

The Advancing Role of Non-Profit Organizations in Drug Development



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The Advancing Role of Non-Profit Organizations in Drug Development



Co-Chairs

Kathy Giusti

Founder and Chief Executive Officer
Multiple Myeloma Research Foundation
Multiple Myeloma Research Consortium

Brock C. Reeve

Executive Director
Harvard Stem Cell Institute

William A. Sahlman, Ph.D.

Professor of Business Administration
Harvard Business School

Participants

Walter Capone

Chief Operating Officer
Multiple Myeloma Research Foundation
Multiple Myeloma Research Consortium

Lon Cardon, Ph.D.

Senior Vice President
GlaxoSmithKline

Michael Curren

Senior Vice President
Cure Alzheimer's Fund

Amy Dockser Marcus

Staff Reporter
The Wall Street Journal

Robert J. Gould, Ph.D.

President and Chief Executive Officer
Epizyme, Inc.

Richard G. Hamermesh, D.B.A

Professor
Harvard Business School

Bob Higgins

General Partner
Highland Capital Partners

Katie Hood

Former Chief Executive Officer
The Michael J. Fox Foundation for Parkinson's
Research

Richard A. Insel, M.D.

Chief Scientific Officer
Juvenile Diabetes Research Foundation

Thomas P. Kanyok, PharmD

Senior Program Officer
Bill & Melinda Gates Foundation

Henry F. McCance

Co-founder
Cure Alzheimer's Fund

Terry McGuire

Co-founder and General Partner
Polaris Venture Partners

Douglas A. Melton, Ph.D.

*Primary Investigator, Howard Hughes Medical
Institute*
Harvard Stem Cell Institute Co-Director
Harvard University

Nitin Nohria

Dean of the Faculty
Harvard Business School

Paul L. Rosenberg

Partner
The Bridgespan Group

Una Ryan, O.B.E, Ph.D., D.Sc.

Chief Executive Officer
Diagnostics For All, Inc.

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Vicki L. Sato, Ph.D.

*Professor of Management Practice
Harvard Business School
Professor of the Practice, Molecular and Cell
Biology
Harvard University*

Nancy Simonian, M.D.

*Chief Medical Officer
Millennium: The Takeda Oncology Company*

Howard H. Stevenson

*Sarofim-Rock Baker Foundation Professor of
Business Administration, Senior Associate
Dean, Director of Harvard Business School
Publishing
Harvard Business School*

Frances Toneguzzo, Ph.D.

*Executive Director, Office of Ventures and
Financing
Partners Healthcare*

Douglas E. Williams, Ph.D.

*Executive Vice President, Research and
Development
Biogen Idec*

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INTRODUCTION

The Changing Drug Development Landscape

Scientific advances have led to greater insight and understanding of multiple disease states; however, for many life-threatening illnesses, targeted treatments and cures remain elusive. Several factors contribute to the inherent challenges in drug development, including the fact that diseases are fundamentally complex, and the scientific and business challenges of developing viable therapeutics are daunting.

The current economic environment has also changed the drug development landscape. Many important players have become more risk-averse. Novel targets and compounds arising from early-stage research are often deemed too risky by companies faced with growing pressure to deliver products quickly. Venture capital is also shrinking, which is limiting the advancement of new research and development companies.

Meanwhile, other demands facing pharmaceutical industry stakeholders are not always in sync with the goal of advancing drug development. Concerns about intellectual property and the results of proprietary research may impede the ability of research teams in academia and industry to share important findings. Moreover, regulatory structures may not be optimal for new drug approvals.

Emerging Opportunities for Non-Profit Organizations

“The goal is to share our strategies, models and deal structures with each other to establish best practices and further our causes across different diseases.”

Kathy Giusti, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium

Confronted with this challenging drug development landscape, non-profit organizations (NPOs) are stepping in to bridge gaps between research and product commercialization and prioritize agendas around what patients need most: longer survival and better quality of life. Across multiple diseases, NPOs have emerged as a driving force in reshaping the ways in which the various constituencies engage with one another. As their roles evolve, the opportunities for NPOs to learn from one another's successes have never been greater.

