

Breakthrough

Medical research is dominated by government and corporate funding. How can philanthropic dollars find a distinctive niche?

By Adam Keiper

IN THE FIELD OF MEDICAL RESEARCH, PRIVATE philanthropy is a relatively small player. According to the Foundation Center, foundations spent less than \$5 billion on medicine in 2007, and less than half of that went to research. Compare either number with the \$40 billion that government agencies, primarily the National Institutes of Health (NIH), spend on biomedical research, as well as the \$80 billion that private pharmaceutical and biotech firms invest in research and development each year.

Even though the philanthropic sector's resources are dwarfed by those of the public and for-profit sectors, private philanthropy still has one crucial advantage. Private donors do not answer to voters or shareholders, and they are not constrained by the peer-review protocols that dominate government funding. They are free to innovate, to experiment, to take risks, and to find and occupy their own distinctive niche.

A Legacy of Leadership

AMERICAN MEDICINE HAS LONG BEEN A partnership between public resources and private philanthropy. The nation's first hospital, established in the 1750s by that congenial organizer, Benjamin Franklin, was funded by both the state assembly and private donors in what is thought to be the earliest instance of a matching grant. In the decades that followed, several other medical institutions were funded by private philanthropy, including most notably the prestigious hospital, nursing school, and medical school underwritten by Johns Hopkins, and John D. Rockefeller's creation in 1901 of the

first American medical research institute.

By the dawn of the 20th century, medicine in the United States was rapidly evolving from a craft and a vocation to a scientific profession. That transition was cemented—and modern medicine was in large part created—by interventions from the nation's large philanthropic foundations in the early 20th century. The Rockefeller Foundation (and its predecessor entities) undertook a series of hugely ambitious campaigns to fight killer diseases around the globe: hookworm, yellow fever, malaria. And a scathing 1910 report commissioned by the Carnegie Foundation helped instigate a massive overhaul of American medical education. Abraham Flexner, the report's author, argued that medical schools should be reformed along more scientific lines—and then worked assiduously, as a Rockefeller Foundation employee, to push for such reforms across the country.

Although the federal government was involved in large-scale public health efforts during the first half of the 20th century, its investment in medical research was minimal. The experience of World War II, however, convinced Washington that government, industry, and science could collaborate successfully on projects of critical national importance. Federal spending on nearly every kind of science and technology rapidly rose in the postwar years—including medicine and health. Thanks in part to lobbying from the Albert and Mary Lasker Foundation, the NIH annual budget

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rose from about \$3 million in 1945 to \$30 million by the end of the 1940s, \$300 million by the end of the 1950s, and \$3 billion by the end of the 1970s. For fiscal year 2010, the NIH has requested a \$31 billion budget.

The rise in government spending on medical research in the last six decades has meant that philanthropic dollars have made up a shrinking proportion of the funding stream for medical research. That, in turn, has meant that philanthropy has had a diminishing ability to control medical research in the United States: it may no longer be possible for philanthropy to create new disciplines practically from scratch, as the Rockefeller Foundation did with molecular biology. (That said, there is still wide latitude for disciplinary innovation. The Whitaker Foundation, which wound down its operations in 2006, seeded the country with centers of biomedical engineering.)

Private donors have instead looked for other ways to achieve breakthroughs in medical research. They are focusing on unconventional hypotheses, enlarging our understanding of human health by asking questions that nobody else is thinking about. They are creating ways for researchers around the globe to share information, working to speed up the process by which cures move out of the lab and into the market, and offering early career support for promising researchers. They are calling attention to diseases that would otherwise be neglected. And they are drilling down on rare and neglected diseases, tackling the problems that neither private enterprise nor public funding has the incentive to confront.

Many of those strategies have already borne fruit, while others that are just getting underway promise to recast private philanthropy as an essential player in research projects that will, over time, save and improve millions of lives.

Investigating Unconventional Hypotheses

PRIVATE PHILANTHROPY IS UNIQUELY SITUATED to break new ground by pursuing unorthodox theories. Because they are not beholden to shareholders, peer review, or political constituencies, donors can direct their resources wherever they wish. So, for example, Peter A. Thiel, the investor perhaps best known for co-founding PayPal and taking an early interest in Facebook, has supported unconventional laboratory and theoretical work through his Thiel Foundation. He has a

6 Strategies for Philanthropic Funding of Medical Research

Investigate unconventional hypotheses.

Private donors are not accountable to voters or to shareholders, and are not limited by the peer-review processes and expectations prevalent in government funding. They enjoy the freedom to think boldly—and to pursue unorthodox theories that have the potential to make real advances in medical research.

Foster collaboration among researchers.

The learning opportunities from scientific collaboration can offer an attractive return on philanthropic dollars. The rise of the Internet has considerably facilitated this task, making it ever-easier to host large quantities of data, available around the world and in real time.

