



# Successful Public/Private Partnerships



*A National Survey and Leadership Perspectives*

Guide to Effective Patient Foundation and  
Life Science Industry Collaborations



by **BAYBÍO**



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## Letter from the President

Disease foundations are playing an increasingly important role in bringing treatments to patients and to market. We believe there is great benefit to encouraging collaboration between life science companies and these foundations. As a starting point, we surveyed and conducted in-depth interviews with industry and foundation executives to understand how these leaders are working together, and what they view as the key opportunities and challenges of industry-foundation collaborations.

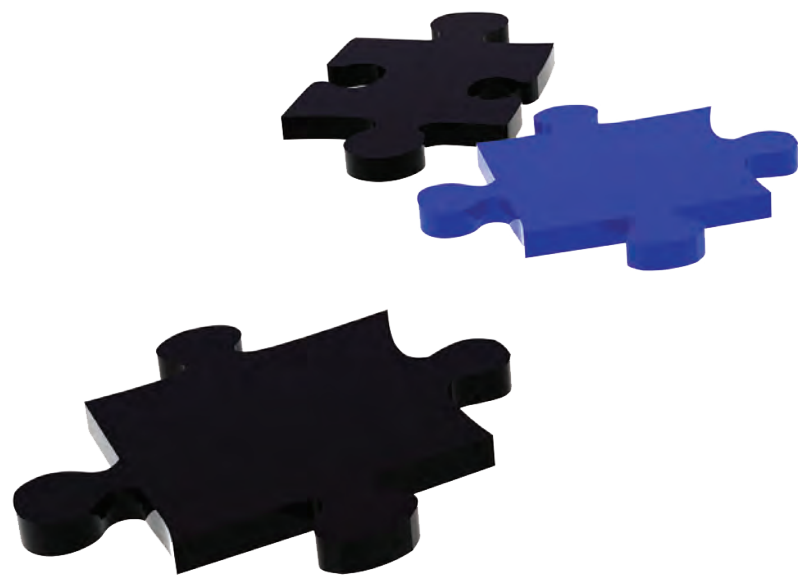
In these challenging economic times, disease foundations are playing a larger role in the strategic funding of early, innovative drug discovery programs. Their unique expertise in specific disease areas can be incredibly valuable in developing and disseminating new research tools, technologies and clinical protocols. Plus, their understanding of standard of care and patient needs, as well as their access to clinical thought leaders and patients, can enhance and accelerate clinical development of new treatments.

This study would not have been possible without the participation from our survey respondents and interview subjects who provided us thoughtful details and insights. We appreciate the generous support of FasterCures, the Biotechnology Industry Organization and our committed advisors. Their efforts reflect their strong commitment to this area.

Our goal is to use the findings of this study to promote and enhance industry-foundation collaborations. We hope to provide practical advice to life science companies and disease foundations on how to form and manage successful collaborations. To encourage more interactions in the coming months, BayBio will conduct a series of seminars and workshops that drill down into key subjects, including collaboration funding models, patient driven clinical trial design and recruitment, and regulatory and payor considerations.

Finally, as we continue to learn of the industry and disease foundations needs and best practices, we encourage you to share your experiences with us, contact BayBio to suggest additional resources and with questions.

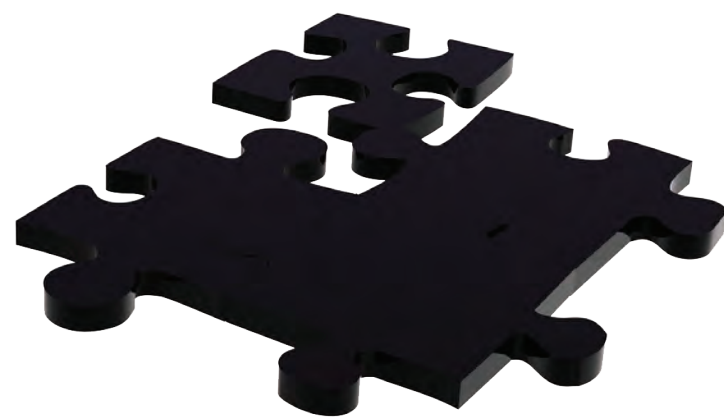
**Gail Maderis**  
President and CEO  
BayBio



Overview:

## The New Power in Partnerships

**B**iomedical companies that can harness the power of patient groups will get medicines, devices and diagnostics to market faster, cheaper and smarter, helping patients and rewarding investors. These new and powerful partnerships are playing an increasingly larger role in determining the success along the continuum of innovation from discovery to commercialization.



Patient advocacy groups and disease foundations, collectively called “patient foundations” or “foundations” in this paper, are shaping a fresh reality – by reducing business risk and even accelerating development. Linking the perspectives and resources of foundations to industry pipeline programs is producing notable and game-changing contributions to disease research, pipeline investment and the commercialization of innovative therapies, devices and diagnostics.

This report addresses how foundations are expanding beyond their traditional roles in advocacy and policy to play a pivotal role in early-stage funding and innovation. For example, FasterCures reports that 55 patient groups in its network dedicated to accelerating medical innovation give \$600 million in medical research grants each year. About half of these groups actively support clinical trials, and nearly 90 percent have partner with biopharmaceutical companies.<sup>5</sup>

Patient foundations can also bring urgency, intelligence and resources such as patient databases and biospecimens, preclinical models, and trial and recruitment support that is essential to advancing the development process. In short, patient foundations can influence research and expedite outcomes.

For example, Kalydeco, a breakthrough therapy approved in 2012 for a rare form of cystic fibrosis, was discovered by Vertex Pharmaceuticals and the Cystic Fibrosis Foundation in a collaboration that began in 1998. The foundation provided significant scientific, clinical and financial support, including a \$75 million investment, throughout the discovery to development process. According to the U.S. Food and Drug Administration’s (FDA) Commissioner Margaret A. Hamburg, M.D., “The unique and mutually beneficial partnership that led to the approval of Kalydeco serves as a great model for

<sup>5</sup> The groups are members of FasterCures’ TRAIN (The Research Acceleration and Innovation Network) <http://train.fastercures.org/>

### Success Stories:

## Orphan Drugs

**Sigma Tau** partnered with several **cystinosis foundations**, resulting in Cystaran™ (cysteamine ophthalmic solution).

**Vertex** partnered with the **Cystic Fibrosis Foundation** resulting in Kalydeco™ (ivacaftor).

“By working with foundations, pharmas can de-risk the drug development process.”

*Robert Goldstein  
Vice President  
Communications & Public Affairs  
ALS Therapy Development Institute*



## Majority of Partnerships “Very Successful”

- 78% of foundation respondents ranked their partnerships as “very successful”
- 59% of company respondents ranked their partnerships as “very successful”

“Partnership is a very interesting interface because both sides can learn, and that increases the pace of acceleration.”

*Matthias von Herrath*  
Director  
Type 1 Diabetes R&D Center

what companies and patient groups can achieve if they collaborate on drug development.”<sup>6</sup>

Companies and foundations share the need for fresh approaches and more efficient pathways to market due to the current business environment. Declining research funds from private and public sources are compounded by the following trends: increased research and development (R&D) costs, avoidance of risk investment, regulatory challenges and the rise of big data.

BayBio believes that such collaborations offer significant opportunities, but awareness of why and how to launch an effective partnership, and how to do it successfully, is constrained by a lack of industry research on best practices. To address this, BayBio has completed the first national survey of nearly 100 biomedical companies and patient foundations

<sup>6</sup> FDA News Release, “FDA approves Kalydeco to treat rare forms on cystic fibrosis,” Jan. 31, 2012

to extract what is working well and what can be improved. The survey was followed by 17 in-depth interviews to gather information on how to explore, form and navigate successful partnerships (please see page XX for a complete list of interviewees). The result of BayBio’s research is this practical roadmap to partnership, with an emphasis on best practices for life science companies and patient foundations, to help partners become even more productive.

BayBio’s research reveals that companies and foundations share top-priority interests in five significant areas: understanding patients, scientific and clinical advice, funding R&D, clinical trial design and patient advocacy. In addition, the two groups align closely on clinical trial recruitment and FDA advocacy as mid-range priorities, and reimbursement policy and post-market research as low priorities.

As regulatory and payor challenges increasingly impact patient access to new and even existing treatments, industry and foundations will need to collaborate on influencing regulatory decision-making and reimbursement policy. Without reimbursement, each group loses: the drug or device may be accessible to only a small subset of patients in need, and company shareholders will lose their return on investment.

While funding of research and development is among the top reasons for partnerships for both groups – ranked by foundations as second and by companies as fourth – foundation respondents said the majority of their total grant and R&D funding is still directed to academia.

However, foundation funding to biotechs has been steadily growing over the past few years. Burrill & Company, a global financial services firm focused on the life sciences industry, reports that grants patient foundations provided to life science sector in the United States grew from \$62 million in 2010 to \$97 million in 2012, and had reached \$88 million by early September in 2013.