In the summer of 2011, the Multiple Myeloma Research Foundation (MMRF) – an organization at the forefront of transforming the traditional role of NPOs in patient advocacy – and the Harvard Stem Cell Institute (HSCI) – the largest collaborative of scientists and clinical experts in stem cell science – convened a roundtable discussion on the advancing role of NPOs in drug development. Hosted by William A. Sahlman, professor of business administration at Harvard Business School, the discussion brought together representatives from NPOs, the pharmaceutical industry, venture capital and academia to share insights and experiences that define best practices for this new NPO model. In addition to the MMRF and the HSCI, other NPOs present included the Juvenile Diabetes Research Foundation (JDRF), the Cure Alzheimer's Fund (CAF), the Michael J. Fox Foundation for Parkinson's Research (MJFF), and the Bill & Melinda Gates Foundation.

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Kathy Giusti, co-chair of the event and founder and chief executive officer of the MMRF and the Multiple Myeloma Research Consortium (MMRC), encouraged participants to learn from each other's successes. She described the MMRF's strategic shift from traditional grant funding to building new models of drug development as an example of a model that others can emulate.

Throughout the day, participants explored the ways in which the altered landscape in drug development has pushed NPOs to adopt new approaches and broker partnerships that have resulted in new knowledge and accelerated progress. They defined a core role of NPOs as being the "trusted third party" that can offer incentives and create deal-based structures amenable to the varying needs and expectations of each collaborator. The roundtable discussion focused on sharing this knowledge with other organizations and discussed best practices to advance these new models and the organizations' agendas to find treatments and cures across different disease states.

The event chairs and attendees believed that the roundtable's best practice sharing should not just be contained to the four walls in which it happened. This resource is intended to share the key insights discussed.

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LEADING NPO MODELS AND BEST PRACTICES

Creating Innovative Funding Structures: Current Grants and Deal Structures

Richard A. Insel, M.D., Chief Scientific Officer, Juvenile Diabetes Research Foundation

Brock C. Reeve, Executive Director, Harvard Stem Cell Institute

*“We need to think of grants and deals as being more than just about money.”
Brock C. Reeve, Harvard Stem Cell Institute*

As NPOs evolve, so too must the incentives and structures that support the new roles they are playing. The roundtable discussion began with a focus on how organizations are creating innovative deal structures that allow them to partner more meaningfully with innovators throughout the drug development continuum. In advancing novel deal structures, NPOs have the opportunity to leverage the talents, resources and strengths of their collaborators to ultimately move early clinical research into treatment and devices that benefit patients.

Roundtable participants highlighted different types of deal structures that target specific stages of the drug development process. Brock Reeve, executive director at the Harvard Stem Cell Institute (HSCI), described that the make-up of a deal structure is largely dependent on the goal of the collaboration, and can allow for partners to share resources beyond just information.

The best deal structures catalyze and retain industry commitments, strive for regulatory clarity and de-risk project deliverables for future partners. By sharing knowledge and exerting influence over drug development pipelines, effective deal structures move the ball forward towards new drug approvals.

Dr. Richard Insel, chief scientific officer at the Juvenile Diabetes Research Foundation (JDRF), described the organization’s innovative deal structures. Dr. Insel pointed out that the JDRF once put nearly all its resources into academic grants. After recognizing the void that existed in translating academic research into a clinical setting, it began devoting roughly 10-15% of its budget to collaborations with industry. JDRF has partnered with 34 biotechnology and pharmaceutical companies, each developed with different structures for different purposes.

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CASE STUDY

Juvenile Diabetes Research Foundation Partners with Pharmaceutical Company to Drive Clinical Development

As part of its early stage research, the Juvenile Diabetes Research Foundation (JDRF) partnered with the Genomics Institute of the Novartis Research Foundations (GNF) on pancreatic beta cell regeneration, proliferation and expansion in Type 1 diabetes.

In collaborating with academic partners on efforts to generate new endogenous beta cells, JDRF faced a translational void: the collaboration had identified promising targets, but resource limitations blocked the path to clinical development. To overcome these limitations, JDRF partnered with GNF, which had an ongoing program in beta cell regeneration and also the resources, motivation and commercial mindset to move the research forward.

JDRF's ambitious partnership with GNF calls for developing several investigational new drugs. The arrangement links JDRF's academic networks, project pipeline and unique position in Type 1 diabetes research with GNF's high-throughput chemistry platform and expertise in drug discovery.

The partnership with GNF is a prime example of an innovative deal structure intended to further research in a meaningful way. GNF and Novartis control the intellectual property and bear responsibility for commercializing and developing products. JDRF will receive a modest return on investment, based on GNF's successful commercialization of products derived from the funded research program. In the 22 months since the collaboration began, new targets and pathways have been identified, and new collaborations have emerged from JDRF's network.

