



Cornerstones of Collaboration

FOUNDATION-LED PARTNERSHIPS TO ACCELERATE R&D



FasterCures
A CENTER OF THE MILKEN INSTITUTE

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ABOUT *FASTERCURES*

FasterCures, a Washington, D.C.-based Center of the Milken Institute, is driven by a singular goal: to save lives by speeding up and improving the biomedical research system. We focus on cutting through the roadblocks that slow medical progress by expanding the science of patient input, fostering policies to support biomedical innovation, and spurring cross-sector collaboration in research to get better results.



Collaboration 2.0

This report is released under *FasterCures*' Collaboration 2.0 program. Biomedical innovation is increasingly complex and requires cross-sector, multi-disciplinary collaboration to make progress. The R&D ecosystem needs to routinely harness collective power and share knowledge and experiences to achieve better results. *FasterCures* champions efficient and effective R&D collaborations by creating and sharing resources and providing platforms for these necessary multi-stakeholder interactions.

ACKNOWLEDGMENTS

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Introduction

Multi-stakeholder collaboration isn't just the participation trophy of biomedical research and development (R&D), a nice way to make sure everyone feels included.

No one organization or sector alone can bring a scientific discovery through product development. The process takes years and often requires participation from patient advocates, academia, industry, and government. We're beginning to appreciate just how hard cracking our biological code is in many instances and that we need to engage partners from diverse disciplines and with a range of skills that have not always worked closely with one another.

Stakeholders in R&D increasingly look to foundations to be the “honest brokers” to lead these collaborations. Building and sustaining a collaborative venture with sometimes competing interests, however, is not for the faint of heart and is a far cry from the individual grant-making process that foundations have focused on in the past.

In April 2017, *FasterCures* convened a select group of foundation leaders and other stakeholders to take the conversation about the partnerships that venture philanthropies are leading to a new level of specificity (see Participant List on page 22). We examined different types of relationships and agreement structures, how the agreements originate and what's necessary to sustain them, and how partners are addressing legal and cultural challenges that can divide them.

THROUGHOUT THE WORKSHOP, PARTICIPANTS DISCUSSED:

- > When is collaboration the right solution, and what forms might it take?
- > How can foundations ensure that their mission and values are preserved while taking into account their partners' differing interests and needs?
- > What are the main obstacles to creating efficient and effective collaborations, and what are examples of when it has worked well?
- > What resources exist—or are needed—to address key issues, such as legal agreements, data sharing, and intellectual property (IP) protection?

We asked participants to provide specifics and to come armed with documents, legal language, templates, and recommendations for tools and resources that they could share with the community. Those documents became the backbone of a new “Foundations as



Collaboration Conveners” toolkit, available on *FasterCures*’ TRAIN Central Station web site, which will be an evolving repository of resources for the community.

PARTICIPANTS IN THE WORKSHOP DROVE THE DISCUSSION TOWARD ACTION WITH AN AGENDA FOCUSED ON:

- > What are the most significant challenges for partners internally and externally that need to be addressed?
- > What tools and resources would be useful in streamlining the planning and execution of collaborative initiatives by foundations?

FASTERCURES’ FRAMEWORK FOR EFFECTIVE CONSORTIA

Consortia serve to advance research efforts that address a shared but unmet need. In 2013, *FasterCures* initiated its Consortia-pedia project to highlight the growing trend of research-by-collaboration, to map the landscape of consortia, and to illuminate the value of these formal collaborations when done well. We developed the following framework to better guide conversations and thinking around these types of partnerships. Alignment of mission and expectations and a clear understanding of the nature of the partnership are key to the success of any consortium.

START-UP: MISSION AND VISION

- Who are my partners? What incentives drive each of the organizations joining this consortium?
- Do we share an unmet need that can advance both a shared goal and our unique objectives?
- Can we coalesce around a shared vision for moving forward?
- What are the outputs and outcomes of this effort? Who are the beneficiaries?

ESTABLISHING AND MAINTAINING RELATIONSHIPS: TERMS OF ENGAGEMENT

- What assets and resources can each partner bring to the effort?

- What resources are needed to augment existing assets? How do partners access those external resources?
- What policies and practices can each partner agree to regarding data sharing, IP, conflict of interest, material sharing, confidentiality, and data access?

EVALUATION AND SUSTAINABILITY

- What accountability measures must be in place to track progress and impact?
 - > Equitable and timely contributions of resources and effort from all participants
 - > Scientific milestones on research projects
 - > Strategic milestones on consortium progress toward mission
 - > Procedures to ensure return-on-investment to participants and sponsors
 - > Other strategic measures and mission-driven considerations
- How will metrics be used to provide real-time feedback, and how will the feedback impact the trajectory of the consortium?
- Are there external factors that must be considered in the near- and long-term that could potentially shift the focus of the consortium or alter the nature of the partnership?

Why are Foundations Becoming Collaboration Conveners?