Accelerate the move from lab to market.

Private philanthropy can hasten the process of turning ideas into cures. Whether by helping researchers clear regulatory hurdles or by connecting innovators with entrepreneurs, private donors have helped get lifesaving drugs and procedures to the people who need them most.

Fund researchers early in their careers.

Given their general low risk tolerance, government grants for medical research tend to gravitate toward well-established researchers. Private donors, by contrast, are at greater liberty to support whomever they think has the most promising proposal—regardless of their standing among peers.

Raise awareness among the general public.

Donors are also well-positioned to call attention to diseases and conditions that might otherwise be overlooked. In addition to promoting prevention strategies, they can raise critically needed funds to elevate the profile of neglected problems.

Focus on rare and neglected afflictions.

Some diseases are so rare that there is little incentive for private investors to pursue, and little deterrent for public figures to ignore, their cures. Donors can occupy this niche by targeting specific diseases and devoting themselves to their cure.



“Rapid advances in biological science foretell of a treasure trove of discoveries this century, including dramatically improved health and longevity for all,” says Peter Thiel. (Photo by Steve Maller; used under a Creative Commons license)

particular interest in research on aging and life extension, explaining that “rapid advances in biological science foretell of a treasure trove of discoveries this century, including dramatically improved health and longevity for all.” Thiel is also the most prominent benefactor of the “Singularity” movement, whose followers advocate human enhancement and the merging of human minds with computers.

The John Templeton Foundation also explores unconventional questions in medicine as part of its namesake’s interest in the “big questions” of science and religion. “Medicine, health, religion, and spirituality are logical places to invest in as we can learn . . . about religion, positive emotions, virtues, and other things typically relegated to religion by studying their impact, good or bad, on health,” says Kimon H. Sargeant, the foundation’s vice president for human sciences.

“Many of the late Sir John Templeton’s favorite maxims, such as ‘when you rule your mind, you rule your world’ or ‘what you think you are, you are,’ teach that the links between our thoughts and our health are profound—and mostly overlooked,” says Sargeant. The foundation has investigated those links through its support for projects like the Duke Center for Spiritu-

ality, Theology, and Health, which has created a network of interested scholars. “The amount of studies that have been done since the year 2000” on spirituality and health, says center director Harold Koenig, “probably exceeds all the research in the 150 years prior to 2000.” One of the findings of all this research has been that religious attendance—“How often do you go to church, or synagogue, or mosque?”—is the “most powerful predictor, of all religious characteristics, on health outcomes, on longevity, on well-being.”

It is too early to “make any definitive claims about causation at this point,” says Sargeant, but “the findings thus far show there are positive correlations between spirituality and health.” Like the Duke Center, the Institute for Spirituality and Health at George Washington University is another important center that trains medical professionals to understand linkages between spirituality and health.

Christina Puchalski, the institute’s founder and executive director, argues that spirituality has become an increasingly accepted part of conventional medicine. The growing acknowledgement of a link between spirituality and health, Puchalski says, is part of a larger shift in medicine away from concentrating on disease to thinking about *illness*—that is, a move to a more patient-centered approach to medicine, one that takes into account the “inner life of the patient.”

The institute has worked with leading medical organizations, like the Association of American Medical Colleges, to increase recognition of spirituality in medical education; about three-quarters of American medical schools now address spirituality and health, either in full courses or as integrated components of other courses. The institute has also developed tools intended for clinical settings, such as a set of questions that can help practitioners understand patients’ beliefs and values, which can then help doctors tailor their therapeutic approach.

In addition to its work on spirituality and health, the Templeton Foundation is funding a variety of projects that pursue unorthodox theories. With support from Templeton, a team at Yale headed by Günter Wagner is studying genetics and the origin of organismal complexity. (The goal of the project is to explore the role of creativity in the genetic programming of higher-order organisms and to show how specific creative mechanisms have been fixed in organisms’ body plans.)

Another team at New Mexico's Mind Research Initiative is investigating the neuroscience of creativity. Still others are pioneering the field of positive psychology. All of them, however, are examples of the freedom of philanthropy to pursue the questions that others cannot or will not.

The Samueli Foundation is likewise engaged in the exploration of heterodox hypotheses. Susan Samueli has had a longstanding interest in homeopathic medicine and has "seen the value of acupuncture in family situations," says foundation executive director Gerald Solomon. Susan and her husband, Henry Samueli, "wanted to fund research to determine the evidence-based support, or lack thereof" for different modes of "integrative and complementary medicine"—including techniques like acupuncture and chiropractic, as well as broader considerations that contribute to well-being, like healthy eating and living.

