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FasterCures: Removing Barriers to Treatments

In mid-2013, as FasterCures celebrated its 10th anniversary as a center of the Milken Institute, Executive Director Margaret Anderson thought about an important question. What should the organization do to ensure it had even more impact in its next 10 years? FasterCures was a non-profit “action tank” whose mission was to speed up the process of moving new therapies from discovery to patients in need. Its predecessor organization, known initially as CaP CURE, had been founded by Michael Milken in 1993 as part of his efforts to accelerate medical solutions.

Over its first decade since it became FasterCures in 2003, Anderson believed that FasterCures had achieved many successes. Early on, it had launched a program to enable individual patients to play a greater role in medical research and it created a web portal to promote the role of tissue banks in research. In 2007, with guidance from Nobel Prize laureate David Baltimore, who was on the FasterCures board, it formed a task force to improve how government research was organized. Over the past four years, it held an annual conference, “Partnering for Cures,” which attracted over 1,000 people and became the “must attend” event for top leaders from all parts of the medical research community. With these and other initiatives, FasterCures had developed a strong reputation for being a catalyst for improving the medical research system, for injecting a sense of urgency into solving problems, and for its ability to get things done.

Anderson, who was widely credited for her stewardship of the organization, had initiated a strategic review of FasterCures and was preparing to present her vision of the future at an upcoming board meeting. Anderson commented:

We could stop now and be happy just doing what we are doing, but I have seen other non-profit groups follow that route and get stale. Partnering for Cures is a huge success, but maybe it’s time we take on greater challenges. For example, investors are increasingly supporting late stage research with higher probabilities of success while funding for riskier early stage research is drying up. Should FasterCures raise money and direct it toward early stage breakthrough therapies? Another issue is that many medical researchers and drug developers are greatly frustrated by the whole clinical trial process. It’s complicated, takes a long time, and is very expensive. Should we organize ourselves to better understand this issue and make a high-level push to improve it? Or perhaps we should look at drug reimbursement decisions.

Professor Richard G. Hamermesh and James Weber, Senior Case Researcher, Case Research & Writing Group, prepared this case. It was reviewed and approved before publication by a company designate. Funding for the development of this case was provided by Harvard Business School, and not by the company. HBS cases are developed solely as the basis for class discussion. Cases are not intended to serve as endorsements, sources of primary data, or illustrations of effective or ineffective management.

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We need to figure out how to make the reimbursement system more efficient and get new, more innovative treatments incorporated into it.

FasterCures is already making a difference, and now we want to leverage our reputation and take on more prominent issues.

Michael Milken and the Precursors of FasterCures

Michael Milken made a name for himself on Wall Street, in philanthropic endeavors, and in the advancement of medicine well before he founded FasterCures. Before earning an MBA from Wharton in 1970, Milken had joined an investment bank. Over time, he developed the use of high-yield bonds and other types of securities to help finance a long list of successful companies and entrepreneurs who had difficulty obtaining financing from traditional sources. In the late 1980s, he became the highest paid executive in the U.S., earning in excess of \$500 million in 1987 alone.¹ In 1989, however, Milken's banking career ended when he pled guilty to six counts of securities law violations, was barred from the securities industry, served 22 months in jail, and paid in excess of \$1 billion in fines and settlements.²

Milken's philanthropic activities began when, as a child in the 1950s, he raised money for the Community Chest (now called the United Way). In the 1970s his mother-in-law's battle with breast cancer and later his father's melanoma (skin cancer) further drew his interest to support medical research. In 1982, to formalize his efforts, he co-founded the Milken Family Foundation to support medical and educational causes.³

Just days after being released from prison in 1993, Milken's support of medical research took a more personal turn when he was diagnosed with prostate cancer and given 18 months to live. Despite his bleak prospects, Milken chose an aggressive medical treatment regimen and added holistic and dietary changes. Soon his cancer went into remission.

Milken was familiar with other forms of cancer because of his philanthropy in the 1970s and 1980s, but his knowledge of prostate cancer was limited, so he set out to learn everything he could about the disease and research in the field. He discovered that prostate cancer was a "medical backwater."⁴ Despite being one of the leading causes of cancer deaths, scientists were conducting very little research into its causes or treatments. The government was providing very little in terms of research grants and with little funding, new Ph.D. graduates chose other areas in which to begin their careers. Additionally, the disease had received little media attention compared with some other forms of cancer.⁵

Milken believed that part of the challenge rested in the way research grants were awarded. The National Cancer Institute (NCI), part of the National Institutes of Health (NIH) which was the world's largest provider of medical research grants, had created a complicated and time-consuming application process. Researchers were often required to spend more than a year gathering enough data to complete an application which could be hundreds of pages in length. The entire process, including NIH review of the application, might take three years from start to finish. During this time, other medical discoveries might render the grant proposal obsolete.⁶

To help spur research, Milken founded the Prostate Cancer Foundation (PCF) in 1993. The PCF was originally called CaP CURE, a name that reflected three interrelated goals. "Ca" was a shorthand notation used to designate cancer, and represented the belief that progress against any form of cancer often led to treatments for other forms of the disease. "CaP" indicated cancer of the prostate. And

“CURE” reflected the organization’s goal of major progress against all diseases. The three components would later become three distinct entities. The “Ca” became C-Change: Collaborating to Conquer Cancer, an organization led by former U.S. President George H. W. Bush and wife Barbara to advance a national dialogue on cancer. The “P” became the PCF. And the “CURE” became the basis of FasterCures.