FasterCures' TRAIN (The Research Acceleration and Innovation Network) program is an affinity network of strategically focused, high-impact disease research foundations. TRAIN provides a platform for foundations to connect with one another and with other stakeholders in the R&D system to learn and collaborate. For more than a decade, we have seen the issues these foundations are compelled to address expand beyond their traditional grant-making missions to encompass building translational infrastructure, supporting early-stage biopharma companies, informing regulatory decision-making, and even influencing the dialogue around the value of and reimbursement for new treatments.

What happened to the “good old days” of raising money and giving it away in investigator-initiated grants? Aren't multi-institutional or multi-stakeholder collaborative initiatives harder for foundations to execute? We asked the workshop participants why they now consider this to be part of their job descriptions.

- > **“BECAUSE WE HAVE NO CHOICE,”** one attendee immediately chimed in. If solving the problems in these disease areas were easy, it would be done by now. The science is complex, the R&D process is challenging, and there are many parties that need to be involved. “None of us can do this work alone.”
- > **BECAUSE “WE HAVE THE END IN MIND,”** said another. “To change patient outcomes, all players need to think backwards from what they're trying to achieve.” Foundations can serve as honest brokers, bringing together the diverse parties necessary to move an effort forward. Foundations have a fundamentally different bottom line than other players in the system. They are focused on moving promising ideas through development and improving patients' lives as quickly as possible, so they can afford to take risks with their capital that other players may not be willing to take. As one participant asserted, “Foundations, particularly private founda-

WORKSHOP WISDOM

Why collaborate?
“Because we have
no choice. **None
of us can do this
work alone.**”

ACCORDING TO RESPONDENTS TO OUR PRE-WORKSHOP SURVEY, SUCCESS IN COLLABORATIONS COMES FROM:

- > Hard work, perseverance, and reasonable common goals
- > Clear objectives and responsibilities
- > Knowledge exchange between trusted partners
- > All partners agreeing to goals from the start and being willing to take some risk

tions that don't have to go out and continually raise money, have an obligation to take on risk.”

- > **BECAUSE FOUNDATIONS CAN BUILD A BRIDGE BETWEEN BASIC AND APPLIED SCIENCE.** While they appreciate the need for fundamental scientific inquiry, a foundation's ultimate goal is always applying science to help patients. “We are interested in advancing basic understanding of disease mechanisms, which [is not the same as] basic science,” said one participant.
- > **BECAUSE FOUNDATIONS ARE DRIVEN BY A SENSE OF URGENCY TO STREAMLINE PROCESSES, REDUCE REDUNDANCIES, LEARN FROM FAILURES, AND ENABLE COMMUNICATION AMONG PLAYERS IN THEIR DISEASE AREAS.** They increasingly want to change the culture of medical R&D by more actively managing the research process and putting funding policies in place that incentivize behaviors, such as collaboration, to accelerate progress across the system.

Defining Collaboration

What do we mean when we say “collaboration?” Technically, it could be as simple as two researchers sharing their independent results, or it could be a hand-off of a research project from one party to another. For the purposes of this workshop, we were focused on more complex, multi-party efforts.

In *FasterCures*’ Consortia-pedia report, we defined consortia as initiatives characterized by:

- > **INTEGRATION OF RESEARCHERS** from multiple sectors (academic, government, industry, nonprofit, clinical care), or researchers from the same sector that normally “compete” with each other;
- > **AGREEMENT ON A MISSION** that addresses a shared need with a strategic and milestone-driven plan to achieve outputs that, in turn, can be broadly used by each stakeholder;
- > A **GOVERNANCE STRUCTURE** that provides each stakeholder with an opportunity to offer input to the partnership’s strategic objectives and operations; and
- > An **INTEGRATED RESEARCH PLAN** that leverages the resources and knowledge from each stakeholder.

Collaborations have diverse research objectives, including advancing knowledge, conducting biomarker research, creating broadly used tools, enabling data sharing, and developing products. The parties and objectives of the collaboration will influence important details of the partnership, such as participation incentives and agreement terms.

While the focus of *FasterCures*’ collaboration workshop was on formal partnerships, informal partnerships are common as well. Formal collaborations involve written agreements, milestones, and accountability. But foundations can foster informal collaboration by provid-

WORKSHOP WISDOM

“If you’re doing it **right**, you’re not just a funder, you’re a **partner.**”





ing forums for, and lowering barriers to, parties working together. One attendee made the important point that foundations don't need to drive every collaboration: "We don't always have to be the hub of the wheel, but we can help facilitate collaboration among sectors without always being the direct convener." Foundations are also creating platforms for collaboration by funding the development of registries, natural history studies, clinical research, and care networks—translational research tools that can attract and incentivize other stakeholders to work together. Finally, as another attendee noted, sometimes working alone is faster—the choice to collaborate should be based on your objectives.

Is it possible to have a universal recipe for R&D collaboration, or is every engagement a custom activity? To some extent every collaboration is unique—one participant noted that "If you've seen one of our deals, you've seen one of our deals." But another attendee noted that each organization needs to have some guiding principles that inform the initiatives in which they are involved. One organization that facilitates a large number of consortia summarized its **GUIDING PRINCIPLES** as:

- > Science is the most important driver.
- > What is our unique contribution—why should we lead this?
- > Clear governance structures and understandings.
- > The ability to walk away.

