Table of Contents

01 Giving Smarter
06 Evaluating Philanthropic Investment
   07 Accountability
   11 Collaboration
   14 Research Effectiveness
   17 Resource Building
20 Catalyzing Innovation in Disease Research
Philanthropists have a unique, powerful role in jump-starting new models of innovation needed to accelerate progress in medical research and development (R&D).

Philanthropic investment in medical research is ideally positioned to make relatively high-risk investments that could significantly move a field of research forward and increase the likelihood that other parties will also invest. Although private philanthropy is only a small share of overall spending on medical R&D in the United States, its flexibility and focus on outcomes have an outsized impact on the medical research enterprise.

But not all research entities that receive philanthropic funding are created equal. This is where informed philanthropic giving can make a transformative difference. By understanding the role nonprofits play along the R&D spectrum and the practices that position them to be most effective, you can maximize the return on your philanthropic investment and make a sustainable impact in the search for cures.

FasterCures, the Washington-based center of the Milken Institute, was built on the premise that to save lives, we must save time in the way we discover scientific breakthroughs, develop those into effective treatments, and deliver those to patients who need them most. But expediting cures cannot happen in a vacuum. All sectors of the medical research system must come together with a renewed focus on improving the health and well-being of the people diagnosed with deadly and debilitating diseases.

We have developed programs to catalyze innovative approaches to disease research, including a specialized track on medical philanthropy. This guide is a key component of that effort, as it presents donors with a framework for evaluating giving decisions and preferences within the broader R&D context. Giving Smarter is meant to serve as a companion to the FasterCures publication Getting Started: The Medical Research and Development Primer, which provides donors with an outline of the process needed to translate scientific discoveries into accessible medical solutions. These tools were designed to help you build a high-impact medical philanthropy portfolio.
Despite 21st century medical and technological advances, from the decoding of the human genome to stem cell science, from health information technology to targeted cancer therapies, many patients continue to receive the same treatment options offered decades ago. For those who are not so lucky, no cures exist at all and even existing treatments may be highly inadequate.

For too many patients and their families, the promise of medical progress remains unfulfilled. The stakes are high and the consequences are measured in life-altering outcomes.

**It takes too long.** On average, it takes 15 years to take a scientific breakthrough from the microscope to the marketplace. But we all know that when a deadly or debilitating disease strikes, we do not have 15 years.

**It costs too much.** For all the financial and human capital flowing through the system, there is remarkably little emphasis on specific goals or milestones to cure disease or achieve tangible clinical results. Although government and industry spending on medical R&D has increased dramatically over the last two decades, the rate of new drugs coming to market has not improved.

**Science is complicated, the process even more so.** What is behind the slow momentum in clinical discovery and application? Science is hard and researchers often insist that “science cannot be managed.” For many diseases, the answers to even the most basic biological questions—such as what causes them and how they attack the body—remain elusive. Even as we learn these things, other factors slow research progress, including:

1. lack of understanding of the process needed to advance early stage research through therapeutic development,

2. little incentive for those engaged in basic science research to translate new knowledge into patient benefit, and

3. limited opportunities for scientists to be a part of cross-sector and interdisciplinary collaborations necessary to advance outcomes-driven medical research.
Finding Big Ideas in Small Places

Medical philanthropy, accounting for only 3 percent of overall U.S. spending on medical R&D (Figure 1), is well poised to play an outsized role in catalyzing and jump-starting innovation. Unlike governments or industry players, private foundations and individual donors are free from obligations to political constituencies, legacy mandates, or shareholders, enabling them to direct their dollars to the projects or organizations they choose. Philanthropy can serve as a reliable source of funds for novel research that might not be able to compete successfully for public funds.

By providing financial incentives, medical philanthropists can change the culture and structure of the medical research enterprise. But philanthropy’s true potential can be realized only through informed, strategic, and measurable giving strategies.

By investing wisely in improved processes and collaborative research, donors can make relatively high-risk investments that could significantly advance a therapeutic option, move a field of research forward, and increase the likelihood that other parties will also invest.