The collaboration builds in a program advisory committee that meets monthly, a steering committee that meets face-to-face quarterly and a scientific advisory board that meets annually. The collaboration is also open to academic groups to translate their discoveries and participate in an annual meeting.

JDRF's collaboration with GNF illustrates how funding that combines non-profit and industry financing can advance biomedical research. Long timelines in drug development often deter venture capital and industry investment, so NPOs are increasingly tasked with securing hybrid funding to bridge financial gaps.

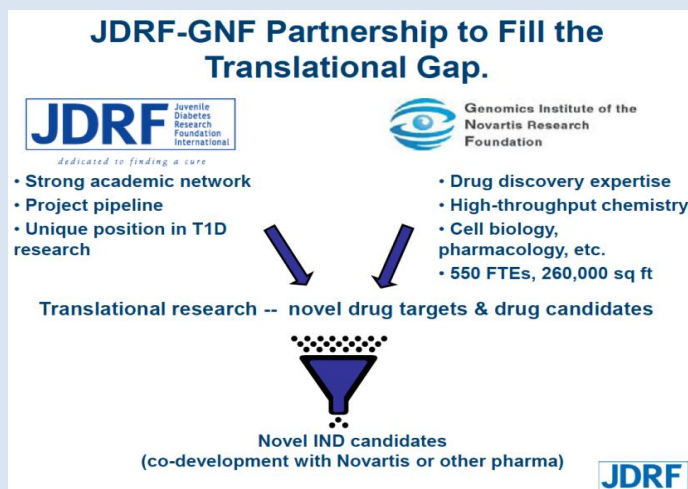


Figure 2: Private-public partnerships like JDRF's collaboration with GNF bring a commercial mindset to NPO-sponsored drug development.

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Building Effective Coalitions between Venture, Industry and NPOs

Vicki L. Sato, Ph.D., Professor of Management Practice, Harvard Business School, and Professor of the Practice, Molecular and Cell Biology, Harvard University

Katie Hood, Former Chief Executive Officer of the Michael J. Fox Foundation for Parkinson's Research
Terry McGuire, Co-founder and General Partner, Polaris Venture Partners

“We need to think about the reward incentive characteristics of all the players in the value chain.”
William A. Sahlman, Harvard Business School

Structuring sensible kinds of deals and partnerships is critical to a clear understanding of what each member brings to, and gains from, the partnership.

Participants from venture capital and industry emphasized that beyond financial currency, the most valuable currency every NPO has is a diverse body of knowledge that spans regulations governing research, the landscape of experts working on a given disease, promising targets and pathways for drug development, trial protocols and factors related to dosing in preclinical studies and ways to de-risk research for industry partners. As “concierges” to this critically important research enterprise, NPOs are valuable partners for different groups because of their ability to open doors and provide high-level access to individuals and organizations.

Katie Hood, former chief executive officer of the Michael J. Fox Foundation for Parkinson's Research (MJFF), explained her perspective on how an NPO brings value to partnerships with industry. She explained that because NPOs have the primary incentive to work towards a cure for patients, they are willing to fund the novel research that may ultimately lead to a disease-modifying treatment. NPOs are working with industry to ensure that what emerges from a given grant is compelling to future partners, thus de-risking research.

Terry McGuire, co-founder and general partner at Polaris Venture Partners, spoke about how venture capital-backed companies can benefit from a partnership and emphasized the credibility an NPO brings to a company. He highlighted that NPOs are experts in specific disease states and they validate a company's technology and approach when they invest. He also outlined that NPOs provide invaluable access to the clinical community, enabling patient enrollment and market research, but would like to see NPOs become more involved with affecting regulatory policy.

Dr. Vicki Sato, professor of management practice at Harvard Business School, and formerly of Vertex Pharmaceuticals, shared Vertex's experience building collaboration with the Cystic Fibrosis Foundation (CFF). Each organization had financial expectations inherent in the success of the partnership; Vertex needed the CFF's resources to fund the transition from assays to clinical development and CFF ultimately needs the Vertex compounds to come to market to capitalize on its investment. Each group made investments in downstream activities important to the success of the partnership and worked together in ways that benefitted both groups.

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CASE STUDY

Michael J. Fox Foundation Conducts Landscape Analysis to Identify Partner

Launched in 2000, the Michael J. Fox Foundation for Parkinson's Research (MJFF) was founded with a mission to promote drug development for Parkinson's Disease (PD). With a celebrity namesake, the MJFF recognized early on that it would garner considerable attention. It set out immediately to leverage its visibility to accelerate clinical research and development. The MJFF sees itself as tasked with de-risking PD research through funding early-stage research, providing clinical expertise and being a knowledge center for the PD research community.

