

The New Power Players in Drug R&D Are Wearing Bright T-Shirts

Luke Timmerman | 10/29/12 | [Follow @ldtimmerman](#)

See all those people signing up for the 10K charity run/walk in your hometown this weekend? Those folks in your Facebook photo album, decked out with colorful T-shirts and uplifting messages about fighting some disease?

You could easily have written off many of these nonprofit fundraisers a few years ago as well-intended, but ultimately ineffective, efforts for coming up with cures. The real action, you could have argued, was only happening in the investor-driven, profit-motivated world of biotech and pharmaceutical companies.

But the gap between the nonprofit and for-profit worlds is narrowing, and it's a good thing. Those foundations raising money in drips and drops at the local 10K are starting to become much less doe-eyed, more sophisticated agents in the quest to develop new medicines. Instead of just raising money, giving it away to academic scientists, and hoping cures would come out the other end, many foundations have wised up. They know that isn't how the world really works. And at a time when so many companies are starving for cash to advance their best drug R&D ideas, many nonprofit foundations know they can make an impact by cleverly applying their own blend of money, networks of patients, and sincere advocacy.

Look at what's happened this year. The Cystic Fibrosis Foundation has basically been on a yearlong victory tour, after it got the credit it deserved for helping Vertex Pharmaceuticals develop the groundbreaking new CF drug ivacaftor (**Kalydeco**). By mobilizing \$75 million of its own money, its network of researchers, its network of patients, and its own organizational moxie, the CF Foundation helped to create a new drug that profit-motivated investors never would have supported. The market looked too small. But now that the drug has been approved by the FDA (and incidentally, the market actually looks pretty big) Kalydeco has become the key case study many other foundations want to learn from. Increasingly, foundations want to say that they, too, financed not just great research, but great products that came from the research.

Nobody has a better handle on this growing trend than the Washington, D.C.-based advocacy group FasterCures. This nonprofit serves as a convener for the **various disease foundations** who are hoping to be like the CF Foundation. FasterCures now counts **55 foundations** in the U.S. that actively form partnerships with biotech and pharma companies. The foundations now collectively put in an estimated \$500 million a year into drug R&D programs. And you can expect the number of foundations getting into this "venture philanthropy" business to grow, because many traditional **biotech venture capital firms are dying**, and startups are looking to find money anywhere they can. Sometimes these foundations give their money in simple grants to companies, sometimes they get equity stakes, and sometimes they strike deals to get royalties from sales of products they support.



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UNDERWRITERS AND PARTNERS



“These foundations are small and scrappy on one hand, but they are mighty and effective on the other hand,” says **Margaret Anderson**, the executive director of FasterCures. “When you talk to Big Pharma, it’s been a bit humbling for a lot of them to look at (CEO) Bob Beall and the CF Foundation, and say ‘here’s a nonprofit that raises money through walkathons, and look at what they did.’ It’s a profound example of how the system can work. It’s gotten people excited. It’s enabled people to think outside the box.”



Margaret Anderson,
executive director of
FasterCures

Conventional thinking at foundations has gone something like this: It was mostly about raising money for a good cause, giving it away to top-notch university researchers like the National Institutes of Health does, and hope for a discovery. If that discovery came, there was faith in the free markets, which logically ought to pick up on, say, a discovery in the field of Parkinson’s disease and attempt to turn it into a moneymaking product.

Some foundations, sadly, still cling to these outdated notions. But as Big Pharma and biotech company R&D operations have suffered so many expensive failures, companies have turned risk-averse, putting their money into drugs only in the late stages of development that look like safe bets. It’s become clear to many forward-thinking foundations that if they really want cures, they have to change their ways, and get involved in both research AND early development at companies, not just research in academic labs. In short, the foundations need to take some risk.

I know what many readers are thinking at this point—the foundations are out of their depth. And if you keep score in purely financial terms, it’s hard to argue. Members of the Pharmaceutical Research and Manufacturers Association put in about **\$50 billion** a year into drug R&D, while the National Institutes of Health finances about **\$30 billion** a year of basic biomedical research. Disease foundations surely give away billions too, but if only about \$500 million goes toward R&D at companies.