**FIGURE 1:** Estimated Health Research Expenditure in the United States by source 2008 in USD billions (100 percent = $130.5B)

Medical philanthropy is well poised to play an outsized role in catalyzing and jump-starting innovation.
Defining Your Philanthropic Priorities

**KEY QUESTIONS**

1. **What disease am I passionate about?**
2. **Who do I want to help (e.g., in what geography, at what ages, what size of patient population, etc.) and why?**
3. **Would I rather fund R&D for new products, or development of tools and resources to support R&D (e.g., health information technology systems, training, etc.)?**
4. **Am I interested in prevention, diagnosis, or treatment?**
5. **How soon do I expect to see a product or tangible outcomes?**
6. **Do I know which stage of the R&D pipeline I should support (Figure 2)?**
7. **Knowing the process, how much risk am I willing to take to potentially transform R&D efforts?**

For many philanthropists just venturing into the complex, challenging process of medical R&D, it can seem formidable. The decision to invest in medical R&D is motivated by a broad range of factors: a personal experience or that of a loved one with a specific disease, the desire to improve health and save lives, the aspiration to improve the status quo.

Regardless of the impetus, investing your philanthropic dollars in medical R&D will require a careful assessment of your own priorities and preferences. Whether the decision to support a nonprofit effort stems from a personal connection, a comprehensive landscape analysis, or a hybrid of both, donors may want to consider their decisions within the context of the R&D process.

There is no one-size-fits-all approach to navigating the process of selecting recipient organizations. The R&D process involved in bringing new diagnostics, drugs, and vaccines to market is long and riddled with uncertainty. Within a given disease or therapeutic area, researchers may pursue dramatically different strategies and approaches in an effort to reach the same endpoint. At the onset, it often is difficult to predict whether a project or approach is likely to succeed and, if so, what its eventual social value might be.

Additionally, the organizations working on medical R&D are distinct and diverse, ranging from academic institutions to medical research foundations, from pharmaceutical and biotechnology companies to product development partnerships that act like virtual, nonprofit pharmaceutical companies that advance a portfolio of candidates through the development process.
Furthermore, within a disease, several potential interventions (i.e., diagnostics, drugs, vaccines) may vie for support, as may many organizations all along the spectrum from basic to translational research and on to clinical development.

Familiarizing yourself with the R&D process and arming yourself with information can help you objectively assess opportunities. Daunting as it may be, 21st Century medicine is within our reach and medical philanthropy plays a critical role in ensuring the medical research system is innovative and responsive.

**THREE STAGES OF MEDICAL RESEARCH**

1. **Discovery research** is the earliest stage of research, carried out for the advancement of knowledge, without necessarily any regard to its application to practical problems.

2. **Translational research** is the process of applying ideas, insights, and discoveries generated through basic scientific inquiry to the treatment and prevention of human disease – the critical bridge between basic research and clinical research. It includes intermediate steps such as identification of biomarkers, target and pathway validation, and development of and testing in animal models.

3. **Clinical research** is research in human subjects aiming toward approved treatments for patients. Clinical research is broken into three key phases: PHASE 1 examines the safety of the product in a very small group of healthy volunteers; PHASE 2 assesses the efficacy and correct dosing in a larger group of patients; and PHASE 3 tests the product in a much larger, more diverse population to determine broader efficacy, develop usage guidelines, and compare with existing products for the same indication. See also the FasterCures publication, “Getting Started: The Medical Research and Development Primer.”

Source: Entrepreneurs for Cures, FasterCures, 2008

**Figure 2: Three Stages of Medical Research**
Assessing the return on your philanthropy will help ensure that you achieve the greatest possible impact. At FasterCures, we developed an evaluation framework to guide philanthropists’ efforts to make more informed strategic decisions about where and how to invest their philanthropic capital.