WORKSHOP PARTICIPANTS ARE EXPERIENCED COLLABORATORS

Many of the participants in the workshop have already convened and led multi-stakeholder, collaborative R&D initiatives with a wide range of objectives and partners. Here is a small sample of such initiatives.

FOUNDATION CONVENER	NAME OF COLLABORATION	PURPOSE	PARTNERS
AMERICAN HEART ASSOCIATION (AHA)	AHA PRECISION MEDICINE PLATFORM	Allows researchers and clinicians to access and analyze vast and diverse data to facilitate collaboration and accelerate breakthroughs in prevention, treatment, and cures for heart disease and stroke.	Amazon Web Services, AstraZeneca, Cedars-Sinai Heart Institute, Dallas Heart Study, Duke Clinical Research Institute, Intermountain Medical Center Heart Institute, International Stroke Genetics Consortium, and Stanford Cardiovascular Institute
AMYLOIDOSIS FOUNDATION	AMYLOIDOSIS RESEARCH CONSORTIUM	Works to accelerate the development of advanced diagnostic tools and effective treatments for systemic amyloidosis.	25 academic research centers
CHILDREN'S TUMOR FOUNDATION	NF PRECLINICAL INITIATIVE	Works to accelerate proof of concept testing of potential effective repurposed drugs in neurofibromatosis- (NF-)relevant models, and to frontload the clinical pipeline with new drug candidates for NF1.	Four leading NF academic laboratories with plans in 2017 to expand to partners in the pharmaceutical industry
COPD (CHRONIC OBSTRUCTIVE PULMONARY DISEASE) FOUNDATION	COPD BIOMARKER QUALIFICATION CONSORTIUM	Pools existing data from clinical studies evaluating various biomarkers to provide sufficient information to qualify them so that the U.S. Food and Drug Administration (FDA) and the European Medicines Agency can use them to evaluate new treatments.	GlaxoSmithKline; Boehringer-Ingelheim; AstraZeneca; Pfizer; National Heart, Lung, and Blood Institute; and FDA
CURE DUCHENNE	COLLABORATIVE TRAJECTORY ANALYSIS PROJECT	Works to unleash the power of collaborative data science on clinical trial design, potentially helping the entire community to bring effective new therapies to patients more quickly.	Pfizer, BioMarin, Shire, Sarepta, PTC Therapeutics, Solid Biosciences, Catabasis Pharmaceuticals, Bristol-Myers Squibb, and Parent Project Muscular Dystrophy
FOUNDATION FOR THE NIH	BIOMARKERS CONSORTIUM	Identifies, develops, and qualifies biomarkers to advance specific applications for diagnosing disease, predicting therapeutic response, and improving clinical practice using new and existing technologies.	National Institutes of Health (NIH), FDA, Centers for Medicare and Medicaid Services, more than 20 biopharma companies, and five nonprofits
JDRF	ENCAPSULATION CONSORTIUM	Develops a product that will hide implanted beta cells from the immune system and allow people with type 1 diabetes to live life as if they don't have the disease.	More than 25 research institutions
ONE MIND	TRACK-TBI	Coordinates a national collaboration among Level I Trauma Centers that will enroll 3,000 patients in the largest longitudinal study of TBI (traumatic brain injury) ever undertaken.	11 research universities
UNITIO, INC.	T1D EXCHANGE	Coordinates a network of clinical care and research centers, combined with a registry, biobank, and social network, offering researchers access to aggregated clinical, biological, patient-reported outcomes, and electronic health record data.	77 clinics, patients, physicians, researchers, and industry representatives

PARKINSON'S PROGRESSION MARKERS INITIATIVE: IT'S A TOUGH JOB, BUT SOMEBODY'S GOT TO DO IT

The Parkinson's Progression Markers Initiative (PPMI), sponsored and coordinated by The Michael J. Fox Foundation for Parkinson's Research (MJFF), is an observational clinical study partnership among researchers, funders, and study participants working toward the goal of identifying progression biomarkers to accelerate Parkinson's disease drug development. This collaboration is based on the model of the Alzheimer's Disease Neuroimaging Initiative and involves 20 industry partners and more than 30 study sites at academic centers. The data from the study are updated weekly and available for researchers to download on the PPMI site, and industry uses learnings from the study to design clinical trials.

Facilitating exchange between partners and researchers, as well as making data publicly available, is expensive: \$75 million over 10 years. Although this study is having an immense impact on Parkinson's disease research, it was, and still is, according

to MJFF, difficult to explain to donors why this collaboration is worthy of investment. The delayed gratification between starting the initiative and seeing returns is a significant struggle.

