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Kathy Giusti and the Multiple Myeloma Research Foundation

This is the model for cancer. It is the model for medicine; of what we have to do.[1]

— Eric Lander, founding director of the Broad Institute

Kathy Giusti (HBS '85) was driving home one evening in January 1996 when she got the call from her doctor.[2] He asked if Giusti and her husband Paul (HBS '85) could come to his office.[3] Sensing bad news, Giusti wanted to know what was wrong.[4] Her doctor relented and told Giusti that she had multiple myeloma (MM), a rare and fatal cancer.[5]

Early the next morning, Giusti went to a bookstore near her Chicago, Illinois home and headed for the medical section.[6] "We were going through these books, and I started thinking 'Oh my God, what just happened to my life,'" she recalled. After her appointment, Giusti's worst fears were confirmed. She could expect to live for just a few more years—three or four at the most.

Up to that moment everything seemed to be going as planned.[7] Since graduating from Harvard Business School (HBS) just over a decade earlier, Giusti had had one career success after another. At the time of her diagnosis, she was the executive in charge of global operations for G.D. Searle & Co.'s (Searle) arthritis drugs division.[8] The Giustis had recently had their first child and just started trying to have a second.[9] Now, all was in flux.

Giusti's mind raced. What should she do for treatments? Should she leave Searle to make the most of the time she had left with her family? Should they move to the East Coast where both her and her husband's family lived? She could not help but think that just a day before her life was perfectly in order. Where did she go from here?

Kathy Giusti

Giusti graduated from the University of Vermont in 1980 with plans to attend medical school but her physician-father dissuaded her.[10] "He thought I was too impatient to cope with medicine's bureaucracy," she recalled.[11] Giusti instead worked for the pharmaceutical firm Merck & Co., left

Professors Richard G. Hamermesh and Joshua D. Margolis and Case Researcher Matthew G. Preble (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.

after three years to attend HBS, and then worked for Gillette, a personal care products company.[12] After marrying and moving to Chicago, she returned to pharmaceuticals with Searle.[13]

In December 1995, Giusti felt unwell and went to her doctor, who did some blood work that led to her diagnosis.[14] The outlook was bleak and the medicine used at the time had for years not made any significant advances.[15] Although some doctors and researchers were investigating MM, it received less funding than other diseases. "You can't be a researcher and go into diseases that have no money because you won't get any grant funding," Giusti noted.[16] Moreover, information flow was limited. "Doctors would say 'Oh, you're going to that center for treatment? Would you ask what they're doing in terms of research?'" remembered Giusti. "It was archaic."

Despite her negative prognosis, it was important to Giusti to have another child.[17] "My whole life changed when I found out I was pregnant [. . .]. I realized that nothing was more important to me than finishing the pregnancy," she recalled.[18] As drugs might risk the health of her unborn child, and because her disease was "inactive,"[a] Giusti decided to delay treatments.[19] Giusti left Searle after having her second child in mid-1997, and moved to Connecticut to be near family.[20]

Multiple Myeloma

MM turned healthy plasma ("the liquid portion of blood") into malignant myeloma cells which created a defective protein.[21] This protein was made in such volumes that it limited production of healthy blood and plasma.[22] These protein cells could move into bone and cause tumors.[23] Quality of life for patients was often poor, as the disease led to a weakening or loss of bone and bone lesions.[24] Patients were at risk of infections, anemia, poor blood clotting, and kidney problems.[25] Drugs were available and some patients received bone-marrow transplants but, as one observer noted, "the cancer cells invariably return, evolving in such a way that they no longer respond to treatment."[26]

The median survival rate was 62 months for patients with Stage I MM, 44 months with Stage II, and 29 months with Stage III.[27] According to projections, approximately 24,050 people would be diagnosed with MM in the U.S. in 2014, and more than 11,090 would die from it.[28] This relatively small number of diagnoses (see **Exhibit 1**) made MM a rare, or "orphan," disease as it claimed fewer than 200,000 patients (see **Exhibit 2** for other examples).[29] Historically, pharmaceutical firms had shied away from these diseases, but in the early 1980s the U.S. government enacted the Orphan Drug Act, which "created financial incentives for the research and production of such orphan drugs."[30] Within roughly two decades, 220 of these drugs were approved.[31]

Launching the Multiple Myeloma Research Foundation

After her diagnosis, Giusti educated herself on MM.[32] She became involved with the International Myeloma Foundation and held a seat on its board.[33] However, she soon left. "I wanted to focus on research and it was focused on advocacy. I wanted to write business plans. The founder asked me to leave, and I remember saying to my husband: 'I just got fired from my volunteer job,'" she recalled.

[a] According to one medical center, "inactive disease does not require immediate treatment. Inactive disease patients do not present with any myeloma-related organ or tissue impairment [. . .]. Symptomatic or active disease requires treatment. Patients with myeloma in Stage II or Stage III disease fall into this category." Trinitas Regional Medical Center, "Multiple Myeloma," http://www.trinitascancercenter.org/multiple_myeloma_diagnosis_and_treatment.htm, accessed January 2014.