This framework is designed to highlight effective practices that we have identified through our ongoing work with innovative nonprofit organizations, and was refined based on our findings from the FasterCures Philanthropy Advisory Service (PAS) program. In its pilot phase, PAS assessed 20 nonprofit disease research organizations in four disease areas — Alzheimer’s disease, malaria, multiple sclerosis, and tuberculosis.

To measure the impact of each organization objectively, we focused evaluation efforts on key drivers of organizational success:

1. Accountability
2. Collaboration
3. Research Effectiveness
4. Resource Building

These four measures of success provide you with information you need to inform your philanthropic priorities and allow you to track the impact of your giving within a functional framework.

There are many factors that influence an organization’s structure and the scientific context in which it operates. To help illustrate how these performance measures may be applied, we have developed fictional case studies that represent real-world scenarios to give you fodder for consideration as you decide how best to leverage your philanthropic capital. Accompanying the case studies are questions to help you dig deeper into the real-life organizations you are considering as partners.

When we invest our money, we look for the best ROI; when we donate it, shouldn’t we look for the best ROP (Return on Philanthropy)?
Accountability

What practices would an exemplary organization employ to ensure maximum accountability in its operations?

Accountability focuses on organizational policies and mechanisms that ensure funds are being used responsibly and effectively to achieve the organization’s goals. Establishing parameters around organizational practices that demonstrate its accountability helps ensure an unbiased look at its practices. Consider the experiences of the Malaria Research and Development Foundation in the following six components of accountability.

1. Strategy and Planning
2. Milestones and Monitoring
3. Management
4. Financial Sustainability
5. Technology Transfer and Commercialization
6. Community Engagement

Accountability Case Study: Malaria R&D Foundation (MRDF)*

Situation Overview
Malaria is a mosquito-borne parasitic disease that kills nearly a million children every year, mostly in sub-Saharan Africa.

Mission
To improve treatment options for malaria patients through the development of new life-saving drugs by bringing together partners from the public, private, and philanthropic sectors.

*MRDF is a fictional nonprofit organization. This case study was developed to illustrate performance measures of nonprofit organizations and not based on a specific organization, person, or situation.
**Strategy and Planning**

MRDF developed a comprehensive, six-year strategic plan designed to deliver five new malaria treatments in a decade and expand the pipeline of drugs in development. An outline of program tactics and activities demonstrate its implementation approach. To ensure scientific impact and relevance, MRDF established two advisory committees—one focused on development comprised of multi-disciplinary scientists, and the other focused on delivery, with mostly endemic country representatives. These committees help guide the strategic planning, implementation, and regular progress tracking.

**Milestones and Monitoring**

MRDF develops an annual plan for each program area that includes an outline of activities, a timeline for implementation, and specific milestones. A designated staff member tracks progress on a quarterly basis through a performance monitoring system. The scientific advisory committee meets twice a year to review ongoing projects, and may recommend funding adjustments, time-frame extensions, corrective actions, or project termination if a specific program is not producing the expected results. The committee also reviews the complete portfolio semi-annually to ensure projects are on track and that pre-determined objectives are being met.
Management

MRDF is led by a management team of qualified, diverse professionals. Its executive director is a former pharmaceutical executive with extensive background in medical product development and has spent significant time living and working in malaria endemic countries. Additionally, MRDF has a strong leadership team that includes scientists, administrators, and business development professionals who bring experience in developing products, managing nonprofits, and dealing with patient populations.

Financial Sustainability

MRDF has a detailed funding strategy that articulates its financial sustainability approach and goals. It has broad-based support from government grants, private foundations, and individual donors. The organization has set specific goals for levels of funding from the different groups and actively works to ensure a diversified funding base so that it is less susceptible to changes in donor priorities or other external circumstances.
Technology Transfer and Commercialization

MRDF’s R&D team creates a separate development plan for each product in the portfolio that lays out the activities, partners, and timeline required for commercialization. Partnership arrangements are negotiated based on an intellectual property policy that is designed to ensure that treatments are affordable and accessible for all patients. The foundation engages partners from academia, government, and industry to secure the right balance of skills and expertise for each stage of a product’s development, and has built long-term strategic relationships with partners in all sectors.