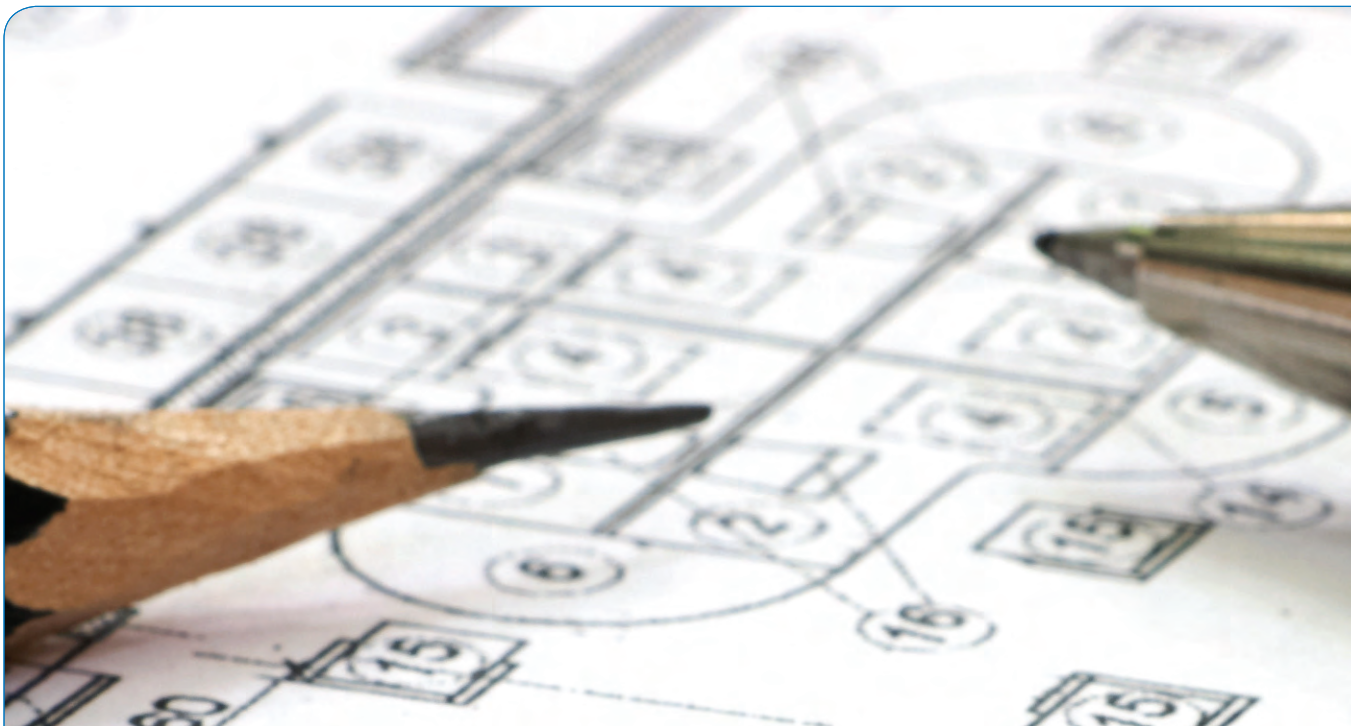
While there are key areas of alignment, the survey uncovers mismatched interests that require expert management for partnerships to be successful. For example, companies seek support for market research while foundations rank marketing as the lowest priority. In addition, dissonance on drug prices is a well known challenge in the field.

Respondents offered advice on what would have made their weaker partnerships more effective. Both sectors said some fundamental improvements are needed in partnership practices for collaborations to succeed. The most cited examples were a lack of understanding of the other’s full capabilities, a lack of knowledge about the drug discovery process and a lack of good communications. The good news is that all three of these are addressable.

In the following pages, this report highlights the benefits of partnership, describes five major best practices that serve as a roadmap for successful partnerships, identifies opportunities for growth, summarizes top takeaways for an effective partnership process, lists key pitfalls to avoid and previews the next steps BayBio plans to take to improve and stimulate more rewarding partnerships.

“Patient groups can do advocacy in Washington that pharmas can’t.”

*Robert Goldstein*  
Vice President  
Communications & Public Affairs  
ALS Therapy Development Institute

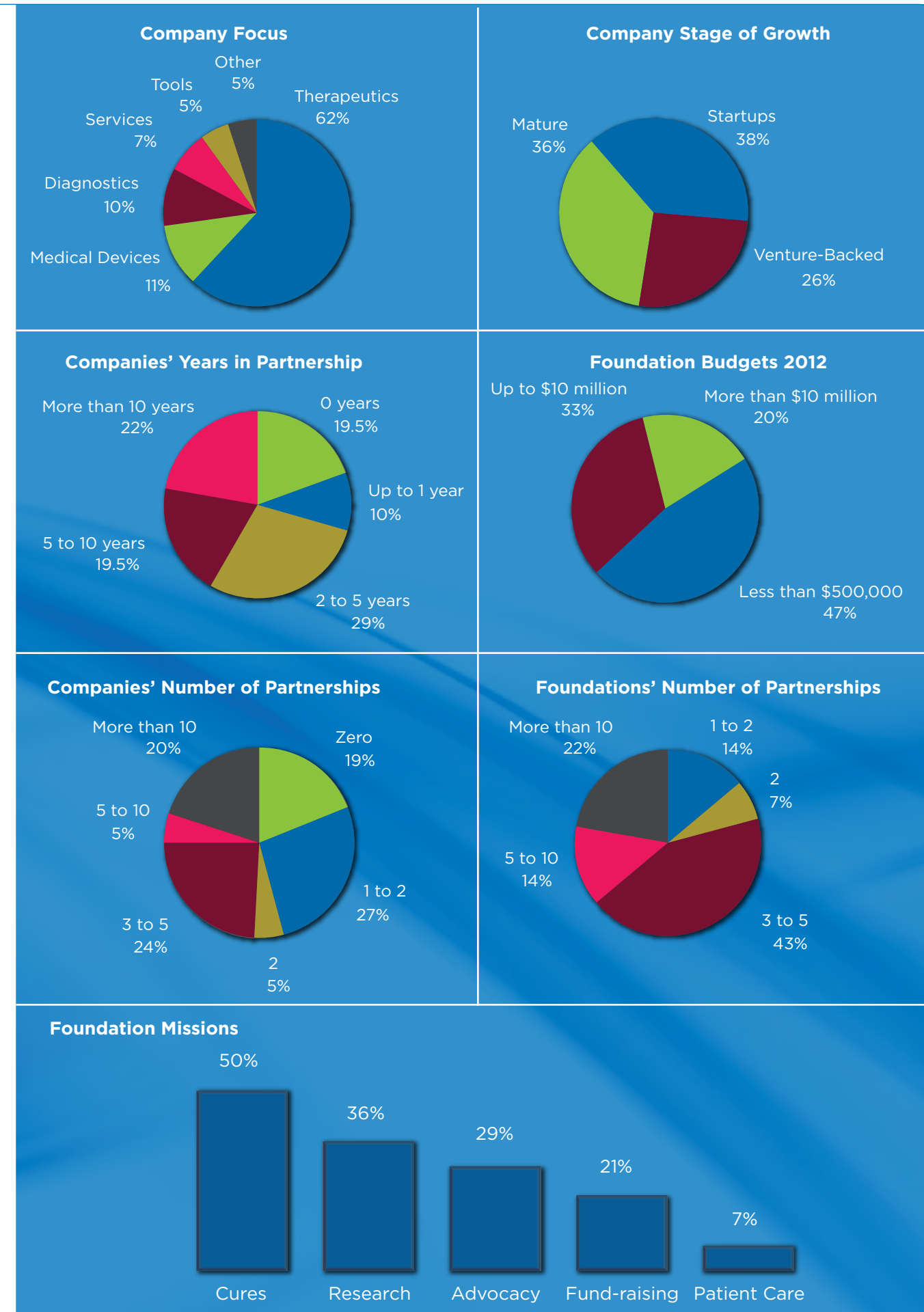


## Methodology

BayBio surveyed nearly 100 biotech and foundation CEOs, vice presidents of research, directors of patient advocacy and others across the United States, and conducted 17 in-depth leadership interviews from November 2012 to March 2013. Of the 63 biomedical companies, 62 percent focused on therapeutics with the rest developing medical devices, diagnostics, services and biotools. Of those, 38 percent were start-up, 26 percent venture-backed and 36 percent commercial, with market caps ranging from \$550 million to \$140 billion.<sup>5</sup> Seventy-four percent of company respondents had existing partnerships with foundations or wanted to develop them.

The 34 participating patient foundations represented missions focused on finding cures, funding research and advocating for patients across a range of diseases including cancers, diabetes and neurological disorders. While 20 percent of the foundation respondents had budgets exceeding \$10 million, nearly half of them had budgets of \$500,000 or less and the remaining third had up to \$10 million. All of the foundation respondents had existing partnerships with companies or wanted to develop them.

<sup>5</sup> Survey Definitions: "Start-up" companies support operations with initial funding, have scientific and research-stage programs, and have little or no sales revenue. "Venture-backed" companies support operations with secondary funding. Sales, development and/or licensing milestones extend capital access. Resources are directed toward internal program development. Products are typically in clinical development with potential for commercial approval and sales. "Commercial" companies are established enterprises with one or more FDA-approved products and/or commercially available products. They typically achieve profitability and actively invest in pipeline and growth strategies.



Benefits:

## Partnerships Can Accelerate and De-Risk Drug Development

A successful industry-foundation partnership occurs when each organization leverages its respective resources to create a greater return on investment. The contributions can be synergistic or additive, but ultimately each party benefits from questions, ideas and resources that sharpen the collaboration and its prospects.

“It’s been a tremendous help for me when raising venture money to say we received grants from foundations. External validation like this made my job a lot easier.

*Jeffrey Ostrove  
President & CEO  
Ceregene*

Foundations can bring assets to a partnership that otherwise would require significant investment by companies. Louis DeGennaro, Executive Vice President and Chief Mission Officer, Leukemia & Lymphoma Society, said: “Because of our access to patients, access to clinical trial sites and access to key opinion leaders, we can frequently resolve a company’s brewing issue.” He continued that for most of the foundation’s partnerships, “We are helping them over a certain hurdle, but we don’t have the resources to fully fund a drug development program.”

In addition to patient access, foundations also contribute monetary resources. In BayBio’s survey nearly 71 percent of company respondents said they received up to \$100,000 from foundations; 15 percent received \$100,000 to \$500,000, seven percent received up to \$1 million and seven percent received more than \$1 million.

Some foundations also provide robust scientific expertise. The Michael J. Fox Foundation for Parkinson Research has a large staff of devoted to research efforts, including ten scientists and one clinician. Their in-house team is responsible for plotting and managing the foundation’s aggressive research portfolio. This applicable knowledge of the science helps to inform partnerships. Certain foundations are able to proactively identify a company that has a technology they are interested in developing, according to David Lubitz, a partner in the law firm Schaner and Lubitz, PLLC, who represents mostly disease foundations.

Marc Bonnefoi, Head, North America R&D Hub, Sanofi, said foundations can offer other resources. “They have tools and models that can also be applied to research. They propose and provide help to design clinical trials and have a fantastic network of experts.”