Meanwhile, Giusti was approached by friends, family, and co-workers interested in helping, perhaps by donating to a foundation dedicated to the disease, but Giusti had no place to direct them. So, Giusti and her identical twin sister considered starting their own organization. "I was not a risk taker, but when I faced my own mortality, my risk profile changed dramatically," she explained.[34]

To kick off their efforts, the sisters held a fundraiser and invited leading physicians in the field. The event raised roughly $450,000. "This showed that we were serious about getting something done and were capable of significant fundraising," Giusti explained. "The doctors understood that if they let me take care of the business side of the company, it would be a great partnership."

Following that fundraiser success, the sisters launched the Multiple Myeloma Research Foundation (MMRF) in 1998. Giusti's business school classmates helped develop the initial business plan, and she enlisted other HBS alumni to the board of directors. "I joined because I knew Kathy, knew what she had done in her business career, her passion for the cause, and the mission of the company," explained Board Chairman W. Dana LaForge (HBS '85 and a classmate of Giusti). The board met twice annually and had five committees: audit and finance, conflict of interests, human resources, programming (how money was allocated), and resource development (how money was raised). (See **Exhibits 3** and **4**.) Dr. Kenneth Anderson, the physician treating Giusti at Boston's Dana-Farber Cancer Institute, joined both the board of directors and its scientific advisory board, which focused on the MMRF's research activities.[35] The MMRF later formed a technology advisory board to identify drugs and novel approaches used to treat other diseases that might work for MM patients.

Early Model

At first, the MMRF was an all-volunteer organization and Giusti worked out of her home. She wanted the MMRF to focus on research, help stakeholders collaborate and communicate, and ultimately lead to a cure. However it was unclear how the MMRF fit within the research community. "I didn't want our organization to duplicate others' efforts, and was trying to determine how our foundation would be most helpful," Giusti recalled.

Wanting to understand how medical research was organized and funded, she volunteered with the National Institutes of Health (NIH). "The NIH was looking for patient advocates, and I viewed it as a training opportunity," Giusti explained. "My work was noticed by others at the NIH and led to me being appointed to the National Cancer Advisory Board (NCAB), a position I held for six years. All of a sudden I had access to the head of the National Cancer Institute."

Revenue came from donations and fundraising (see **Exhibit 5**). Giusti and her board recognized that the MMRF could not compete for funding and attention with more prevalent cancers (see **Exhibit 6**), nor did they want to. The initial focus was to support vaccine development and new immune therapies (to strengthen the immune system) in order to facilitate the best treatment for patients, which at the time was either a bone-marrow or stem-cell transplant.[36] To support research, the MMRF funded scientists with small awards (of between $45,000 and $75,000). This strategy was supported by the board. "I think that because Kathy didn't have to worry too much about funding and worked on getting the business model right helped us in the long-term," LaForge said.

In the late 1990s, the drug thalidomide, which helped prevent MM cells from developing, emerged as a potential treatment and gave patients new hope.[37] The MMRF played an important part in helping move the drug towards U.S. Food and Drug Administration (FDA) approval, which was important because of the drug's troubled past: it had caused birth defects decades earlier when prescribed to pregnant women as an anti-nausea drug.[38] As a foundation, the MMRF was not bound by the same stringent regulatory and legal requirements as pharmaceutical firms. "When I worked in

the pharmaceutical world, every meeting was based around 'can we say this?' or 'can we say that?' because people worried about what we could legally communicate to patients via our marketing efforts. The MMRF is a foundation; I can talk directly to patients to make them aware of new advances in the field. There is no obstacle to what I can send out in a newsletter," Giusti explained. "The hardest part of getting a drug approved is finding patients and getting them into a trial. We help create a groundswell of knowledgeable patients aware of what is going on in terms of research and clinical trials so that potentially good drugs do not languish for lack of patients in trials."

The MMRF compiled a patient database (see **Exhibit 7**), so that as thalidomide neared clinical trials and approval, Giusti could share this information with patients. She had waited to discuss the drug with any patients until the medical community had investigated the drug and published findings indicating that it may be helpful for MM patients. "I had doctors review my messages, so nothing was sent without careful scientific review. The MMRF became their way to talk with patients," Giusti said. "When we talked about the benefits of the new drug, we also made sure patients understood the potential risks." The drug's eventual approval, under the brand name Thalomid, also shifted the way Giusti thought about how to best treat patients; from a focus on vaccines to one on novel drugs.

The MMRF was ultimately involved in helping four drugs gain approval. An example of the MMRF's role in this process was its work on the drug Velcade. The drug was developed by Julian Adams, a chemist at a small biotechnology company, and in an early clinical trial, a MM patient went into a full remission. Adams asked Giusti if he could present his findings at an MMRF meeting, and the attendees (which included physicians) were so impressed that they used much of the meeting to develop the protocol for how to run the Velcade clinical trials, thereby saving months of discussion between these dispersed persons.

Raising Awareness and Building Connections

The foundation received its first major donation in 2000 when a board member and HBS classmate donated $1 million in shares from his company, which the MMRF immediately sold. "This was more powerful than we could have imagined," LaForge explained, "and helped change who we were. This donation enabled us to build the organization and create the need for additional funding." Giusti was also gaining confidence that the MMRF offered unique value. "When I went calling on major pharmaceutical companies for donations and started getting five-figure sums, I could tell we were starting to have an impact, and said to myself 'We'll figure this out.'"

