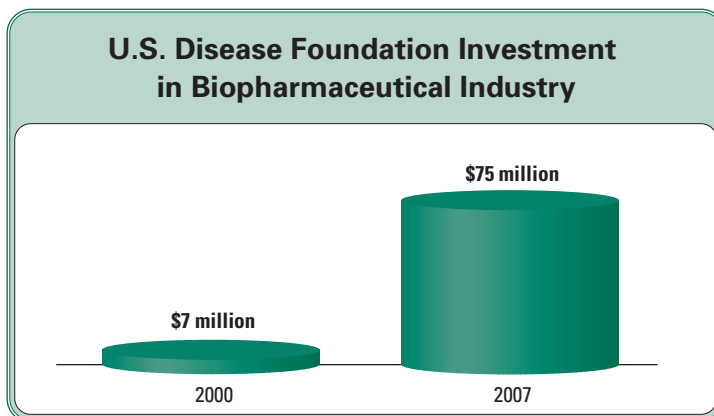


Venture Philanthropy on the Rise

► U.S. Disease foundations will invest about \$75 million this year in biopharmaceutical companies to fund discovery and development of new drugs and therapies for their corresponding diseases. This year's figure represents a 10-fold increase since 2000, and the figure should continue to rise. This new avenue of industry funding has been dubbed "venture philanthropy."

► The amount of money that disease foundations contribute to industry research would not pay the development costs of even one drug today. But, the strategic timing of the funding as well as the additional resources and expertise that the foundations provide serve to enable drug discovery and development that wouldn't be undertaken otherwise and perhaps to speed that development as well.

Venture philanthropy is a term coined in the past few years to describe the funding that disease foundations provide to biopharmaceutical companies to research poten-



Source: CenterWatch estimates, 2007

tial drugs and therapies in their respective diseases. U.S. disease foundations' investment in the biopharmaceutical industry this year will be about \$75 million—10 times as much as its investment in 2000. The figure should continue to rise.

These foundations believe in metrics, milestones and accountability. They don't just want to fund drug development; they want to change it. In the biotech sector, they may have met their perfect match.

But why are non-profits giving money to for-profit biotechs in the first place? On its face, it's counterintuitive, but a number of factors have created the perfect environment for venture philan-

thropy. Disappointed by academia's inability and lack of incentive to translate discoveries into the clinic as well as a reduced National Institutes of Health (NIH) budget, foundations realized they had to bring industry into the equation or let promising research languish in the lab.

Biotechs have been staring for years into the so-called "Valley of Death"—the preclinical stage of development that presents a too high-risk investment to attract venture capitalists anymore. These companies couldn't conduct translational research in rare diseases without external funding sources. Foundations are the only organizations willing and able to fill that

see [Venture Philanthropy](#) on page 7

Venture Philanthropy

continued from page 1

funding gap, but not without careful consideration and a firm understanding of what they want from biotechs in return.

But, disease foundations do not just give money to industry. Money doesn't solve problems, such as protocol development and patient recruitment, on its own. In addition to money, disease foundations provide valuable resources and expertise to further de-risk the joint venture between them and biotech.

This article will focus on five of the leading foundations that have a venture philanthropy component. Their programs promise to be an increasingly important part of the drug development landscape in the decade to come. They are: The Cystic Fibrosis Foundation, Multiple Myeloma Research Foundation, Muscular Dystrophy Association, Juvenile Diabetes Research Foundation, and The Michael J. Fox Foundation for Parkinson's Research. Their work and money will have positive implications not only for patients who will benefit from the therapies that result, but also for the industry as a whole.

Lost in Translation

These five foundations have all created programs targeted to industry because, after years of funding academic research, they learned that it is very difficult to translate promising academic discoveries into the clinical setting, or they were aware of the venture capital funding gap that had created the "Valley of Death," or they knew that NIH wasn't going to step in with funding, or some combination of these factors. Potential drugs and therapies getting lost in translation—or not even getting that far—drove some foundations to re-evaluate their granting mechanisms and start new programs to ensure industry could get into the mix.

