficiency and streamline regulatory review.
- **Comparator drug supply networks:** Establishing infrastructure to support member company purchase of verified comparator drugs from other members to enhance patient safety and reduce the risk of counterfeit drugs.
- **Shared investigator platform:** Building an online platform that allows participating member companies and clinical investigators to share data through a central standardized platform with greater efficiency.

In February 2016, TransCelerate launched BioCelerate, a subsidiary that aims to improve efficiency in pre-clinical research. Its first initiative, *Toxicology Data Sharing*, is focused on “enabling access to a broader cross-company set of toxicology data ... to modernize toxicology to enhance product safety.”<sup>15</sup>

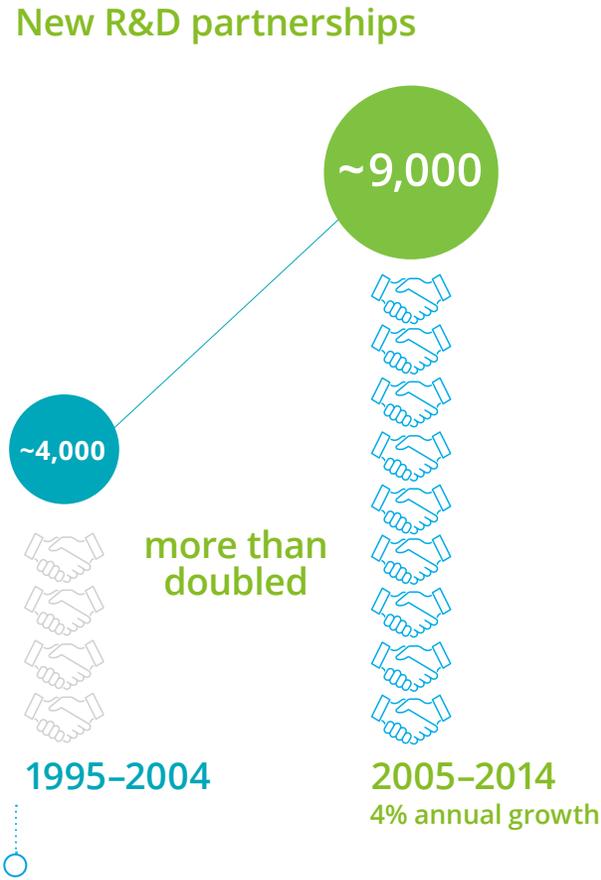
### Potential value to partners

- **Biopharmaceutical:** Participating companies can share R&D leading practices to optimize results and shorten the time needed to discover, develop, and deliver new therapies to patients who need them.
- **Regulators:** An important endpoint will be a potential shift to a more standardized format to enable more efficient regulatory review, R&D efficiencies and, ultimately, earlier approvals of needed new medicines.
- **Other:** Industry organizations such as CDISC are collaborating with TransCelerate to drive their objectives to a broader audience; for example, establishment of new data management standards to enable sharing of relevant clinical data across disease types to improve efficiency across all participants.

## Partnership models and trends

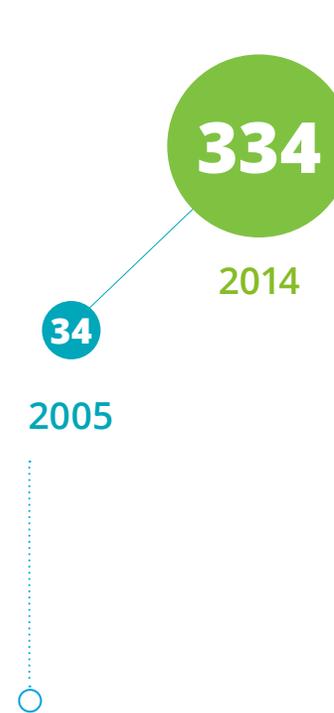
### Substantial partnership growth among biopharmaceutical ecosystem stakeholders

Using our comprehensive database, we assessed the number of *net new* partnerships launched in a given year (versus the number of currently *active* partnerships at any given point in time) and found that partnerships in the biopharmaceutical R&D ecosystem have grown substantially over the past 10 years.