In 1995, Milken and the PCF hosted the first Cancer Summit in Washington, which convened 250 leaders of the cancer movement. At the Summit, Milken called for a “rethinking” of the War on Cancer, from a “war of attrition” to a “plan of attack.” He outlined a ten-point plan:⁷

1. Internationalize the war on cancer;
2. Invest more;
3. Recruit a world-class scientific team;
4. Coordinate worldwide cancer resources;
5. Accelerate technology transfers;
6. Push the technological envelope;
7. Create a “world library of organic chemicals”;
8. Accelerate approval of new drugs;
9. Get product to market faster; and
10. Mobilize patients and families.

The Summit led to a 1998 March on Washington in support of increased funding of biomedical research. Over the five years following the March, Congress increased the resources of the National Institutes of Health from \$13.7 billion per year to \$27.2 billion.⁸

The PCF reflected Milken’s business mindset in contrast with the more academic mindset of many scientists: researchers were expected to pursue measurable, time-specific targets that could lead to treatments rather than to focus on publishing papers in basic science and earning tenure. For example, the PCF developed a simplified and faster grant application process. Applications were five pages long and individuals selected for a grant received a funding check within 90 days of submitting. Grant recipients were required to share their findings at annual PCF scientific retreats with other scientists within one year. This sharing conflicted with traditional research norms where scientists tended to keep their research private until published in a journal. The PCF encouraged applicants to pursue their “dream” research rather than research that was “safe” and likely to earn grants from traditional sources. It also sought to award grants to early stage ideas that then enabled researchers with promising results to obtain larger grants for clinical trials from other sources.⁹

Beyond grant funding, the PCF also raised awareness of prostate cancer, pressured Congress to provide funding, and brought together powerful people in government, finance, and medicine to increase the urgency around achieving results. The PCF removed barriers to progress where it found them. For example, it initiated an effort of the leading cancer research centers to standardize, and make more efficient, the protocol for handling human tissue samples—something Milken could not believe had not been done previously.¹⁰

The efforts of the PCF earned Milken high praise. In 2004, Fortune magazine credited Milken with “speed[ing] up science” and called him “The Man Who Changed Medicine.” A foremost prostate cancer surgeon stated, “Mike’s done more for prostate cancer research than anyone in America.” The PCF also got results. Between 1993 and 2004, the per-capita death rate from prostate cancer fell 24%—despite an aging population more susceptible to prostate cancer—four times the decline of the overall cancer death rate.¹¹ Milken continued to chair the PCF in 2013, by which time it had become the

leading philanthropic organization for prostate cancer.¹² Between 2010 and 2012, the FDA approved six new prostate cancer drugs that had been supported by PCF research.¹³

FasterCures

Milken founded FasterCures to apply what had been learned from his cancer work to all life-threatening diseases. Milken stated, “We want to look at the whole system and ask ourselves what policies could be improved in order to accelerate cures.”¹⁴ FasterCures was established in Washington, DC to be close to centers of political power and leading medical research organizations such as the NIH. (The Milken Institute, FasterCures’ parent organization, was located in Santa Monica, California.)^a Milken committed \$1 million in initial funding to launch FasterCures and hired Greg Simon to lead the organization.

Simon, a lawyer by training, had previously served as Vice President Al Gore’s chief domestic policy advisor, had held senior positions in the U.S. Senate and House of Representatives, and had served as a strategy consultant and lobbyist in the technology sector. Simon envisioned modeling FasterCures after a successful think tank whose “influence didn’t come from lobbying, but rather because their ideas had power.”¹⁵ Simon added that part of FasterCures’ mission was to get proponents of medical research working together to increase funding levels and to ensure that funding was “spent wisely.”¹⁶ In a 2003 press release he explained:

[FasterCures] is an independent, nonpartisan organization that will examine the entire medical research process. It will seek ways to shorten the path to cures and improved treatment outcomes for the most deadly and debilitating diseases.... It will mobilize economists, medical researchers, clinicians, biologists, ethicists, genomics specialists, chemists, physicists, mathematicians, computer scientists, patient advocates, legislative analysts and others to evaluate the entire research process, publish concrete policy recommendations, and provide leadership for their implementation.... [It] will point to new efficiencies that can accelerate scientific discovery [and] propose changes in misplaced priorities, sometimes inefficient regulations, and conflicting incentives that slow progress on finding treatments and cures.¹⁷

How to deliver its mission was less clear. Kathi Hanna, a consultant to FasterCures’ founding team and later a FasterCures Fellow, explained that FasterCures was not designed to conduct or fund research or focus on any one specific disease. Hanna recalled, “There are disease-specific organizations that are very busy doing their own thing and thinking about their own constituencies. FasterCures does not have a constituency or a disease. This gives us the luxury of looking across all of these groups and asking what problems are they all having. We then figure out how we can help.” (In recent years, non-profits had increasingly become involved in advancing medical research. The organizations tended to be philanthropic foundations or charities, patient-driven, and focused on developing treatments for a single disease. See **Appendix** for details on a few of these organizations.)

Early Years

In its early years, while the FasterCures team spent time thinking about what the organization should do, it also began to develop a network of individuals in the field. Hanna recalled:

^a The Milken Institute was a 501(c)(3) public charity. In 2011, it had total revenues of \$30.2 million, total expenses of \$21.1 million, and total assets of \$55.8 million. Source: The Milken Institute IRS Form 990, via GuideStar, accessed October 2013.

