

# Issue Brief

## Investing in Innovative Medical Research

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For more information about *FasterCures*, please visit: [www.fastercures.org](http://www.fastercures.org)

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### ISSUE CONTEXT AND DESCRIPTION

Philanthropists are essential supporters of medical research, providing funds to fight diseases that afflict their loved ones and communities, and raising awareness about medical issues that affect us all. Most university research centers and medically focused foundations have long depended on donors for the resources they need to advance their work. Now, forward-thinking philanthropists have an opportunity to help drive innovation in the practice of medical research itself—to support changes that could lead to the development of novel treatments and cures.

Over the last decade, scientific advances have set the stage for such medical breakthroughs. Scientists have mapped the human genome, increasing the potential to unlock the genetic roots of diseases. Since then, philanthropic dollars and nonprofit muscle have translated genetic knowledge of a few diseases, including cystic fibrosis and some types of cancer, into treatments for patients.

Yet other than a few successes, recent efforts have produced relatively little progress in developing effective new treatments and cures. Though the research budget at the National Institutes of Health (NIH) has grown from \$15 billion in 1996 to \$30 billion in 2009,<sup>1</sup> the rate of new drug approval has not increased. What's more, many pharmaceutical companies are now tweaking existing drugs rather than investing in big new discoveries.<sup>2</sup> Meanwhile, moving new treatments through testing and regulatory mechanisms currently takes up to 15 years, leaving patients without treatments that could improve or save their lives.

In 2007, the philanthropic sector provided \$918 million to support medical research.<sup>3</sup> While this amount is small compared to the billions spent by the NIH and pharmaceutical companies, philanthropists often operate with fewer constraints than they face. To date, donors have often missed opportunities to use their comparative flexibility to support higher-risk, yet potentially high-impact research—work that is crucial to the successful pursuit of breakthroughs in medical care. By investing strategically in improved processes and collaborative research, donors can impact the broader field of medical research even as they help improve the lives of those suffering from the diseases they seek to defeat.

## CHALLENGES

Efforts to improve the existing medical research process face multiple hurdles.

### **Lack of incentives for translational research:**

Currently, there is a major lack of incentives for “translational research”—work that bridges the gap between basic science and later-stage clinical research needed to produce new treatments and cures. Researchers actually refer to this gap as the “Valley of Death” because many sound scientific developments do not find further funding or champions for commercial development.

**Institutional constraints:** As research and development costs have skyrocketed and success rates have declined, companies have become increasingly risk-averse. In many cases, they are investing later along the research continuum and becoming more conservative in their decisions about what to fund. Government-funded academic researchers, meanwhile, have strong professional incentives to focus on basic science. Such science is essential to the discovery process, but it is not sufficient to develop therapies for patients.

**Roadblocks to collaboration:** Pressure to publish, intellectual property concerns, tenure and the peer review system currently used

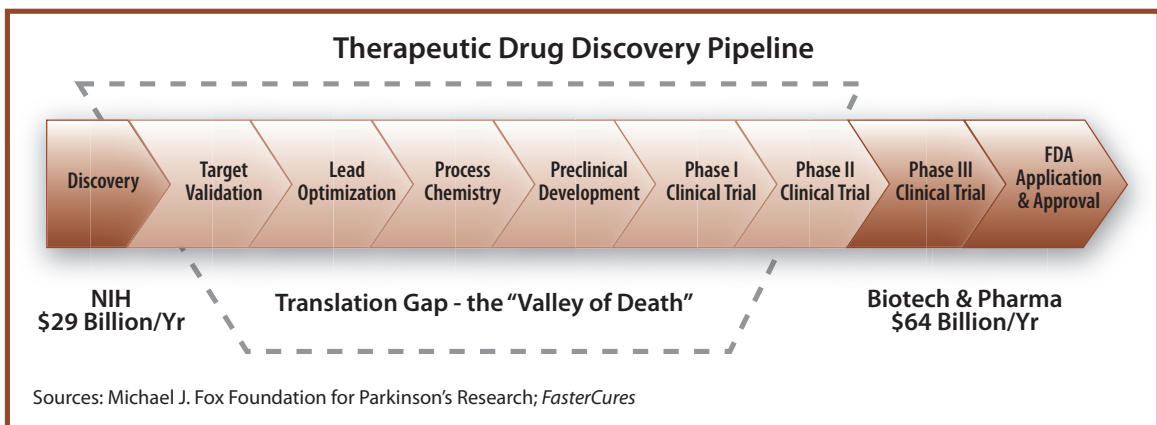
to determine grant funding often discourage scientists from collaborating. Too often, scientists have neither the opportunities nor the incentives to participate in cross-disciplinary approaches that could further a discovery and unlock its potential for treatment.

**Lack of information:** Donors currently find it difficult to get information on what research is being done, how it will make an impact, and where the greatest needs in medical research lie. Additionally, few useful frameworks exist for measuring one medical research opportunity against another one, and the lengthy timeframe for generating clear results makes assessing impact difficult.

## SUCCESSSES

Despite these challenges and limitations, new practices in medical research are yielding promising results.

**Application of management tools:** Donors to the Michael J. Fox Foundation for Parkinson’s Research have provided funds used to expedite grant processes, establish benchmarks for progress, apply continuous evaluation practices, and invest in a portfolio of promising research initiatives simultaneously. Philanthropists’ growing support for such management tools



and performance measurement requirements is helping to reduce the overall risk of research investments, create new incentives for scientists and change the culture and expectations around medical research.