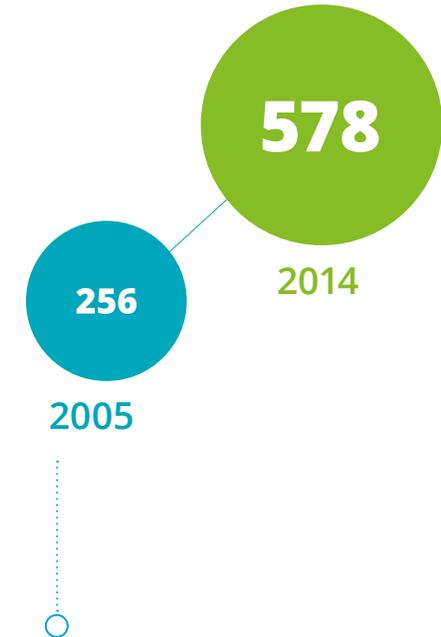
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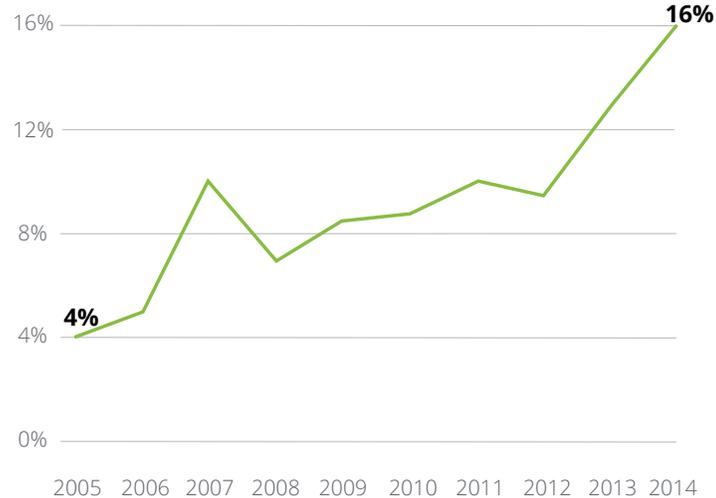
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## Partnership models and trends

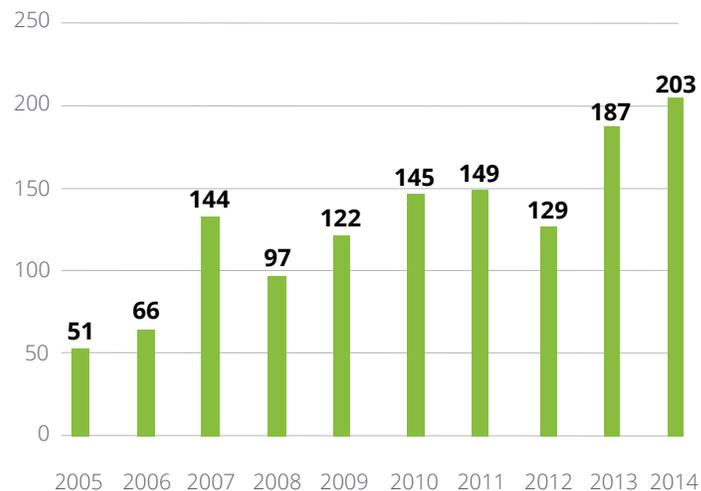
Further analysis of all partnerships formed in recent years provides greater insight into how “typical” partnerships have changed over time. In the last 10 years (2005–2014), there has been a notable increase in more collaborative partnership models such as JVs and consortia (Figure 6).<sup>1</sup> More traditional R&D in-licensing and acquisition-based partnerships declined somewhat over the same period, but still remain a common way to partner.

Figure 6: New joint ventures and consortia, 2005-2014<sup>1</sup>

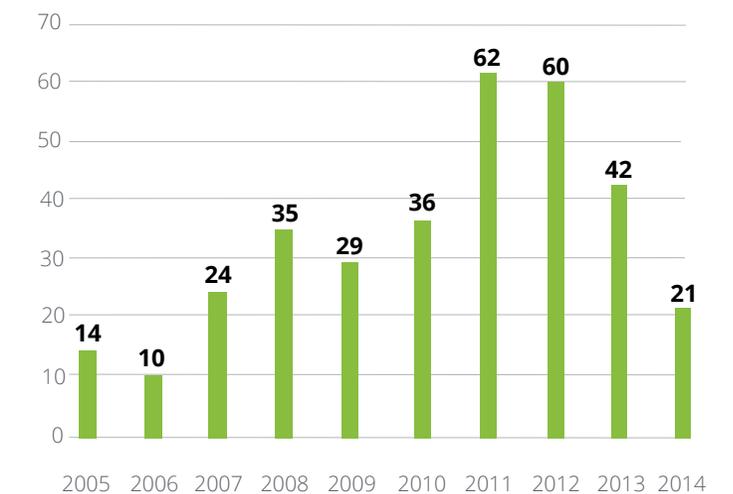
### JV as a % of all new R&D partnerships, 2005–2014



### Number of net new JVs, 2005–2014



### Number of net new consortia, 2005–2014



# Ecosystem collaboration and emerging health care threats

The R&D ecosystem is central in identifying treatments and vaccines for life-threatening illnesses and public health threats such as malaria, Ebola, and the Zika virus. Often, many different stakeholders are engaged in the complex search for solutions but may be challenged by operational and logistical hurdles, conflicting priorities, and scarce resources. Cross-ecosystem collaborations can help address this by bringing stakeholders together to achieve a mutual goal.