“We know we can bring patients to the table for clinical trials, and that makes it a lot easier for companies.”

*Sharon Hesterlee  
Vice President, Research, Parent Project  
Muscular Dystrophy*

Foundations also call on a wide range of companies’ assets. The survey showed that 25 percent of company respondents provided their expertise to foundations and 25 percent gave funding, equity or royalties. Other offerings were products, products to the foundation’s primary stakeholders at below-market prices, research and clinical data, preclinical product development services and public recognition.

In addition to these, some foundations requested intellectual property rights, funding for educational programs and conferences, and collaboration on social media strategies.

Tracey Mumford, Senior Associate Director, Research Partnerships, The Michael J. Fox Foundation, reflected the sentiments of many foundation respondents when she spoke of the key benefits of working with the biotech industry. She said: “We get really interesting science from people who are zeroed in on getting treatments to the patients. I think some of the most interesting ideas come from biotech organizations. They’re in the business of science and so they understand the steps they need to commercialize it.”

## Benefits of Partnership

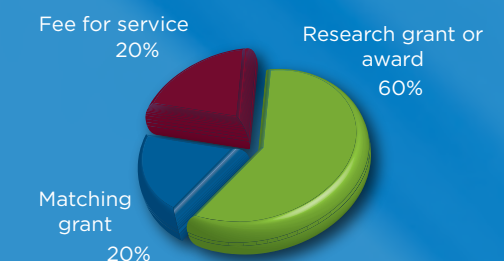
Foundations bring . . .

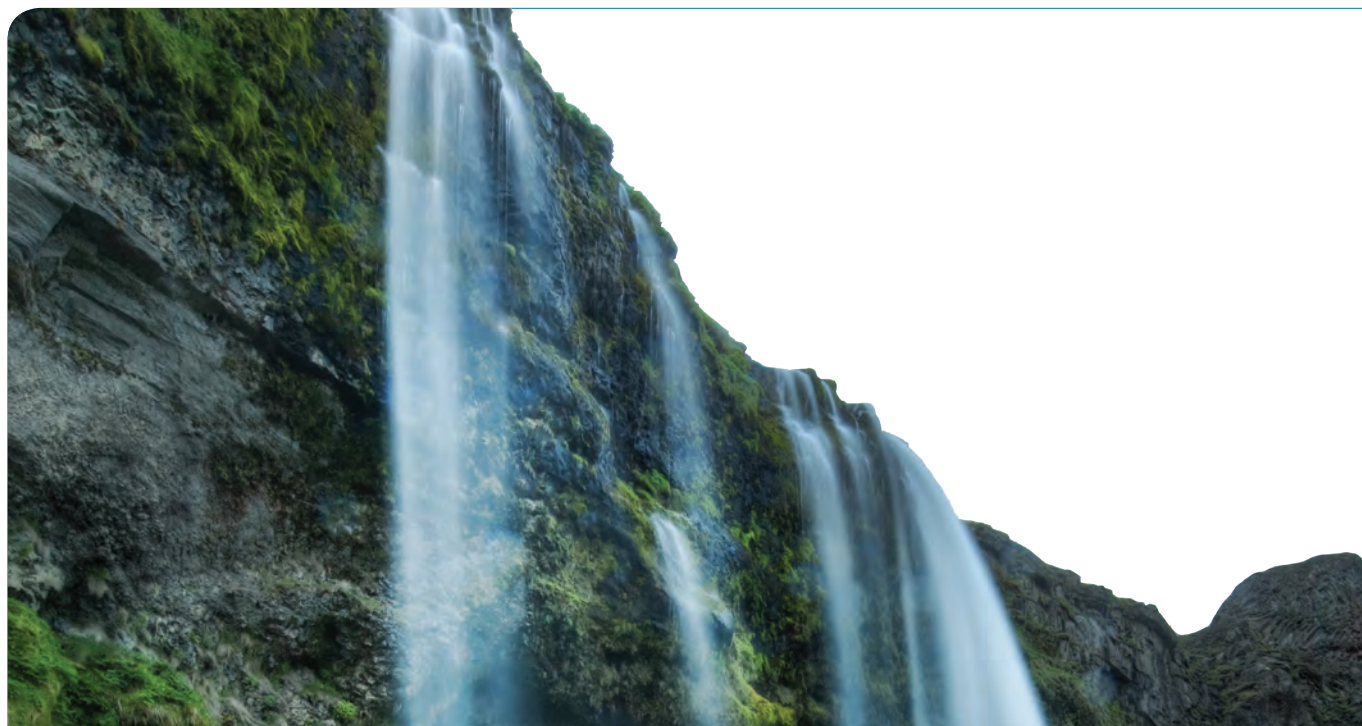
- Focus on patients’ needs
- Validation to venture capitalists of company’s direction
- Disease expertise
- Patient data like bio-specimen repositories
- Deep insight into patients, caregivers and FDA issues
- Access to patient populations for trials
- Advocacy to regulators
- Contacts with academia and media
- Funding

Companies bring . . .

- Commercialization expertise: getting treatments to patients
- New technologies
- Commitment to discovery
- R&D expertise
- Experienced scientists and equipped labs
- Knowledge of how private sector works
- Funding

### Type of Funding Companies Receive from Foundations

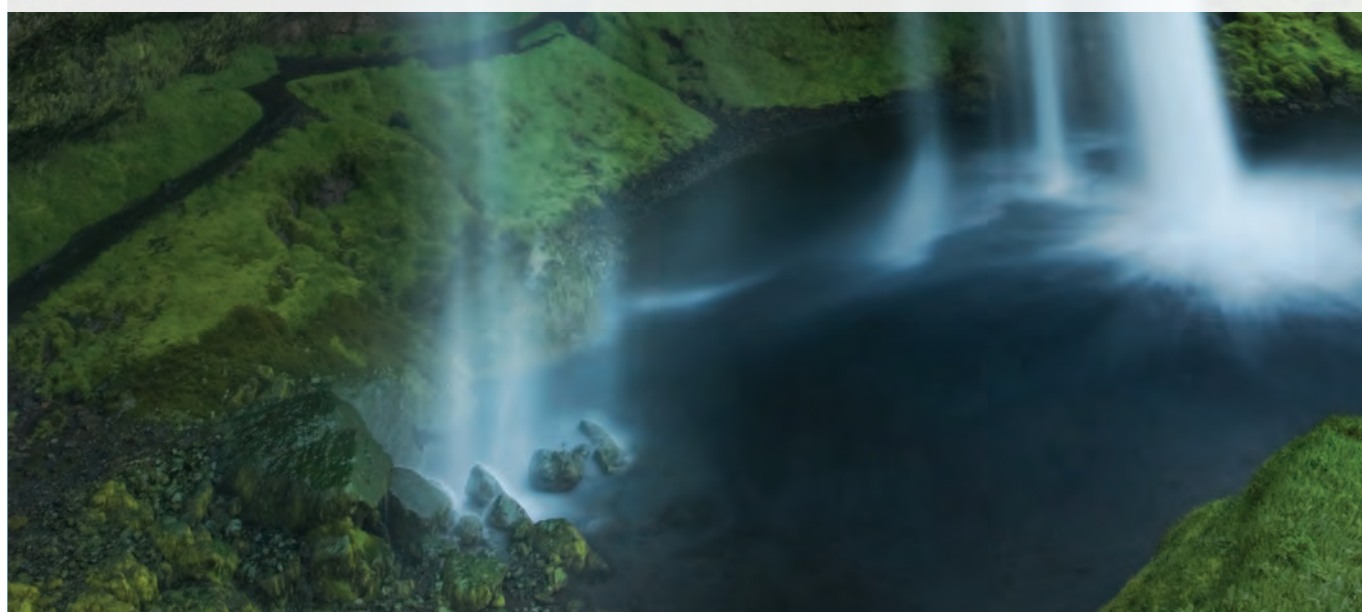




Opportunities:

## Tap New and Unexpected Resources

**B**ased on survey findings, companies anticipate ramping up their project-driven relationships with foundations and, concurrently, foundations anticipate increasing their budgets.



BayBio's survey shows that 85 percent of company respondents plan to increase the number of their partnerships. They cited the following needs for new resources as drivers for their expansion: the lack of private equity funding, cost reductions, more available patient information that could inform new disease models and biomarkers, clinical trial design and recruitment assistance, patient input to evaluate orphan indications, foundations' interest in product development, more stringent requirements from the FDA, and entry into new therapeutic areas.

Additionally, foundations are learning from their peers who have a track record of successful partnerships, such as the Cystic Fibrosis Foundation, Leukemia & Lymphoma Society, and The Michael J. Fox Foundation. Foundations that seek to explore partnerships are gaining a better understanding of how they can establish relationships with corporations by studying how their peers have done so.

“Sophisticated foundations know what they want and are moving to find biotechs that have assets they want to work with.”

*David Lubitz  
Partner  
Schaner and Lubitz, PLLC*

The opportunity to tap new resources is available at each stage of a product's lifecycle from discovery to market. For example:

- **Pre-Competitive:** To explore the potential of tapping new resources, Matthias von Herrath, MD, Vice President and Head, NovoNordisk Type 1 Diabetes R+D Center Seattle; Professor, La Jolla Institute, said: “I find we can do a lot in a nonconfidential space to understand where

### New Opportunities for Partnership

Foundation respondents said:

- 93% plan to increase budgets
- 36% plan to increase grants for translational science
- 14% plan to allocate more to research
- 14% plan to focus on cost efficiencies
- 7% plan to increase budgets each for regulatory, reimbursement, grants and new technologies

Company respondents said:

- 85% plan to increase number of partnerships

Of those 85%, more partnerships will focus on:

- 79% on specific diseases
- 33% on research
- 21% on patient advocacy
- 12% each on clinical trials and funding

something would go before you make thousands of CDAs or restrict space to operate.”