Networking helped us develop as an organization. We spent a lot of time talking to a lot of different people to identify what the issues were and what people cared about. We did some work on electronic medical records and found that getting a foot in on one issue allowed the organization to start making connections. We realized that we could both push on an issue and leverage and increase the size of the network and our visibility. This would enable us to be more effective on other issues. So while electronic records seem mundane, at the time, that's what everyone was talking about.

In 2004, FasterCures launched "Patients Helping Doctors" (PHD) as its first initiative. PHD educated the patient community about what individual patients could do to help in the research process. Examples where patients could help included participating in clinical trials, and making their tissue samples and medical records available for study by researchers. FasterCures pointed to studies that showed that most clinical trials faced delays because of difficulties related to recruiting patient volunteers. For its part, FasterCures planned to coordinate with and support existing organizations working to increase patient education and involvement in research. FasterCures announced the PHD initiative at a conference sponsored by the Milken Institute at which the U.S. Secretary of Health and Human Services spoke in support of the initiative.¹⁸

In 2005, FasterCures, in partnership with several medical technology partners, announced that it would create a web portal, called "BioBank Central," that would provide a central location for information and collaboration among all the constituencies of bio banks (human tissue banks). The constituencies included the bio banks, researchers, lab equipment manufacturers, medical ethicists, government agencies, and patients. The goal of BioBank Central was to speed up cures by disseminating information, lowering barriers to obtaining information, and education.¹⁹

In 2006, the organization sponsored a conference in partnership with the Agency for Healthcare Research Quality and the NIH's National Center for Research Resources to examine the use of electronic medical records for research purposes. Following the conference, FasterCures published a report that made several recommendations including that researchers be granted confidential access to electronic medical records, that such records be used to monitor the effects on patients of drugs recently approved by the U.S. Food and Drug Administration (FDA), and that researchers be allowed to use their access to electronic medical records to identify potential research subjects. Simon stated that these electronic records should be "part of a common database across the country that we can all mine for information."²⁰

FasterCures also formed a Philanthropy Advisory Service (PAS) to help philanthropists looking to support medical research causes determine where to invest to achieve the greatest return on their philanthropy. Melissa Stevens, FasterCures' deputy executive director, explained, "The original intent was to look across all diseases and identify the greatest unmet needs, then profile organizations working in those areas. We have since targeted our efforts in this area, and have begun working with individual philanthropists to identify high-impact research opportunities in specific disease areas." FasterCures accepted donations from philanthropists for customized advisory services. It also developed a set of tools and metrics, called its Giving Smarter Framework, which other donors could use to make more informed and strategic investment decisions. FasterCures received initial financial support for PAS from both the Robert Wood Johnson Foundation and the Bill and Melinda Gates Foundation.

In 2007, FasterCures received a major boost when philanthropist Sumner Redstone announced he would donate \$35 million, spread over five years, to advance the organization's work. FasterCures

used these funds and other donations to expand its existing programs and begin to add new programs. It also increased staffing levels to 16 individuals over a several year period.

In looking at what FasterCures had accomplished, Anderson pointed to three other achievements of which the organization was quite proud: The Research Acceleration and Innovation Network, The Task Force on NIH's Intramural Research Program, and the Partnering for Cures annual conference.

The Research Acceleration and Innovation Network (TRAIN)

FasterCures launched TRAIN in 2004 and grew the program significantly after the Redstone donation. TRAIN was a group of nearly 60 non-profit disease-focused organizations brought together by FasterCures to improve the research environment and solve common problems. It had two fundamental purposes: share best practices and connect these organizations to other stakeholders in the system. To achieve this, FasterCures held conferences and maintained a TRAIN website where members (and the public) could view webinars, participate in discussion groups, learn about other TRAIN members, and find other information relevant to the non-profit medical research community.

Sharing best practices FasterCures believed that certain best practices set apart the most impactful organizations. In general, this meant taking an entrepreneurial, mission-driven, strategic funding approach. For example, the Cystic Fibrosis Foundation, a participating TRAIN organization, had pioneered the approach of making early-stage capital investments into biotechnology companies working on cystic fibrosis. An increasing number of non-profits were following this approach or thinking about doing so, but it was not easy. TRAIN provided a safe forum for these organizations to discuss this approach. Kristin Schneeman, FasterCure's program director for TRAIN, explained:

The organizations that are attracted to TRAIN cover a range of diseases from the common to the rare. They all developed in their own ways and have taken many different approaches, but their goals are the same, to improve treatments or find cures for their disease. They come to TRAIN around common interests that are distinct from their disease interest. They don't have a lot of bandwidth to devote to thinking about their strategies, their processes, or things that aren't specific to their disease state. TRAIN provides them a venue for doing that.

Connecting organizations FasterCures also used TRAIN to connect the disease specific non-profits with other stakeholders such as pharmaceutical and biotechnology companies, academic leaders and institutions, government policy makers (NIH, FDA, and the Centers for Medicare & Medicaid Services (CMS)), and healthcare payer organizations. Schneeman stated, "We have come to be acknowledged as an honest broker for these other parties that are interested in interacting with this broad range of foundations. They value our mediating role. They know that the groups participating in TRAIN tend to be very action-oriented."