Community Engagement

MRDF has created a specific committee comprised of leaders from malaria-endemic countries that focuses on addressing the affected communities’ priorities—from access to affordability. This committee works alongside a team of MRDF staff that is responsible for building awareness and advocacy around the disease and directly supporting new products when they are introduced. Clinical trials conducted in endemic countries also provide a key opportunity for the organization to interact with patients, caretakers, and other affected populations.

KEY QUESTIONS

1. Does the organization have an intellectual property policy that includes provisions for the transfer of technology or commercialization of products?
2. Does the organization partner with industry to help advance technologies through the R&D pipeline?
3. How will the organization engage with industry if it produces early-stage discoveries that have development potential?
4. Does the organization have deeply-rooted ties to the communities it serves?
5. Does the organization conduct activities aimed at understanding the needs of affected communities?
6. Does a representative of the affected community sit on the organization’s board, or is there a separate community/patient advisory structure?
7. Does the organization have mechanisms in place to regularly communicate with the affected community?
Collaboration

How can an organization demonstrate its commitment to collaboration?

Collaboration is critical to successful innovation in disease research. Some of the most exciting work in biomedical science lies at the intersection of disciplines. To assess an organization’s collaboration capacity with a wide range of partners, consider the Foundation to Fight Alzheimer’s Disease scenario and the following four components.

1. Knowledge Sharing
2. Cooperative Research
3. Global Research
4. Strategic Partnerships

Collaboration Case Study: The Foundation to Fight Alzheimer’s Disease (FFAD)*

Situation Overview
Alzheimer’s disease is the most common form of dementia, characterized by protein deposits in the brain, which affects millions of older people in the United States and abroad.

Mission
To identify and advance promising pathways to new treatments for Alzheimer’s patients through early-stage research.

*FFAD is a fictional nonprofit organization. This case study was developed to illustrate performance measures of nonprofit organizations and not based on a specific organization, person, or situation.
Knowledge Sharing

Since its inception, FFAD has had a written knowledge sharing policy that helps ensure that the impact of the research it supports is maximized and magnified. Implementing this policy started with developing mechanisms for sharing data, experiences, and resources.

Among its activities, FFAD holds annual scientific seminars, developed an online platform for discussion, and created an accessible database of medical presentations and publications. These efforts are implemented to facilitate early-sharing of data, allow real-time feedback on research process, results analysis, or process adjustments, and track outcomes and impact.

Cooperative Research

Recognizing that effective research is becoming more interdisciplinary and multi-institutional, FFAD is focusing its funding on cooperative research efforts. More than half of FFAD’s grants are directed to research networks that include various institutions and departments. FFAD prioritizes grant applications from multidisciplinary teams both within an institution and among multiple institutions. Additionally, FFAD leadership actively seeks out opportunities to facilitate collaboration — through discussion forums and targeted networking opportunities.
Global Research

Disease knows no boundaries and a growing number of research programs span continents. FFAD puts a premium on novel ideas and has sought out research proposals globally to ensure it continues to fund cutting-edge, forward thinking ideas. Many of its research networks include institutions from Europe and Australia, with 15 percent of funding directed to research programs based in other countries. FFAD also actively creates opportunities to engage the international research community through conferences, special events, scientific working groups, and advisory boards. It also has developed a global database of Alzheimer’s researchers and centers to help keep the research community connected and keep patients and funders informed.

Strategic Partnerships

Constantly in pursuit of partners that could ensure it stays true to its mission and maximize the return on its own investment, FFAD developed a long-term collaboration with a leading pharmaceutical company to co-fund early-stage R&D activities in academia and small biotechnology companies. The aim is to facilitate the transfer and commercialization of new technologies to significantly advance the process. Also, FFAD both convenes and participates in roundtable discussions and other meetings that bring together various sectors to support existing efforts and develop new solutions.
Research Effectiveness

How do you objectively measure an organization’s effectiveness and impact?