- **Drug Discovery:** In an early stage collaboration to accelerate a cure or treatment for multiple sclerosis (MS), the Myelin Repair Foundation recently joined efforts with Gencia Corporation to assess the myelin regenerating capabilities of the company's proprietary therapeutic compounds for MS.
- **Preclinical Development:** The Michael J. Fox Foundation (MJFF) is sponsoring a phase 1B study with a Sanofi deprioritized compound. MJFF included Sanofi representatives at their pre-IND meeting with the FDA. The IND was





- > Robust biobanks, registries & natural histories
- > Patient-led research networks
- > Patient-centered ideas for new treatments
- > Patient-focused scientific agenda

- > Validation of most promising compounds
- > Understanding of clinical practice guidelines

- > Access to patient group global, regional & national clinical research networks and KOLs
- > Patient-centered development input
- > Development of target product profile

- > Clinical trial accrual
- > Early-stage advisory panel

- > Public policy advocacy
- > Regulatory agency engagement
- > Reimbursement

- > Access programs
- > Media spokespersons
- > Brand and issue advisory boards
- > Disease awareness and patient education
- > Adherence
- > Screening and treatment guidelines

approved, so MJFF was able to immediately start the study and recruitment.

- **Clinical:** The Alzheimer’s Association, MJFF and JDRF, formerly known as the Juvenile Diabetes Research Foundation, have web sites to match patients with clinical trials to assist biopharma with recruitment. The Fox Trial Finder website has more than 23,000 registered volunteers willing to participate in Parkinson’s disease clinical trials.
- **Clinical:** During clinical trials IRBs often limit the total dollars that can be spent as well as what they can be spent on by companies. Childcare and travel for associated family members is often not covered. However these restrictions can limit clinical trial participation, especially in rare disease cases where you have to recruit outside your local area. To help companies with enrollment, Parent Project Muscular Dystrophy worked with trial sites directly by providing block grants between \$5,000 and \$50,000 for expense reimbursement, boosting company recruitment efforts.
- **Regulatory:** Neuraltus Pharmaceuticals worked with patient groups such as ALS Treat Us Now to garner their support for a meeting with the FDA on data from its Phase 2 clinical trials on NP001, a drug for amyotrophic lateral sclerosis (ALS). After meeting with the FDA, Neuraltus is now planning Phase 2B trials.
- **Commercial:** NPS Pharmaceuticals, a biotech that developed orphan drug Gattex for short bowel

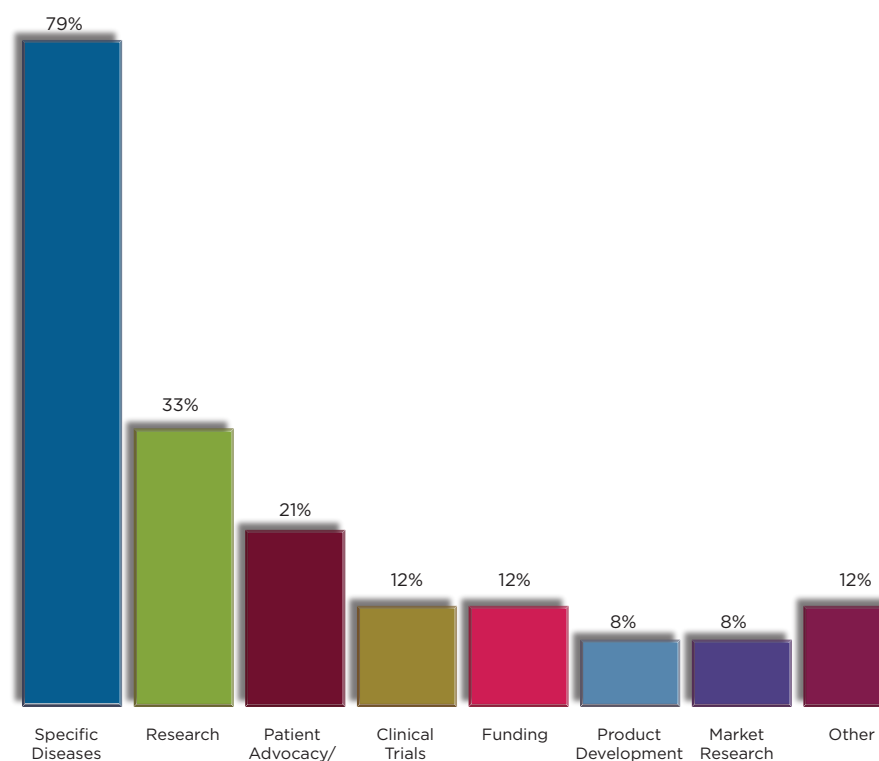
syndrome has worked closely with the Short Bowel Syndrome Foundation. The founder of the foundation strategized with NPS marketing employees on how to promote Gattex to its social network of 1,000 patients and caregivers. “It’s a nice, close relationship,” said founder Andrew E. Jablonski. “I’m an asset to them and they’re an asset to me.”<sup>5</sup>

Opportunities for partnership exist not only with companies that focus on specific diseases, but also with companies that develop platform technologies. Lindy Fishburne, Executive Director, Breakout Labs/Thiel Foundation, said: “There’s a huge opportunity for foundations to be engaged in innovation more broadly. If a platform works, then it has phenomenal implications for major diseases. I think a lot of big breakthroughs will actually come at the platform level.”

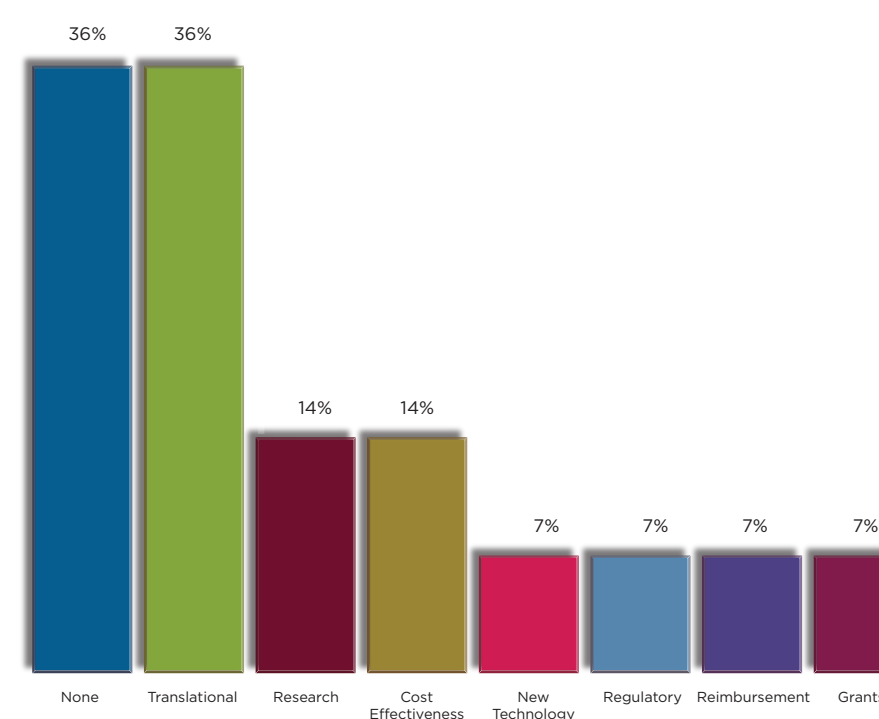
Disease foundation and biotech company collaborations are a growing field open to innovators who can see partnership opportunities that fill unmet medical needs. As the sidebar on the previous page indicates, challenges in the biotech industry have led some companies and foundations to reassess their priorities in working with the other sector. This, in turn, will open new opportunities for public-private partnerships.

<sup>5</sup> The New York Times, “Making ‘Every Patient Counts’ a Business Imperative,” Jan. 30, 2013

How Companies will Change Focus with Foundations in the Future



How Foundations will Change Missions in the Future





Roadmap

## Best Practices Can Greatly Enhance Outcomes

The national survey and leadership perspective produced a unified view of the five best practices that can be applied as a roadmap to structuring and managing successful partnerships. From the outset, each partner must have: a stake in the success, decision-making power and a commitment to execution.

“Once you have one good collaboration experience, it opens the door to many other great experiences going forward.”

*Tracey Mumford  
Associate Director  
Michael J. Fox Foundation*

BayBio's survey of companies and foundations reveals five high-priority areas where the two groups shared significant interests for collaboration, along with lower-ranked priorities. These survey results can serve as a guideline for constructing common goals, while facilitating a fundamental consensus on a shared vision and specific objectives.

### Best Practice #1. Vision and Goal Alignment: Aim for Same Bull's Eye

While industry and foundations enter into partnerships with different, but complementary, agendas and resources, they are united in their vision and goals to find new therapies and produce a return on investment for their stakeholders.

Visions inspire and goals motivate all healthy organizations whether the vision is narrowly defined or break-through. The following examples illustrate the scope visions can take:

**On a targeted scale,** Novo Nordisk and JDRF are working together to create an official repository of negative data for Type 1 diabetes. "Think about how much less we'd spin our wheels if we all knew what had already been done," said Matthias von Herrath, MD, Vice President and Head, NovoNordisk Type 1 Diabetes R+D Center Seattle; Professor, La Jolla Institute.

**On a larger scale,** JDRF and Medtronic Diabetes, a business unit of Medtronic, share a vision of creating an artificial pancreas. One of their mutual goals is to review redundant sensing technologies. "Ultimately, we're trying to move quickly and expeditiously to get closed-loop systems to the market," said John Mastrototaro, Vice President Research, Technology and Business Development/Chief Technology Officer, Medtronic Diabetes.

**On a systemic scale,** The Michael J. Fox Foundation organized a workshop with 16 representatives from the FDA and 40 key opinion leaders on cognitive

### Companies and Foundations Align Priorities

BayBio's survey showed:

High-priority shared interests:

- Understanding patients and caregivers
- Scientific and clinical advice
- Funding R&D
- Clinical trial design
- Patient Advocacy

Mid-priority shared interests:

- Clinical trial recruitment
- Patient advocacy

Low-priority shared interests:

- Reimbursement policy
- Post-market research
- Creating an innovative database
- Advancing translational science
- Learning why a treatment failed
- Failing faster

impairment from industry, the NIH, patient advocacy groups and disease research communities. The result was a consensus strategy for therapeutic development in cognitive impairment that will speed the testing and review of drugs. MJFF plans to publish the findings in a peer reviewed journal to share with the larger scientific community.