While most foundations will continue to award the larger portion of their research funds to academia—where important discoveries are made—the percentage of foundation research funds targeted to industry will rise.

Sharon Hesterlee, Ph.D., vice president translational research, Muscular Dystrophy Association, said, "We'd get progress reports every year, and I noticed some grantees all started to sound familiar. We had one person in particular who was developing a gene therapy approach for a disorder using all kinds of techniques—vectors and particles—and had had reasonable success using animal models using various techniques, and every year we got a new grant [application] for a new technique. I pulled a lot of these files out and started calling the investigators to find out what are the next steps, and if you're not planning to move forward with therapy development, why not?"

"It very quickly became clear that the investigators were interested in doing something with this therapy but didn't know what, so what they were doing was just sending new grants that incrementally improved on the technology. They were very much driven by the technology and whatever is the chief hypothesis in the lab and keeping the lab funded. They didn't really know how to take the next step forward and develop a new therapy, especially with a new indication, a drug that hasn't been in humans before," said Hesterlee.

Peter Lomedico, Ph.D., director of industry partnerships at the Juvenile Diabetes Research Foundation, also realized that various forces were working against successful translation. "A few years ago, we looked at the research portfolio and realized that there was considerable effort on the academic side, the discovery research side, and good progress was being made there, but in terms of translating the discoveries,

getting more things into the clinic and driving toward development with the eventual goal of reaching the marketplace, there wasn't a whole lot there in terms of trying to deal with that. Certainly the changing investor climate for biotech, changes in the venture capital community and also within big pharma really made clear that there is this gap, whether you refer to it as 'The Valley of Death' or some other phrase. Clearly there is a challenge for an organization like ours to see the fruits of the discovery research mostly coming from the academic side and how to get that translated over to companies so they can take on programs and drive through clinical development and through the approval process," Lomedico said.

Kathy Giusti, founder and chief executive officer, Multiple Myeloma Research Foundation, has a unique vantage point: She has worked in the pharmaceutical industry, worked for government, worked with academia, and is a multiple myeloma patient. "I've seen all elements of drug development from NIH to industry to academia to being a patient myself, and I think it's that that has given me this ability to understand nobody has had intent, it's just an old and broken system that really needs to be updated. I hope that we can play a major role in that," said Giusti.

It's Not Just About the Money

While each foundation provides funding to biotechs to give them the ability and the incentive to conduct research that could lead to a cure of their disease, just throwing money at the problem of translation isn't going to fix it. These foundations are well aware of the fact that they bring a lot more value to the biotechs than simple dollars and cents can provide. CFF and MMRF have

see [Venture Philanthropy](#) on page 8

Venture Philanthropy

continued from page 7

patient databases and work to match biotechs with sites where they will find the right patients to study in. All of them have access to deep expertise in their disease as well as the knowledge of where their money will potentially do the most good toward reaching the goal of getting drugs and therapies approved.

Deborah Brooks, president and chief executive officer at The Michael J. Fox Foundation for Parkinson's Research, described the role of funding as a part of the value of what her foundation contributes to industry.

Brooks said, "Funding is an aspect of how we get defined, but it's not the only way we engage and provide value to industry. We use our capital, in particular with industry, through the lens of how we can keep PD [Parkinson's Disease] prioritized in their portfolio of activity—that's everything from supporting specific therapeutic ideas as well as investing in tools that de-risk broadly. We look at the drug development pipeline, and we try to understand the landscape as it relates to Parkinson's in as much detail as we can. We probably have among the best cat-bird seats in the world because it's our daily focus. We think about what's missing, what are we learning, what do we know, what action can be taken, and how can we use our resources and expertise and connect the dots? And that translates into a portfolio of activity. We bring the money, but we're also bringing the expertise in that access to additional information and resources and problem solving."