In addition, launching PPMI caused a shift in organizational focus toward increasing efforts to engage patients in order to recruit nearly 2,000 participants for the study. The foundation had to invest internally in human capital to manage the operations of PPMI. Today, there are five dedicated staff for the initiative. MJFF also encountered challenges when trying to bring other stakeholders onboard since its internal policies on data sharing and other issues were more open than its partners were comfortable operating under.

While launching and supporting PPMI has taken effort, MJFF has focused on reinforcing its common goal and providing leadership as a neutral convener, which has enabled the study to grow in scale and scope and contributed significantly to the field.

CHDI FOUNDATION: MANAGING SCIENCE TOWARD BETTER OUTCOMES

CHDI Foundation, a privately funded, nonprofit research organization focused on Huntington's disease (HD), considers itself to be a "collaborative enabler." It employs a "novel virtual model that encourages scientific collaboration to more directly connect academic research, drug discovery, and clinical development." It does this in part by making all resources developed with its support freely available to all in the HD research community and ensuring those researchers have freedom to operate in the HD space.

CHDI aims to fundamentally change the way science is done for the disease, which has gone without a disease-modifying treatment since its discovery in the 1870s. CHDI Foundation focuses on making processes more efficient and managing efforts, rather than simply focusing on funding the science. Likening its role to the managers of complex scientific projects like the moon

landing, CHDI emphasizes effective management and leadership as much as the intended scientific outcomes, and has a staff of more than 90 managers and scientists to drive the work forward.

CHDI has also focused its endeavors on creating an IP landscape that allows partners the freedom to operate and on improving the quality of scientific studies. Rather than concentrating on patents and controlling IP resulting from research programs, one of CHDI's primary efforts is ensuring that all researchers in the HD field have license to use the products that CHDI is involved in. A focus on improving the quality of research has led to hiring a director of experimental design to work with grantees on study designs and the creation of an Independent Statistical Standing Committee to provide unbiased evaluation and advice to any HD researchers to improve the quality and reproducibility of their work.

Collaboration Life Cycle: Start-Up


Gathering all the smartest people around an issue and funding them to work together produces good outcomes, right? Not necessarily. There are many issues that need to be thought through at the beginning of a collaboration to build a strong foundation for its success. While not every twist in the road can be anticipated, time invested in thinking through the pathway with “the end in mind” and the resources that need to be in place at each step will pay significant dividends. Among the start-up issues that participants discussed most frequently at the workshop were:

Mission and Governance

- 1 **ALIGNMENT OF GOALS AND EXPECTATIONS** consistently came up throughout the workshop as being among the most critical steps in forming a collaboration with diverse stakeholders. Alignment is also a persistent challenge. Clarity about the problem the group is working together to address and appropriate expectations about what can and will be accomplished must be a focus at the outset.
- 2 A **FORMAL, TRANSPARENT GOVERNANCE STRUCTURE** outlining decision-making authorities and management responsibilities builds confidence among the participants and funders. It explains how issues related to resource allocation, project selection, oversight, and tracking outcomes will be handled. (*See IBD Plexus Case study and Relevant Resources on page 13.*)
- 3 There was robust discussion at the workshop about **HOW MUCH CONTROL A FOUNDATION SHOULD HAVE** in setting the terms for a collaboration in the near-term and how much decision-making control it should have about its activities in the medium- to long-term. There were a number of advocates in the room who argued that “if you’re creating scarce resources, you should have some input into how they’re used.” If, for instance, one of your guiding principles is that you want any resource created in the course of a collaborative project broadly shared, you need to put policies

WORKSHOP WISDOM

“To think **outside the box**, you first have to figure out where the box is.”



Keeping all parties focused on the mission of the collaboration and accountable for their activities might necessitate a more active role for the foundation.

in place that will prevent other partners from using your samples or data resources in ways that restrict further access for research. Keeping all parties focused on the mission of the collaboration and accountable for their activities might also necessitate a more active role for the foundation. In contrast, a funder might not want to dictate too many conditions of participation if it's trying to create a more flexible environment that supports the goals and motivations of a variety of players, where everyone “plays nicely in the sandbox.” No matter the approach, clear communication by funders goes a long way to explain why they are requiring certain things—what their “guiding principles” are—and allows for some flexibility if there is a more accommodating way to achieve the same ends.

Human Capital

- 1 CAREFULLY CONSIDER HOW THE INITIATIVE WILL BE STAFFED.** Most foundations are limited in the size of their professional staff. Hiring additional staff to manage large collaborative efforts might be required, although it can sometimes be difficult to explain the need to donors. The foundation might also need to outsource activities that require expertise the foundation does not have. One beneficial solution for getting the work done and building an engaged team is to leverage the time and expertise of partner organizations' staffs. If in-kind contributions of partners' staff time are expected, this could be explicitly called for in agreement language.
- 2 DIFFERENT PARTNERS BRING DIFFERENT SKILL SETS**—and different priorities that are important to them. “Don't push people out of their areas of expertise,” recommended one workshop participant. Understanding the priorities and incentives of all the players is absolutely key to the success of any partnership.