The California-based foundation's most prominent project in this arena has been the Samueli Institute, a nonprofit organization founded in 2001. The institute, which has a \$15.9 million annual budget, is housed in Alexandria, Virginia. Its "sole purpose is to examine the science of healing and health," as a sort of independent think tank, says Solomon. For example, the institute found from a survey of the scientific literature that there is "clear evidence that acupuncture reduces nausea," Solomon says. This means, for example, that chemotherapy patients might be encouraged to try acupuncture to mitigate the side effects of their treatment. The institute is also working to help develop standards for training and educating people involved in distributing nutritional supplements.

In a sense, the Samueli Foundation is revisiting a debate about the merits of homeopathic medicine that was cut short nearly a century ago by the Flexner report on medical education. At the turn of the last century, the homeopathic school contended against the allopathic school that emphasized science and pathophysiology. The Flexner report came down hard on the side of allopathy, which became the basis of mainstream medicine. The Samueli Foundation's effort to prod at those central tenets is among the clearest examples of philanthropy's freedom to pursue heterodox but promising pathways in medicine.

The Connecticut-based Stanley Family Foundation has also pursued unusual scientific theories. Impelled by their son's experience with mental illness, Theodore and Vada Stanley two decades ago asked E. Fuller Torrey—a psychia-

trist and the author of the book *Surviving Schizophrenia*—to help them develop a mental-health research program. The result has been the Stanley Medical Research Institute, which Torrey now directs.

From the beginning, the institute focused on two diseases—schizophrenia and bipolar disorder—that Torrey says were "really being massively under-researched" by the National Institute of Mental Health (NIMH). Although NIMH has since increased its funding, the Stanley Institute was, up until a few years ago, underwriting "at least a third" of the U.S. research on bipolar disorder and "close to half" of the research on schizophrenia, he says.

The Stanleys have also "been exceedingly generous and willing to take a chance on research that NIMH would not have," Torrey says—research outside "the established paradigm," like Torrey's own investigations into the possibility that bipolar disorder and schizophrenia are caused by infectious agents.

Also, Torrey adds, since the Stanleys committed their money over time, the institute has been able to launch projects of a scale too large for NIMH—most notably the Stanley Brain Collection. The institute has more than 600 post-mortem brains, gathered between 1994 and 2005 with the cooperation of medical examiners and the consent of next of kin. With its focus on brains from people with schizophrenia and bipolar disorder, it is the world's most widely used brain collection for researchers studying those diseases. Since 1996, the institute has sent, without charge, over 200,000 pieces of tissue to more than 240 researchers in the United States and 20 foreign countries.

The other major Stanley goal is to find more effective treatments for these diseases. It



Ted and Vada Stanley were impelled by their son's experience with mental illness to fund research into the brain. (Photo courtesy of Mr. and Mrs. Stanley)

currently supports 60 treatment trials of drugs that are off-label or already generic and thus of limited interest to pharmaceutical companies. One especially promising drug for bipolar disorder is currently undergoing additional testing for possible marketing.

Fostering Collaboration among Researchers

THE STANLEY BRAIN COLLECTION HIGHLIGHTS yet another strategy that donors can use to advance medical discoveries: supporting big projects that make possible a range of investigative research. These “enabling projects” require resources and patience that government and industry might not muster, but they provide tools for researchers around the world, very often free of charge.

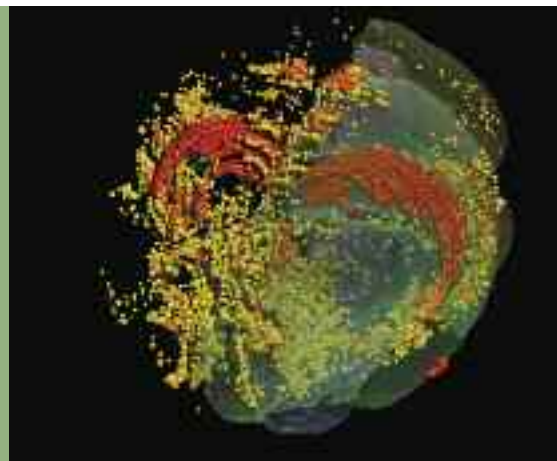
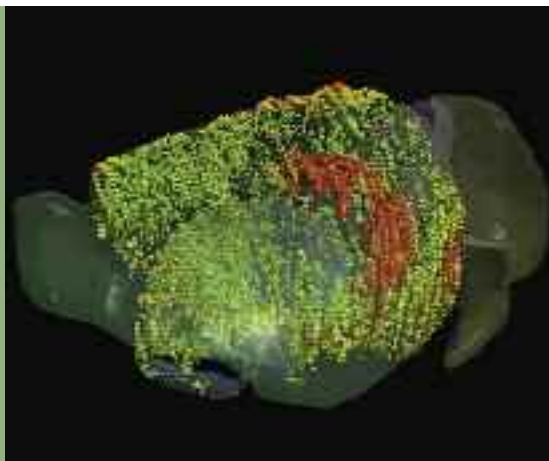
Another recent example of a major enabling project also involves brain science. The Allen Institute for Brain Science, launched in 2003 with \$100 million in seed money from Microsoft co-founder Paul G. Allen, invested \$41 million in developing a three-dimensional “atlas” of the gene expression patterns in the adult mouse brain. His vision had two critical requirements, according to Elaine Jones, the institute’s chief operating officer. First, Allen wanted the brain atlas to be “totally free, totally open access; all the data and all the tools for manipulating and managing the data are given out free on the Web.” And second, he wanted the brain atlas to be available “in real time,” as soon as the quality of the data was checked. Thus, even though the brain atlas wasn’t formally published in a scientific journal until January 2007, scientists around the world had

access to its data during the three years it was under construction.