After several years in the field, MJFF's sponsors saw gaps in existing health research enterprise. The Foundation performed a comprehensive "landscape assessment" of everyone associated with PD research, and it began to investigate and provide what these specific groups need to further research.

As part of this "landscape assessment," MJFF began collaborating with a biotechnology company investigating neurotrophic factors for PD treatment. A subsequent clinical trial testing this approach failed, but for technical reasons that, the investigators believe, was related more to dosing problems than the actual treatment. Patient recruitment for a new trial based on an alternate dosing mechanism is now underway.



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Becoming Knowledge Brokers: Pre-Competitive Consortium

Walter Capone, Chief Operating Officer, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium

Nancy Simonian, M.D., Chief Medical Officer, Millennium: the Takeda Oncology Company

“We must work together to forge critical partnerships between academia, scientists and drug development if this is going to be sustainable industry. We need to look out ten years and invest in people today.”

Nancy Simonian, M.D., Millennium: The Takeda Oncology Company

Transparency, knowledge sharing and driving innovation forward are crucial to progress in drug development, but they do not always come easily to a research enterprise that often depends on secrecy to achieve proprietary outcomes, either in the form of patents or peer-reviewed publications. The industry is faced with a double-edged sword: to compete amongst peers and spawn innovation, proprietary research remains an important asset; however, secrecy is problematic when potentially useful data remain out of reach of the broader research community.

The participants recognized the need to create an environment where the elements of successful drug development, including collaboration and data sharing, can happen effectively. Walter Capone, chief operating officer of the Multiple Myeloma Research Foundation (MMRF) and the Multiple Myeloma Research Consortium (MMRC), introduced the concept of the pre-competitive consortium, which is a system that enables competitors to share early stage research that will ultimately benefit all involved by enabling disease research to happen in a comprehensive and efficient manner. These arrangements pool resources, drive innovation, build enabling platforms and spur product development.

Mr. Capone began with an overview of successful pre-competitive consortiums that have made major advances in other industries, such as the wide-band optical networks created through a partnership between the Defense Advanced Research Projects Agency, the Digital Equipment Corporation and AT&T as well as discovery-enabling consortium CERN, the European Organization for Nuclear Research.

Dr. Nancy Simonian, chief medical officer at Millennium: the Takeda Oncology Company, outlined Millennium’s experiences working in pre-competitive consortiums with other pharmaceutical companies and NPOs, like MMRF. She highlighted Millennium’s involvement in the I-SPY trial, a national study identifying biomarkers predictive of response to therapy that is testing multiple drugs from various sponsors in one trial, as an example of how companies can work together in a pre-competitive consortium to move research forward. She also cited Millennium’s work with the MMRF on its Personalized Medicine Initiative and CoMMpass (Relating Clinical Outcomes in MM to Personal Assessment of Genetic Profile) trial, in which MMRF, Millennium and other industry partners will participate in a planned “pre-competitive industry consortium” to identify the molecular origins of multiple myeloma and enable physicians to match specific treatment approaches to the patients most likely to benefit. Dr. Simonian believes these types of partnerships are paramount to the future of drug development as companies realize the importance of sharing information to make important clinical and therapeutic advances.

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Mr. Capone continued with a discussion highlighting the important role NPOs have in enabling these research partnerships across industry. Since no entity can corner the market on all the approaches needed to make advances happen fast enough, the MMRF created a consortium to harness the power of personalized medicine for multiple myeloma patients. The design of the consortium was important to its overall success in garnering participation and driving results.

Other participants added that:

- Windows of data access can incentivize industry partners
- NPOs must establish themselves as “keepers of the data” that will enable the data to be used for discovery and development
- Pre-competitive consortium need solid business plans to be successful
- Industry partners need to see what benefits they will get at various stages of the collaboration
- Pre-competitive consortium need to have straight-forward plans for handling intellectual property

Participants discussed how an emerging role of NPOs is to act as knowledge brokers that can play useful roles facilitating data access and ensuring that research partnerships balance transparency with the needs of collaborators. NPOs are motivated solely by the search for better treatments and cures, and do not seek intellectual property or promote any one treatment over another. NPOs can ideally win the trust of their partners and develop governance structures that incentivize collaboration – for instance, with windows of data access or innovative licensing arrangements – while fostering a level of transparency that galvanizes wide-spread research.