### Examples include:

- **Global Alliance for Vaccines and Immunizations (GAVI):** The organization was founded in 2000 when the World Health Organization (WHO), UNICEF, academia, biopharmaceutical companies, and other funding providers came together to address vaccine access challenges in developing markets. Since its inception, GAVI support has helped countries to immunize 500 million children and prevent seven million deaths.<sup>16</sup>
- **Global Health Vaccine Center of Innovation:** Created in 2015 by Sanofi, the Bill & Melinda Gates Foundation, and the Infectious Disease Research Institute (IDRI), this collaboration is focused on new vaccine development and reducing the costs associated with production and distribution. The collaboration aims to capitalize on partners' collective expertise by integrating vaccine development and production knowledge, international development and commercialization capabilities, and partners' broad influence and financial backing.<sup>17</sup>
- **PATH malaria vaccine initiative (MVI):** Created in 2009 by several biopharmaceutical companies, academic, government, and other industry organizations through a grant from the Bill & Melinda Gates Foundation, this initiative aims to accelerate the development of effective malaria vaccines and catalyze timely access in endemic countries. MVI maintains a portfolio with a breadth of preclinical and early clinical projects, with at least one advanced therapy in the pipeline.<sup>18</sup>

## Partnership models and trends

### Already, existing consortia have made progress across the following key challenges and opportunities:

- **Understanding difficult diseases at the molecular level:**  
Growing understanding of the inner workings of complex diseases is revealing important pathways for new research but also unearthing new challenges. The Alzheimer’s Disease Neuroimaging Initiative (ADNI) was formed in 2004 to advance understanding of this devastating disease in order to develop new treatments to slow or stop Alzheimer’s progression. The initiative, formed by the NIH, National Institute on Aging (NIA), FDA, and numerous industry, academic, and non-profit organizations, has made tremendous strides in Alzheimer’s Disease (AD) detection, helping to elucidate the underlying pathways of AD progression, and improving the efficiency of clinical trials related to addressing AD ([see ADNI case study](#)).<sup>19</sup>
- **Quickly and effectively diagnosing and tracking disease progression:** The non-profit Cure Huntington’s Disease Initiative (CHDI) Foundation was created in 2002 by several biopharmaceutical, academic, and contract research organizations (CROs) to focus on developing therapeutics to track and slow the progression of Huntington’s Disease (HD).<sup>20</sup> One accomplishment includes developing an assay to measure the build-up of a protein that is known to be harmful for patients with HD ([see CHDI Foundation case study](#)).
- **Using biomarkers to monitor patients before, during, and after trials:** The Biomarkers Consortium, formed in 2006, is focused on identifying, developing, and qualifying biomarkers for cancer, inflammation and immunity, metabolic disorders, and neuroscience. The term “biomarker” is a hybrid of “biological marker;” it refers to a broad subcategory of medical signs which can be measured accurately and reproducibly. Medical signs stand in contrast to medical symptoms, which are limited to those indications of health or illness perceived by patients themselves.<sup>21</sup> The Biomarkers Consortium includes 30 government, biopharmaceutical, academic, patient advocacy, and other non-profit organizations, and is managed by the Foundation for the National Institutes of Health (FNIH) as a public-private partnership. Some notable achievements via the application of biomarkers to research include development of approaches to accelerate trials for breast cancer, defining sarcopenia (loss of muscle tissue) as a disease (versus simply an effect of the aging process), and predicting long-term results of statins use to treat patients with high cholesterol and/or risk of developing cardiovascular disease ([see Biomarkers Consortium case study](#)).<sup>22</sup>

## Partnership models and trends

- **Developing new ways of conducting clinical trials to take advantage of recent technological and operational advances:** As we move toward an era of increasingly targeted and personalized treatment, the traditional randomized, controlled trial presents some significant challenges. New trial designs, which capitalize on the growing understanding of the underlying biology of disease, are beginning to present new paradigms for conducting clinical research. The Lung Master Protocol (Lung-MAP) consortium is a public-private partnership formed in 2014 by several biopharmaceutical companies, government, and nonprofit advocacy organizations to develop a novel, multi-drug clinical trial for patients with a specific, difficult-to-treat form of advanced lung cancer (squamous cell carcinoma). The trial design leveraged genetic profiling to assign patients to one of five trial arms.<sup>23</sup> Applications of this type of study design have helped to advance precision medicine in oncology and generate interest from biopharmaceutical companies and others in tackling difficult cancers with a personalized approach ([see Lung-MAP case study](#)).
- **Evaluating the potential for combination treatments:** Over time, researchers often identify additional benefits of medicines when used in novel combinations with other drugs. In many cases, the treatments may confer greater benefit together versus when used individually. Recognizing the value of these expanded therapeutic options, academic

centers, biopharmaceutical companies, and nonprofits are collaborating through a partnership called CoNNCT (Collaborative Novel-Novel Combination Therapies) to accelerate identification of effective drug combinations for cancers. The goals of this collaboration are to make it easier to test multiple combinations of new drugs, reduce the cost of investigational studies, shorten the time to demonstrate proof of concept and, ultimately, to accelerate the development of novel treatments in other diseases and conditions ([see CoNNCT case study](#)).<sup>24</sup>

- **Accelerating translational research:** Translating early research findings into therapeutic advances is a challenging process that requires both in-depth understanding of the science as well as specific regulatory and development capabilities. The California Institute for Biomedical Research (Calibr) is a not-for-profit collaborative that is bringing partners together to accelerate translational research in order to develop new medicines for patients with unmet needs across a broad range of disease areas.<sup>25</sup> Building on the success of early, open collaboration, Calibr also has a unique structure that enables commercial partnerships later in the development process ([see Calibr case study](#)).