But these groups bring more than money to the table. Some of them bring big-name recognition, connections, and clout. The Michael J. Fox Foundation for Parkinson’s Research, the Multiple Myeloma Research Foundation, the CHDI Foundation, the Leukemia & Lymphoma Society, Accelerate Brain Cancer Cure, the National MS Society, and the Juvenile Diabetes Research Foundation are a few of the foundations that mean business when they talk about “venture philanthropy.” You could even throw the **Bill & Melinda Gates Foundation** into this conversation, as it has shown increasing interest lately in financing companies that are working on **vaccines** and other tools for fighting infectious disease in the developing world.

Joshua Boger, the founder and former CEO of Vertex, says foundations are becoming a much bigger force than ever before in drug development. Well-run foundations can help attract scientific experts to a drug development program, and help companies understand their patients much better at an early stage of development. Down the road, if a project is successful, no one will advocate for it more fiercely, or more effectively, with regulators and payers. Who wants to stare a dying patient in the eyes and say sorry, your drug looks good, but you need to run another 2-year clinical trial to increase the statistical rigor of a dataset?

“They definitely bring more than money. That’s a huge advantage to their investment that a purely financial investor can’t bring,” Boger says. “It’s really valuable. It can make a big difference.”

These are still early days, however, for most foundations exploring the drug development world. Many are experiencing growing pains. Some foundations struggle with a portfolio strategy, in which they have to spread their bets around to be diversified, but not get

spread so thin, Boger says. Sometimes they can also get, shall we say, greedy, just like profit-driven companies. Foundations can sometimes go astray when they give their money, and expect lucrative future returns, just like a Big Pharma company. Instead of thinking in purely financial terms, the foundations need to remember their mission is to develop drugs, not build up a monster endowment. "They sometimes forget what their mission is," Boger says.

There are some tough cultural issues that foundations and companies need to think hard about before sending off a check to a drugmaker. Some foundations struggle getting out of first gear, just because of the perception that they, as nonprofits, are good, and companies, as for-profits, are bad. That's an oh-too-simple position to take, but there are also serious gray areas to think about. Disease foundations are supposed to advocate for the health and wellbeing of their members, and if they believe their members have been treated badly by a drug company they have invested in, how do you handle that?

It might sound like too much of a hornet's nest, so why bother getting involved? The hard truth is that many of these foundations have been around long enough to know that another 40-50 years of ineffective basic research isn't going to cut it. They are motivated to figure out ways to cooperate with companies, calling up guys like Boger and Beall for advice on how they did it. Some of them are motivated enough to even hire biotech industry insiders to show them the way.

One of those types of folks is **Max Wallace**, the CEO of **Accelerate Brain Cancer Cure**. This foundation, co-founded by AOL pioneer Steve Case and his brother Dan, naturally gravitated to can-do folks in business for help in making progress against brain cancer. So the foundation hired Wallace, an experienced biotech executive, to help put \$2.5 million of donations per year to work.

In one example, ABC2 recently put **\$175,000** into Cambridge, MA-based Agios Pharmaceuticals to run experiments on whether drug for a cancer metabolism pathway might be useful for brain cancer. That amount of money might sound trivial, and the amount wasn't even disclosed in a 2009 press release (probably because the PR folks thought guys like me would dismiss the grant as irrelevant). But as Wallace says, the grant helped convince the deeper VC pockets on the Agios board to commit more company resources to that program once it started showed more promise. And Agios, surely, appreciates that ABC2 can help open some important doors to patients that it will need along the drug development journey.

Sure, there's no guarantee of a payoff in this collaboration. But the motivation of disease foundations, their absolute staying power, isn't something you can measure on a balance sheet. I saw the passion and purpose on display a couple years ago at a conference when **Josh Sommer** of the Chordoma Foundation implored a group of researchers to get cracking on new pathways, on collaborations, on new drug candidates. He wants them to help develop a product that will save his life.

It would be a mistake to think that foundations will somehow replace venture capital or the public stock markets as the main source of investment capital for drug development. But foundations do have a lot to offer, and the potential to help develop drugs the market would never otherwise support. If we're lucky, this new surge of nonprofit/for-profit collaboration just might lead to a bunch of new Kalydecos.

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