By monitoring its Web traffic, the institute can track which parts of the atlas researchers are accessing—so they know which genes are getting the most attention. There are already hundreds of journal citations mentioning the atlas, Jones says, in papers on subjects ranging from memory and learning, to obesity, to diseases like multiple sclerosis. From studying the statistics and by heeding feedback from researchers, the institute can constantly improve the online tools it has built to present the data.

The institute has since taken on several other enabling projects, including another mouse brain atlas that tracks gene expression starting at the embryonic stage; an atlas of gene expression in the mouse spinal cord; and a gene-expression atlas of the human brain, with a projected budget of \$55 million. These projects are all funded following the seed-money model Allen established, with additional resources coming from other foundations, nonprofit organizations, biomedical firms, and government.

Enabling projects such as the Stanley Brain Collection and the Allen Institute’s atlases are too distant from any prospect of profit for private industry to take them up. And although NIH has certainly funded some enabling projects—the Human Genome Project being the best example—it generally “funds hypothesis-driven basic research,” says Jones. For philanthropists with patience and trust in the creativity of scientists, fostering collaboration can be an attractive way to push forward progress in medicine.



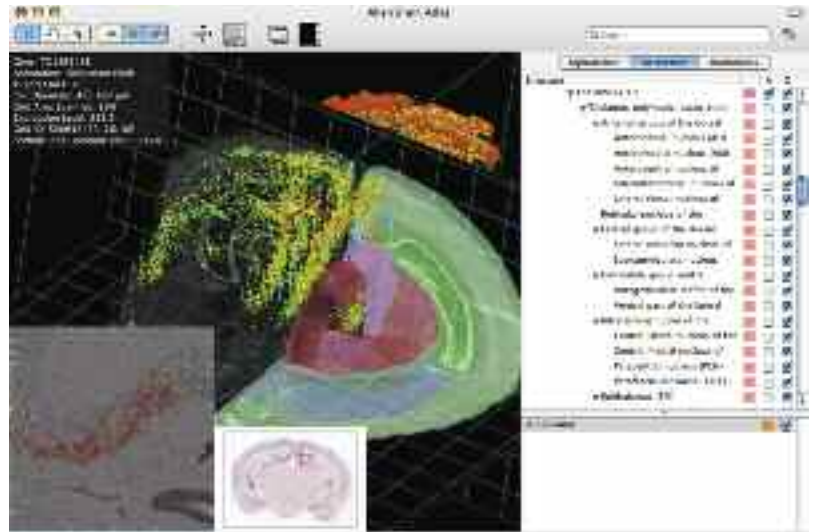
Images from the Allen Institute’s atlas of the mouse brain.

Accelerating the Move from Lab to Market

SOME FOUNDATIONS PREFER TO CONCENTRATE on more immediate results, targeting their resources on projects that try to speed new treatments to market. The path from the laboratory bench to the patient's bedside is a long, hard slog. Drug development—from the initial synthesis of a promising compound, through preclinical research, through clinical research and trials, through the government drug-approval process—can take upwards of a dozen years. On average, it costs more than \$800 million. So many potential drugs disappear during the development process that it has been nicknamed the “valley of death.”

One of the most effective voices for reform has been that of Michael Milken. The Wall Street icon had been funding medical research since the 1970s, and the Milken Family Foundation launched the Cancer Research Awards in the 1980s. But he redoubled his efforts after he was diagnosed with advanced prostate cancer in 1993. He learned that prostate cancer research was stalled in a vicious cycle: funding wasn't flowing to the field, which meant that it wasn't attracting scientists, which meant that there were no advances, which was keeping away the funding. In the words of Andrew C. von Eschenbach, who went on to head the National Cancer Institute and direct the Food and Drug Administration, “We were in this quiet corner no one wanted to be associated with.”