Equally important was building awareness of this relatively unknown disease. Because of her prior position at Searle, Giusti knew people in the medical and pharmaceutical industries around the world, and her husband also had a large network of professional contacts. Moreover, living in southwestern Connecticut put them in proximity to prominent individuals (in pharmaceuticals, healthcare, and numerous other fields) working in New York City, as well as a number of wealthy neighbors and friends. Giusti and her husband also sent letters to everyone in their HBS class. This network was not just a source of potential donations, but enabled Giusti to connect with people—such as Joseph Hogan, an executive then in charge of General Electric (GE) Healthcare—to speak at fundraisers, help her make new connections, and build awareness. Other early speakers included journalists Ann Curry and Katie Couric, television celebrities from NBC, then owned by GE.

Of greatest importance, though, was the work of her sister Karen to help launch the MMRF, both for her legal expertise—she was an attorney with the media company Time Inc.—and for the connections she had by virtue of her position at Time, as the company contributed its resources and

employee's time to the young organization. "The MMRF wouldn't have gotten off the ground without Karen," Giusti said.

Giusti attributed the MMRF's success in raising awareness and money to the urgency of its mission: "I felt like I was going to die because all the people around me were. I was living day by day, test to test. So every time I worked on something, the urgency had to be there, because if I got a bad test result I was worried it wouldn't get done. Everything had to be done that day."

Building the Multiple Myeloma Research Consortium

To encourage greater collaboration among researchers, Giusti wanted to start a network of the leading hospitals working on MM. There was no natural incentive for an institution to start such a formal partnership on its own, because physicians and researchers understandably wanted to publish their own results or advance the goals of their institutions. This was inefficient for MM, as no single institution had access to enough patient tissue to make meaningful discoveries, nor could any one hospital afford to dedicate such resources to one disease.

In building the network, the MMRF had to identify what it could offer to encourage hospitals to join. "There was no way we were going to change the 'publish or perish' mentality, so this was about layering on another set of incentives," noted Anne Quinn Young, vice president of development and strategic partnerships. One of those incentives turned out to be access to tissue samples.

Giusti had learned the value of tissue samples early on. Whenever she had bone marrow extracted as part of her treatment, Giusti told the doctor to take as much as he or she wanted, and noticed that other researchers would turn up to take samples. "What do pharmaceutical firms and academic centers need to do their job better? Tissue and data," Giusti explained.

The new clinical network was named the Multiple Myeloma Research Consortium (MMRC), and its first major project was to build a repository of pooled tissue samples to which all member institutions donated and had access. Members met frequently as a group to discuss findings, share new ideas, and advance treatments. "What the MMRC brings to the process is scale and acceleration. Kathy had a plan, and she was adamant that no one institution could accomplish their goals for MM alone," explained Dr. William Dalton, CEO of the biotech firm M2Gen and formerly the president and CEO of the Moffitt Cancer Center in Florida. "We all had common goals, but what we didn't have was an individual to unify our efforts. Kathy created a neutral entity where everyone could come together, but she respected the autonomy and individuality of each partner."

The MMRF managed the MMRC's administrative functions and allocated research funding. Centralizing research enabled the MMRF to track progress and establish metrics to measure success. The MMRC launched in 2004 with four founding members: The Dana-Farber Cancer Institute in Massachusetts; the Mayo Clinic in Minnesota; Moffitt Cancer Center in Florida; and Princess Margaret Cancer Centre in Toronto, Canada. Legally, it was a separate entity from the MMRF with its own executives and board of directors (though there was overlap in these two categories with the MMRF). The MMRC ultimately grew to have a 10-person steering committee and 29-person project review committee by 2014 (see **Exhibit 8** for the MMRC's leadership).

The Multiple Myeloma Research Consortium and Clinical Trials

The FDA's drug approval process[b] required multiple clinical studies involving many patients.[39] However, for certain diseases that lacked treatments, the FDA sped up its process: it aimed to complete a "priority review" within six months, while the "standard review" of a typical drug took 10 months.[40] But it was still difficult to find enough patients. A full 50% of all clinical trials never actually began because they enrolled just one or no patients. Here, the MMRC could leverage the MMRF's patient database (providing a ready source of patients for clinical trials), its relationship with key opinion leaders, and its member institutions' ability to conduct clinical trials. Through such assistance, the MMRC was able to facilitate faster launches of early stage clinical trials.

For the MMRC's member institutions, the organization served as a trusted third party, especially given its policy to investigate any drug with the potential to help patients. "We don't care what company or drug wins; we want the patient to win. We want everyone to win," Giusti explained. Through 2013, the MMRC was involved in 46 clinical trials, testing 24 drugs, of which two were ultimately approved. For its part, the MMRF was more than a matchmaker connecting patients with clinical trials; it also worked closely with pharmaceutical companies, biotechnology firms, and the FDA to critique a drug's protocol. One patient explained how he had benefitted from the work of the MMRF and MMRC. Miles Stuchin was diagnosed in 2009 and treated with a standard drug protocol. This worked until 2012, when the drugs in this protocol stopped working for him:

> I was no longer responding and my condition was rapidly deteriorating. It was a terrible situation. My great fortune was that less than three months earlier the FDA had approved a new myeloma drug, Kyprolis. I started taking it in October 2012 when it was first coming to market and it immediately worked for me. Kathy and the MMRC had been very involved with the clinical trials for Kyprolis for a long time and she understood the urgent need of patients to have this drug available right away. Because of Kyprolis's success in trials, the FDA allowed Kyprolis [to move forward] under its accelerated approval program, which brought the drug to market months, or possibly years, earlier than would ordinarily be the case. For me, having the drug available when I needed it was literally the difference between life and death.