At the BIO2007 conference held in Boston, Robert Gallotto, vice president, strategic planning and alliance management, Altus Pharmaceuticals, said during the "Venture Philanthropy and Foundation Deals" panel presentation, "Capital is only

one part of the equation. It's much more the intellectual capital that was important for us."

Daniel Grau, chief operating officer of CombinatoRx, a Cambridge, Mass.-based biotechnology company, said at BIO2007, "We also look for access that most venture philanthropists can provide in spades in their various indication fields, to various tools and to leading advisors and expertise. This is a very important benefit." CombinatoRx is a biotechnology company focused on developing new medicines built from synergistic combinations of approved drugs. In less than six years, the company has discovered and advanced into clinical trials a portfolio of six product candidates targeting multiple immuno-inflammatory diseases and cancer, at a total investment, including development of its proprietary screening technology, of less than \$50 million. The company has four venture philanthropy deals ongoing, one each with Accelerate Brain Cancer Cures, Cystic Fibrosis Foundation, CHDI [Huntington's Disease], and the Spinal Muscular Atrophy Foundation.

Added Grau, "What's been beneficial to us is that in some cases with these deals we're moving deeper into a therapeutic area that we already have a presence in. Oncology would be an example of that. We were not active in glioblastoma multiforme, brain cancer, but we had an existing set of products in the cancer field, so this allowed us to go deeper into that area. In other cases, we're diversifying and building out new R&D verticals."

In addition to bringing money, resources and expertise, foundations make sure that the companies they give funds to are meeting their obligations. The foundations have built in the power to withhold payment for performance that doesn't measure up, and they aren't afraid to use it. Grants are milestone-driven, and each foundation has met-

rics they use to judge a company's performance on a grant-by-grant basis.

"We set the milestones in a logical sequence. The point was that if you can't accomplish this step, there's no sense moving forward or providing further money. That gives us tighter control. With an academic grant, we get yearly progress reports. There's some merit to that maybe, but with a for-profit company, we're taking donor money and giving it to a for-profit company and we feel we have a fiduciary obligation to make sure we have a lot of oversight over how it's being spent. It's worked out well. We have a very good idea of where the company is. Often, milestones are delayed. That just happens, and we have the option of withholding payments when milestones aren't accomplished. For a very small startup company, that can mean that people don't get paid. I've found that if you want something done in record time, you can use that carrot-stick approach," said MDA's Hesterlee.

Biotech Advantage

Biotechs often find themselves in a Catch-22: If they do not have proof-of-concept data, they cannot attract venture capital, but if they do not have capital, they cannot supply those data. To compound the difficulty of the situation, when biotechs work toward getting these data with big pharma, they have to choose between getting their research costs funded or dilutive activities such as giving up their product rights, but venture philanthropy money does not require that biotechs make that choice. Sometimes foundations' money is even the difference between a biotech's existing or not.

"Venture philanthropy has actually played a very important role for us in building our business," said Grau. "Funding from the bench through patient proof-of-concept, plus commercial rights. That's one of the crystal clear virtues of working with venture

philanthropists. If you were to work with a specialty pharma or large pharma organization, it's very unusual that you would have their commitment to cover all your research costs and at the same time they would provide you with all the product rights. Those things typically don't go together, but they can go together in the venture philanthropy space, and that's a very interesting opportunity for biotech companies."

Altus Pharmaceuticals' Gallotto also described the importance of his company's venture philanthropy deal with CFF. Altus Pharmaceuticals is focused on developing and commercializing novel protein therapeutics for patients with chronic gastrointestinal and metabolic diseases. The company is developing a portfolio of products based on its novel protein crystallization technology and has the potential to redefine the use of biopharmaceutical proteins for gastrointestinal or metabolic diseases by allowing the oral and parenteral delivery of high value protein replacement products.