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*“The thing is to not build more silos – at the end of the day, we need a cure.”
Walter Capone, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium*

CASE STUDY

Multiple Myeloma Research Foundation Pre-Competitive Consortium

As its primary goal, the Multiple Myeloma Research Foundation (MMRF) aims to cure multiple myeloma. Along those lines, the MMRF recently established its Personalized Medicine Initiative, which seeks to spur the development of diagnostics and treatments that are tailored to each patient's unique tumor biology. This approach builds on platforms developed by MMRF and its affiliate, the Multiple Myeloma Research Consortium (MMRC), that segment patients by individual characteristics for the purpose of applying targeted therapies.

Within this pre-competitive consortium, multiple industry partners will conduct strategic planning in support of the Personalized Medicine Initiative, and perform genomic/molecular profiling and data analysis for an MMRF-sponsored landmark study of multiple myeloma's clinical progression. The CoMMpass (Relating Clinical Outcomes in MM to Personal Assessment of Genetic Profile) study is designed to identify molecular features that predict relapse and benefits from treatment, as well as biomarkers for assessing drug response. Ideally, the study will generate data that translates quickly into actionable drug targets.

The MMRF expects the consortium will generate the richest dataset in multiple myeloma, and offer unparalleled opportunities for analyses that advance better treatments and cures. As incentives, industry partners have limited windows of access to data that subsequently will be made available to the broader research community.



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The Future: Public and Private Partnerships

William A. Sahlman, Ph.D., Professor of Business Administration, Harvard Business School
Thomas P. Kanyok, PharmD, Senior Program Officer, Bill & Melinda Gates Foundation
Paul L. Rosenberg, Partner, The Bridgespan Group

The last session put the day's discussions about the changing roles of NPOs into the bigger context of where the industry is moving in the future and the interplay between NPOs and government entities. Dr. William Sahlman of Harvard Business School set the stage for the discussion by reminding participants that the work being done in the drug discovery and development space assumes local and national importance when one considers the rising costs associated with chronic disease. As the U.S. government considers ways to decrease the deficit, it is important to note that research costs to develop treatments are much lower than the cost for long-term disease management. This makes the work that NPOs are doing in terms of innovative early stage research funding and partnership building one of the most important ways to combat these diseases. Participants were encouraged to think about ways to create more public/private partnerships to make an impact on disease research.

The Bill & Melinda Gates Foundation is the world's largest philanthropic foundation with about \$33 billion at the end of 2010 to fund research to combat communicable diseases globally. The Foundation works with various government groups, including the National Institute of Health, the U.S. Food and Drug Administration, the U.S. Agency for International Development, the President's Malaria Initiative and the President's Emergency Plan for Aids Relief in the U.S., in addition to its international work. As Dr. Thomas Kanyok described, the Foundation is based on the mission of helping people achieve their maximum life potential, which includes treating or working to eradicate diseases. Partnerships with the public sector have helped the Foundation work towards its mission and Dr. Kanyok encouraged the NPOs present to think of ways to work with government entities to make real changes for patients.

Paul Rosenberg, a partner in The Bridgespan Group, a non-profit strategy consulting company, concluded the session with four key areas in which NPOs usually look for guidance:

1. How to allocate resources among research, advocacy, patient services and prevention;
2. How to build influential networks;
3. How to develop the appropriate funding models; and
4. How to partner in ways that generate the biggest impacts.

He challenged participants to think of ways to get more disease foundations to take on these issues and to collaborate with one another to solve problems across disease states.

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CASE STUDY

Bill & Melinda Gates Foundation Impact on Disease

Guided by the belief that all people should have the ability to live their best life, The Bill & Melinda Gates Foundation uses its philanthropic dollars and resources in an effort to make the most impact in the diseases affecting the global community. The Foundation's philosophy is to take big risks, knowing that a vast majority of what they invest in may not work, but its philanthropic backing gives it the flexibility to take multiple shots on goal.

The Foundation measures return on investment based on impact to a disease and not monetary returns. It may use the disability-adjusted life year, a measurement of overall disease burden, or eradication of a disease to determine the return on the investment.

BILL & MELINDA
GATES foundation

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OPPORTUNITIES FOR NPOs

As the drug development landscape changes, the role of NPOs will continue to evolve. Participants identified key areas where NPOs may focus and affect change to help spur drug development, build stronger partnerships with collaborators and become leaders.