As with any field, having the right policies and practices in place is important, but even more crucial is the ability to demonstrate effectiveness and impact both on the scientific landscape and ultimately on patients’ well-being. Measuring effectiveness may start by looking at an organization’s ability to achieve its own objectives and may require input from external scientific experts who can help you understand how an organization’s R&D portfolio fits into the needs of the specific disease area, as well as the demonstrated or potential value of its scientific contributions. Consider the Multiple Sclerosis Treatment Research Fund’s experiences in three areas that can help assess its research effectiveness.

1. Strategic Achievements
2. Portfolio Congruence
3. Scientific Advancement

Research Effectiveness Case Study: Multiple Sclerosis Treatment Research Fund (MS Fund)*

Situation Overview
Multiple sclerosis (MS) is a chronic, debilitating disease of the central nervous system in which the body’s immune system attacks the covering insulating the nerves, resulting in neural dysfunctions and disability.

Mission
To identify promising drug targets for the development of future MS treatments.

*MS Fund is a fictional nonprofit organization. This case study was developed to illustrate performance measures of nonprofit organizations and not based on a specific organization, person, or situation.
**Strategic Achievements**

When it was founded three years ago, the MS Fund convened a group of leading MS and central nervous system experts to help develop a detailed five-year roadmap for its activities, complete with goals and timelines for each activity. This framework has allowed the MS Fund to track its strategic achievements and articulate its progress. For example, to date, it is able to say that all but one of its milestones are on or ahead of schedule. For the one milestone lagging behind, the fund, with the help of its scientific advisors, re-assessed available data and determined that the pathway was less promising than originally expected and, as a result, decided to de-prioritize the project in favor of other, stronger programs. Looking forward, the fund appears to be on track to continue to achieve its objectives as planned.

**Portfolio Congruence**

All MS Fund projects fall into one of three activity areas that were established in the research roadmap. This demonstrates a high level of congruence between the fund’s portfolio and its plans and goals, which is necessary to be effective. The majority of projects address translational research questions, mirroring the priorities outlined in its roadmap and annual research plans. To stay on track, the MS Fund’s scientific advisors review its portfolio biannually to evaluate both the progress of individual projects and the balance of those projects among areas of focus and strategic objectives. The MS Fund recently reported that because of this approach, its scientific team plans to target specific areas in sourcing new projects in order to ensure that the portfolio remains aligned with the roadmap.
Scientific Advancement

Researchers supported by the MS Fund have identified 20 potential targets in the first three years of operations. To date, five of the targets have been transferred to biotechnology companies that are proceeding with preclinical development. Negotiations are underway to transfer three additional targets in the coming months, and the MS Fund is seeking partners to take on development of the remaining targets. Although it is unclear how these targets may eventually impact patients, outside scientific experts indicate that this rate of return is exceptional and indicate that the targets are high quality and hold high potential.

KEY QUESTIONS

1. Is the organization making an impact in advancing medical progress in its disease area?
2. What are the organization’s most important scientific milestones?
3. Do outside experts consider these to be significant contributions to moving the field forward?
4. If the organization has delivered products, is there evidence that they are reaching patients?
Resource Building

What elements should you examine to assess an organization’s capacity to build resources necessary to advance a robust research agenda?

Some, though not all, nonprofit organizations that fund or conduct medical R&D also engage in efforts to fill critical resource gaps that limit scientific progress in their fields. However, it is important to note that needs vary by field, and each organization should work to identify what, if any, investments in this area are most likely to advance its own mission. We examined the Tuberculosis Vaccine Coalition’s experience in resource building by looking at its tools and resources and training and career development.

1. Tools and Resource Development
2. Training and Career Development

Resource Building Case Study:
The Tuberculosis Vaccine Coalition (TVC)*

Situation Overview
Tuberculosis (TB), the second leading infectious cause of death globally, is a bacterial infection that is found worldwide but imposes its burden predominantly in developing countries and emerging economies.