Managing the Multiple Myeloma Research Consortium

For the MMRC, member engagement was a priority. "We have to make the work of the MMRC so compelling and meaningful that members are afraid of being left out by not participating," explained Giusti. Many members reported the experience of attending a roundtable, being pleasantly surprised by the quality of participants and discussion, and not wanting to be left out of future gatherings. Leadership also made a point of publicly acknowledging and thanking members, particularly those who were the first to join or contribute to research or initiatives.

The MMRC hosted research roundtables which served several purposes. "Kathy asks us regularly what the next big question is the MMRC should fund. So we end up serving as both a focus group and a steering committee; the MMRC is the engine, providing finance, operations support, and

[b] The FDA had a three-stage approval process. In Phase I, the drug was tested on 20 to 80 healthy individuals to learn how the body processed the drug and find any harmful side effects. If the drug passed, it moved to Phase II to see how the drug worked in potentially hundreds of patients with the disease it targeted. The FDA became involved between phases II and III to see how the drug would be used in studies potentially containing thousands of patients. U.S. Food and Drug Administration, "Drug Approval Process," http://www.fda.gov/downloads/Drugs/ResourcesForYou/Consumers/UCM284393.pdf, accessed December 2013.

project management," explained Dr. A. Keith Stewart, dean for research at the Mayo Clinic in Scottsdale, Arizona. At these roundtables, people also talked about their work and early findings; information they normally would not share. "The research people discuss isn't necessarily at a stage where it's ready for publication, but this is more about getting ideas out there," said LaForge. Attendees also had financial incentives to participate. "They know we have money," Giusti noted. "They're sharing because they want their research to be appealing and all the normal competition that fuels secrecy starts going away." The MMRC also held a roundtable every year with lawyers from member institutions to identify legal barriers standing in the way of research.

Bringing together a group of academic hospitals was challenging. The MMRC had to design agreements around how tissue was contributed and stored, who owned it, and how intellectual property (IP) was handled.[c] From a budgetary standpoint, establishing the MMRC had been a difficult decision as it required multi-million dollar commitments. "We had to determine if we had the capabilities to manage and support this structure," LaForge recalled.

Moreover, despite the successful relationships built through the MMRC, Giusti and her fellow executives had to be constantly vigilant to ensure that members fully participated. An institution might, for example, conduct its own clinical trial without sharing the information. "This is frustrating: we're building this network, helping fund its researchers, referring new patients to them, and they go behind us," Giusti explained. "I have to call them out when this happens. This is hard because as a leader, I don't want to come across as beating up on them." Instead, Giusti deployed a soft touch to maintain accountability. "Kathy holds individuals and institutions accountable. If a project is not progressing as planned, Kathy will sit down with that member and ask about milestones, timelines, etc. She never accuses them of doing something wrong; she just asks questions. It's an effective way of keeping people and institutions accountable," explained Dalton.

Every three months, the MMRC gave each member a scorecard detailing how much and how quickly it brought tissue in, whether it shared any new ideas or insights, and whether it attended meetings. The MMRC compiled and analyzed this data, and shared it with members (in an anonymized form) to show each institution how it performed relative to the others. "I felt like I was a regional sales manager leading a sales force again," Giusti said. Underperforming members were quietly moved out.

The MMRC faced different challenges with pharmaceutical firms. Some firms already had the functions the MMRC offered, while others were skeptical of the organization. But the MMRC/MMRF could provide a valuable service. "They are an independent, objective third party. There is no question about the MMRF's mission. The MMRF provides access to patients and the credibility only it can bring. There's never any doubt [about] who it represents: the patient," explained Dr. N. Anthony Coles, the former president, CEO, and chairman of Onyx Pharmaceuticals, Inc.[41]

For firms that lacked the resources of larger pharmaceutical companies, the MMRC offered another benefit. "Kathy can give small companies with promising drugs access to the MMRC network, help them build relationships, and even help with the specifics of a trial such as contracting. A small company working alone might not get this same level of attention from the academic community," explained Dr. Deborah Dunsire, president and CEO of En Vivo Pharmaceuticals and the former president and CEO of Millennium Pharmaceuticals.

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[c] All tissue was sent to the Mayo Clinic in Scottsdale, Arizona. This location was chosen for its state-of-the-art facilities, capacity to store the tissue, and because it had expressed an interest in housing the tissue bank. The MMRC helped build processes and procedures to enable the Mayo Clinic to operate the tissue bank, and invested in its infrastructure.

Ultimately, Giusti and her organization let their work speak for itself, as she detailed its accomplishments: "We open trials 60% faster than the oncology industry standard. Every drug we've taken in progressed along the pathway to approval. This tells you we're picking the right drugs." The MMRC eventually grew to include 16 hospitals in the U.S., and one in Canada.