Milken threw himself into funding medical research with the same brook-no-delay attitude that had characterized his career at Drexel Burnham Lambert. He launched the Prostate Cancer Foundation and began transforming the field almost overnight. A 2004 *Fortune* magazine cover story entitled “The Man Who Changed Medicine” summarized his approach:

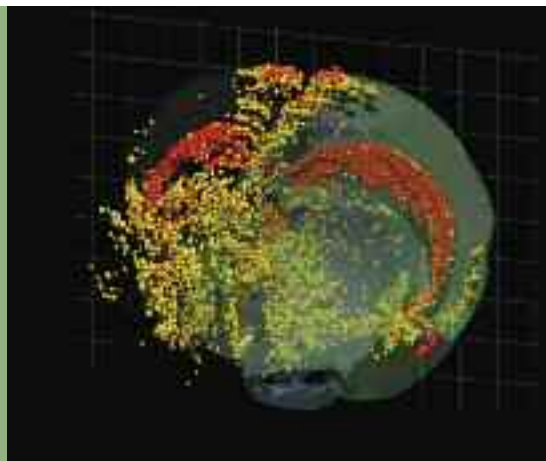
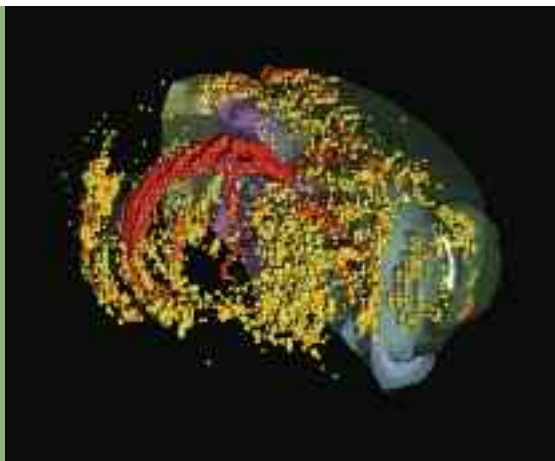


The Allen Institute's Brain Explorer interface allows researchers to study features of the mouse brain, here rendered in 3-D. (Image originally published in open-access BioMed Central; used under a Creative Commons license)

The Milken model, in a nutshell, is to stimulate research by drastically cutting the wait time for grant money, to flood the field with fast cash, to fund therapy-driven ideas rather than basic science, to hold researchers he funds accountable for results, and to demand collaboration across disciplines and among institutions, private industry, and academia.

Since its inception, the Prostate Cancer Foundation has funded more than 1,500 programs at nearly 200 research centers in 20 countries. While the disease remains a major killer, survival rates have risen dramatically—thanks indisputably to the foundation's work. (Milken's own cancer is in remission.)

For potential donors who want to follow in his footsteps, Milken advises direct personal involvement. “There's no substitute for rolling up your sleeves and working with the people who can make



Before You Write the Check ...

Map the field.

Ask yourself: Where is philanthropic capital most needed? Where can your funds leverage the greatest gains, and what existing philanthropic investments could use additional support? Consider directing your funding toward areas not currently covered by major grantmakers, government programs, or private capital. Philanthropists enjoy the freedom to invest where they think best. Take advantage of it.

Determine your risk tolerance.

Donors need to calibrate their expectations to their funding. If the goal is to secure incremental gains, consider structures that will yield consistent results. If the goal is transformative breakthroughs, be prepared for frustration and a higher probability of failure.

Decide on a pathway for your funding.

A key decision point is whether to fund individual research teams or to work with established research centers, leaving resource allocation decisions to them. If you want to support pioneering research in an area that has not received much attention, the first route may be preferable. If you want to advance research in a field that has already attracted substantial funding, it may make more sense to fund institutions that are already involved in current research.

Get expert opinions.

Many top medical philanthropists have engaged leading researchers and scientists as formal or informal advisors. These individuals know the research arena better than anyone and can direct your gifts to the most promising projects and grantees. Look for an advisor with deep knowledge of the field and a reputation for candor and discretion. A peer-review system can sift the best proposals.

Consult research platforms.

Researchers have an increasing number of resources for sharing and disseminating their findings. It may be worth reviewing some of the shared platforms to get a sense of where dollars are already flowing—and find out where they aren't.

a difference,” he has written. “They get the benefit of your participation and you gain a direct understanding of the real problems and potential solutions, which makes you a more informed giver.”

Knowing that treatments for other diseases are being slowed by many of the same institutional troubles that once beset prostate cancer research, Milken in 2003 founded an “action tank” intended to combat the “tangle of regulation, misplaced and misaligned priorities, and conflicting incentives that permeate the medical research environment.” Although technically part of the California-based Milken Institute, the new organization, called FasterCures, is housed in Washington, D.C.—in recognition of the policy component of the problem, says executive director Margaret Anderson.

“The hallmark of the new medical philanthropy is that it’s very informed, it’s engaged, and it’s really results-oriented,” Anderson says. Several of the major disease-focused funds, such as the Michael J. Fox Foundation for Parkinson’s Research and the Multiple Myeloma Research Foundation, have emulated some or all of the Prostate Cancer Foundation’s program. Many of these “passion capital” groups, as Anderson calls them, try “to eliminate wasted energy, effort, time—starting to apply business principles.”