## Case study: Alzheimer’s Disease Neuroimaging Initiative (ADNI)

### Overview

The Alzheimer’s Disease Neuroimaging Initiative (ADNI) was formed in 2004 as a global research effort to improve understanding and diagnosis of Alzheimer’s Disease (AD), a condition that affects a large number of people and currently has no effective cure. A range of collaborators, including the NIH, NIA, FDA, and numerous industry, academic, and non-profit organizations share study data as they work to better understand AD and its precursors, and define AD progression through collaborative, longitudinal, multicenter research that assesses clinical, imaging, genetic, and biospecimen biomarkers through the process of normal aging to early mild cognitive impairment to late mild cognitive impairment to dementia or AD.<sup>19</sup>

*“Promoting biomedical research, especially for a complex disease like Alzheimer’s, requires the resources and expertise of many partners in both the public and private sectors working together toward a common goal.”<sup>26</sup>*

**Steve Paul, MD, Chairman, FNIH**

Among other accomplishments, ADNI has developed standardized methods for clinical tests, magnetic resonance imaging (MRI), positron emission tomography (PET), and cerebrospinal fluid (CSF) biomarkers; improved clinical trial efficiency; developed

methods for early detection of AD; and helped to reveal some of the mechanisms of normal aging. All of ADNI’s obtained data is made broadly and publically available—collaborators relinquish any patent rights.

In November 2016, the University of Pittsburgh and Pfizer Inc. announced a partnership to develop a computational model that will help identify the drivers of schizophrenia, AD, and related brain diseases and enable researchers to better understand and treat these diseases. The study will use the publicly available datasets of ADNI, which contain images, genetic and biological information, and clinical observations of patients, to develop software that can be used to associate the images with gene patterns.

*“The exciting thing about this type of translational research with Pfizer is that it expands the research impact of what we do at Pitt, inclusively involves participation across our campus, and meets the core missions of both our University and industry partner ... we wouldn’t be able to do this specific research without an industry partner, and we’re thrilled to have Pfizer’s collaboration.”<sup>27</sup>*

**Donald Taylor, PhD, Assistant Vice Chancellor, University of Pittsburgh**

### Potential value to partners

- **Academia:** Focused support, community, and funding enable AD research, with the latest technologies and capabilities provided by a diverse set of consortium participants.
- **Biopharmaceutical:** Promotes better disease understanding and collaboration to accelerate diagnostic capabilities and discovery of potential therapeutic solutions, access to a broader patient population with varying degrees of AD progression and needs.
- **Federal research:** Facilitates collaboration among public and private sector researchers to validate the use of biomarkers for diagnosing, treating, and monitoring AD.
- **Non-profits:** Builds greater awareness of disease nuances among various patient populations and potentially, faster progress toward more effective treatments through support of AD-focused studies by patient advocacy groups such as the Alzheimer’s Association.

## Case study: Cure Huntington’s Disease Initiative (CHDI) Foundation

### Overview

Huntington’s Disease (HD) is a fatal genetic disorder that causes the progressive breakdown of nerve cells in the brain. It deteriorates a person’s physical and mental abilities, often during their prime working years, and currently has no cure.<sup>28</sup> In 2002, a number of biopharmaceutical, academic, and contract research organizations (CRO) partnered to create the non-profit Cure Huntington’s Disease Initiative (now CHDI Foundation). Its goal is “to rapidly develop therapeutics that slow the progression of Huntington’s Disease (HD).”<sup>20</sup> CHDI Foundation funds HD researchers in universities worldwide, manages internal therapeutic programs through CRO collaborations, partners with biopharmaceutical companies for drug development, and enables researchers to engage in HD work.

Since 2002, the CHDI Foundation has made significant strides towards understanding and treating HD, including:

- **Partnering with a biopharmaceutical company to discover and develop an antisense drug for HD.** The company has initiated a Phase I/IIa clinical study of a drug candidate in patients with HD, which is the first potential therapy intended to directly target the cause of the disease by reducing the production of the protein responsible for HD.<sup>29</sup>

- **Developing an assay to measure the build-up of a harmful mutant protein in patients with HD.** Being able to detect and measure the amount of mutant huntingtin (the HD gene) present in the nervous system will help us assess whether the gene-silencing drug is hitting its target and has the intended effect.<sup>30</sup>

*“A lot of what we’ve been doing the last 10 years has reached a stage of maturity—programs are entering later stages of pre-clinical and the early stages of clinical. We are emboldened and are very happy that we have a portfolio that is large in number and crafted for HD.”*