Anticipating Future Needs

- 1 No one can peer into a crystal ball to see how any research initiative will play out. But consideration should be given to whether there are **NEEDS, OR WANTS, DOWN THE ROAD THAT CAN BE ANTICIPATED AND PLANNED FOR UP FRONT**. This might include different types of data that will be needed or different partners that will need to be brought on board. Action might be required in the near term, such as aligning informed consent documentation and Institutional Review Boards (IRBs) with your future plans.
- 2 Many workshop participants advocated for the **IMPORTANCE OF BRINGING IN INDUSTRY, REGULATORS, AND EVEN PAYERS EARLY ON IN THE PLANNING** so their perspective can inform the work of the collaboration, even if they are not (yet) active collaborators.

RELEVANT RESOURCES IN THE 'FOUNDATIONS AS COLLABORATION CONVENERS' TOOLKIT

- > Consortia-pedia: An In-Depth Look at the Research-by-Consortium Trend in Medical Research and Development (*FasterCures*)
- > IBD Plexus Governance Structure (Crohn's & Colitis Foundation)
- > IBD Plexus: Partnering to Accelerate Science (Deloitte)

For more, go to train.fastercures.org/toolkits

IBD PLEXUS: INVESTING IN A STRONG FOUNDATION

The Crohn's & Colitis Foundation is about to launch IBD Plexus, a large-scale information exchange platform and biobanking system intended to accelerate inflammatory bowel diseases (IBD) research and engage all stakeholders—patients, providers, and both academic and industry researchers. In its role as convener and catalyst in IBD, the foundation is collaborating with researchers and clinicians to build and run the system in-house, as well as assembling a broad-based coalition of stakeholders to contribute to and support the resource, and then use the platform for research into the diseases.

Building such a large system from scratch was compared to gutting and renovating a house. After demolition, there is a vast, empty space, and immediately the architect and contractor ask the homeowners question after question, many of which require expertise the homeowners don't have, or have downstream im-

plications the homeowners can't anticipate. Knowing it wouldn't have all the answers, the foundation consciously chose to use a transparent, dynamic governance process with working groups composed of all stakeholders.

The foundation was also keenly aware of long-term sustainability from the onset. The Helmsley Charitable Trust generously provided start-up funds, but the foundation would need to find other means to support IBD Plexus over the long-term. The foundation decided that partnerships with industry were essential for sustained viability, and so early in the building process created cross-functional working groups with representatives from academia and industry to ensure that decisions made would be acceptable to both groups. In particular, the foundation focused on ensuring that near-term decisions would not preclude industry partnerships down the road.

Collaboration Life Cycle: Building Relationships

WORKSHOP WISDOM

“You need a few ‘bell cows,’ a few leaders who say ‘we can do this.’”

As challenging as the start-up phase of a new partnership can be, identifying a significant unmet need and some great partners with shared objectives could turn out to be the easy part. Next is the challenging and time-consuming work of creating the structure and agreements that maximize your chances of achieving your goals and that create the norms of behavior for the group. What policies and practices can help foundations reconcile their interest in sharing data and resources with academia’s professional incentives for publication and industry’s need for IP protection? What are some major stumbling blocks you can anticipate?

Workshop participants focused much of their discussion on the following issues:

- ① **BUILDING TRUST** is key in any relationship, of course, and must happen from the earliest conversations about collaboration. Foundations that have built credibility in their disease communities as key opinion leaders have a head start in that process.
- > A number of participants highlighted the importance of making sure the **MILESTONES** in agreements (and all agreed that milestones are critical to multi-stakeholder initiatives) are decided upon together and appropriate to the task at hand, the stage of research, etc. While the scientific outcomes of the work cannot be dictated, milestones can ensure the research process is proceeding apace (e.g., have essential personnel been brought on board, have key experiments been conducted, etc.). These are essential for building trust.
- > As has been noted, understanding the **INCENTIVES** that drive the sectors, organizations, and even individuals participating in a collaboration is important, even if you aim to modulate some of those incentives. As one participant said, “Everyone wants to think outside the box, but first you need to understand where the box is” for all your collaborators.

- One workshop attendee urged the group not to overlook the seemingly obvious, but often neglected, work of **TEAM-BUILDING**, instilling in the group a sense of shared purpose and even pleasure in being part of the team. Foundations are uniquely well-suited to this task since they can bring patients into the equation, bringing to life the importance of the work the group is doing.

2 **DATA SHARING AND REUSE** was cited in the pre-workshop survey responses as the most common objective of collaborative initiatives and also the most common challenge. It is not easy to overcome resistance to sharing by both academic and industry partners. And it is expensive to provide the infrastructure for data sharing, to compensate researchers for the expenses associated with sharing data, and to maintain the data-sharing infrastructure over time. If this is a primary objective of foundations in creating multi-stakeholder collaborations, these issues must be front and center in agreement negotiations. One participant called for more education for funders about the issues involved in data sharing to help build an understanding of the concerns partners might have about it.

3 The right approach by foundations to the **PROTECTION AND MANAGEMENT OF IP** is still the subject of much debate, in the workshop and the R&D field more broadly. Survey respondents told us that they are more likely to be working with industry partners than academic partners on collaborative ventures, so IP issues are critically important to them.

