

Successful Public-Private Partnerships

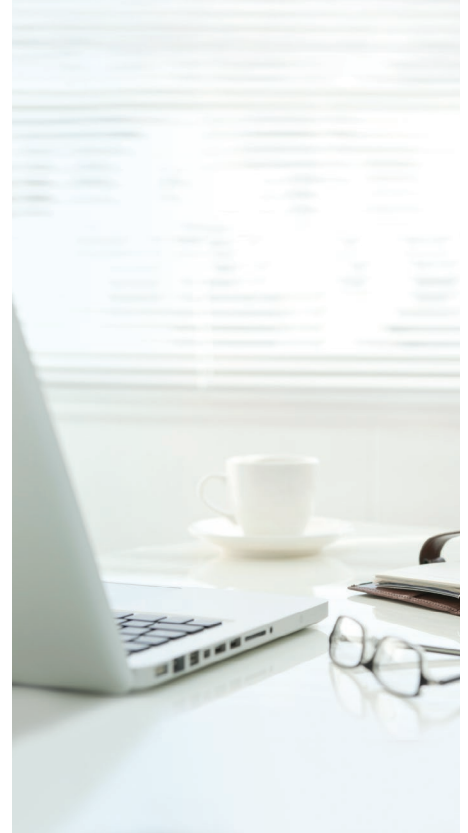
A guide to effective patient foundation and
life science industry collaborations

A National Survey and Leadership Perspectives



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Biomedical 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. Foundations bring urgency, intelligence and resources like patient data and biospecimens, preclinical models, and trial and recruitment support that can be essential to progressing key steps in the development process. In short, they can influence research.

According to FasterCures, a nonprofit dedicated to accelerating medical innovation and research, 55 patient groups in their network gave \$600 million in medical research grants in one year, about half of the groups supported clinical trials and nearly 90 percent currently partner with biopharmaceutical companies.

Given declining research funds from private and public sources, increased R&D costs, regulatory challenges and avoidance of risk investment, companies and foundations share the need for fresh approaches and more efficient pathways to market. While industry and foundations enter into partnerships with different, but complementary, agendas and resources, they are united in their goal to find new therapies and produce a return on investment for their stakeholders.

Partnerships can accelerate innovation and derisk process

These partnerships are shaping a new reality for all involved by reducing business risk and accelerating treatments. This contemporary model is producing notable and game-changing contributions to disease research, pipeline investment and the commercialization of innovative therapies, devices and diagnostics.

Success stories such as the orphan drug Cystaran™ from Sigma-Tau, which partnered with several cystinosis foundations, and Kalydeco™ from the Vertex Pharmaceuticals/ Crohn's and Colitis Foundation of Canada partnership illustrate the effectiveness of this new business model.

To help foster more of these partnerships across the industry, BayBio recently completed the first national assessment of such collaborations—to extract what is working well and what may lead to failure—from a strong sample set of case studies and relationships. The purpose of the study was to highlight the benefits of partnership, identify opportunities for growth, share best practices and describe key pitfalls to avoid. Through this work, BayBio intends to provide a roadmap—both

for life science companies and patient groups—of how to explore, form and navigate successful outcomes. This report is an executive summary of a larger, more detailed report to be issued in the coming months.

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

– Linda Molnar
Founder and CEO
Kirhla, Inc.

Survey profile

BayBio surveyed nearly 100 biotech and foundation CEOs, vice presidents of research, directors of patient advocacy and others across the United States, and conducted 12 in-depth interviews. 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 startups, 26 percent mezzanine, and 36 percent mature, with market caps ranging from \$550 million to \$140 billion. 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 spent up to \$10 million. All of the foundation respondents had existing partnerships with companies or wanted to develop them.

Key findings: significant shared interests

Our key findings revealed that companies and foundations shared top-priority interests in four significant areas: understanding patients, scientific and clinical advice, funding R&D and patient advocacy. Each brought expertise and resources the other lacked, and advanced science together. Nearly 60 percent of respondent companies ranked their partnerships as successful, and offered BayBio advice on what would have made the weaker partnerships more effective.