Mission
To develop and deliver a new, more effective vaccine to prevent TB infection and disease.

*TVC is a fictional nonprofit organization. This case study was developed to illustrate performance measures of nonprofit organizations and not based on a specific organization, person, or situation.
As part of its strategic planning process, TVC conducted interviews and in-depth research to identify critical gaps in tools and resources that would limit its ability to advance vaccine candidates through the development pipeline. This effort determined that one of its biggest obstacles is the lack of a clinical trials infrastructure to test vaccine candidates in countries where TB is highly endemic. TVC decided that if it were to succeed in its mission, it would need to invest in building and maintaining the necessary capacity to conduct its trials.

TVC identified existing institutions in several countries as potential partners, assessed infrastructure and equipment needs at each institution, and invested in upgrades to fill those gaps. Six partner institutions located in disease endemic countries in Africa, Asia, and Eastern Europe now meet the required standards and are ready to conduct trials. These sites are linked through a common information technology system that facilitates communication and data sharing, and leadership and staff from the institutions meet on a regular basis, typically in concert with major scientific conferences, to share best practices and discuss common challenges. TVC also has worked with the partner institutions to identify other product developers and market their services to those groups.

**Tools and Resources Development**

**KEY QUESTIONS**

1. Does the organization’s mission or strategy include developing tools and resources as a priority? If not, why not?

2. Does the organization contribute to developing needed tools and resources such as predictive animal models, interoperable research databases, comprehensive biobanks, patient registries, clinical trials infrastructure, information technology platforms, and data standards and protocols?

3. Has the organization identified and invested in other types of tools and resources to support the field?

4. Are the tools and resources developed available to the broader research community, and is there evidence that they are being used by outside parties?

5. Do the tools and resources developed address critical gaps identified by experts in the field?
As TVC began to develop its clinical trials network, it found that there were not enough skilled clinical researchers and support staff in many of the countries where its partner institutions were located. To help address this gap, the coalition provided financial support for staff members at these institutions to attend courses and workshops to obtain training in good clinical and laboratory practices, as well as other areas that would help to support the network’s efforts. Additionally, the coalition found partners that could help secure support for advanced scientific training for several young researchers. Upon training completion, they can then apply their skills at network institutions in their home countries. The coalition also provides support for partner institutions seeking grants to cover such career development and professional advancement activities.

**Training and Career Development**

**KEY QUESTIONS**

1. Does the organization’s mission or strategy include supporting training and career development as a priority? If not, why not?

2. Do the training and career development opportunities supported address critical gaps identified by experts in the field?
MEDICAL RESEARCH IS BADLY IN NEED of more innovative, high-risk approaches with high-reward potential. All sectors of the medical research enterprise — government, industry, nonprofit, and philanthropy — have critical roles to play to catalyze these approaches.

For medical philanthropists, informed and strategic decision-making could help ensure maximum return on philanthropic investment. By having performance measures and standards in place for accountability, collaboration, effectiveness, and resource-building, donors are cultivating a culture that is mission-driven, results-oriented, and focused on the true bottom line: preventing, diagnosing, and curing disease.
About FasterCures and its Medical Philanthropy Program

FasterCures/The Center for Accelerating Medical Solutions is a nonprofit think tank and catalyst for action that works across sectors and diseases to improve the effectiveness and efficiency of the medical research enterprise. FasterCures, a center of the Milken Institute, is committed to accelerating the process of discovery and development of new medical solutions for deadly and debilitating diseases.

Philanthropic investment in medical research, though small in size, plays a unique, critical role in finding medical solutions. Philanthropic capital fills funding gaps in research that is high-risk but also has potential of high return. For some diseases, nonprofit funding models are virtually the only source for innovative research.

FasterCures’ Medical Philanthropy Program was created to help philanthropists make informed investment decisions and have in place a framework to assess the return on their philanthropy.

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