- The success of the Cystic Fibrosis Foundation in collecting royalties from the development of Vertex's drug Kalydeco has excited and challenged many

TRANSCCELERATE BIOPHARMA: CREATING VALUE FOR PARTNERS

TransCelerate Biopharma Inc. is a nonprofit organization with a mission to collaborate across the biopharmaceutical research and development community to identify, prioritize, design, and facilitate the implementation of solutions to drive efficient, effective, and high-quality delivery of new medicines, improving the health of people around the world. TransCelerate was founded in 2012 and has maintained strong support from industry. Members provide resources in terms of an annual membership fee, as well as providing their own staff to work on TransCelerate projects.

This sustained success is enabled by the collaboration's structure and decision-making process. The consortium focuses on projects that will improve R&D processes, systems, efficiencies, and pain-points felt commonly by the membership. Currently, TransCelerate has more than 15 projects that aim to improve the site investigator experience, enable harmonization of clinical trial processes, facilitate sharing of information, enhance sponsor efficiencies, and improve the patient experience. TransCelerate's successful framework for collaboration has also sparked new opportunities in preclinical development (operating under the subsidiary BioCelerate), and most recently, in pharmacovigilance. TransCelerate continually evaluates its strategic focus to ensure that it is continuing to provide value to the members. TransCelerate also provides significant value-add services to the consortium: industry-quality program management, legal guidance to ensure that the collaboration is not violating anti-trust or other laws, and digital/social platforms to share project results and solutions as widely (and publicly across the industry) as possible.

RELEVANT RESOURCES IN THE 'FOUNDATIONS AS COLLABORATION CONVENERS' TOOLKIT

- > FNIH Biomarkers Consortium General Intellectual Property and Data Sharing Principles (NIH)
- > Parkinson's Progression Markers Initiative Research Documents & SOPs (MJFF)
- > CHDI Agreement Templates
- > Sample Funder's Addendum: Access to Research Tools (*FasterCures*)
- > Confidential Disclosure Agreement for Research Consortium (MJFF)
- > Key Research Agreement Terms and Definitions (*FasterCures*)
- > Venture Philanthropy Legal Report: The Importance of an Interruption License (Schaner & Lubitz)
- > 2014 Annual Summit White Paper (One Mind)

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organizations. One workshop participant said that while he believed a reasonable financial return on investment (ROI) is a legitimate expectation in some situations, the recent focus by some foundations on how to maximize it—even when advancing their philanthropic missions—has become a distraction and is complicating relationships with partners.

- > Other attendees said they have **NO INTEREST IN “OWNING” IP** (though foundations rarely own IP; they want to be able to benefit if it's commercialized). But understanding when and how IP may come into play in an initiative—whether it's a desire to appropriately protect inventions that may arise from the work and be commercialized, or a desire to preserve the freedom to operate among partners and even outside parties—is extremely important.

- ④ **USE THE RIGHT TOOL FOR THE TASK.** One participant advised others to not become wedded to a particular tool or approach (e.g. “march-in rights,” which gives a foundation a claim on IP if it's not developed by its owner). Instead, consider what your objectives and guiding principles really are, communicate clearly with your partners about your objectives, encourage your partners to communicate clearly with you about their requirements, and work toward a solution that fits your purpose. (*FasterCures* has developed a set of guiding principles and points to consider in negotiations, particularly around IP, among nonprofit, academic, and industry partners; see Appendix for more information).

Collaboration Life Cycle: Evaluation and Sustainability

How do we know if these initiatives are worth sustaining—and if they are, for how long? What intermediate and long-term metrics are reasonable to measure the performance of the collaboration? These are evolving areas of thinking and research, as multi-stakeholder R&D collaborations become a more prominent feature of the landscape.

- > **DEFINING SUCCESS.** Several participants noted that it's important to define success up front, in the service of transparency and accountability. Others appealed for a certain level of flexibility to allow teams to pivot in response to the scientific results of the project and potentially “creating a dialogue” among the researchers. However, “you can't sunset it without knowing” how you're defining success, said one participant, and it can be easy to define success post hoc to make any effort appear successful. Other attendees talked about having a responsibility to educate external constituencies along the way about what to expect from the project and how to manage expectations appropriately. This includes conversations about the potential for ROI; one participant urged others not to lose sight of the ROI of moving the work forward—or ending the project if it's proving unfruitful.
- > **MAINTAINING FOCUS.** A number of people throughout the day talked about the challenges of maintaining focus, particularly in lengthy and large-scale projects, and guarding against mission creep. This connected back to the earlier discussion about how much control foundations can and should have over the initiatives they've launched. It can also be challenging to keep things on track when partners' personnel or priorities change.
- > **“SURROGATE MARKERS” OF SUCCESS.** There have to be measures of a successful collaboration other than the achievement of its objectives, which can be a long time coming and are not scientifically assured. What evidence of progress can foundations offer their partners, sponsors, and donors to keep them engaged? One participant noted, “Process evaluation is almost as

WORKSHOP WISDOM

“Process evaluation is almost as important as impact evaluation.”