Giusti reflected on the MMRC's success: "It's about finding leaders who want to innovate, and once one came on board, the others followed. We just had to find the right person within the organization to go to the hospital's attorneys and say, 'this is important, and it is going to happen.'" Furthermore, researchers and doctors had altruistic and professional motivations. "This is about the joy of working together to cure a fatal disease," said Giusti. "It brings these researchers to prominence, because MM is not the focus at most of these individual hospitals."

Giusti Searches for a President

As the MMRC was getting off the ground, Giusti's disease became active and she needed a stem-cell transplant. Given that Giusti would be out for months recovering from the procedure, she looked to bring in a president to support the MMRF during her absence. A candidate was then found and approved by the board. In January 2006, Giusti received her identical twin sister's stem cells via transplant. The procedure and recovery left her physically weakened and exhausted, and she was unsure when she would have the energy to return. Three months later, though, Giusti felt strong enough to resume work.

She had stayed informed of what was happening at the MMRF during her recovery and was uneasy with its direction, noting that advocacy had started to replace research as the president's priority. Giusti had also heard from people within the organization who were concerned that the president's actions were moving the MMRF away from its founding mission. "When I returned to work full time, we both went through coaching sessions to see if we could create a workable solution to our different approaches. However it became clear that we both had very different ideas for the company," Giusti said. The MMRF's board then did its own interviews and confirmed Giusti's belief that the president was not the right person for the position, and the president was asked to leave.

Growing the Organization

The following year, 2007, was pivotal for the MMRF as Giusti realized she needed to hire an expert in research and build an organization to match. The MMRF hired a chief scientific officer (CSO), a director of translational research (with academic and pharmaceutical experience), and a team of researchers. The CSO focused on member projects that involved the MMRC's shared tissue, and served as a disinterested party to examine each of those trials. This was important to ensure that institutions did not exhibit an undue bias towards a certain drug.

The CSO oversaw the MMRF's research team, which focused on tissue, genomics, and drug validation. Later on, the MMRF built a clinical team (overseen by a chief medical officer (CMO) hired in 2009) which focused on drug trials, ensuring execution, and regulatory compliance. The two teams met twice a year to prioritize which drugs the MMRF should target, and then helped encourage clinical trials. These in-house groups were not designed to replace its MMRC partners, but rather to develop internal capabilities around drug discovery and to reduce relying on these centers as the MMRF's sole source of information.

The MMRF's growth meant that it had to re-evaluate its personnel as its needs changed. "We had a person who came from academia and was crucial in helping us with membership agreements and

other legal obstacles when we built the MMRC," Giusti explained, "but as the MMRC turned to the clinical application of research, he was not the right person for that position."

Kathy Giusti's Leadership

Colleagues described Giusti as a demanding leader with a strong, no-nonsense personality. "She has high expectations of herself so it's understandable that she has high expectations for everyone else," observed Young, who elaborated, "Kathy is looking for people to give more than what they think she wants to hear—she wants their counsel or guidance."

Giusti also possessed the combination of skills necessary to bring together different organizations. "She is smart, business savvy, and someone I could establish a partnership with. We were fundamentally aligned on working for what was in the best interest of the patient," said Coles. Dunsire elaborated: "She has a unique understanding of who her audience is, what they are working on, where their gaps are, and how to overcome these problems. A big part of the MMRF's success has been Kathy's ability to address her audience's goals and align incentives."

Giusti had a strong connection and credibility with patients. "She turned her personal tragedy into an opportunity for others," Anderson said. "In sharing her personal story, Kathy makes herself so accessible and so vulnerable. It is extraordinary that Kathy has done so well living with MM. You have to admire and respect what she has done, and it makes you want to do anything in your power to help her mission," explained Coles.

The MMRF was not designed to be a patient advocacy organization. "The best thing I can do for patients is to help find a cure," Giusti explained. However, LaForge noted that Giusti and other executives had close relationships with a number of patients. "Kathy talks with patients all the time, and they call her at the really tough times in their lives," LaForge said. Giusti also sent out hundreds of Christmas cards every year featuring her children. "I want to thank them for the year they gave me with my family," she explained. "Some people I come in contact with see me as some crazy tyrant pushing all their buttons. I want them to see that I'm a patient and a mother."

However the MMRF's high-profile had the potential to rankle some within the academic and business communities. "In order to raise the public awareness of MM, the organization needs to promote its achievements. This is good for the MMRF, but the criticism has been that it's been quick to claim credit, especially around FDA approval of drugs," explained one observer.

Business Focus

The MMRF's business orientation helped it overcome the stereotypes of non-profits—that they lacked professional management and a concern about results. Many credited that to Giusti's operational focus. "She is mission driven," Coles said, "and has the business know-how to effectively represent herself and the MMRF to physicians and business." Giusti believed that her background brought credibility to the MMRF and made others willing to contribute. "I don't do anything without a business plan. When I contact people," she said, "I have a plan laid out; otherwise, it's a waste of time. I'm only asking for their time and energy. I take care of everything else." Dunsire added another reason for Giusti's effectiveness: "Kathy has a clear and compelling vision of what she wants to accomplish and is willing to challenge the status-quo. She focuses relentlessly on making relationships win-win: 'How can I make it a win for your business to get what I want?'"