When Altus started working with CFF in 2000, it was a pre-IND company and didn't have the infrastructure to develop an effective product. It was in the typical biotech catch-22. CFF committed funding in the early stages. "They were helpful for us to bridge the gap from an idea to animal proof-of-principle data with a \$1 million grant in 2000. From there, in 2001 we were able to develop a broad collaboration and alliance agreement with the CF Foundation. It allows the CFF Foundation to fund up to \$25 million in milestone-based grants. This was important for us because most of that funding was done prior to phase II... This cash was non-dilutive. The ability to retain rights to your molecules is uniquely important to any biotech company," said Gallotto.

Although this kind of money is a very large investment for a foundation, it is relatively small in the investment world. But,

aside from making translational research possible, venture philanthropy and foundation resources can attract venture capital farther down the road.

"Venture capital organizations like the fact that you're working with these groups that have unique perspective of the disease that otherwise companies themselves would not have. You have unique access to physicians, nurses, patients who have a complete understanding of these diseases and understand how to design protocols. This was very important from our perspective when we went later on to raise outside capital from the venture community. We took this collaboration with the CF Foundation with the animal data that we had and our plan for our IND, and we were able to raise \$51 million at the end of 2001 through a Series B round. Without the CF funding prior to IND, to phase I being completed, to the end of phase II being completed, we may never have gotten as far as we did in terms of this product or even as a company with respect to even existing," said Gallotto.

Altus finished its initial public offering (IPO) last year and has initiated phase III clinical trials of its enzyme replacement therapy in people with cystic fibrosis and pancreatic insufficiency.

Foundation Models

Although venture philanthropy is a collective term to describe disease foundation investment in biotechnology for the development of drugs and therapies in their respective indications, it actually takes many forms. Following is a description of the different foundation models.

Cystic Fibrosis Foundation (CFF).

Year founded: 1955

Money invested in research since founding: \$735 million (through 2006)

Industry program: Cystic Fibrosis Foundation Therapeutics Development Program (1998)

Money invested in industry drug discovery & development since 1998: \$230 million

Money earmarked for industry drug discovery & development in 2007: \$43 million

Biotech collaborators: 24+

Drugs in CF pipeline: 30+

CF patients in database: 23,000

Sites in clinical trial network: 64

The gene that causes cystic fibrosis was discovered in 1989, and researchers know much about the basic defect. This is the good news. The difficulty getting pharmaceutical companies or biotechnology companies interested in researching therapies for the disease is that, even though it is the most common fatal genetic disease in the U.S., it affects only 30,000 in this country and 70,000 worldwide. Beall of The Cystic Fibrosis Foundation knew that the foundation would have to establish a new business model for his non-profit if it wanted to interest pharma and biotech to enter the field of cystic fibrosis.

"We felt that the best thing we could do was reduce these companies' financial risk by supporting early stage drug discovery. Our business model is to effectively translate the understanding of the basic defect in the gene into new therapies for our patients," said Beall.

He began in 1998 by investing in a therapeutic development program comprising a network of seven clinical research centers, designated as the Cystic Fibrosis Foundation's Therapeutics Development Network that

see [Venture Philanthropy](#) on page 10

Venture Philanthropy

continued from page 9

specialize in conducting phase I and II studies for treatment of cystic fibrosis. The network also had a coordinating center. In 1999, CFF received a \$20 million grant from the Bill and Melinda Gates Foundation, and in 2000, the foundation launched Cystic Fibrosis Foundation Therapeutics (CFFT), its non-profit drug discovery and development affiliate that administers contracts with biotech companies. TDP is a program of CFFT.

Initiation of the collaborations with industry, level of investment and deal structure vary. CFFT agrees to fund, on a matching basis, the development of products/compounds for the purpose of identifying new drugs for cystic fibrosis. CFFT may approach a biotech studying a compound being studied in another indication and give that company anywhere between \$50,000 and \$25 million to test it in cystic fibrosis. Some deals with biotech are royalty-based—if the drug is approved, the foundation gets a multiple back. Sometimes the deals are a combination of royalties and a multiple. The CFF also requires additional compensation for extraordinary sales results. If there is a suspension in development activities, the CFF obtains worldwide rights to develop the product with agreement to negotiate royalties to the original partner after CFF's investment is returned.