ARTIFICIAL PANCREAS PROJECT: TAKING THE LONG VIEW

JDRF launched the Artificial Pancreas Consortium in 2006, bringing together physicians, scientists, engineers, medical device manufactures, and the FDA to accelerate the development of this new technology. The foundation invested \$116 million to develop and test the technology between 2006 and 2016. In 2016, the FDA approved the first artificial pancreas system. Along the way, the consortium developed many shareable resources that will benefit researchers, developers, and regulators inside and outside the type 1 diabetes field. This commitment of more than a decade required a long-term view of how to evaluate and sustain investments. Even with the exciting approval of a product, work continues to improve the technology, to maximize the outcomes for patients, and to ensure that patients have access to it.

JDRF's long-term view factored into the IP agreements the foundation established with the researchers and institutions it funded in the artificial pancreas project. While JDRF started with simple grants to academic researchers, as the technology matured, the foundation was concerned that conflicting IP claims could be developed by universities and block down-stream commercial development of new devices. The foundation didn't want to own the IP, but it did want to have a seat at the table when licensing decisions were made so that it could ensure that its investments were ultimately leading to products that would benefit patients.

important as impact evaluation” in these situations. Some other types of “surrogate markers” cited by attendees included creating new collaborations based on the perceived success of existing ones, and attracting the attention of industry to your disease area of interest. Metrics need to be appropriate to the stage of research, as previously mentioned.

- **SUSTAINABILITY.** Not surprisingly, there was quite a lot of discussion about how foundations can sustain what are usually fairly expensive ventures and much bigger bets than grants to individual academic investigators. Most look to the biopharma industry to provide the long-term financial support many of these initiatives require, and there was some discussion about emerging thinking on how to redefine the value proposition to engage industry's interest and support. But as one participant put it, “If it's important to you, then sustainability might be on you.” Another attendee made the important observation that we have to think about the sustainability not just of a specific collaboration but also of the outputs of that collaboration, which might include developing data standards, research resources, or additional assets that will require maintenance in the future.

RELEVANT RESOURCES IN THE 'FOUNDATIONS AS COLLABORATION CONVENERS' TOOLKIT

- > Grant Milestones and Deliverables, MJFF
- > Report on Socio-Economic Impact (Innovative Medicines Initiative)
- > Bibliometric Analysis of Projects (Innovative Medicines Initiative)

For more, go to train.fastercures.org/toolkits

Action Agenda: Opportunities to Streamline Collaborations

Throughout the workshop, participants surfaced many challenges they face in creating and sustaining collaborative R&D efforts. But they also had many potential solutions to offer and ideas for tools and resources that could help streamline the process.

CHALLENGES CITED BY WORKSHOP PARTICIPANTS

What are the most significant challenges for partners internally and externally that need to be addressed?

START-UP

- > Engaging interest and buy-in from the right partners, including regulators
- > Understanding and influencing stakeholder incentives
- > Alignment of goals and expectations
- > Need for different types of talent, management, and special expertise
- > Governance structure
- > Managing conflicts of interest for foundations, investigators, and companies
- > Length of time to launch

ESTABLISHING AND MAINTAINING

- > Internal resources to manage the initiative
- > Due diligence on choosing projects
- > Data policies and practices, and allocating resources to support sharing
- > IP policies—what is the foundation's role, what solutions serve objectives
- > Maintaining focus, guarding against mission creep
- > Quality of science across all partners

EVALUATING AND SUSTAINING

- > Tracking IP and commercialization of funded research
- > Getting negative data shared publicly
- > Measuring financial and non-financial ROI
- > Metrics for organizational success, how you measure your contribution
- > Educating and communicating with stakeholders, setting realistic expectations
- > Process evaluation in addition to impact evaluation
- > Mechanism to sunset large collaborations
- > Measuring the impact of the scientific strategy versus the impact of the collaboration—do they need to be separate?
- > Demonstrating the value to donors
- > Sustainability model with industry

WORKSHOP WISDOM

“‘If you want to go fast, go alone; if you want to go far, go together.’ We’re asking the question, ‘Can we go quickly together?’”

SOLUTIONS, TOOLS, AND RESOURCES CITED BY WORKSHOP PARTICIPANTS

What tools and resources would be useful in streamlining the planning and execution of collaborative initiatives by foundations that do not currently exist?

START-UP

- > More thinking about role of foundation in management, governance of collaboration
- > Sample organizational guiding principles
- > Access to common agreements and common infrastructure (IRBs, trial networks) developed by NIH
- > Templates for contracts, protocols, etc.
- > Mentoring, peer-to-peer network for information and guidance
- > Case examples and contacts of organizations that have done specific types of projects (e.g., trial networks, biomarker initiatives, etc.)
- > Governance models, including information about international consortia
- > Catalogue of resources that could benefit pre-competitive collaborations (e.g., preclinical animal study databases, etc.)