**Robert Pacifici, PhD, Chief Scientific Officer, CHDI Management, Inc. / CHDI Foundation, Inc.**

*“The initial development of this antisense drug for Huntington’s Disease came out of a longstanding productive partnership between Ionis and CHDI.”*

**Robi Blumenstein, President, CHDI Management, Inc.**

### Potential value to partners

- **Academia:** Academic researchers can seek funding from the CHDI Foundation and gain access to resources (e.g., reagents, antibodies, mouse models, clinical samples) and datasets that facilitate discovery and development of promising new drugs.
- **Biopharmaceutical:** Companies can collaborate with CHDI Foundation to accelerate the development of potential new drug candidates.
- **Non-profits:** The CHDI Foundation serves as a non-profit organization to fund and support therapeutic development for HD through a virtual platform intended to facilitate greater connectivity between academic research and clinical development, with the goal of accelerating development of new approaches to managing the disease.
- **Vendor and contractors:** CROs can partner with CHDI Foundation staff scientists who manage internal drug programs through trials that the CROs themselves can run (in addition to those run with biopharmaceutical companies).

## Case study: Biomarkers Consortium

### Overview

The potential role biomarkers can have in accelerating the detection, diagnosis, and treatment of diseases is well recognized; however, as of 2007, few biomarkers had been qualified for clinical use.<sup>31</sup> Developing biomarkers requires a diverse set of capabilities: deep knowledge of disease risk, natural history, and outcomes; sufficiently large samples; and ability to properly analyze biomarker data.

In 2006, 30 government, biopharmaceutical, academic, patient advocacy, and other nonprofit private-sector organizations combined to form the Biomarkers Consortium, a public-private, pre-competitive partnership managed by the Foundation for the National Institutes of Health (FNIH). This collaboration seeks to rapidly identify, develop, and qualify potential high-impact biomarkers, particularly to enable improvements in drug development, clinical care, and regulatory decision-making.<sup>22</sup>

The consortium is currently focused on cancer, inflammation and immunity, metabolic disorders, and neuroscience—there is a dedicated steering committee for each of these areas with representatives from all involved stakeholder groups. Each steering committee is responsible for identifying and moving forward promising pre-competitive biomarker projects for implementation by the consortium, as well as overseeing each individual project under its purview.<sup>22</sup>

The Biomarkers Consortium has launched a number of projects across its focus areas which use biomarkers in medical diagnostics and new drug development, including:

- *I-SPY 2 Trial (cancer)*: An adaptive, multidrug phase II trial in high-risk breast cancer patients that seeks to accelerate the trial process. Each patient's tumor is molecularly profiled then a potential treatment likely to work for that tumor profile is assigned. Two drugs completed testing in 2013 and five additional drugs are in active testing with results expected through 2019.
- *Sarcopenia Project (metabolic disorders)*: In 2013, this project generated the first evidence-based comparison of criteria for clinically relevant muscle weakness that goes beyond what might be expected via the aging process.
- *Atherosclerosis In Silico Modeling (metabolic disorders)*: An atherosclerosis (thickening of artery walls) model will be developed in silico (via computer modeling) using published data to identify a set of short-term biomarkers that predict long-term clinical outcomes after statin therapy, as well as biomarkers of residual risk after statin therapy. This can help better target which patients may or may not benefit from taking statins to treat high cholesterol and/or reduce the risk of developing cardiovascular disease.<sup>27</sup>

### Potential value to partners

- **Biopharmaceutical**: Member companies can develop evidence to help qualify biomarkers for specific applications in diagnosing disease, predicting therapeutic response, and improving clinical practice, which can have value across many of their current and future drug development programs.
- **Government**: Researchers can collaborate with biopharmaceutical companies to validate biomarkers in certain disease areas and assess the use of biomarkers in disease identification, monitoring, and treatment.
- **Non-profits**: Patient advocacy and other nonprofit groups support studies in particular disease areas to advance scientific understanding and more rapidly identify patients needing treatment.
- **Regulators**: The FDA can use consortium-driven studies to better understand the role of biomarkers in certain diseases and facilitate regulatory approval for submissions that include a biomarker component.

## Case study: Lung Cancer Master Protocol (Lung-MAP)

### Overview

About 80 to 85 percent of lung cancers are non-small-cell lung cancers. Despite advances in non-small-cell lung cancer treatments, few alternatives to surgery currently exist to treat squamous cell carcinoma, which comprises between 25 and 30 percent of all lung cancers. A number of mutations have been found in squamous cell lung cancer and researchers are increasingly focusing on developing cancer therapies to target certain mutations.<sup>32</sup>

In 2014, several biopharmaceutical, federal research, and non-profit/advocacy organizations came together to develop a novel, multi-drug clinical trial called the Lung-MAP clinical trial, which is focused on patients with recurrent stage IIIB-IV squamous cell lung cancer. This first-of-its-kind clinical trial uses a targeted method to match patients with studies of a number of new treatments being researched. Patients with advanced or incurable stage IIIB/IV squamous cell carcinoma are assigned to one of five trial arms based on the genomic profile of their tumors.<sup>23</sup> This design strives to increase efficiencies in several areas by allowing multiple drugs to be tested simultaneously, giving some form of treatment to all patients, and easing the burden associated with recruiting patients for multiple distinct studies.