FasterCures aims to bridge the “valley of death” in part by getting the different parties involved in medical research to talk to one another. One such initiative, Partnering for Cures, brought together mission-driven disease foundations and biopharmaceutical companies at its first meeting in December 2009 in New York City. FasterCures hopes that foundations will find investors willing to usher promising therapies to market, and that the drug companies will find projects they can afford to pursue since the foundations will have assumed some of the early risk. For philanthropists, Anderson says, such meetings are an opportunities to “learn about best practices” and to keep tabs on the “innovation happening all over.”

Connecting researchers to industry and investors is also a key project of the Ewing Marion Kauffman Foundation. (For more information about Kauffman, please see *Philanthropy*, Fall 2009.) The foundation—which is dedicated to the cultivation and promotion of entrepreneurship, rather than to medical research specifically—has funded or seeded several projects to

help move medical innovations from the laboratory to the market.

Kauffman's iBridge Network, begun in 2007, is an effort in this vein. "iBridge is like Craigslist for innovation and ideas," says Lesa Mitchell, vice president of advancing innovation at the Kauffman Foundation. "It's an online innovation catalyst that lets university-based researchers connect and collaborate with other experts, promote their ideas and discoveries to businesses and entrepreneurs who can offer funding and, ultimately, license their innovations for practical benefit."

Mitchell points out that American universities receive some \$40 billion each year in federal grants, but much of the resulting research remains tucked inside the black box of the academy, largely invisible or inscrutable to outsiders, especially outsiders who might wish to translate the research into useful (and profitable) products. For biomedical research—and a whole range of other subjects, from computer science to energy research—iBridge uses Web 2.0 techniques to help break innovations out of the black box.

Funding Early-career Researchers

"OF ALL THE PROGRAMS WE'VE SUPPORTED OVER the last generation," says Michael Milken, "the biggest payoff in terms of social benefit has come from the awards to young investigators." Milken spent years seeking out and funding promising early-career cancer researchers. Realizing that such researchers were often tempted to pursue more lucrative clinical practices, he provided grants aimed at keeping them in the lab, conducting cutting-edge research.

Throughout the 1980s, Milken supported an array of scientists who later went on to make revolutionary breakthroughs in cancer research. He funded Lawrence Einhorn, who went on to develop a highly successful chemotherapy regimen for testicular cancer, and who later treated Lance Armstrong. He supported Charles Myers, who subsequently went on to become chief of the clinical pharmacology branch of the National Cancer Institute. He funded Dennis Slamon, who later discovered Herceptin, a major advance in the treatment of one type of breast cancer. He backed Bert Vogelstein, who went on to conduct pathbreaking work on the p53 gene, whose mutant form is believed to be involved in more than half of human cancers. And he funded Owen Witte, whose subsequent work provided the basis for the development of Gleevec.

Another donor who specifically targets promising young researchers is Edward Netter. Netter is the chairman and CEO of Geneve Cor-



"There's no substitute for rolling up your sleeves and working with the people who can make a difference," says Michael Milken. From left, Milken, Missouri Governor Jay Nixon, and Komen for the Cure founder Nancy Brinker. (Photo courtesy of the Prostate Cancer Foundation)

poration, a private diversified holding company. With his wife, Barbara, he co-founded the Alliance for Cancer Gene Therapy in 2001. They were motivated in large part by the experience of watching their daughter-in-law fight and eventually lose an 11-year battle against breast cancer. (Please see "Challenging the Status Quo in Cancer Research," *Philanthropy*, November/December 2006.) The experience was devastating to their family. Watching a loved one fight cancer brings on "a feeling of helplessness," says Barbara. "And to be able to do something about it is healing."

As part of their efforts to do something about it, the Alliance for Cancer Gene Therapy has awarded more than \$20.1 million to fund 33 research projects. The goal: to harness the potential offered by cell- and gene-based therapies to accelerate the effective and safe treatment of all types of cancers.

The alliance awards grants to individual researchers—not to institutions. The practice reflects a core conviction of the alliance: that it is the scientists themselves who are the visionaries. To that end, one of its signal grant programs is the Fund for Discovery, which awards up to \$500,000 to tenure-track assistant professors conducting innovative exploratory research. The award distributes funds over three years, inclusive of a maximum of 10 percent indirect costs. Funds may be used at the recipient's discretion for salary, technical assistance, supplies, or capital equipment.

Why does Netter focus on early career researchers? As he explained to *Philanthropy* in

2006, “Whether it’s in the business world or in the pursuit of scientific research, it’s important to critically examine the status quo and relentlessly pursue a better way.” Finding and funding bright, ambitious researchers—those who are not yet established heavyweights in the field—is one strategy for relentlessly pursuing a better way.