ESTABLISHING AND MAINTAINING

- > Good approaches to IP policies and management
- > Training in how data sharing works
- > Tools for system and stakeholder mapping
- > Data and sample sharing policies and agreements
- > Model consent language
- > Examples of IRB efficiencies (e.g. reliance agreements)
- > Antitrust policies for industry in collaborations
- > Information about liability insurance for foundations engaged in health research activities
- > Toolkit of resources other foundations have developed to address issues
- > List of law firms, consultants, etc. who can provide support for tech transfer, statistical analysis, etc.
- > Ideas for how to engage more data scientists
- > Models for engaging with regulators, e.g. Research Roundtables, drug development tools meetings

EVALUATING AND SUSTAINING

- > Examples of evaluation, success criteria
- > Develop tools to more easily track IP generated from research funding
- > Models for sustainability plans for registry operating costs

Key Takeaways

- 1 **COLLABORATION IS NECESSARY.** Though not always the right solution to every problem, the complexity of science and need for diverse skills and disciplines to overcome shared challenges are making multi-party collaborative R&D an increasingly prominent feature of the landscape.
- 2 **COLLABORATION IS NOT FOR THE FAINT OF HEART.** Building and managing collaborations require different skills than traditional grant-making. Hands-on project management is fundamental to keeping programs on track.
- 3 **FOUNDATIONS ARE UNIQUELY WELL-SUITED TO, AS WELL AS UNIQUELY CHALLENGED BY, BEING COLLABORATION CONVENERS.** Their long-term view and patient-centric drive can make them ideal honest brokers to bring together diverse interests. Their financial and human capital limitations, however, can make sustaining and managing initiatives a high-risk undertaking.
- 4 **INVESTING IN A STRONG FRAMEWORK IN THE START-UP PHASE IS WORTH THE TIME AND EFFORT.** Having a clear sense of your organization's guiding principles, communicating them clearly to your partners, and operationalizing them through the terms of engagement and governance structure will pay dividends.
- 5 **RESOURCES TO STREAMLINE COLLABORATIONS EXIST, BUT MORE ARE NEEDED.** While every collaboration is unique, foundations have much to learn from one another and their partners. *FasterCures* has long been in the business of providing platforms and opportunities for collaboration across sectors, organizations, and diseases; in addition to creating the "Foundations as Collaboration Conveners Toolkit," we will continue to enable stakeholders to share their experiences and resources on this topic, and we will address some of the needs expressed in this Action Agenda.

Under the umbrella of our new Collaboration 2.0 program, *FasterCures* will be focusing more explicitly on how to maximize the potential of partnership, what the most important success factors are, and how we can improve collaboration to achieve important goals such as sharing of data and knowledge and reducing the time and cost of clinical trials.

By collecting the wisdom of leaders across sectors, analyzing the challenges, and guiding effective solutions, we aim to help the biomedical R&D field go further, faster, together.

Participant List

We are grateful to the following participants for sharing their time and expertise during our workshop. Titles and affiliations were accurate as of April 2017, when the workshop took place.

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Appendix

FASTERCURES RESOURCES

- Web site: TRAIN (The Research Acceleration and Innovation Network) Central Station (<http://train.fastercures.org/>)
- Toolkit: Foundations as Collaboration Conveners toolkit (<http://train.fastercures.org/toolkits/foundations-as-collaboration-conveners>)
- Program: Collaboration 2.0 (<http://www.fastercures.org/programs/collaboration/>)
- Report: Consortia-pedia: An In-Depth Look at the Research-by-Consortium Trend in Medical Research and Development (<http://www.fastercures.org/reports/view/39>)
- Report: Honest Brokers for Cures: How Venture Philanthropy Groups are Changing Biomedical Research (<http://www.fastercures.org/reports/view/15>)
- Report: Unlocking Intellectual Property: Principles for Responsible Negotiation (<http://www.fastercures.org/reports/view/24>)

EXTERNAL RESOURCES AND CASE EXAMPLES REFERENCED

- Alzheimer's Disease Neuroimaging Initiative (<http://www.adni-info.org/>)
- CHDI Foundation (<http://chdifoundation.org/about-us/>)
- Crohn's & Colitis Foundation: IBD Plexus (<http://www.crohnscolitisfoundation.org/science-and-professionals/research/IBDPlexus/>)
- Cystic Fibrosis Foundation: Royalty Sale Will Be Transformational for People with CF (<https://www.cff.org/About-Us/Media-Center/Press-Releases/Cystic-Fibrosis-Foundation-Royalty-Sale-Will-Be-Transformational-for-People-with-CF/>)
- JDRF: Artificial Pancreas Project Consortium (<http://jdrfconsortium.jaeb.org/ViewPage.aspx?PageName=Home>)
- Michael J. Fox Foundation for Parkinson's Research: Parkinson's Progression Markers Initiative (<http://www.ppmi-info.org/>)
- TransCelerate Biopharma, Inc. (<http://www.transceleratebiopharmainc.com/>)



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