**Leveraging information technology:**

Funders, nonprofits and researchers alike are using new technologies to increase data gathering and information sharing. The Drugs for Neglected Diseases *initiative* (DNDi) is an organization designed to improve treatment of diseases that primarily affect low-income populations in the developing world. DNDi recently implemented a web-based system that collects results of any testing on a particular molecule worldwide, so that partners on six different continents can easily access up-to-date research data.<sup>4</sup> Other groups have begun using electronic medical records, online forums, social media and other information technologies to share data and patient experiences.

**Increasing recognition of collaboration's value:**

Developing new treatments requires the convergence of assets and expertise across sectors. Recognizing this, medical research foundations, individual philanthropists and the private sector came together in December 2009 at the first annual "Partnering for Cures" conference, convened by *FasterCures*. The conference was designed to facilitate strategic collaborations needed to expedite development of new medical solutions.

**TRENDS**

Small but important changes in the culture of medical research could lead to new innovations in treatment development.

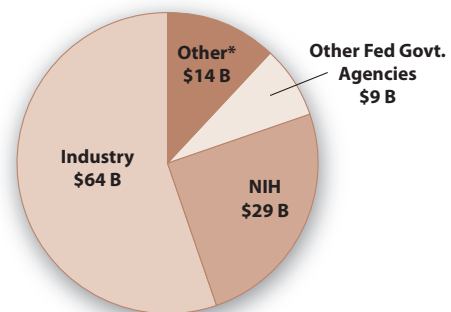
**Increasing public awareness:** The need for an accelerated process for finding medical solutions is gaining traction with the broader

public. A recent *Research!America* poll found that a majority of Americans want more funding for research and believe that research is a solution to rising healthcare costs.<sup>5</sup>

**New business models:** Innovators are developing new ways to integrate science and business practices and experimenting with new ways to develop cures and treatments. The Myelin Repair Foundation's ARC (Accelerated Research Collaboration) model is designed to bridge the gap between academic laboratories and the pharmaceutical industry, using best business practices and ongoing management oversight to help drive new drug discoveries into the commercial pipeline.<sup>6</sup>

**More partnerships:** Partnerships between nonprofit foundations and private companies are an increasingly popular means of sharing the risks associated with developing new treatments and cures. The Global Alliance for TB Drug Development, for example, is a nonprofit product development partnership that brings together public and private research and development organizations worldwide in an effort to find new drugs and therapies for tuberculosis.<sup>7</sup>

**U.S. Health Research Expenditures:  
\$116 Billion**



\* Universities, State and Local Government Contributions, Foundations, Independent Research Institutes, Voluntary Health Associations

Source: *FasterCures*, 2008

## FUNDING OPPORTUNITIES

Philanthropists can assume greater levels of risk than other funders and encourage innovation in existing organizations.

**Help close the translational gap:** Donors can play a critical role in accelerating the search for cures by funding research that bridges the gap between basic science and later-stage clinical research. This may include funding efforts to collect data and samples from patients sharing the same disease, supporting programs to expand networks of clinical trial sites or investing in new models for testing treatments.

**Invest in validation:** To ensure a product's commercial relevance, companies and investors need to see replicated results or published papers indicating a discovery's promise. Philanthropists can earmark funds for nonprofits to use on validation work that attracts more investment around promising discoveries.

**Fund collaboration:** Donors can provide operational funding to support organizations as they develop processes for sharing data and establish metrics for measuring success. They can fund online tools and infrastructure needed to facilitate collaboration. They can also support efforts that bring scientists together to exchange ideas and share best practices.

**Tell the story:** Philanthropists can support documentaries or education programs that raise awareness and help the general public understand the current system and its limitations.

1. National Institutes of Health, Office of Budget, <http://officeofbudget.od.nih.gov/pdfs/FY11/Approp.%20History%20by%20IC%20%28FINAL%29.pdf> (accessed February 25, 2010).

2. Maggie Fox, "In Tough Economic Times, NIH Head Looks to Clinic," Reuters.com, January 25, 2010, <http://www.reuters.com/article/idUSTRE60O40J20100125> (accessed February 25, 2010).

3. "2008 Investment in U.S. Health Research," *Research!America*, <http://www.researchamerica.org/uploads/healthdollar08.pdf> (accessed February 25, 2010).

## Donor Considerations

As donors give to organizations working on the diseases they care most about, they should consider the following.

**Ask to see a research and development strategy:**

Make sure that any organization you fund has a plan for ensuring that its scientific discoveries lead to treatment development, and an access and delivery strategy for getting the right treatments to the right patients. The organization should also be considering commercialization potential for its scientific discoveries.

**Hold organizations accountable:** Before funding, ask to see a management plan, list of advisors, and information on how the organization measures progress. Also, look for evidence of commitment to collaboration and information sharing.

**Manage with interim milestones:** While the bottom line is curing a disease, donors can measure progress incrementally based on pre-defined benchmarks. Getting to breakthroughs takes time and patience, but having a framework for evaluating your investment is key to measuring impact and success.

**Be flexible and learn from failures:** Be open to midcourse adjustments as the research process unfolds. Trust the original grantee to make changes in the plan should the need arise. Science is unpredictable, and the variables are many. Learning what does not work could lead you to what does.

Funders can also bring together existing nonprofit or industry coalitions to discuss reform opportunities, science education and strategies for engaging more people to help improve the medical research system.

4. Drugs for Neglected Diseases *initiative*, <http://www.dndi.org/> (accessed February 25, 2010).

5. "America Speaks," *Research!America*, <http://www.researchamerica.org/uploads/AmericaSpeaksV10.pdf> (accessed February 25, 2010).

6. Myelin Repair Foundation, [http://www.myelinrepair.org/research\\_model/research\\_process.shtml](http://www.myelinrepair.org/research_model/research_process.shtml) (accessed February 25, 2010).

7. Global Alliance for TB Drug Development, <http://www.tb.alliance.org/about/mission.php> (accessed February 25, 2010).