Raising Awareness among the General Public

OTHER PHILANTHROPIC EFFORTS ON BEHALF OF medical research have concentrated on raising awareness about specific diseases. The best-known nonprofit in this category is probably Susan G. Komen for the Cure (formerly the Susan G. Komen Breast Cancer Foundation). In 1982, Nancy G. Brinker founded the nonprofit organization to honor a promise to her sister, who had died of breast cancer. “I knew that we not only had to have a fundraising organization to support the very best research,” she says, but that “it was extremely important to raise the profile” of breast cancer. Americans were well aware that nearly 58,000 U.S. servicemen perished in the Vietnam War. What was not widely known, however, was that nearly six times that number of Americans died of breast cancer during those same years.

“We had to change the culture,” Brinker says, but “almost to a person, in the beginning, I was told that we couldn’t do it.” Now, 28 years later, the pink ribbon is the ubiquitous icon of breast cancer advocacy, and there are Komen “Races for

the Cure” across the country. According to the organization’s statistics, the five-year survival rate for cancers that have not spread from the breast has risen from 74 percent in 1982 to 98 percent today. Since its founding, says Brinker, there hasn’t been any significant advance in breast cancer therapy that has not been touched by Komen.

“And on the research side,” says Brinker, “the next five years will be the most exciting since President Nixon announced the ‘War on Cancer’ in 1971.”

Focusing on Rare and Neglected Diseases

SOME DONORS INVEST BROADLY IN BIOMEDICAL research with a full spectrum of initiatives. For instance, the Howard Hughes Medical Institute (a private operating foundation with an \$831 million operating budget and a \$14 billion endowment) has for decades sponsored a wide variety of research programs. The Bill & Melinda Gates Foundation has likewise pursued broad agendas, with support for research on curing and preventing malaria, tuberculosis, and HIV.

Other foundations have instead chosen to concentrate on one specific disease (or family of diseases). From 1987 to 1996, for example, the primary project of the Aaron Diamond Foundation was the Aaron Diamond AIDS Research Center, which made a series of fundamental discoveries about HIV, including helping to develop the multi-drug “cocktail” therapy that has proven enormously beneficial.

The Los Angeles-based Eli and Edythe Broad Foundation has adopted both the wide and narrow approaches to research. The Broad Institute, a world-class interdisciplinary research shop established in 2004 in cooperation with Harvard and the Massachusetts Institute of Technology, was launched with an initial gift of \$100 million from the Broads; since then, they have given \$500 million more to the institute, which seeks clinical applications for discoveries in genomics. The foundation has also funded three stem-cell research centers in California universities.

But the Broads have also taken a narrower approach, prompted by a relative’s experience with Crohn’s disease. The foundation has since 2001 operated the Broad Medical Research Program, which offers grants to researchers studying Crohn’s and ulcerative colitis. Karen Denne, a spokeswoman for the Broad Foundation, says the program acts as a sort of “farm team for the



With events like the Race for the Cure, Susan G. Komen for the Cure has invested more than \$1.5 billion in breast cancer research since 1982. (Photo courtesy of Susan G. Komen for the Cure)

NIH,” supporting “very early research that wouldn’t qualify for NIH funding.”

Some of the most exciting developments in medical philanthropy are just now taking shape, including a new initiative that NIH is launching to find treatments for rare diseases. There are more than 6,800 rare diseases, defined as diseases affecting fewer than 200,000 Americans. These range from the well known, like cystic fibrosis, to the obscure, like progeria and Niemann-Pick disease. Although NIH estimates that rare diseases cumulatively affect more than 25 million Americans, each separate disease is so uncommon that drug companies have little incentive to try to cure it; as of today, only about 200 of these rare diseases have effective pharmacological treatments.

At The Philanthropy Roundtable’s 2008 Annual Meeting in Naples, Florida, acclaimed geneticist Francis Collins spoke of the need for partnerships between the government, private industry, and the philanthropic sector to tackle these diseases. Such partnerships may soon be facilitated by a program called Therapeutics for Rare and Neglected Diseases (TRND, pronounced “trend”) that NIH announced in May 2009. Collins—who has since been appointed Director of NIH—explained in an email to *Philanthropy* that

the discovery of the genetic basis of thousands of rare diseases, and the determination of the genome sequence of many pathogens of the developing world, provides a new and exciting list of potential drug targets. But the limited market potential means that these translational opportunities may lie unexplored unless the academic sector primes the front end of the pipeline, and develops an effective strategy to “de-risk” these projects for downstream private sector investment. NIH is prepared to do just that, with programs like the Roadmap Molecular Libraries initiative, and the new TRND program.

Work on TRND is still in its earliest stages, explains Stephen C. Groft, the director of the NIH Office of Rare Diseases Research: assembling a team, setting up a facility for lab work, and so on. Once it is up and running, TRND will look to collaborate with private philanthropy. “The opportunities are there,” Groft says, for disease-oriented foundations to help shepherd the process along.