In addition to sharing best practices and connecting organizations, TRAIN also helped inform FasterCures. Anderson explained, "We recently set up an advisory council of TRAIN members that provides us with a feedback loop. Members can tell us what they are worrying about and what they think we should be working on. We feed off them very much in terms of what they are seeing."

The Task Force on NIH's Intramural Research Program

In 2007, FasterCures decided to examine how the NIH's intramural program operated. The NIH was part of the U.S. Department of Health and Human Services and was the lead agency for medical research with an annual budget of \$28 billion. The NIH was divided into two main segments: extramural and intramural. Extramural, which accounted for 80% of the budget, awarded grants to

outside institutions (universities, hospitals, and private labs, for example) to conduct research. Intramural, with 10% of the budget, operated government-owned labs, employed scientists, and conducted research using NIH funds. Intramural also operated the NIH Clinical Center, the world's largest, and perhaps most sophisticated, clinical research hospital.²¹

Anderson described FasterCures' involvement:

At that time, there was widespread agreement in the research community that intramural's \$3 billion budget was not being deployed as effectively as it could be. Greg [Simon] decided to create a task force to think about how that could be improved and David [Baltimore] agreed to lead the effort. We are a non-partisan organization so we saw our work as a set of recommendations to the incoming [U.S.] president, whoever that person might be.

The task force, made up of high-level stakeholders, held a series of discussions and published a report outlining core principles on how NIH could be more effective. The principles were seen by the task force as "quick wins" that could be implemented by the incoming administration. For example, one problem was that the NIH Clinical Center was only operating at about 50% capacity. Utilization could be improved if the intramural program could apply for grants from the extramural program; however, there was a general belief that such comingling of funds across the two programs was not allowed. The task force hired a lawyer to investigate this belief and found that this could happen. Anderson called it an "urban legend" that everyone had long believed was true. Another example was that the intramural program did not have a mission statement. Anderson continued:

We found that intramural could access extramural funds and now they are doing it. We talked about the lack of a mission statement and now they have one. The task force pointed out the inadequacies in their structure and metrics and now they are moving forward on those points. That was an example of something that we put some attention on, and really exerting very little power, we were able to make changes.

It was an interesting use of "power." Just by convening a group of individuals, and saying what we were looking at, created change. I think that's the secret sauce of FasterCures. It is this little engine that could—small not-for-profit, headed by Mike Milken, with Gary Becker and David Baltimore (both Nobel Prize winners) on the board, and good working relationships with bioscience leaders such as Peggy Hamburg (Commissioner of the U.S. FDA), Francis Collins (Director of the NIH), Chris Viehbacher (CEO of Sanofi pharmaceutical), Sue Desmond-Hellman (Chancellor of the University of California, San Francisco (UCSF)), and others with a strong interest in our mission. Suddenly everybody started asking; "Who is this group, and what are they going to do next?" (See **Exhibit 1** for FasterCures board.)

Hanna added:

The task force got the recognition of the federal government, the research community, and other patient groups looking at specific diseases. I think they saw for the first time how FasterCures can get something done.

Partnering for Cures Conference

In 2009, building on its experience of bringing leaders in the research field together, FasterCures held the first of what became its annual Partnering for Cures conference. The conference, held in New York, drew 1,000 attendees each year. These included representatives from disease groups (patient groups), policy makers, philanthropists, big pharmaceuticals, biotechnology companies, academics,

investors, government agencies, and others. The conference was sponsored by funding from foundations and pharmaceutical and biotechnology companies.

The conference included 30 presentations that highlighted novel cross-sector collaborations—for example an academic institution working with an investor to develop a new public/private partnership. These presentations emphasized the collaboration and the impact on patients and not the market potential from an investor point of view. Potential presenters had to submit an application that was reviewed by a committee to ensure there was a true collaboration and one that offered something not seen before. The conference also had education sessions and discussion panels. Another key aspect was a customized partnering matching system that helped bring together interested parties in beneficial ways. Anderson explained, “We seemed to have developed a good reputation and track record of getting the right people in the room and then really lasering in on what needs to happen.”

Susanna Ling, associate director of development, added,

The Partnering for Cures event is our flagship conference and allows us to showcase the amazing work of all these patient groups, the venture philanthropists, and other events that have transpired throughout the year. It’s unlike any other industry conference. The focus is on innovation and how attendees get through bottlenecks in the process. People have been talking about the many problems that exist in medical R&D for a long time, but we’re focused on trying to find solutions.

Margaret Anderson

Anderson, who joined FasterCures in 2004 as chief operating officer, played a lead role in developing and implementing many of the organization’s initiatives. In 2009, she was named executive director when Simon left to take a position in a large pharmaceutical company.

Anderson explained that she had long felt a connection to work in healthcare. “Growing up, I had several family members in healthcare. My mother was a head operating room nurse so that was what I was exposed to as a child.” Anderson found she was more interested in the policy side of healthcare, and the impact of science on people and society, than she was in being a practicing scientist or medical professional. After earning an undergraduate degree in political science and a master’s degree in science, technology, and public policy, Anderson started her career at the Biological Applications Program of the Congressional Office of Technology Assessment (OTA). The OTA advised congress and developed policy options relating to important scientific issues. Anderson recalled, “When I was at the OTA, the biotechnology industry was emerging and questions such as the use of DNA in criminal trials and the creation of DNA databases were at the fore. It was an exciting time and I found I had a passion for science.” Anderson later worked in several health-related non-profit organizations: the American Public Health Association, the Society for Women’s Health Research, and finally the Academy for Educational Development. In these positions she sometimes worked with patient groups and advocates. She continued, “I worked in the area of HIV prevention in the early days of the HIV/AIDS epidemic and I saw how passionate and committed many of these patient groups were. Some of that rubbed off on me and I developed a strong belief that patients mattered and needed to be at the table when addressing healthcare issues.”