While it may be obvious for potential partners to identify a shared vision, it is essential to be clear on matters of misalignment. Those are the areas that can get a partnership into trouble. For example, the survey shows that the most common mismatch in

“Build relationships with patient groups before you need them. There may come a day when they could help you with the FDA or Congress.”

*Andrew Gengos  
President, CEO and Director  
ImmunoCellular*

priorities between companies and foundations was market research: companies ranked it number three and foundations ranked it number nine.

A fundamental source of misalignment can stem from different missions and priorities of foundations and companies. Foundations tend to focus on a single or family of related diseases, while many companies focus on maintaining diverse pipelines that reduce dependence on one outcome and risk exposure. Foundations concentrate primarily on supporting patients through finding cures, educating stakeholders and raising public awareness; companies concentrate on driving value for investors. Other areas of misalignments may surface in different expertise, leadership commitment, resources and experience in partnering.

Not surprisingly, company respondents that were more closely aligned with foundations advocated for early engagement. Companies reported that forming relationships prior to late-stage development generated a number of important advantages including tracking with patient needs, and helping corporate scientists understand the urgency and importance of programs in development and motivation. Executives also reported that involvement in fundraising and volunteer activities were high on their radar screens. To that end, 75 percent of Opexa Therapeutics employees and their families participate in National Multiple Sclerosis Society walks, facilitating informal discussions and

### What Defines Success?

“Don’t bite off more than you can chew,” advises John Mstrototaro, VP Research, Technology and Business Development/Chief Technology Officer at Medtronic Diabetes.

“The final milestone may not be ‘commercialize this product.’ It might be ‘complete this clinical trial with these metrics.’”

Each partnership will define its own measures of success, which may include:

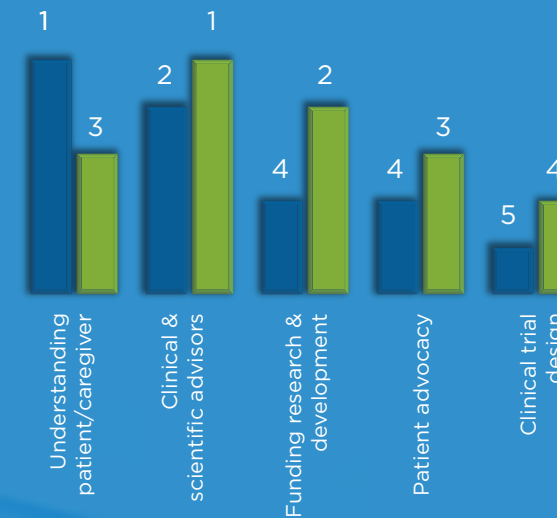
- Creating an innovative database
- Collaborating on designing a trial
- Learning why a treatment failed
- Advancing translational science
- Failing faster

personal connections, and motivating employees as to the urgency of their mission to impact progressive MS. Other companies have engaged in a broad range of awareness and fundraising activities, which bring together leadership and patients to interact and learn from one another. Some companies host a patient appreciation day by inviting patient groups and health care professionals to tour the company’s labs, followed by a discussion with scientists.

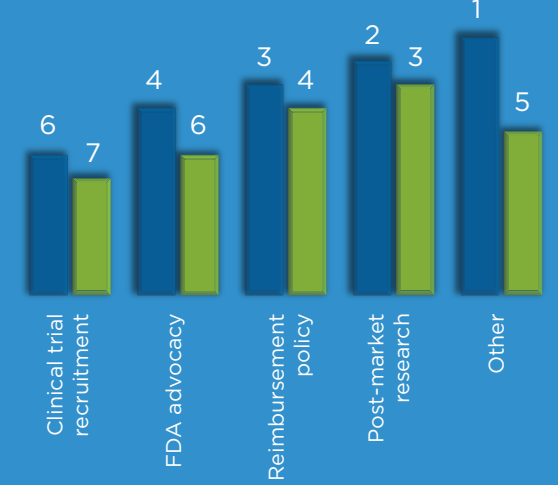
### Company/Foundation Priorities on Vision Alignment

Company Rank Foundation Rank

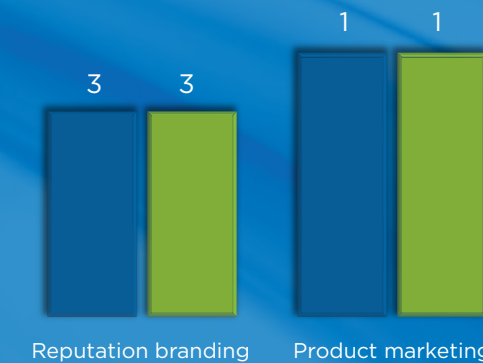
#### Top 5 Matches



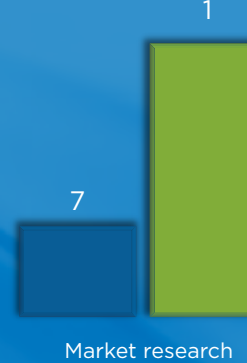
#### Close Alignment - Mid-Priorities



#### Match - Low Priorities

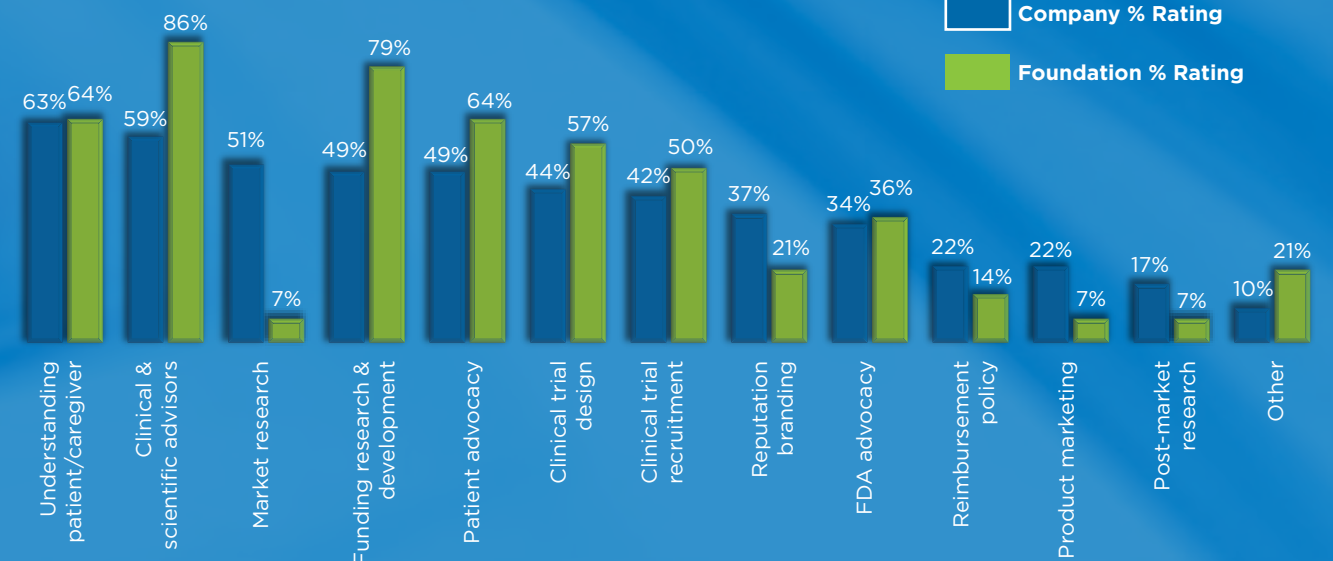


#### Top Mismatch



### Company and Foundation Raw Data

Company % Rating Foundation % Rating





## Best Practice #2.

### Resource Alignment: Get It Right from the Start

Resource alignment focuses on identifying the scientific, financial and organizational needs of the project, assessing the available joint resources and agreeing on how they can be effectively applied to project outcomes. Resources include funding, personnel, knowledge, skill sets, data, contacts and time.

The partnerships that worked best had equal commitment on both sides to adequately resource the projects and bring the right talent to the table,

“Do you have all the gaps filled by bringing the two parties together?”

*Linda Molnar, Founder & CEO  
Kirhla, Inc.*

according to Louis DeGennaro, Executive Vice President and Chief Mission Officer, Leukemia & Lymphoma Society. “I don’t think it’s a mystery. Resources on both sides need to be adequate to get the job done.”

It is crucial for prospective partners to focus on clarity and specificity when discussing resources. John Mastrototaro, Vice President, Research,

### Foundation Funding Focus

Of the foundation funds company respondents received:

- 60% was for grants or awards
- 20% was for matching grants
- 20% was for fees for service

(For dollar amounts, please see chart on page XX.)

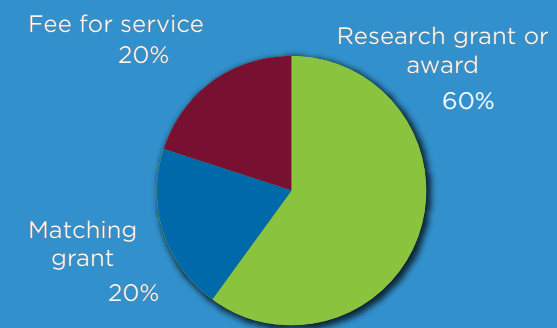
Technology and Business Development and Chief Technology Officer, Medtronic Diabetes, said: “I think there needs to be a lot of clarity when going through the process of establishing the relationships, in terms of what the exact objectives are and making sure they’ve really mapped out fully what resources and timelines they believe they’re going to hit.”

Louis DeGennaro, Executive Vice President and Chief Mission Officer, Leukemia & Lymphoma Society, pointed out the implications of inadequate resources. “If we find ourselves bumping up against the same milestone time after time, you’re forced to ask yourself: ‘Do we have it resourced properly? Are we asking the right questions? What else do we need to bring to the table?’”