Negotiated portions of the monetary awards from CFFT are dependent on the accomplishment of predetermined, success-driven milestones. CFFT may withdraw from the project under certain circumstances, including failure to achieve milestones. CFFT requires the establishment of a Scientific Advisory Council made up of CFFT, sponsor and joint representatives.

The foundation has not taken any equity in any of the companies to this point. The

foundation re-invests all the money it makes into more research. When a drug was approved by the U.S. Food and Drug Administration [FDA] a few years ago, the foundation flipped the royalties and sold the future rights to it for about \$18 million and used that money for the initial capitalization for its high throughput screening program.

CFF has also created a new funding vehicle, called the Technology Access Program, for emerging drug discovery technology. The foundation awards up to \$1 million per year for three years to focus on new or complementary approaches to targets that may promote maturation of F508 CFTR. The cystic fibrosis transmembrane conductance regulator (CFTR) is mutated in patients with CF. The most common CF-associated mutation is deletion of phenylalanine at residue 508, CFTR delta F508. Two awards were made last year.

A biotech or pharma that has a product or idea it wants to test in cystic fibrosis can use the network run by the Therapeutic Development Program. CFF helps companies conduct clinical trials and does the early phase drug discovery programs.

In February, CFF expanded its investigative site network, run by its Therapeutic Development Program (TDP), with a \$3 million award distributed among 45 new research centers in 20 states nationwide. The money is being used to build each site's infrastructure, expand its staff, or help with patient recruitment operations. It is a two-year program, which means sites will have to perform studies and follow Good Clinical Practice (GCP) procedures in order to stay in the network the following year. CFFT has established an operational center in Seattle to coordinate with the network, which is staffed with more than 40 personnel and can assist in protocol development and monitoring. The investment is meant

to prepare the new sites to become part of CFF's existing network of 18 sites. By 2009, CFFT hopes to have all 45 sites, plus additional sites added in the coming years.

"People like our business model because we hold ourselves accountable. We are the only voluntary health organization that measures success in terms of our pipeline. The pipeline itself is our metric for future success. Our medical programs have continued to grow at a rate of nearly 19% for each of the last three years," said Beall.

Multiple Myeloma Research Foundation

Year founded: 1998

Industry program: Lead grant

Research entity: Multiple Myeloma Research Consortium (MMRC, 2004)

Academic centers in MMRC: 13

Money earmarked for drug development in 2007: \$11 million (industry share=\$2 to \$3 million)

Biotech collaborators: 30+

Compounds: 37 single agents/combinations

Patients in database: 30,000

Tissue bank: 1,000 bone marrows and matching peripheral blood

In 1998, when Kathy Giusti founded the Multiple Myeloma Research Foundation, fewer than five drugs were being evaluated in multiple myeloma. Affecting 45,000 people in the U.S., it is a heterogeneous disease. For this reason, Giusti believes that "myeloma is probably going to be cured by a cocktail of therapies. It may not be exactly the same for every patient. As a result, we need a significant repertoire of really good, targeted therapies from biotech and pharmaceutical companies. And unfortunately with mini-

mal funding from NIH or reduced funding from NIH and the difficulty in raising [venture capital] funds, we thought we needed to step in to build the bridge between the validation of drugs and clinical trials.”

In 2004, Giusti founded a non-profit affiliated with MMRF called the Multiple Myeloma Research Consortium comprising 13 academic centers that have signed membership agreements. MMRF conducted a request for applications for centers. Those that had the highest number of patients, clinical expertise, in the right geographic area and had a good blend of translational research were the ones selected. MMRF developed the model and all policies and procedures for the consortium, and the foundation raises the money to fund it. Each center is held accountable to specific milestones and timelines. Metrics include: the time that a trial comes to MMRF as a concept to the point which a protocol is written to the time of IRB approval to first patient enrolled to study close. Each center is given a scorecard rating its performance

MMRF employs five people dedicated to facilitating clinical research conducted at the consortium’s academic centers and is able to call on the skills of multiple myeloma experts as well. Giusti has identified problem areas in the clinical research process, including protocol development and contracting. For both areas, Giusti offers solutions to speed the drug development process.