Under Anderson’s leadership, FasterCures continued to demonstrate its ability to convene key players at the highest levels of industry, government and academia. Two examples stood out. First, the 2011 Lake Tahoe Retreat, titled “Accelerating Innovation in the Bioscience Century.” FasterCures

helped convene what one participant described as “the board of directors of the bioscience ecosystem,” including the NIH director, FDA commissioner, the majority leader from the U.S. House of Representatives, CEOs of international pharmaceutical companies, and heads of major medical research institutions.²² One key recommendation from that event was establishment of the National Center for Advancing Translational Sciences (NCATS). With information and encouragement from FasterCures, Congress created NCATS within months and President Obama signed it into law early in 2012. The second example came from September 2012 when FasterCures hosted the three-day “Celebration of Science” conference in Washington, DC, which convened 1,200 of the most senior people in government, academia and industry. Weeks later, FasterCures brought together hundreds more in London and Singapore. The participants represented a broad range of perspectives but shared the common belief that bioscience should be high on the agenda.

Thinking about FasterCures’ 10th anniversary, Anderson asked herself, “What next?” FasterCures had developed and continued to operate several important and successful programs that had made a difference in advancing medical progress. And it had done so with a small staff, a modest budget of a few million dollars a year, and perhaps their secret weapon—Milken, who had critical analytical skills and who knew nearly all the important players in the field. When he called on them, they responded. Anderson needed to decide how much more to take on and was thinking about three new areas of focus: clinical trials, drug reimbursement, and early stage funding. Each area represented key parts of the system that brought new treatments to patients and improvements in them could bring those treatments faster or at lower cost.

Clinical Trials

Before being approved for use, manufacturers tested potential new medical devices and drugs for safety and efficacy through a series of clinical trials involving human subjects. Clinical trials were a major expense in both time (they could take a decade) and money (costs could approach \$1 billion). Designing and managing trials and then extracting useful data was highly complex and FasterCures believed that any improvements to the clinical trials system could provide significant benefits to patients. Anderson commented, “This is a hard issue to get your arms around, but we need to find practical actions that can make things better, things that are so logical that there is little reason not to do them.”

In early 2013, Anderson hired a medical researcher to help the organization better understand the clinical trials issue and potentially identify ways FasterCures might get involved. So far, FasterCures had identified several areas of interest—two were clinical trials data transparency and patient recruitment for clinical trials—where practical steps could be taken to improve the process.

Transparency The FDA, and its European counterpart, the European Medicines Agency (EMA), required extensive data collection relating to clinical trials. Historically, much of this data, particularly data for trials involving drugs that did not receive approval, remained unavailable to the public. There was an ongoing effort by the EMA and the European Parliament to make clinical trial data more readily accessible to outside researchers, and since medical products were developed with an international market in mind, the FDA was paying close attention to its European counterpart. There were many potential benefits to data transparency and some groups were in favor of increased transparency. From failed trials, other researchers could learn what went wrong and in the process, gain a better understanding about human biology and disease. In some cases, it might be possible to use data from placebo groups from failed trials to make future trials more efficient. For approved drugs, physicians could have more confidence in the drugs they prescribed if they knew the trials data had been examined by researchers beyond just those at the pharmaceutical company that

developed the drug. Some pharmaceutical companies had indicated a willingness to share some trial data with outside researchers and explore sharing data with competitors. While these companies often considered the details of the molecule under study to be competitive information, sharing clinical trial methods and processes, as well as placebo/control arm data could help make all trials more efficient.

Some researchers and patient advocates were opposed to transparency or at least concerned about certain aspects of it. One issue was privacy. What type of data would be made public and would it be possible to use that data to identify individual patients? Regulators also had concerns. For example, some regulators did not want their decisions to approve or reject a drug to be second guessed. There was also the issue of informed consent—clinical trial patients acknowledged that they understood how their data would be used within the objectives of the study, but what happened when the future use of their data was unknown or to-be-determined?

Recruitment The second practical area was recruitment. It was very difficult for drug researchers to identify and recruit patients to participate in clinical trials. This raised costs and delayed the start of many trials which slowed down research. Anderson stated, “There is great frustration in many quarters, including among the TRAIN groups, around this issue. In the cancer space, less than 5% of eligible participants for trials actually participate and it is worse in other diseases. If you could increase that to 25%, how much faster would trials get done?” Other organizations had made attempts at addressing the issue. Some venture philanthropy organizations had tried pushing people towards trials for their own diseases. There was also interest in the healthcare field about using electronic health records to match potential trial participants with relevant trials and then alert the potential participants’ physicians and have the physicians reach out to their own patients. Anderson continued, “People want to serve, and certainly people with diseases want to serve, but so far they don’t really know how.” She continued:

For FasterCures to be helpful, we would have to find ways to make the process more efficient without getting in the way of the doctor-patient relationship or being self-serving for the non-profit disease groups, but while ensuring the informed consent of trial participants. On the other hand, for people with a disease and a potential new drug, it is a pity that so much time and money is wasted getting people to join the trial.