At first hesitant to join a non-profit, President of the MMRF Walter Capone—who had been the vice president of commercial development and operations at Progenics Pharmaceuticals—soon saw that "working with donors was very similar to working with investors."[42] Capone had built his career in the pharmaceutical and biotechnology worlds with Bristol-Myers Squibb and Wyeth, and was recruited to the MMRF because of his work in these fields, particularly in bringing drugs to market.[43] The MMRF's leadership was also impressed that he had worked in the field of AIDS during his career, and thus had primary experience working on a disease with an engaged patient community and where there was collaboration between multiple parties on moving drugs and research forward.

Bill Bowes (HBS '52), founding partner of U.S. Venture Partners and a MMRF board member, met Giusti when they were both at HBS in 2009 to receive an Alumni Achievement award.[44] "I was impressed by her story. To go from being an executive at a large pharmaceutical firm to a non-profit startup is a big move," he said. Giusti followed up with Bowes after the event and flew to California to discuss the MMRF in greater detail. "Kathy was smart, had experience in pharmaceuticals, and had enormous energy. It's that combination of traits and abilities you look for in a CEO," Bowes said. Bowes had no family history with the disease, but donated close to $5 million. "I was impressed by what they'd done in spotting promising drugs early, helping to set up clinical trials, and shepherding them through the approval process. This is a big process even for major companies," he explained.

Move to Personalized Medicine

Genetic sequencing, the process of examining DNA at the molecular level, had generated great research interest because it promised to enable doctors to tailor treatment to an individual patient based on his or her genetics—a practice known as personalized medicine. The MMRF's first effort in this area came in 2005 with the founding of the Multiple Myeloma Genomics Initiative (MMGI): a $12 million undertaking to collect 400 tissue samples for sequencing.[45]

The idea to sequence the MM genome came to Giusti while listening to a presentation on genetic sequencing given by Eric Lander. At the time, Giusti was a member of the NCAB and Lander was the founding director of the Broad Institute (the Broad) and had led the effort to sequence the human genome. "I knew the NIH would never do it for MM. I called Eric up afterwards and asked what it would take for the Broad to partner with us," Giusti recalled. "He told me how much tissue, how much money, and how many clinical experts we would need, and I told him that we already had all of this." In the course of preparing the proposal, Giusti contacted leading sequencing experts to review the submission. "I wanted the Broad to know we were serious. Having the experts review the proposal was also a way of telling the Broad: 'make this the best program you can because we are sharing our most precious research resource—tissue—with you,'" she explained.

By early 2011, 38 MM genomes were sequenced and a team of researchers published their findings in *Nature*.[46] One revelation was that 4% of the patients had mutations on the BRAF V600 gene, a mutation shared with melanoma skin cancer patients. This indicated that drugs used to treat melanoma might also be effective for these MM patients. "This shifted our thinking of these patients as having MM, to their having a BRAF V600-related cancer," Giusti explained. Starting in April 2012, MM patients were being included in a study of vemurafenib, a drug targeting the BRAF V600 gene.[47]

Heterogeneity of Multiple Myeloma

As researchers better understood the genetics of the disease, it became clear that MM was not a single disease. Instead, it had 10 or so different subtypes, and when patients relapsed there was not

one genetic mutation common among all of them but some 50 to 60. The recognition that MM was a heterogeneous orphan disease meant that although a few drugs might work well for many patients, therapies that targeted specific subtypes or mutations were necessary to treat patients individually.

Reaching this level of care required many more tissue samples representing a variety of patients, subtypes, and mutations. "The data is in the tissue, but the ones we had collected were just snapshots of a single moment in time," Giusti explained. "We knew when we started the first study of the MM genome that we would eventually need a clinical study. The gold standard for this type of genomic study is for it to yield a clinical, longitudinal, and molecular dataset."

Forming CoMMpass

In 2011, the MMRF launched a new initiative: Relating Clinical Outcomes in Multiple Myeloma to Personal Assessment of Genetic Profile (CoMMpass). The goal was to collect tissue from newly diagnosed patients at three key stages (diagnosis, best response to treatment, and relapse), sequence each sample, and then track each patient for five years to examine his or her response to the disease and treatment. Because treatments were ineffective for 15% of patients, the study required a sample size of 1,000 patients for findings to be statistically significant.

CoMMpass was expected to be a $40 million investment, and early discussions raised a number of issues. Some involved potential legal risks: one MMRF executive who was also a doctor was concerned about her name being tied to the program in case of a malpractice suit. "We had to make it clear that we were not running a drug trial," Giusti explained. "This was a longitudinal study and we were banking patient's tissue; we were not developing a drug or treating patients."

Management and money were also important considerations during board discussions. "How do we influence and control this entity in a way that generates meaningful results? From a financing perspective, we couldn't risk the MMRF on one investment," LaForge recalled. Management had to quickly address these questions. "There was an urgency to act," explained Capone. "Unlike in the corporate world, where we waited until all the budgets lined up and the answers to questions were fully known, we had to move. Lives were on the line."