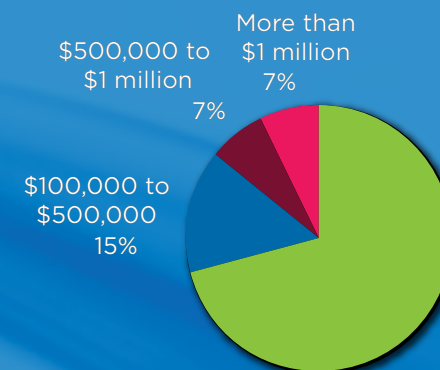
“It doesn’t cost a lot of money to partner with foundations. You just have to spend the time and have a genuine interest in patients.”

*Andrew Gengos  
President, CEO and Director  
ImmunoCellular*

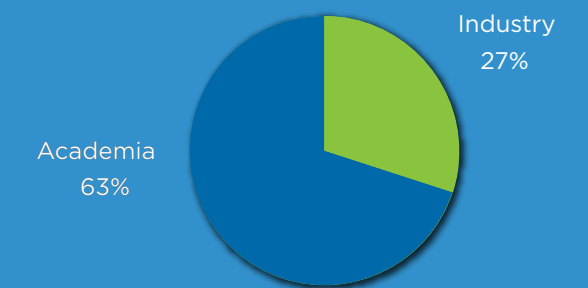
### Type of Funding Companies Receive from Foundations



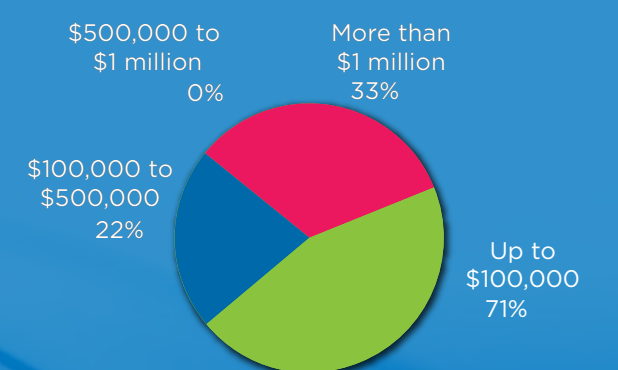
### Amount Companies Received from Foundations



### Foundation Funding to Academia and Life Science



### Amount Foundations Gave to Companies



Vertex Pharmaceuticals has adopted a portfolio approach towards foundation relationships with multiple partners to fill in their resource gaps, which also can offer validation at the national patient advocacy level. Dr. Youssef Bennani, Vice President, Research and Development, Vertex Pharmaceuticals, said in reference to the partnership with the Crohn’s & Colitis Foundation of Canada: “We’re funding basic research projects that meet our objectives based on scientific merit, obviously, but also on translatability. We want to leverage the few dollars we have.”

Thomas P. Sellers, Senior Director Patient Advocacy & Corporate Philanthropy, Millennium: The Takeda Oncology Company, said partnerships work best when a company has part of its organization

clearly responsible for managing relationships with patient organizations. “It’s not something that I think generally works to be 25 percent of the job of somebody in marketing,” he said. “You need dedicated staff, but it doesn’t need to be a lot of staff.”

An effective approach is for each partner to identify representatives at equal levels of seniority because that conveys the importance the organization puts on the project. In the end, Klaus Romero, Director of Clinical Pharmacology, Critical Path Institute, summed up the most important point on resource alignment: “As long as you identify the right people within the different organizations, it flows.”



## Best Practice #3.

### Partnership Structure: Prevent Predictable Problems

Public-private partnerships can be challenging due to different operating styles and values. Each partner is shaped by its mission, priorities, leadership, expertise, resources, and experience in partnering with the other sector. Therefore it is important to agree from the beginning on issues of governance, roles, responsibilities and accountability.

Governance, the rules and processes that guide an organization, focuses on who or what body has the authority to make decisions. The simplicity or complexity of governance depends on the needs of the partnership and the culture each partner brings. Matthias von Herrath, MD, Vice President and Head, NovoNordisk Type 1 Diabetes R+D Center Seattle; Professor, La Jolla Institute, favors keeping it simple. "It's good if only a few people manage the partnership. That's how we're doing it with the JDRF. You get other departments - legal and other things - involved as needed if you have a complicated transaction."

“ We both have skin in the game. We set up win-win relationships so we both have incentives for success.”

*Kevin Glasgow, CEO, Crohn's & Colitis Foundation of Canada*

More complex projects, or a portfolio of partners, require a more structured and systematic approach, as described in Best Practice #4 - Management Models.

Regardless of the project, there is a common universe of big and small matters that industry-foundation partnerships must negotiate in advance of signing a memorandum of understanding or a contract, according to David Lubitz, a partner in the law firm Schaner and Lubitz PLLC. It is important that the partnership is clear on who makes or contributes to what type of decision.

Big issues include such decisions as:

- How will a foundation be paid if a technology becomes commercially viable?
- How can the parties reach a mutually agreeable solution, if a biotech company decides that its technology will not be profitable enough to continue, but the foundation wants to make use of the technology?
- What if the biotech fails for business reasons?
- Small issues include:
  - How does the partnership handle research licenses for outcomes that develop as part of the technology, like data or research tools, but are not at the core of the invention?
  - At what level should that material be available to other scientific researchers?
  - If a biotech is sold to a pharma, should the terms of the foundation partnership be changed?

Roles and responsibilities, like governance, should also be defined at the beginning of a partnership. A complicating factor for small organizations is that employees often cover several different roles and thus need to be clear on how to allocate their time. "It involves collaboration at a lot of different levels," said Thomas P. Sellers, Senior Director Patient Advocacy & Corporate Philanthropy, Millennium: The Takeda Oncology Company. "There are different kinds of roles based on the stage of the continuum from discovery through commercialization."

Furthermore, for a successful partnership to develop, accountability should be specified for milestones: how they will be measured, frequency of meetings and communications, deliverables and timetables.

Finally, a theme that emerged from BayBio's in-depth interviews was that some foundations feel they are not receiving the same respect as other kinds of partners or investors. Sharon Hesterlee, Vice President, Research, Parent Project Muscular Dystrophy, said: "We've had good experiences with some companies, where we felt like we were treated as business partners and equals, and others where they just didn't understand the value we brought to

the table, because it wasn't just funding - it was also our extensive networks."

Hesterlee urges both partners to put everything into writing. She said, "We like contracts. It's nice to have a roadmap that lays out how success will be defined and measured as well as, in the worst case scenario, how everything gets divided up and distributed."

## Best Practice #4.

### Management Models: Build on Successful Business Models

Effective alliance and project management models, with formal structures and systems, drive results and generate value for organizations and their stakeholders. Honed by the private sector during the last 30 years, alliance management is now an accepted part of most companies' competitive and growth strategies. It is a business function with disciplines and metrics, like any other business process, and the alliance manager has emerged as a key player in guiding a project or product to success.

BayBio's interviews with foundation and industry executives revealed that successful partnerships have moved to adopt private sector alliance management models and methodologies. Such an approach is essential because two partners may have a shared vision and goals, but have very different views and operational approaches on how to reach those goals. Good models clearly answer the question: "How?" Both partners should agree on how they define objectives, motivate efforts, coordinate activities and allocate resources.

Survey respondents agree that a steering committee, advisory board or project management team is a key management structure to direct and manage alignment on progress, issues and concerns, and to avoid miscommunications. The committee purpose, structure and skills set depended on the project requirements.

The system of joint committees and scientific and technical milestones works well to gage progress of collaborations between large pharmaceutical and small biotech companies. Relationships between industry and foundations can be structured in a similar way to ensure success.

Following are management systems adopted by three successful partnerships:

- **Vertex Pharmaceuticals and Crohn's & Colitis Foundation of Canada (CCCF):** Youssef Bennani, Vice President, Research and Development, Vertex Pharmaceuticals has a sharp focus on what Vertex wants to accomplish and thus needs an effective management structure to do so. In partnering with CCCF, he said they created a structure that allowed them to "move forward pretty fast." Vertex put together what Bennani calls a "mini-equisystem," of about 100 people from foundations and universities all focused on inflammatory bowel disease, a new disease area for Vertex, and supported it with "inter-institutional" research agreements. Representatives of each partner meet regularly as a group to examine the progress made by each one. They also have a joint research committee "trying to define translatability. We all feel that it's not as well translated or transcribed as it needs to be."
- **Ceregene and several ALS groups:** Jeffrey Ostrove, President and CEO of Ceregene, was part of a steering committee that included several ALS groups and the Robert Packard Foundation. He said their regular meetings became a kind of think tank where he would report on the status of his team's progress and data. "The groups asked a lot of good questions," he said. "We had a very positive interaction trying to move the field forward."
- **Leukemia & Lymphoma Society and various companies:** Louis DeGennaro, Executive Vice President and Chief Mission Officer, L&LS, said every biotech partnership they have centers around a committee called the Research Advisory Committee with members from the company and the foundation. He said: "That committee is really there not so much to oversee how the project goes but to anticipate issues or problems or challenges and bring the collective resources of the two organizations together to anticipate what those problems might be and hopefully deal with

them ahead of time. We think that makes for a very effective partnership."

Another process commonly cited in BayBio's survey and interviews is the need for a point person or alliance manager representing each party. This dedicated person would be a project manager, facilitator and gate-keeper to ensure the team, as well as the internal organization, is consulted, informed and held accountable.

John Mastrototaro, Vice President Research, Technology and Business Development/Chief Technology Officer, Medtronic Diabetes, said: "One of the things you need to establish is an identified alliance partner - in other words, one point of contact whose job is to assure that the communication between us and the other agency is effectively managed. We've found that if there is one point of contact on both sides, and those two people develop a relationship and rapport, then it leads to a much better alliance."

The key characteristic needed for an effective point person is one who has experience in managing projects and driving the process. Correspondingly, the point person needs specific skills when representing a company or a foundation.