When launched, the trial anticipated screening between 500–1,000 patients each year for over 200 cancer-related genes.<sup>33</sup>

As of February 2016, Lung-MAP is offered at over 700 US medical centers and community hospitals.<sup>34</sup> This approach is helping to advance the development of targeted therapies tailored to specific patient genetic traits—and has addressed the traditional patient recruitment burden, particularly in disease areas with unique or small patient populations.

*“This trial is breaking down the old paradigms of traditional clinical trials, allowing multiple enrollees to be tested and assigned to the treatment most likely to work for them ... Lung-MAP takes advantage of new advances in biomarkers, targeted therapies, and advanced-stage cancer treatments while tailoring these treatments to those who need them most—patients.”<sup>34</sup>*

**Ellen V. Sigal, PhD, Chair and Founder, Friends of Cancer Research**

### Potential value to partners

- **Biopharmaceutical:** Participating companies can enhance their capabilities in genomic profiling and its application to identify likely to benefit from treatment, potentially addressing a known unmet need for treatments for certain types of lung cancer.
- **Federal research:** Using genomic profiling to establish clinical trial arms is a novel way of potentially increasing clinical trial efficiency, which can help evolve clinical trial standards and facilitate the regulatory approval process.
- **Health care providers:** Participation may increase treatment options for their patients.
- **Non-profits:** Patient advocacy associations often support collaborative approaches such as this to accelerate the development of potential new treatments.

# Case study: The CoNNCT Initiative

## Overview

Recognizing the value of novel drug combinations for the treatment of various cancers, academic centers, biopharmaceutical companies, and nonprofit entities are collaborating on a new paradigm to accelerate and broaden the clinical testing of drug combinations to facilitate early go/no-go decisions and assess effective drug combinations. This model distributes development risk across participants and has the potential to improve the traditional clinical trial model in terms of efficiency and cost. The goals of this collaboration include making it easier to test multiple combinations per agent, reducing the cost of early investigational studies, and shortening the time to demonstrate proof of concept.

In 2016, a planning workshop was held with a variety of stakeholders ranging from biopharmaceutical companies, diagnostics companies, academic clinicians, and representatives of cancer nonprofits, FDA, and other organizations to outline focus areas for the collaboration. Recommendations resulting from the workshop include establishing that CoNNCT (Collaborative Novel-Novel Combination Therapies) should focus on a pilot study (or studies) around defined hypotheses

of combinations where one core or anchor product is combined with multiple products, with agreement that the scope would expand and evolve over time. Participants also agreed on the organization and funding structure, concluding that a nonprofit entity would lead the initiative; that participants' roles would be clearly defined; and that an agreed-upon funding model could facilitate each participating biopharmaceutical company making equal contributions and that other sources of funding would be sought to help offset costs. The group also reached consensus on approaches to contract negotiation and clinical study design.<sup>24</sup>

*“CoNNCT is the first time a group of this kind is focusing on novel-novel drug combinations for potential treatments that have not yet been reviewed by the FDA. Our goal is to work together to speed the decision-making around go/no-go decisions in oncology combination drug development using higher throughput and early signal finding.”<sup>24</sup>*

**Keith T. Flaherty, MD, Director of Developmental Therapeutics, Massachusetts General Hospital and Director of the Termeer Center for Targeted Therapies**

## Potential value to partners

- **Biopharmaceutical:** For participating large companies CoNNCT can provide a new platform for expediting multiple, new, cross-country novel-novel drug combinations at the same time. Small companies could gain access to more diverse pipelines and more streamlined partnerships for accelerating trials of their own pipeline products.
- **Health care providers:** Clinicians may be able to test more novel drug combinations in a more efficient manner; in addition, they could benefit from streamlining the collaboration process.
- **Non-profits:** These groups, which often contribute to similar initiatives, provide important insights as well as impartiality with participating biopharmaceutical companies and other members which, in return, play a pivotal role in advancing the goals of the partnership. CoNNCT makes use of a nonprofit oversight construct to govern the collaboration so members' contributions and benefits are of equivalent value.
- **Regulators:** The FDA is involved in this partnership at an early point and will be able to work with the sponsors of the pilot studies to address concerns and clarify potential approval paths for novel-novel drugs under development.

## Case study: Calibr

### Overview

California Institute of Biomedical Research (Calibr) was formed in 2012 via investment by Merck & Co., Inc., as a nonprofit organization focused on developing innovative therapies that address unmet patient needs. Calibr strives to maximize academic and nonprofit scientists' involvement. Calibr has a unique operating model; the organization distributes profits resulting from discovery sales equally among its scientists and partners, regardless of how the discovery is used or licensed.<sup>25</sup>

Calibr tracks progress through the generation and development of therapeutic candidates and increasingly strives to demonstrate its ability to translate lab-based discoveries into tangible patient outcomes. Current projects include CAR-T cell therapies for cancer and drug candidates for multiple sclerosis, cystic fibrosis, chronic obstructive pulmonary disease (COPD), and tuberculosis.<sup>35</sup> In 2015, Calibr was awarded funds to be used to conduct trials on a treatment that could modify the progress of osteoarthritis, which afflicts 27 million Americans. The therapy could also potentially be used to treat damaged cartilage in knees and elbows.