Many of the same concerns surrounding the start of the MMRC emerged again, falling into two broad categories: constituencies and operations. With regard to constituencies, the MMRF had to determine whom to partner with in collecting the tissue and data, and how to draw upon these partners for research, drug development, and patient treatment. On the clinical side, how would CoMMpass engage patients and caregivers? With regard to operations, questions arose as to which hospitals would collect tissue, where it would be stored, who would do the sequencing, and what processes would ensure timely and uniform collection, sharing, and access to data. A new information technology (IT) platform also had to be designed.

CoMMpass also shifted the MMRF from working primarily with major research hospitals to working closely with community hospitals. Historically, most patients went to major research hospitals where oncology specialists could treat MM in ways that local community hospitals could not. As the effectiveness and availability of drugs improved, high-quality care could increasingly be delivered at the local level. Thus, the MMRF needed the patients from the community centers, combined with the knowledge and expertise of its long-time academic partners to review the data. Giusti found that many community hospitals were eager to join. Doctors at these hospitals did not focus on research and publishing, so IP was not a primary concern. They were more interested in treating patients, so the new referrals and publicity the MMRF brought were highly valuable.

To design CoMMpass's protocol, the organization enlisted the help of leading experts from academia, bioinformatics (software that analyzed and interpreted genomic information), community hospitals, and genomics. Participants agreed to give up IP so that all data would be available to the public, with the MMRF owning the tissue and data. "Giving up tissue samples and IP goes against everything physicians and researchers know," Capone noted. "We negotiated an agreement with one center that they would be able to see that all their material made it to the public domain. They went from initially saying they wouldn't participate under any circumstance, to participating so long as it all went into the public domain."

Pharmaceutical companies were also willing participants. In addition to helping them fill out clinical trials via new connections to community hospitals, CoMMpass provided access to sequenced genetic information that gave those firms targets to work towards in developing drugs. "By working with us, they're able to view thousands of samples," Giusti said. Dunsire gave another reason companies were eager to join: "Kathy has recognized that some cancers require a combination of therapies to treat, but it's hard for two different companies to get together and do this on their own."

Four pharmaceutical firms—Millennium Pharmaceuticals, Onyx Pharmaceuticals, Bristol-Myers Squibb, and Janssen Pharmaceuticals (listed in the order each one joined)—were partners in a pre-competitive consortium (PCC) as part of CoMMpass. These members paid between $4 million and $5 million over a period of four years for a window of prioritized access to data from the study (which was updated every six months) and to participate on conference calls with the MMRF's scientific staff. PCC members could follow up with the scientists if they wanted more information, such as how patients were responding to a certain drug.

Insurers, too, would benefit from CoMMpass as it became clear which drugs and treatments were suited to different patients given the stage of their disease, individual demographics, and the specific genetics of their MM. "This will improve care and help insurers attain cost savings through more effective treatments," explained Capone.

CoMMpass's Outcome

The first patient enrolled in July 2011, and by the spring of 2014, 895 patients were screened, 550 were enrolled, and 85 hospitals in North America and Spain were participating. The plan was for all 1,000 patients to be signed up by the end of 2014.

The CoMMpass IT platform had two distinct functions: the Researcher Gateway and the patient-facing Community Gateway. Both went live in September 2013. The Researcher Gateway was designed for doctors, researchers, and bioinformaticians to examine discrete genomic data in the context of complete patient clinical data. "For the first time, these individuals will be able to examine comprehensive, longitudinal clinical data and the most complete genomic data at the same time on a substantive population of MM patients," Capone explained. A goal of the system was to help doctors assess the differential outcomes of MM patients based on the most detailed, longitudinal data attainable, to help in current and future patient management. "Doctors will be better able to better understand the factors leading to attaining the best outcomes for a specific subtype of MM. This information will lead a doctor to determine the best treatment for a particular patient," Capone explained. The Community Gateway enabled patients to connect with one another through discussion groups on a range of topics, with experts moderating each topic and responding to questions. When registering on the site, patients were asked to complete a robust profile to add to the clinical information being collected, and to enable content directed to their specific circumstances—from disease stage, to treatments, side-effects and symptoms.

The Search for Sustainable Funding

As a foundation, the MMRF relied heavily on donations (see **Exhibit 9** for the MMRF's financials). Money came in year round, but two-thirds was raised in the last three months of the year. Over its 15-year history, funding had come from three sources: 40% from individual donors, 30% through events, and 28% from pharmaceutical firms (see **Exhibit 10** for sources of funding for select years).

Raising money was an intensive process and most donations were for relatively small amounts. "About 1,000 new patients contact the MMRF every month and maybe two of them can afford to donate $10,000 or more," Young said. Securing such donations required the right approach. "We're not selling a product, so donors have to see value in what we're selling: either in extended life expectancy or a cure in a meaningful timeframe," explained Capone. "Conveying this value proposition is much more difficult than the typical sales process. Integrity and transparency are paramount. You're speaking with patients about their disease, while also asking for financial support. It's a melding of the emotional and financial side of the business." LaForge echoed the emphasis placed on what donors could expect from their investment: "We don't promise results. What we do promise is effort, direction, focus, and that we'll spend the money wisely. This is a return-on-investment business. It has to be."