“One of the major obstacles in drug development is developing a protocol. A new biotech may not have the expertise to develop a protocol in myeloma and to understand which sites would be best for them to work with. We immediately offer them that expertise. We work with the gentlemen who have done all the leading myeloma trials who can provide all the protocol expertise. We also know exactly what

trials are going on at all of our sites, and if competing trials are happening. We can tell our pharma partners who we think can move quickly and would have capacity to get this trial done.

“Contracting is one of the major obstacles in getting trials done quickly, so we developed master templates here so that we could speed the contracting process. I hired my own attorney and I pay outside counsel to get all the contracting done,” said Giusti.

Communication is key for the centers, which are required to be on monthly teleconferences and at quarterly face-to-face meetings. On calls and at meetings, there are usually also companies presenting their compounds or discussing the protocol or patient enrollment. Ongoing trials require a weekly teleconference.

A unique feature of what MMRF offers its consortium is access to its tissue bank, patient database and its patient navigator program.

Much of what MMRF does entails matching biotechs with the right academic center. The foundation will allocate \$11 million for research investment this year. Two to three million dollars of that amount will be allocated to MMRF’s lead grant program, which funds go directly to biotechs that MMRF selects from those that apply.

“Speed to market is everything we’re about because no company is going to come to myeloma because it’s a big market. But, they would come here because they have a foundation like ours that can facilitate the process so much that you say, ‘We could get our drug to market faster because this group will help us from concept to enrollment to the education of the drug, and even with FDA relations because we do FDA roundtables as well. We take great pride in being an end-to-end solution for biotech and pharmaceutical companies.’” said Giusti.

Muscular Dystrophy Association

Year founded: 1950

Money spent on research since founding: \$670 million

Industry Program: Translational Research Program (TRP)/corporate grant (2004)

Money invested in industry since start of TRP program: \$5.5 million+

Projected industry funding in 2007: \$3-\$4 million

Biotech collaborators: 2

Patient database: No, but working toward it

Industry projects: 1 active, 2 completed

The Muscular Dystrophy Association (MDA) is an umbrella organization for about 40 neuromuscular diseases that in the aggregate affect close to 500,000 people in the U.S. but, individually, they are all orphan diseases. There have been no drugs approved by the FDA specifically for muscular dystrophy ever.

“We tailored a program to address specific categories of this gap we saw in the translational aspects of drug development,” said Hesterlee.

The MDA created the Translational Research Program (TRP), which has four categories of funding: IND Planning Grant, Clinical Research Training Grant, Infrastructure Grant and Corporate Grant. The first two grant categories are designed for sites, the third is to fund a national patient database and the fourth category of grant money is dedicated to industry. “We designed a mechanism to fund companies directly that are involved in early stage drug development—all the steps leading up to an IND (investigational new drug application) and then a phase I or phase II clinical trial. We saw it as affirmative action for rare

see [Venture Philanthropy](#) on page 12

Venture Philanthropy

continued from page 11

disease. If we can put some money into this high-risk early stage, the idea is to move things along until there's some efficacy data in humans, and then [companies] can start getting venture capital money." All four programs are designed to increase the number of biotechs working in muscular dystrophy.

Two biotechs have completed TRP corporate grants and one of them and a new organization are working on a current grant. MDA does not pre-allocate funds every year, except for the research training grants. MDA requires matching funds from the biotechs. Companies need to match MDA funds for a project with at least 50% of their own money going into that same budget.

Both MDA and funded organizations choose a group of outside experts in the field to form a steering committee that will oversee the grant milestones. "We find that the steering committee meetings function not just to make a judgment on the milestone, but they're very good advisory committees. The companies find them to be a good resource," said Hesterlee.