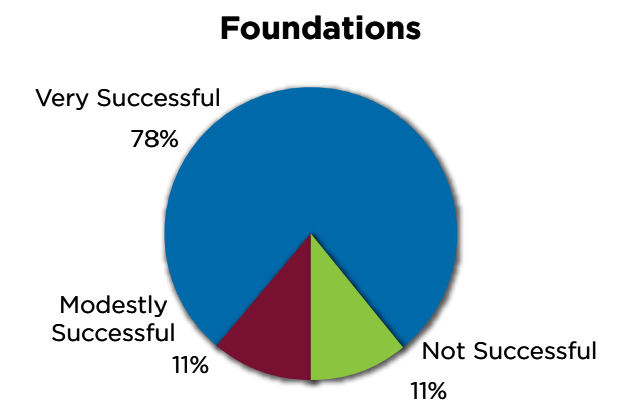
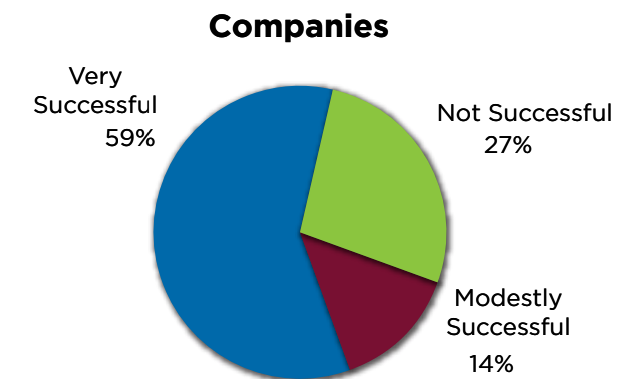
Linda Molnar, Founder and CEO, Kirhla Inc., said the company point person does not need a clinical background, but should be interested in understanding issues from the patient's perspective, dedicated to knowing the foundation, willing to work with the foundation on a regular basis and committed to following the company's engagement plan.

The foundation point person is best served by understanding the discovery and commercialization process relevant to the project. This person must also be able to get the foundation to act quickly when the project calls for it.

As part of BayBio's survey, companies and foundations were asked to rate the success of their partnerships and add comments on their reasons

for their ratings. Both groups identified that a well-managed process was crucial to success.

Like other best practices, one size does not fit all due to such factors as stage of organizational growth, experience in partnering, number of partners and funding. The partnership needs to discuss and decide management choices based on the unique factors in their collaboration.



Looking ahead, Stuart Kilman, Partner, Vantage Partners, said that alliance management will increasingly become more of an imperative. As the biomedical business landscape becomes more complex and interdependent, as science becomes harder and the stakes stay high, he said organizations will need to think beyond alliance management as a function and instead design the organization itself around alliance capabilities.<sup>5</sup>

<sup>5</sup> Cambridge Health Institute, podcast with Stuart Kilman, 2013



## Best Practice #5.

### **Open & Frequent Communication: Make it Authentic**

Communications can make or break any team, especially one that combines different cultures, missions, organizational styles and areas of expertise. It is crucial to agree, before signing any contracts, how communications will work in terms of frequency, mode of communication, agenda-creation, type of information to be shared, definition of transparency and key team contacts for all these purposes.

It is up to each partnership to define transparency and confidentiality. A good checklist for transparency is to define what the following components mean: disclosure, accuracy, clarity, timeliness of information, and inclusivity. The bottom line is: No surprises.

The compelling rationale for transparency is it fosters trust and respect, requirements for any successful partnership. A mutual commitment to transparency drives clarity, invites more

“By far, transparency of communication is the most important element of a successful project.”

*Louis DeGennaro  
Executive Vice President  
Chief Mission Officer  
Leukemia & Lymphoma Society*

information sharing, improves decision-making, and increases team motivation and productivity. As Robert Goldstein, Vice President, Marketing Communications & Development, ALS Therapy Development Institute, said: “There’s nothing more important than having your partner trust what comes out of your mouth.”

Given how common misinterpretations are, even in everyday life, a team comprised of different cultures needs to be clear on all definitions. For example, in one partnership, a team member did not know that a “naïve patient” is one who has not taken a specific compound or drug to be tested in a clinical trial. That misunderstanding led to accusations of

“abusing patients” before the misunderstanding could be cleared up.

Another key lesson from the survey is the importance of companies communicating with their partners even if they do not have anything new they can share. Sharon Hesterlee, Vice President Research, Parent Project Muscular Dystrophy (PPMD), said that when a company is under stress and pressure, it sometimes “circles the wagons and

“Commit to weekly calls and never miss one. Go with an agenda and action items. Don’t ever take a partnership for granted.”

*Robert Goldstein  
Vice President, Marketing,  
Communications & Development  
ALS Therapy Development Institute*

stops talking to anybody and everything goes south. Nobody trusts anybody.” She explained: “Silence is the worst. “The patient community assumes the worst: the drug has failed. And then it gets all over Facebook.”

Hesterlee added that trusted communications can encourage a foundation to act like an early warning system for the company. “They can tell the company what the chatter is among patients and on the internet, and urge the company to address the issue.” She cited an example from her experience at PPMD when a company halted a clinical trial for efficacy reasons, but the patients still wanted access. The company wanted to create a continued-access program so, she said: “We funded it for them, which took the heat off of the company because they were getting killed from the patient community. If we can work with a company, we can help solve problems.”

Several interviewees mentioned the advantages of using social media to connect with patients, healthcare professionals and media. John Mastrototaro, Vice President Research, Technology and Business Development, Chief Mission Officer, Medtronic Diabetes, recommends investing in social media activities to educate stakeholders about what the company is doing to help them, which also could assist in building a future market. “We’re heavy into Facebook, YouTube and Twitter. We’re partnering with patient advocacy groups that may have online diabetes sites or blogging sites.” He has given several educational talks about Medtronic’s pathway to an artificial pancreas in partnership with JDRF and posted them on the web. The online venue allows viewers to watch the presentation in real-time or to access it later with viewers’ comments.

Lastly, given the different cultures in the partnership, an appropriate communication style for collaboration is necessary. Both partners need to have the ability and willingness to be flexible, negotiate and compromise.



## Top Takeaways for Successful Partnerships

Respondents offered advice on how to make the partnership process more effective and productive. Their comments below elaborate on the survey results.

### 1. Educate Your Partner

“Once you recognize what everyone can bring to the table – not only financial value, but intellectual capital and personal connections -- I think you very quickly get on the same page.”

*Tracey Mumford, Senior Associate Director, Research Partnerships, The Michael J. Fox Foundation*

Both partners need to educate each other on their capabilities, priorities, what they need and what they can do. Opportunities for companies: biotech executives surveyed said 59% of foundations do not understand the R&D process or do not understand it well. Explain the key

elements of your business and how the R&D process works. When foundations understand your business better, they will be able to identify ways their capabilities can help the company that you may not have considered.

Opportunities for foundations: Foundation executives said 75% of companies underestimate foundations’ full capabilities. Explain all the ways you can add value, including your competitive advantage. Learn the company’s priorities because that is where their interests lie. Look at their website and learn what is on the market and which patients they target. Craft your presentations to those priorities.

### 2. Communicate, Communicate, Communicate

“We always knew what Genzyme was doing so we could give that message to the clinicians who would be treating and prescribing.”

*Sharon Hesterlee, Vice President Research, Parent Project Muscular Dystrophy*

Good communications build trust. Opportunities for companies: In the in-depth interviews, foundations often mentioned the need for better communications, including transparency, clarity and frequency. Let foundations know what you are doing. The foundations talk to their donors, venture capitalists, regulators, payors, prescribers, patients and the media.

Opportunities for foundations: The interviews showed that biotech executives are not clear on what information or when foundations needed to know it. Be specific about your expectations during discussions on alignment. In addition, it is important to communicate to your patient stakeholders that biotech works in milestones, not in “get this drug to market.” Communicate to patients the R&D process and timelines.

### 3. Agree Early When Data can be Shared Outside the Partnership

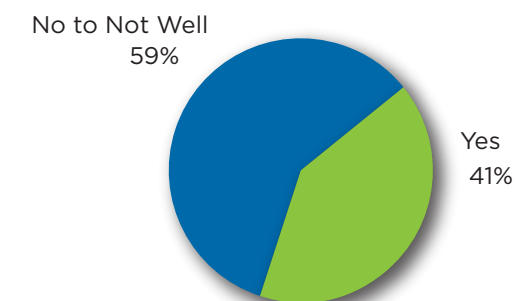
“Biotech companies typically want to keep everything confidential while disease foundations want to make it public. Certainly foundations understand we work in a commercial system. But they have a higher interest in getting potentially helpful research to others in the field.”

*David Lubitz, Partner, Schaner and Lubitz, PLLC*

Resolve this issue early. Opportunities for companies: In interviews, foundations said they can interact with you better if you are willing share information you have generated on the joint project. That can be a challenge given legal issues and a foundation’s level of scientific knowledge. However, it is worth the investment of time because foundations say the more information they have, the more helpful they can be.

Opportunities for foundations: The interviews highlighted that the more you understand the biotech business, the better you will understand how and why biotech executives communicate – or not – as they do. Ask questions, but accept that there are times when publicly-held companies cannot share information that is bound by SEC laws.

### Do Foundations Understand R&D?



### Do Companies Understand Foundations’ Capabilities?





## Lessons Learned

David Lubitz, Partner, Schaner and Lubitz, PLLC, said that unrealistic expectations from each partner often originate from a lack of experience in partnering or lack of knowledge in the field. To avoid common pitfalls, below are a few tips from the experts.

### Lessons for Companies

1. “The number one mistake that companies can make is to only reach out to patient groups when they want something from them.”  
*Andrew Gengos, President, CEO and Director, ImmunoCellular*
2. “If the company expects that a patient organization is just going to be an extension of their marketing team, then they are sorely mistaken.”  
*Thomas P. Sellers, Patient Advocacy & Corporate Philanthropy, Millennium: The Takeda Oncology Company*
3. “Don’t ever hype the research or timelines. Be honest. If you’re not going to make it, then you need to communicate that.”  
*Jeffrey Ostrove, President & CEO, Ceregene*
4. “Go speak to the families, meet the patients. That really makes it clear what the goal is. Get out and talk to the people who are actually going to buy it.”  
*Linda Molnar, Founder & CEO, Kirhla Inc.*

### Lessons for Foundations

1. “Foundations need to understand what’s reasonable. If you give a startup \$50,000 to do a pilot study, you don’t own half the company.”  
*Sharon Hesterlee, Vice President Research, Parent Project Muscular Dystrophy*
2. “One of the things the biotech world does really well is catalog and prioritize projects. Foundations fail at getting their projects into that queue.”  
*Robert Goldstein, Vice President, Marketing,*

*Communications & Development, ALS Therapy Development Institute*

3. “You have to learn to speak the language of the guy sitting at the other end of the table. You may get some constituent feedback that ‘Hey, you’re looking too much like a pharma.’ That hurts. But if your goal is to get the drug to the patient, ignore the feedback.”