Managing Regulatory Issues

***“If you want to give us half a million dollars, I’d say,
‘Keep the money and set up camp at FDA.’”***
Terry McGuire, Polaris Venture Partners

Regulatory barriers that slow drug development can deny patients access to new treatments and cost industry millions in lost revenue. As much as NPOs need technical expertise in disease biology, they may be able to use their position in the drug delivery space to hold conversations with regulators about ways to accelerate approvals, without risking safety.

Sharing Information

“Can we share our failures to help each other make breakthroughs?”
Richard A. Insel, M.D., Juvenile Diabetes Research Foundation

Collaborative drug development depends on data sharing and transparency. Moreover, donors that want timely updates on how their investments advance treatments frequently insist on transparency, even though it can strain relationships among research partners. How to resolve the tension between transparency and collaborator needs for profit and intellectual property remains a fundamental challenge. NPOs can set up governance structures that incentivize collaborators while pushing boundaries for openness in research.

Managing Intellectual Property

***“This is about passing the baton and reaching the finishing line.
All that matters is that someone crosses it. We don’t care who it is.”***
Kathy Giusti, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium

NPOs must create effective mechanisms for intellectual property transfer, which ensure that collaborators aggressively strive to meet their stated obligations. NPOs do not control intellectual property, yet they must deploy business models to ensure that intellectual property is handled in ways that retain profit motives for collaborators without impeding progress towards cures.

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Cultivating Talent and Expertise

“We fund people, not projects.”

Nancy Simonian, M.D., Millennium: The Takeda Oncology Company

NPOs can count clinical networks and internal experts among their most valuable assets. Expertise revolves around this human capital, so attracting the brightest talent is imperative. Katie Hood, formerly of the Michael J. Fox Foundation for Parkinson's Research (MJFF), described how the organization set out to hire junior-level scientists trained with the mindset of finding a cure; this same group strives to imbue MJFF's collaborators with a shared sense of urgency.

Encouraging High Return with High Risk Projects

“We need to carve out money for risky experiments -- that's how you get multiple shots on goal.”

Katie Hood, formerly of the Michael J. Fox Foundation for Parkinson's Research

Drug development timelines, frequently in excess of a decade or more, can exceed investor tolerance. At the same time, a pervasive shortage in new drug approvals has scared off investors, particularly for early-stage research. NPO funding is important for drug development, and equally valuable currency comes from expertise, credibility, access to clinical networks and patients, and the capacity to influence regulators.

Creating New Business Models

“Our governance structures need to be painfully transparent.”

Michael Curren, Cure Alzheimer's Fund

While NPOs bring credibility, expertise, and the capacity to do proof-of-concept research in actual patients to the table, collaborators bring additional funds and resources needed to move new treatments through the pipeline. Participants agreed that partnerships must be founded on sophisticated business plans that guarantee a level of predictable value to potential collaborators.

For-profit companies, in particular, need assurance that resources injected into collaborations will be met with valuable assets on the other side, and also at various stages of development. Strategically-oriented, activist NPOs, such as those present at the roundtable discussion, are defining these new models.

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CONCLUSIONS

“Our whole business model depends on us being patient-driven and pure of heart.”
Katie Hood, formerly of the Michael J. Fox Foundation for Parkinson’s Research

“If disease-area oriented foundations can continue to deepen their thinking about strategy, to make difficult resource allocation choices with a very clear view of intended outcomes, we all can have an even greater impact in helping those affected by these terrible diseases.”
Paul L. Rosenberg, The Bridgespan Group

“What we are about is the cure, and if that wasn’t our primary goal, we couldn’t raise the funds to get this done.”
Kathy Giusti, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium

By moving away from a primary emphasis on grant-funding and towards deal-based structures that engage academics, industry, philanthropists and others around a cure-based agenda, NPOs assume the role of a “trusted third party” that puts patients first and creates a new model for research and development. With their resources and experience, NPOs are well-positioned to overcome these barriers and advance treatment beyond a plateau of discovery.

Participants highlighted that going forward, NPOs need to cultivate new forms of philanthropic support and other types of evergreen funding with an eye towards lessening the amount of time they spend raising capital. Philanthropists and others should be encouraged to support innovation, and NPOs should continue the process of sharing knowledge, developing networks and providing access to thought leaders who can help to guide and improve the drug development process.

The discussion concluded with the need for stakeholders to identify priority areas and research opportunities and engage in conversations with the National Institutes of Health and other funding entities to ensure that creative, high-risk/high-potential opportunities in translational research have adequate support. The path to accelerated drug development will come from new models and deal structures that leverage productivity, accountability, efficiency and transparency.