Recently, Calibr entered into a global strategic collaboration with Pfizer Inc. to develop treatments for heart failure. Calibr has a proprietary antibody fusion technology that enables a modular approach to developing long-acting biotherapeutics.<sup>36</sup>

*"This collaboration leverages Calibr's ability to progress the program rapidly through first-in-human studies and provides access to ... state-of-the-art therapeutic development capabilities."*

**Peter G. Schultz, PhD, Chairman and President, Calibr<sup>36</sup>**

### Potential value to partners

- **Academia:** Researchers can collaborate with and are supported by biopharmaceutical representatives affiliated with Calibr, and are further supported via funding from the NIH for select programs. In addition, Calibr has an alliance with the Scripps Research Institute to create a more effective "bench-to bedside model that accelerates the time and decreases the cost of developing new medicines for the public benefit."<sup>25</sup>
- **Biopharmaceutical:** Participating companies have funded and entered into strategic alliances to license therapeutic compounds of interest or support development in specific disease areas. Calibr provides them access to promising drug candidates that can be developed and brought to patients in need more quickly.
- **Non-profits:** Nonprofits often aim to harness advances in science and technology to save lives in developing countries, and work with partners such as Calibr to develop and deliver to these regions affordable and reliable vaccines, drugs, and diagnostics.

## 4. Biopharmaceutical R&D partnerships: What lies ahead?

Our research and the results of our interviews with key ecosystem leaders point to a growing recognition of the value and importance of collaboration across members of the R&D ecosystem to address the most pressing scientific and technological challenges and harness complementary strengths to bring new treatments to patients. In the coming years, we expect to see continued expansion in disease area-focused consortia, including growing emphasis on more “open” arrangements with respect to structure, control, risk sharing, and other business arrangements. Regulators and the health care delivery system, particularly health plans, need an ever-larger evidence base (including patient-generated data, patient-reported outcomes, and RWE) to inform review and approval of drug applications. Additionally, evolving innovative coverage and payment

models could fuel an even broader range of partnerships in the future.

### **Growing diversification of partnership models and approaches**

Active innovators seek to develop a portfolio of partners with a diverse mix of scientific and operational capabilities to support non-asset-based partnerships. Through this approach they are spawning a variety of open partnership structures and governance models, with diverse objectives and ways to measure progress and success (see additional example joint ventures and consortia in Appendix, Table 1).

Notable achievements from existing pre-competitive collaborations—many described in this report—show the potential for sharing knowledge and capabilities across peers

without jeopardizing any participating organization’s opportunity to succeed. Lessons learned from these accomplishments can be used to enable more pre-competitive collaboration in additional research areas, increasing the potential for new breakthroughs.

Over the past decade, partnership models have increasingly included more open and collaborative structures and objectives, particularly via JVs and consortia that are designed to bring together the right expertise and resources to propel innovation and bring new medicines to patients in need more quickly and safely. R&D ecosystem stakeholders also have been partnering earlier in the new product development lifecycle, integrating innovative research approaches and resources with proven drug development capabilities to accelerate insights. Going forward, we can expect to see additional results and lessons learned from existing partnerships incorporated into new partnership approaches.

## 5. Appendix:

Table 1: Selected joint ventures and consortia

Therapeutic area	Partnership; year formed	Primary stakeholders	Objectives, example results to date
Cardiovascular	<b>Cardiovascular Genomics and Drug Discovery Research Collaboration; 2015</b>	Bayer HealthCare, Broad Institute of MIT and Harvard	This collaboration leverages insights from human genomics to create new cardiovascular therapies. Participants collaborate on genetic discovery, target validation, and drug discovery activities. It is looking to leverage the Broad Institute's genomic analysis experience to further study factors not related to lifestyle choices but rather in-born factors such as age and severity of onset. <sup>37</sup>
	<b>One Brave Idea; 2015</b>	American Heart Association, Google Life Sciences (Verily), AstraZeneca	This \$75 million research project will focus on causative factors and consequences, cardiovascular risk factors, and restoration of cardiovascular health. <sup>38</sup>
Oncology	<b>The CoNNCT Initiative: Accelerating Novel Combinations for Cancer; 2016</b>	Over 40 academic centers, biopharmaceutical companies, and nonprofit entities	This partnership is intended to accelerate identification of effective drug combinations for cancer. The goals of this collaboration are to make it easier to test multiple combinations of new drugs, reduce the cost of investigational studies, and shorten the time to demonstrate proof of concept. <sup>24</sup>
	<b>Lung Cancer Master Protocol (Lung-MAP); 2014</b>	National Cancer Institute, Foundation for the National Institutes of Health (FNIH), several biopharmaceutical companies and industry organizations	This public-private partnership seeks to develop a novel, multi-drug clinical trial for patients with squamous cell carcinoma. The trial design leveraged genetic profiling to assign patients to one of five trial arms. Applications of this type of study design have helped to advance precision medicine in oncology. <sup>23</sup>
Neurology	<b>Parkinson's Disease Education Consortium; 2016</b>	Michael J Fox Foundation (MJFF), several biopharmaceutical companies, and other ecosystem stakeholders	This consortium seeks to offer new educational assets including a "Stages of Disease" toolkit, educational webinars, and podcast series. The objective is to increase patient and caregiver education, potentially leading to better health outcomes through specialist care, use of new treatments, and participation in clinical research. <sup>39</sup>
	<b>Multiple Sclerosis Partnership; 2015</b>	Google Life Sciences (Verily), Biogen	The partnership's primary objective is to explain how and why multiple sclerosis progresses differently from patient to patient. Using sensor data and software, the companies seek to draw insights by pooling data, running queries, and developing a better understanding of biomarkers and safety markers. <sup>40</sup>