Melissa Ashlock, a senior consultant on TRND who has worked on drug discovery at the Cystic Fibrosis Foundation for the past



At The Philanthropy Roundtable’s 2008 Annual Meeting, acclaimed geneticist (and now Director of the National Institutes of Health) Francis Collins spoke about the need for partnerships between the government, private industry, and the philanthropic sector to tackle rare diseases.

decade, says that TRND, with its relatively small \$24 million budget, isn’t going to eliminate the vast expense of developing new drugs. “It’s not that we’re absorbing a large amount of the financial risk,” she says. But by assuming even a little of the early cost, TRND can help get the ball rolling, moving potential treatments “to the point where someone else can” study and develop them. By taking “a ‘systems’ approach to these diseases,” she adds, TRND will hopefully arrive at pathways and best practices that can be used across multiple diseases.

Meanwhile, donors considering moving into medical philanthropy for the first time—or wishing to make existing medical philanthropy projects more effective—will find a promising new resource in the Health Research Alliance (HRA). Formally incorporated in 2005, HRA germinated from discussions among several private research funders in the late 1990s. Its member roster includes both endowed foundations (such as the Burroughs Wellcome Fund) and voluntary health agencies that raise money from the public (such as the American Heart Association).

Most of HRA’s member organizations focus on specific diseases (for example, the American Cancer Society, the Alzheimer’s Association, and the Muscular Dystrophy Association); some fund a breadth of health and medical research (such as the Howard Hughes Medical Institute and the Donaghue Foundation); and a few fund several projects beyond medicine (e.g., the Doris Duke Charitable Foundation and the Avon Foundation).

HRA now has 40 member organizations, a figure likely to keep rising. (HRA’s counterpart in the United Kingdom, the two-decade-old Association

of Medical Research Charities, has well over 100 members. Then again, in Britain, philanthropy actually funds a greater share of medical research than government.) In total, HRA's members invest more than \$1.5 billion in research each year, supporting over 5,500 scientists at every stage of the research process, from "blue-sky" studies to immediate searches for cures.

HRA was animated by a concern that medical foundations "haven't historically been collaborative," says Nancy Sung, the founding board chair of HRA and a senior program officer at Burroughs Wellcome, which has helped incubate HRA by providing office space and some staffing. A core aim of HRA has been "convening and providing a venue for communication among these foundations."

Biomedical research funders that want to participate in HRA, Sung says, must be entirely private, receiving no government funding at all,

and must be completely committed to a transparent peer-review structure; that is, they must be willing to publish details about the researchers that are receiving grants and how they come by them. "Those of us involved in HRA," Sung says, "want our processes to be as transparent as possible so that those who are applying [for grants] know that the playing field is level."

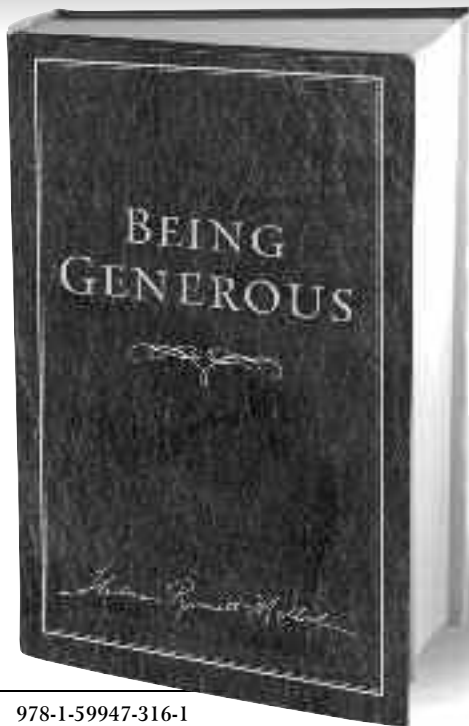
HRA's members do not have identical grant-review processes—"a one-size-fits-all approach is not consistent with the foundation community in this country," Sung says—but those processes must be published, for the benefit of grantees, and also so that other donors can learn from the example. To make the learning process even easier for researchers and other donors, HRA is developing "Grants in the HRA Shared Portfolio," an unprecedented unified database of all its members' grant awards.

"Many people have the impulse to support medical research," Sung says, "but deciding what kind of research is really useful, valuable, and important is not simple or trivial." By joining HRA, donors can learn from the examples of the group's members—their successes and challenges, and their varied funding models. "The sort of people who work in this field are collaborative and generous people," she says.

Sung characterizes HRA's intent with a word from the vocabulary of science: catalytic—"something that's small in size but sets things in motion," with end results greater than might not intuitively have seemed possible.

"Catalytic." It aptly describes the broader challenge of the new medical philanthropy as well: to use its relatively small resources to shape a vast research enterprise that holds the promise of saving and improving millions of lives around the world. **P**

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