*Robert Goldstein, Vice President, Marketing, Communications & Development, ALS Therapy Development Institute*

## Summary

**BayBio’s survey and interviews show that companies underestimate the full range of assets a foundation can bring to the drug development process, and foundations underestimate their influence with regulators and payors.**

“It really begs the question: Why weren’t we doing this from the very beginning?”

*Linda Molnar  
Founder & CEO  
Kirhla Inc.*

Industry-patient foundation partnerships have the potential to drive product outcomes and change patients’ lives. The collaborations represent a dynamic, developing area of business opportunity and innovation. There are ample ways to mitigate industry challenges by partnering across the discovery-to-commercialization value chain. Each partner can bring expertise and resources the other lacks, advance science together, develop treatments and get products to patients.

BayBio’s “Successful Public-Private Partnerships” is intended to provide insights and guidance to companies who have never created a partnership or enjoyed a truly successful collaboration.

## Looking Ahead

In the coming months, BayBio will conduct seminars, workshops and webinars on key subjects including business models for funded partnerships, patient-driven clinical trial design and recruitment, reinvestment in foundations, regulatory and payor considerations, and alliance management. We welcome hearing your feedback and experiences at [misha@baybio.org](mailto:misha@baybio.org).

## Additional Resources

### Where to Start

If a company has never engaged in a partnership with a patient foundation, it can be overwhelming on where to start. Here are a few tips:

- Talk to similar-sized organizations in your field about how they began partnering.
- Conduct research on biotechs or foundations in your disease area.
- Get introduced to a biotech or foundation for an informal talk.
- Attend a BayBio seminar or workshop.
- Check an organization’s website for sections like “Opportunities for Partnership.”

### How to Find Patient Groups with Partnering Experience

FasterCures, a nonprofit dedicated to accelerating medical research, has an inventory of 55 patient groups with partnering experience in its TRAIN program (The Research and Innovation Network) at <http://train.fastercures.org/traininventory>. TRAIN is a platform for venture philanthropy for medical research. The inventory of organizations is designed to “help potential collaborators better

understand the landscape of nonprofit disease research foundations and engage in meaningful partnerships with them. Using a set of common metrics, it highlights key details about each organization’s research portfolio, collaboration efforts, and financials.”

### How to Improve Alliance Management Knowledge and Skills

Organizations that specialize in alliance management frequently host conferences and offer classes. Other venues can be found through professional organizations.

## Acknowledgements

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- Robert Goldstein, Vice President, Marketing, Communications & Development, ALS Therapy Development Institute
- Sharon Hesterlee, Vice President Research, Parent Project Muscular Dystrophy
- David Lubitz, Partner, Schaner and Lubitz, PLLC
- John Mastrototaro, Vice President, Research, Technology and Business Development / Chief Technology Officer, Medtronic Diabetes
- Linda Molnar, Founder & CEO, Kirhla Inc.
- Tracey Mumford, Senior Associate Director, Research Partnerships, The Michael J. Fox Foundation
- Jeffrey Ostrove, President and CEO, Ceregene
- Klaus Romero, Director of Clinical Pharmacology, Critical Path Institute
- Thomas P. Sellers, Senior Director, Patient Advocacy & Corporate Philanthropy, Millennium: The Takeda Oncology Co.
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- Marc Bonnefoi, Head, North America R&D Hub, Sanofi
- Kevin Glasgow, MD, MBA, MHSc, Chief Executive Officer, Crohn's and Colitis Foundation of Canada
- Todd Sherer, Chief Executive Officer, The Michael J. Fox Foundation

Finally, we want to express our deepest appreciation to organizations that helped to bring this paper to life:

### FasterCures

FasterCures, a center of the Milken Institute, is an 'action tank' that works to improve the medical research system – so that we can speed up the time it takes to get important new medicines from discovery to patients. We want the system to move faster. How do we do this? We convene innovators, we make sure our national policy supports accelerated research, we develop programs with a laser focus on what is working, we identify problems standing in the way of progress, and we turn to disruptive ideas and best practices to pave the path toward solutions.

To learn more, visit [www.fastercures.org](http://www.fastercures.org).

### BIO

The Biotechnology Industry Organization (BIO) is the world's largest biotechnology trade association. BIO is a 501(c) (6) non-profit organization headquartered in Washington, D.C. providing advocacy, business development, and communications services for more than 1,100 members worldwide. BIO's mission is to be the champion of biotechnology and the advocate for our member organizations - both large and small. BIO members - many of whom are small emerging companies - are involved in the research and development of innovative healthcare, agricultural,

industrial and environmental biotechnology products. They work every day to improve the human condition by curing the sick, feeding the hungry or developing cleaner, safer and healthier sources of energy.

To learn about BIO and its service to the industry, please visit [www.bio.org](http://www.bio.org).

### Merrill Corporation

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### About BayBio

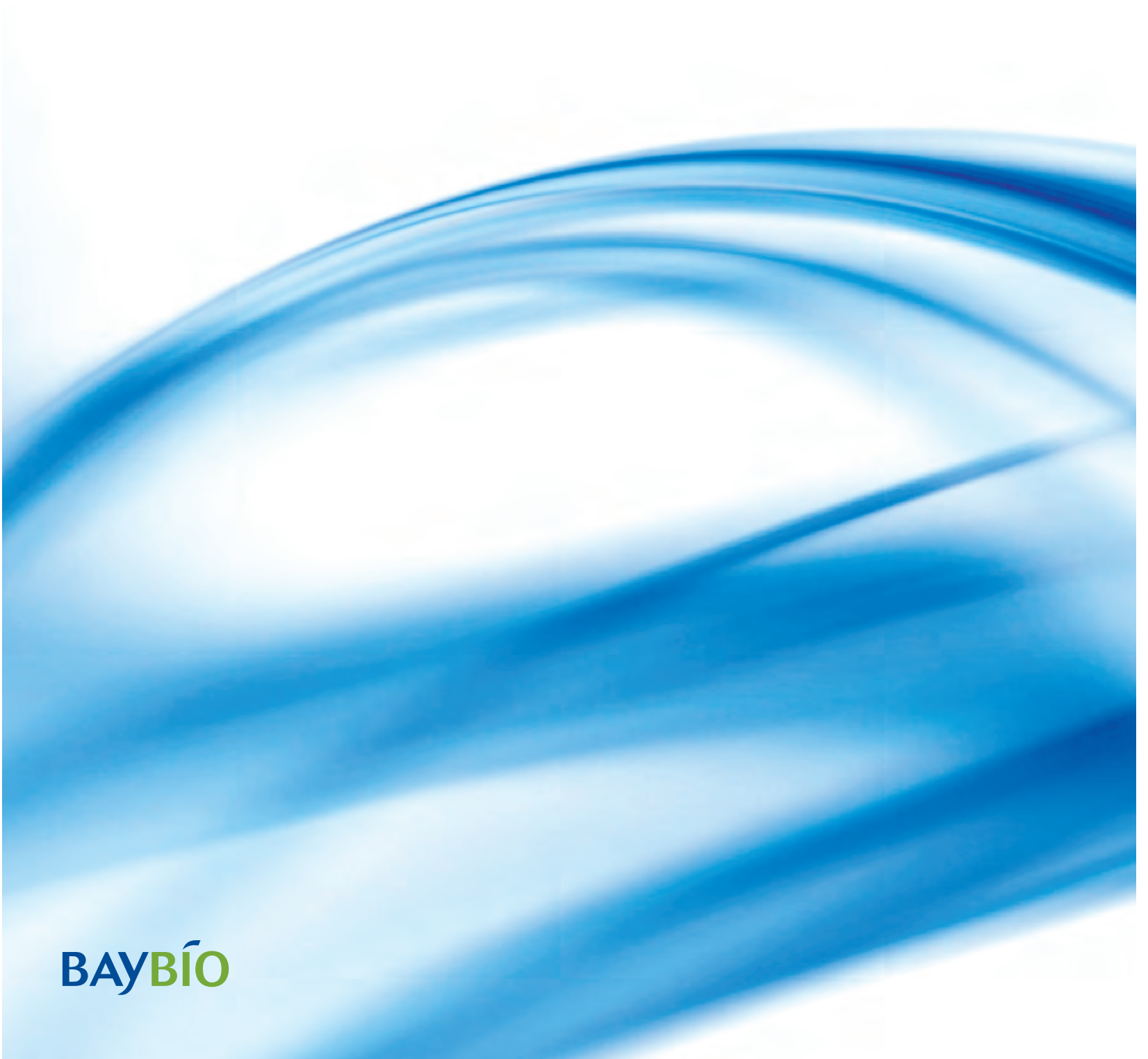
BayBio brings together the collective strength and experience of the world's most innovative and productive life science cluster, helping companies grow, connect and advocate to solve some of humanity's most pressing challenges. BayBio provides comprehensive support and solutions tailored to the unique needs of nearly 1000 Bay Area life science companies and institutions, delivering tangible value through group purchasing savings, capital access, government affairs & advocacy, networking and best-practice sharing. BayBio also supports the future of bioscience innovation through the BayBio Institute's work in science education, career development and entrepreneurship.

Please visit us at [www.baybio.org](http://www.baybio.org).

### About the Writer

Nancy Nielsen, a BayBio Senior Advisor, founded and built the global Corporate Responsibility department at Pfizer Inc., with a strong focus on developing alliances with patient advocacy groups and nongovernmental organizations. Previously she was Vice President and Spokesperson, Corporate Communications at The New York Times Company, and a consultant at McKinsey & Company.





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