At the same time, there were areas of mismatched interests that needed to be managed properly for a partnership to be successful. It was clear that companies were seeking support for market research, product marketing and post-marketing research, while foundations ranked marketing as the lowest priority. Respondents from both sectors said some fundamental improvements were 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 some foundations' knowledge of the drug discovery process and a lack of good communications. The good news is that all three of these are fixable.

Best practices can greatly enhance outcomes

The most successful partnerships were ones that followed best practices in setting mutual objectives, milestones and timelines; creating effective partnership structures and management systems; and communicating openly and frequently. For example, The Michael J. Fox Foundation for Parkinson's Research is sponsoring a phase 1B study with a Sanofi compound and was joined by representatives from the pharmaceutical company at the pre-IND meeting with the FDA to make sure they are planning the study appropriately.

"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,
VP Research
Parent Project Muscular Dystrophy

To lay the foundation for best practices to thrive, both parties must start with a stake in the process, members with decision-making power and a commitment to the process. Following are highlights from the respondents' insights on five best practices:

- 1 Vision alignment: start with the same bull's eye.**
The key is to align on goals, objectives, expectations, milestones, how to measure milestones, timelines, frequency of communications and type of contracts needed (business, scientific) – and put it into writing. Like any project, identifying a senior champion for each partner will help insure success.
- 2 Resource alignment: get it right from the start.**
Much like corporate partnerships, due diligence is needed in discussions on assessment of resources, personnel, advisory groups, access to decision-makers and organizational support. The survey showed funding is not the only reason companies collaborate.
- 3 Partnership structure: prevent predictable problems.**
Public-private partnerships can be challenging due to different operating styles and values. For that reason, it is important to agree upfront on issues of governance, roles,

Benefits of partnership

Foundations bring...

- Focus on patients' needs
- Validation to VCs 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

responsibilities and accountability. It is also essential to create a joint steering committee and assign a point person for each partner.

4 Management models: create a competitive advantage.

Good management models clearly answer the question: “how?” Both partners should agree on how they define objectives, motivate efforts, coordinate activities and allocate resources. One size does not fit all due to factors like stage of growth and experience in partnering.

5 Open and frequent communication: make it authentic.

Transparency is mandatory for success. 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 upfront on how communications will work with contact points, frequency, and how and what information is to be shared.

“If a company expects a patient group is just going to be an extension of their marketing team, they are sorely mistaken.”

– Thomas P. Sellers,
Senior Director
Patient Advocacy & Corporate Philanthropy
Millennium Pharmaceuticals, Inc.

There is ample opportunity to mitigate industry challenges by partnering with patient foundations across the discovery-to-commercialization value chain. In fact, the data suggest that companies underestimate the assets a foundation can bring to a strategic plan, and foundations underestimate their influence with regulators and payors. BayBio’s “Successful Public-Private Partnerships” is intended to help guide companies who have never created partnerships by making them aware of the transformational benefits of partnership, new opportunities and effective management systems. It also aims to help experienced companies improve their outcomes for patients and investors.

Looking ahead

Industry/patient foundation partnerships are a dynamic, developing area of business opportunity and innovation. A sample of disease organizations with experience in partnering can be found at <http://train/fastercures.org/trainInventory>.

New opportunities for partnerships

- 93% of foundation respondents plan to increase their budgets
- 85% of company respondents plan to increase number of partnerships with patient foundations
- 36% of foundation respondents plan to increase grants for translation science
- Key areas of expansion are research, collaborations on specific diseases and patient advocacy

Following the release of BayBio’s full national assessment with case studies, a series of focused reports will be issued on key subjects including partnering for funding, patient-driven clinical trial design and recruitment, and regulatory and payor considerations. To that end, we would welcome hearing your feedback and experiences at misha@baybio.org and nmnielsen@baybio.org.

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