Drug Reimbursement

The second area for potential focus for FasterCures was drug reimbursement. Once the FDA approved a drug, the pharmaceutical company decided how to price it and drug payers decided whether and at what price they would reimburse the drug. The dynamics of these decisions however were shifting. In past decades, many drugs treated common diseases. These drugs were expensive to develop, but the development costs could be spread across large patient groups. For example, the cholesterol-lowering drug Lipitor was taken daily by millions of patients. A month’s supply had a wholesale cost \$150 per patient and the drug generated annual sales over \$11 billion.²³

In recent years, as researchers learned more about disease mechanisms, pharmaceutical companies focused less on finding drugs that might offer marginal improvements over existing drugs for large groups, and focused more on developing drugs that gave hope to patients with less common diseases that had few treatment options.²⁴ This led to a growing trend where new drugs were used in smaller groups of patients, but development costs remained the same. With fewer patients to share the costs, pharmaceutical companies needed to charge higher prices to recoup development costs and earn an attractive return on investment. In 2012, 11 of the 12 cancer drugs

introduced in the U.S. carried prices greater than \$100,000 per year of treatment.²⁵ One result of the high prices has been increased unevenness in payer reimbursement decisions. For each drug, different payers might reimburse at different rates to different patient groups or might decide to not reimburse at all.

FasterCures had begun taking steps to explore this area. In 2013 it co-hosted a workshop on reimbursement with the Cystic Fibrosis Foundation that brought together TRAIN members with representatives from pharmaceutical companies, government regulators and administrators, and payer organizations. Anderson stated, “People in healthcare, particularly the pharmaceutical companies and the payers, tend to be very reluctant to talk about pricing. What they will talk about is what constitutes value in the payer community. Is a drug making a significant impact and improving lives? What data do we need for decision making?” From this workshop, FasterCures was in the process of producing a formal report, much like it did with the task force on NIH’s intramural research, which Anderson hoped would clarify what progress would look like on the reimbursement issue. Beyond its report, FasterCures was focused on increasing understanding and raising awareness across the key stakeholders, but it needed to determine what role it could play beyond that. Anderson continued:

This is a tremendous problem and it can’t be solved unilaterally by anyone. Our hope is, through reasoned conversation among the relevant constituents, all of whom have legitimate points of view, that a better solution can be achieved. But there are risks. All these venture philanthropy groups that are working to accelerate medical solutions and advances in science are making that possible, but what happens if the payers don’t want to pay for it at the other end? Or worse, what happens if we simply can’t afford it?

Early Stage Funding

The third issue FasterCures was considering dealt with early-stage funding. In recent years, many venture capitalists and pharmaceutical companies had stopped investing in early stage research for both medical devices and drugs. Several factors, including low returns on investment, the low probability that the research would result in an approved device or drug, uncertainty over reimbursement rates, and the time from initial investments to eventual returns, had caused this pullback.

In 2011, FasterCures took beginning steps to explore this issue by convening a group of investors, financial engineers, researchers, and pharmaceutical executives to discuss early stage research funding and look at new emerging financing models that might help address this issue. This meeting was also a Milken Institute Financial Innovations Lab, a multidisciplinary group convened by the Milken Institute to address financing or policy questions. Since that meeting, FasterCures developed a few case studies that profiled some of the new financial techniques. In mid-2013, FasterCures was trying to decide whether to take more concrete action. Deputy Executive Director Stevens explained, “I think we now understand the problem, we have seen some potential solutions, and we are thinking about diving in. We are conceptualizing a new kind of venture fund in which FasterCures would play an advisory and convening role to bring together the funding and the expertise to deploy capital into this space.”

FasterCures’ idea was to help create several investment funds, each dedicated to a specific disease state, that would combine venture philanthropy money with traditional venture capital to invest in high-potential early-stage ventures. To make the funds attractive to venture capitalists, the philanthropy money would both bear a higher portion of early investments and losses and take a lower portion of any upside gains. This would lower the risks and enhance the returns for venture

capital investors. For the philanthropists, the funds would leverage their research investments by combining them with venture capital funding. Because most of the philanthropies focused on only one disease, each fund would similarly focus on one area of research. For example, a fund looking to invest in cystic fibrosis drugs might attract philanthropy money from cystic fibrosis foundations. Because some of the venture philanthropy groups are intimately familiar with translational research efforts, they can also source deal flow for the fund. FasterCures estimated that the funds would need to be in the \$25 million to \$50 million range and that philanthropy money might represent 20% of the fund. The hope was that this investment could move potential drugs far enough to attract traditional funding sources to promising candidates. If the funds proved successful, FasterCures believed the funding model would spread to many diseases.

While some questions remained, to make this idea work FasterCures might act as a promoter and matchmaker. It could find an existing venture capital firm to oversee the efforts for a specific disease and then that firm, with the help of FasterCures, would collect philanthropy money and combine it with its own. The firm would also find the specific investment opportunities and manage the fund.

As Anderson considered the three options—clinical trials, reimbursement, and funding—she needed to determine whether FasterCures should get fully involved, what it should do, and whether it could make a difference. To pursue any one issue, Anderson would have to hire two to four new staff members; to pursue all three meant nearly doubling the size of the 16 person organization. She believed that if she could make a convincing argument to her board in favor of moving ahead on one or more of the issues, increased funding would be available to support new initiatives. Anderson thought, “We have been successful so far and I know we should do more, but a lot of what needs doing is much more expansive in scope, more difficult to solve, and involves more risk for FasterCures.”