In addition to what it offers through its corporate grant program, MDA has discovered some creative ways to enable and speed drug development through strategic funding. MDA has found a way to help both Genzyme and Wyeth conduct clinical trials in muscular dystrophy patients. In each case, MDA approached the companies and asked what it could do to help them conduct clinical research in muscular dystrophy. Patients enrolled in the companies' studies were often very ill and needed medical care outside the clinical trial while in the hospital. Because insurance companies would not pay for this medical care, and the companies were not allowed to, MDA

offered to give grants out of its translational research budget to each of the sites enrolling patients for the companies' respective studies. Two of Wyeth's sites accepted funding that totaled more than \$55,000 last year. The money in this case could also be used for travel in addition to clinical trial subjects' hospital costs. Three of Genzyme's sites were each given a grant of \$50,000 in 2003 to cover clinical trial subjects' hospital costs.

"Maybe there's more than one way to get from point A to point B, but just funding research through a standard NIH model was never going to get us the drugs. That was clear. I think that we had to do this. I don't think it was a choice. It's our mission to develop therapies and cures for these diseases, not just find promising research. It's to see them all the way through the clinics. If we don't do this type of program, then we're letting people down," said Hesterlee.

MDA is planning a formal evaluation of the program this year. Because much of the funding has been focused on Duchenne's muscular dystrophy, MDA is going to look at whether it's increased the number of phase I/II clinical trials in that disease. Secondly, MDA is going to look at whether the number of biotechnology and pharmaceutical companies working in this area has increased. "Having drugs approved is the long-term goal. It's something we might use in 10 years to see how the program's progressing," said Hesterlee.

Juvenile Diabetes Research Foundation

Year founded: 1970

Investment in research since founding:
\$1 billion

Industry program: Industry Discovery and Development Partnership Program (2004)

Projected industry funding in 2007:
\$14 million

Industry collaborations: 15

Product candidates: 5 in clinical trials, 10 in preclinical discovery and evaluation

Patient database: No

The Juvenile Diabetes Research Foundation (JDRF) has awarded more than \$1 billion to research of type 1 diabetes, most of it to academia. In that time, it has come to understand some of the basic biology of the disease and some of the aspects of its pathogenesis. In 2004, it launched its Industry Discovery and Development Partnership Program (IDDPP) specifically to fund proof-of-concept in animal models and proof-of-concept in man studies in type 1 diabetes sponsored by industry.

IDDPP's grants are milestone-driven, and the foundation asks the companies to match their funding with an equal or greater amount. JDRF has an industry application process. The foundation accepts inquiries and applications on a rolling basis. JDRF also approaches companies.

"If your goal is to impact disease progression and patient quality of life and effect a cure for a particular disease, the best way that's going to happen is for companies to get involved and take therapeutics or diagnostics or medical devices through development, through the approval process and out into the marketplace... The academic investigators who are necessary to start the

process can't do it by themselves. We really need to work together and engage industry to take over some of these opportunities and drive the marketplace... We want to see more clinical research programs and more product candidates moving toward development, so one metric of success is as we shift more and more into supporting clinical development programs, that means there are more product candidates moving toward the marketplace," said Lomedico.

Michael J. Fox Foundation for Parkinson's Research (MJFF)

Year founded: 2000

Investment in research since founding:

\$94 million+

Industry program: Therapeutics Development Initiative (2006)

Money awarded to industry (2006):

\$4.6 million

Money committed to industry (2007/8):

\$5 million

Patient database: No

Parkinson's disease affects approximately 500,000 people in the U.S. Its target is unknown. The foundation has funded more than \$94 million in research directly or through partnerships since it was founded in 2000.

"We don't understand a single cause of PD, we don't see in the natural history of the disease the same experience for every patient. We can't tell you that there are subtypes of the disease, but we suspect it. We are trying to do some investigating to understand that better, which means an improved therapy might work for a small group of patients or it might work for a broad group of patients. It might help treat the underlying cause of the disease, or it might manage symptoms better. It's such a mix of potential positive outcomes that we

maintain a portfolio that is advancing as many of those things that we think are sensible and we can afford to do," said Brooks.