## 5. Appendix:

Table 1: Selected joint ventures and consortia

Therapeutic area	Partnership; year formed	Primary stakeholders	Objectives, example results to date
Multiple, Other	<b>Accelerating Medicines Partnership (AMP); 2014</b>	NIH, FDA, various biopharmaceutical companies and nonprofit organizations	This partnership's goal is to redesign the model for diagnostic and treatment development through the identification and validation of promising biological targets. The collaboration focuses its efforts in three disease areas: Alzheimer's Disease, type 2 diabetes, and autoimmune disorders of rheumatoid arthritis and systemic lupus erythematosus. AMP aims to streamline development and create cost efficiencies, provide a more thorough understanding of biological targets, and increase the effectiveness of new targeted therapies by reducing failures in Phases II and III. Partners have agreed to make all data and analysis publicly available for the biomedical community. <sup>41</sup>
	<b>Coalition for Accelerating Standards and Therapies (CAST); 2012</b>	Clinical Data Interchange Standards Consortium (CDISC), Critical Path Institute (C-Path), Association of Clinical Research Organizations (ACRO), Innovative Medicines Initiative, National Cancer Institute, TransCelerate BioPharma Inc., various biopharmaceutical companies, FDA	This joint initiative of C-Path and CDISC collaborates with numerous organizations (including other consortia) to promote research in therapy areas with significant potential public health impact. It intends to accelerate clinical research and medical product development by redefining and streamlining data standards, tools, and methods. CAST has published reports outlining standards for disease areas such as Alzheimer's, asthma, influenza, diabetes, and cardiovascular disease. <sup>42</sup>
	<b>GRI Joint Research Collaboration; 2016</b>	Juvenile Diabetes Research Foundation (JDRF), Sanofi, academia	JDRF and Sanofi entered an up to \$4.6 million agreement with four research organizations to develop Glucose Responsive Insulins (GRIs) to improve treatment of type 1 diabetes. Sanofi's Research and Translational Medicine team will provide insulin research to researchers funded in this collaboration, with JDRF providing overall guidance. <sup>43</sup>
	<b>PatientsLikeMe-AstraZeneca Research Collaboration; 2015</b>	PatientsLikeMe, AstraZeneca	Partners signed a five-year agreement to allow AstraZeneca to access PatientsLikeMe's global network and patient-reported data to guide drug development, with the intent of improving patient engagement and outcomes through utilization of patient-reported data. The project will initially focus on respiratory disease, lupus, diabetes, and oncology, and includes data from 250,000 patients. <sup>44</sup>
	<b>Project Data Sphere (PDS); 2013</b>	Various biopharmaceutical companies, FDA, NIH	This independent, not-for-profit initiative of the CEO Roundtable on Cancer's Life Sciences Consortium (LSC) is aimed at driving innovation and accelerating research by connecting researchers affiliated with biopharmaceutical companies, hospitals, and institutions, as well as independent researchers, so that they may share, integrate, and analyze patient-level, comparator arm data from academic and industry-sponsored cancer trials. The project recently achieved a one-year goal to integrate data from 25,000 patient lives. <sup>45</sup>
	<b>Reagan-Udall Foundation for the FDA; 2012</b>	FDA, patient groups, academia, various biopharmaceutical companies, Bill & Melinda Gates Foundation	This independent, not-for-profit organization was created by Congress to advance regulatory science; designed to be a vehicle for bringing an array of resources and perspectives to bear on high-priority FDA regulatory science projects. An example initiative is the Critical Path to Tuberculosis Drug Regimens (CPTR), a partnership with the Bill & Melinda Gates Foundation, the Critical Path Institute, and the Tuberculosis (TB) Initiative. The initiative intends to accelerate the development of new TB multi-drug regimens. <sup>46</sup>

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