Exhibit 1 FasterCures Board of Directors^a

Michael Milken, M.B.A, Chairman

Margaret Anderson, M.A., Executive Director

David Baltimore, Ph.D. Baltimore is a professor of biology at the California Institute of Technology and the 1975 Nobel Prize recipient for work in virology. He was founding director of MIT's Whitehead Institute for Biomedical Research, president of Rockefeller University, and head of the National Institutes of Health AIDS Vaccine Research Committee. David also earned the 1970 Gustave Stern award in virology, the 1971 Eli Lilly and Co. award in microbiology and immunology, the 1999 National Medal of Science, and the 2000 Warren Alpert Foundation Prize. David earned his doctorate at Rockefeller University.

Ernest Bates, M.D. Bates is a board-certified neurosurgeon and founder, chairman, and CEO of the American Shared Hospital Services, a publicly traded healthcare company providing services to hospitals in 22 states. Bates received the Kjakan Award for his contribution to the spirit of entrepreneurial capitalism. He is a board member of the University of Rochester and the University of California, San Francisco School of Nursing, an emeritus trustee of Johns Hopkins University, and a member of The California High-Speed Rail Authority and The Brookings Institution. Bates earned his medical degree at the University of Rochester School of Medicine.

Gary Becker, Ph.D. Becker is a professor of economics and sociology at the University of Chicago and recipient of the 1992 Nobel Prize in Economic Sciences. He is recognized for his expertise in human capital, economics of the family, and economic analysis of crime, discrimination, and population. Becker was at Columbia University and the National Bureau of Economic Research for 12 years. He earned his doctorate from the University of Chicago.

Leon D. Black, M.B.A. In 1990, Black founded Apollo Advisors, L.P. and Lion Advisors, L.P. which managed \$15 billion for institutional investors. He also co-founded Apollo Real Estate Advisors, L.P., which has invested in more than \$5 billion of real estate-related assets. From 1977 to 1990, Black held senior positions at Drexel Burnham Lambert Incorporated including managing director. Black is a director of United Rentals Inc. and Allied Waste Industries, a trustee of Dartmouth College and Mt. Sinai Hospital, and a member of The Council on Foreign Relations and The Partnership for New York City. He graduated from Dartmouth College and Harvard Business School.

Nancy G. Brinker Brinker is regarded as a leader of the global breast cancer movement. In 1982 she founded Susan G. Komen for the Cure, now the world's largest organization committed to action against breast cancer. To date, the organization has invested more than \$1.9 billion in breast cancer research, education, screening, and treatment. In 2009, President Barack Obama awarded Brinker the Presidential Medal of Freedom, the nation's highest civilian honor. She previously served as U.S. chief of protocol and U.S. ambassador to Hungary in the George W. Bush administration. She was named one of TIME magazine's "100 Most Influential People."

Larry Flax, J.D. Flax was co-founder of California Pizza Kitchen (CPK) and served as its co-CEO since 2003 and as co-chairman since 1985. Previously, Flax practiced law as a principal in Flax and Rosenfield, Inc., served as an assistant United States attorney of the Department of Justice and Chief Criminal Division, and was chief of the Civil Rights Division of the United States Attorney's Office in Los Angeles. Flax is a board member for the University of Southern California Law School.

Michael L. Klowden, J.D. Klowden is CEO of the Milken Institute. Previously, he was vice chairman, president, and chief operating officer of Jefferies & Company, a large institutional securities brokerage and investment banking firm, and had been a senior partner at the law firm of Morgan, Lewis & Bockius. Klowden is a trustee of the University of Chicago. He earned his law degree at Harvard Law School.

Shmuel Meitar Meitar is director of the Aurec Group and vice chairman of Aurec Ltd., a leading provider of communications, media, and information services. Meitar has been a director of the Jerusalem Post and Golden Publications, Ltd.

Richard Merkin, M.D. Merkin is CEO and founder of Heritage Provider Network. He pioneered the development of medical networks. Merkin received the Marquis Award for healthcare from the Southern California Foundation for Health, Education, and Research, and he is active on the advisory board for the chairman of the Assembly Subcommittee on Health, and the American College of Physician Executives. He earned his medical degree at the University of Miami School of Medicine.

Allan Schweitzer, M.B.A. Schweitzer is an executive managing director at Beach Point Capital Management. Previously he was chief investment officer at Post Advisory Group, a senior high yield analyst at Trust Company of the West, and an analyst at Putnam Investments. Schweitzer received a bachelor's degree from Washington University at St. Louis and an MBA from the University of Chicago. He currently serves on the board of Bet Tzedek and FasterCures.

Greg Simon, J.D. Simon is CEO of Poliwoog, an online crowd-financing marketplace that matches young companies with sophisticated investors. Previously, he was the founding president of FasterCures, held senior positions in both chambers of Congress and the White House, and served as senior vice president at Pfizer for world wide policy and patient engagement.