The foundation launched its Therapeutics Development Initiative (TDI) last year to expand its industry investment. This is the foundation's only program that is specifically targeted to industry, though other funding commitments often have an industry component. Last year 10 industry research teams were awarded \$4.6 million. These research teams are focused primarily on the development of neuroprotective treatments and cell replacement approaches. In 2007/08 the foundation plans to award \$5 million through TDI. The program has two grant cycles—autumn, 2007 and spring 2008.

Grantees meet in-person for mid-point assessments of their projects. MJFF's metrics focus on the accountability of individual grantees, making sure that they do what they promised to in their applications. Mid-point meetings bring the grantees together in one place and also offer an opportunity to solve problems together.

"There's a real unmet need to capture the kernels of these ideas at very early stages that could be transformative and powerful and really innovative solutions and shepherd those along by identifying them early, providing capital in places where there's no capital to be had because it's just too risky and see if we can breathe some life into these opportunities with the patients in mind," said Brooks.

Future Outlook

Disease foundations have been learning from each other, sharing best practices and are committed to spreading the word about venture philanthropy. While they don't have financial relationships with each other, their common purpose—to discover and develop therapies for incurable disease—binds them together and has created a strong community.

Robert J. Beall, president and CEO of the Cystic Fibrosis Foundation, is considered the catalyst for investing strategically in industry research, and he has been generous with his time and ideas. He was the first to launch an industry program and has helped others navigate the shoals of the venture philanthropy model.

"Bob Beall from Cystic Fibrosis sits on our board of directors, and we've learned so much from him. If I hadn't had Bob helping me, I might have wasted time, energy and money. I'm a big believer in foundations learning from each other because it makes certain that the money invested has a broader impact," said MMRF's Kathy Giusti.

MJFF's Brooks has also benefited from the experiences of other foundation heads and been able to share some innovative ideas of her own. She said, "We learn so much from hearing about the strategies that other groups have crafted as problem solvers. There are a handful of these like-minded groups that are aggressively trying to solve a problem that is stunningly complex and has essentially been delegated. The natural market doesn't solve it for small diseases or complex diseases. These groups are comparing notes and not everything that every group does is relevant to what every group does. It has a lot to do with the state of your science, what your budget is and that might have to do with how big your community is and how wealthy they are. It has been advantageous to have other people working aggressively in this field that we can learn from and share with. We've been both a great beneficiary of other people's wisdom, and we've figured out some interesting things that other people are excited about hearing from us on."

Foundations' shift from basic research funding that went to academia alone to funding drug discovery and development by industry as well has brought about changes in

see [Venture Philanthropy](#) on page 14

Venture Philanthropy

continued from page 13

the way stakeholders view the drug discovery and development process. “This was a real change in culture. One of the things we do is get the basic scientists at the table with [biopharmaceutical companies] and it really gives them a sense of engagement in the process. They’re not threatened by us moving from largely a basic research organization to one where we’re very involved in translation. It’s been the nucleus of a major gifts program. You tell businessmen about our model, how we hold ourselves accountable through that pipeline, and people are willing to invest in that process,” said Beall at BIO2007.

In the future, these five foundations will be called upon to share their expertise with other foundations not yet participating in the venture philanthropy model.

“The message is getting around to other foundations, and other patient groups are seeing the important role that industry plays. And they’re looking to learn from their colleagues, including us and the other foundations, so they can learn how to engage industry more effectively,” said Lomedico.

While the dollar amount invested in industry research is quite small, foundations have found the strategic places to make a small amount of money work the hardest. Without it, in most if not all cases, the chance of developing a new therapy would not exist. An investment by a foundation is capable of attracting tens of millions of dollars of venture capital afterward.

Hesterlee concluded, “Venture philanthropy is a small amount of money in the overall sea of money floating around out there, but I think because all the groups are really trying to apply it so strategically, it’s going to make a big difference—sort of like the lever Archimedes said could move the world.”

—Sara Gambrill