Jonathan W. Simons, M.D. Simons is an internationally recognized physician-scientist, oncologist, acclaimed investigator in translational prostate cancer research, and CEO of the Prostate Cancer Foundation. He was a professor at the Emory University School of Medicine and professor at the Georgia Institute of Technology. He is the founding director of the Winship Cancer Institute at Emory University and co-director of the National Cancer Institute Center for Cancer Nanotechnology Excellence at Emory and Georgia Tech. Simons earned his medical degree from The Johns Hopkins University School of Medicine.

David A. Steinberg Steinberg is founder and CEO of CAIVIS and XL Marketing. Previously he founded InPhonic, the largest seller of communications products and services on the Internet and number one on the *Inc.* 500 list of fastest growing companies in 2004. Steinberg also served as chairman and CEO of Sterling Cellular and was named the Greater Washington Ernst & Young Entrepreneur of the Year for communications. He sits on the boards of the Greater Washington Sports Alliance and Washington & Jefferson College, and previously served on the board of the U.S. Chamber of Commerce. Steinberg holds a bachelor of science from Washington & Jefferson College.

Source: Extracted and adapted from company documents.

^a Because FasterCures was a unit of the Milken Institute, its board of directors served as an advisory board.

Appendix

The following non-profit organizations worked to support specific diseases.

Cystic Fibrosis Foundation (CFF) Founded in 1955 by the parents of children with cystic fibrosis (CF), the foundation's mission was "to find a cure for cystic fibrosis and to improve the quality of life for people living with the disease." Robert Beall had headed the organization since 1994. The CFF was the world's leader in the search for a CF cure. It funded more CF research than any other organization and nearly every drug available to treat CF was made possible at least in part by the foundation. The CFF had nearly 30 new drugs in its drug development pipeline, had \$375 million invested in for-profit companies for CF drug development, and had a grassroots network of 250,000 fundraising volunteers.

In 2011, the CFF had total revenues of \$137 million, program services expenditures of \$110 million, total assets of \$216 million, and 501 full-time employees.^a

Juvenile Diabetes Research Foundation (JDRF) The JDRF was founded in 1970 with the mission of curing Type 1 diabetes. As it became clear that a cure was not close at hand, the JDRF shifted its mission to help people manage and prevent the disease. It funded over \$100 million per year in research and worked to stop the progression of the disease in newly diagnosed patients, avoid or reverse complications, and prevent the disease in at-risk groups. The JDRF had also entered into 50 partnerships with biotechnology companies and pharmaceutical companies that were contract-based, milestone-driven, research-focused collaborations. Concerned that regulatory delays would hinder industry commitment to the field, the foundation also worked to improve the regulatory side of the drug and device development process.

In 2011, the JDRF had total revenues of \$205 million, program services expenditures of \$168 million, total assets of \$215 million, and 624 full-time employees.^a

Michael J. Fox Foundation for Parkinson's Research (MJFF) Founded in 2000 by actor Michael J. Fox, the MJFF focused on finding new therapies for clinical development and had funded over \$300 million in Parkinson's research. The MJFF also provided tools and clinical data to facilitate Parkinson's research to academic and industry researchers at low or no cost, and created a collaborations program to bring together researchers and industry contacts to form drug development partnerships. In 2010, the MJFF launched a \$45 million five-year study to identify biomarkers in patients with and without Parkinson's disease.

In 2011, the MJFF had total revenues of \$65 million, program services expenditures of \$60 million, total assets of \$96 million, and 60 full-time employees.^a

Multiple Myeloma Research Foundation (MMRF) Kathy Giusti founded MMRF in 1998 with the mission to "relentlessly pursue innovative means that accelerate the development of next-generation multiple myeloma treatments to extend the lives of patients and lead to a cure." Its strategy to achieve this mission had evolved over the years from a grant funder for research to acting as the lead investigator on two significant data collection projects that would integrate genomic and clinical data and make this data available to the multiple myeloma research community. The MMRF described itself as a virtual organization that relied on strategic partnerships to help it execute its strategy. As of early 2013, the MMRF had raised over \$220 million since its inception, funded 250 researchers, achieved 60% faster opening of clinical trials and 12% faster enrollment of patients into trials, opened 42 phase 1 and phase 2 clinical trials and had seen five drugs earn FDA approval.

In 2011, the MMRF had total revenues of \$26 million, program services expenditures of \$23 million, total assets of \$27 million, and 24 full-time employees.^a

Muscular Dystrophy Association (MDA) The MDA was the largest non-profit organization focused on muscular dystrophy and other neuromuscular diseases. Founded in 1950, the MDA funded medical research for treatments and cures for neuromuscular diseases, supported families impacted by these diseases, ran youth summer camps, provided education for both families and medical professionals, and served as an advocate for the neuromuscular disease community. It also ran public awareness campaigns and was well-known for its annual Labor Day telethon fundraiser, long hosted by comedian Jerry Lewis, which had raised nearly \$2 billion between 1996 and 2013. MDA management believed that nearly every treatment under development in 2013 for neuromuscular diseases had been supported by MDA funding.

In 2011, the MDA had total revenues of \$157 million, program services expenditures of \$176 million, total assets of \$98 million, and 1,500 full-time employees.^a

Source: Unless otherwise noted, extracted and adapted from “Advancing the Role of Non-Profit Organizations in Drug Development,” May 17, 2013, Harvard innovation lab, Harvard Stem Cell Institute, Harvard Business School, and Multiple Myeloma Research Foundation, unpublished conference paper. MDA source: MDA website, <http://mda.org/>, accessed October 2013.

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