In 2013, the MMRF earned revenue through many sources, including an expanded access program (EAP), fee-for-service, the PCC, and other small revenue streams. To ensure long-term financial security, Giusti had been trying to develop new, sustainable sources of revenue. CoMMpass was generating a trove of valuable data, so she and her executive team wondered if there was some way to monetize parts of this information. The MMRF also had other assets it had historically given away, such as the research and business development work it conducted. The team considered whether they could build income streams from these functions, such as by creating unique research reports.

Capone had led the challenging negotiations with participating pharmaceutical firms and CoMMpass's clinical and research partners in organizing the PCC. All parties ultimately agreed to give up any claims to tissue or IP created through CoMMpass. For their participation, the four pharmaceutical companies that comprised the PCC received five months of exclusivity to review new data. Then, the data became available to the hospitals and researchers participating in CoMMpass for a period of six months. After this second window of exclusivity closed, there was a 30-day period for researchers to start working towards publishing any findings they may have uncovered through analysis of this data. After that, data and results became publicly available. However, several of the MMRF's executives wondered if it was appropriate for for-profit companies that had not participated in the PCC to receive free access to this information, even after the periods of exclusivity expired. As one member of the team asked, "It's one thing for a researcher to get free access, but is it fair for a company not in the PCC, with significant resources, to get access to data and findings for free?"

At the same time, Giusti, Capone, and Young always returned to the organization's mission: to help all patients, even if it posed a challenge to revenue. With EAPs for example, the MMRF served as a conduit through which money from pharmaceutical firms flowed to clinical trials, in order to expand trials so that critically ill patients would have access to drugs that were close to gaining FDA approval. This influx of money showed up in the MMRF's financial statements, and could move the organization's reported annual revenue up and down, though all but a nominal fee for administrative services flowed right back out to fund the clinical trials. The fee was small relative to the amount of time and manpower the MMRF dedicated to EAPs. "We do this because it's the right thing to do for patients," Giusti explained, "but it can have an unintended impact on how the MMRF is rated by organizations that evaluate charities. Because this money shows up as revenue, it sets the bar for how

we are measured by such evaluators. If we run fewer EAPs one year, we have to make up the 'revenue gap' through additional fundraising, so that it doesn't look like we are raising less money or being less effective in our spending. If that happens, we see our charity rating decline."

Meanwhile, Giusti debated how, and even if, the MMRF could charge for access to its data: "We need to stay true to our principles of open access and enabling data sharing, but by the same token, is there a way to monetize parts of what we do without being hypocritical?" The MMRF had historically shared information and worked with all partners for free. "We've helped open 46 clinical trials through the MMRC, testing 24 different drugs," Giusti explained, "and we've gotten nothing back financially from that investment because we've never asked."

She highlighted the experience with Onyx's drug Kyprolis as one such example. The MMRC worked with Onyx (and its predecessor, Proteolix) to help with protocol design, establish clinical trials, enlist patients, and help it through the approval process. In July 2012, Kyprolis received FDA approval, and a year later Onyx was bought by Amgen for $10.4 billion.[48] The MMRF had received money from the company to fund grants, and the partnership was successful as it gave hundreds of patients access to the new drug via an EAP. However, some within the MMRF wondered, upon reflection, if the relationship could have been organized to help fund future research. As Young explained, "we didn't structure anything into our relationship with Onyx, so we don't get anything financially from this sale. I think this sticks out in our minds as a lost opportunity."

As Giusti and her team explored funding possibilities, they observed how other non-profits had established financial partnerships with pharmaceutical firms. The Cystic Fibrosis Foundation (CFF), for instance, received royalties from its work with Vertex Pharmaceuticals on its CF drug Kalydeco, approved for use in 2012.[49] The CFF sold part of those royalty rights for close to $150 million in 2012.[50] Other organizations, such as the ALS Association and the Muscular Dystrophy Association, had also earned revenues from royalties.[51]

Moving to MMRF 3.0

Since its founding, the MMRF had raised over $250 million, facilitated the collection of 5,000 tissue samples, organized a consortium of medical centers, helped in sequencing the disease's genome, and played a role in multiple clinical trials. Patient life expectancy had increased, and six new MM drugs were on the market (see **Exhibit 11**). Giusti was excited about CoMMpass and how it would drive the organization to her future vision of the MMRF (see **Exhibit 12**). "CoMMpass is about taking research and applying it at the clinical level. If a patient comes into the program and we know they have a specific mutation, we'll be able to get them into the right trial. The next iteration of the MMRF, what I call MMRF 3.0, is about precision medicine."

Getting to that point would not be easy. First, management needed to formulate options for creating sustainable revenue streams. With some options in front of them, Giusti and her board needed to decide whether this made sense, or if it threatened the organization's mission and standing. Second, Giusti and her board needed to rethink the talent they needed to reach MMRF 3.0. For LaForge and his fellow board members, this raised an even more central concern: "I don't care how good your leader is, how do you keep them motivated? My biggest challenge is how do I make sure Kathy has the energy to keep being a leader? I try to provide the emotional and intellectual energy to help her be extraordinary; the energy that is imparted to Kathy from bringing in top caliber employees and board members is critical." The stakes were high for everyone involved. As Dalton put it bluntly, "If Kathy and the MMRF are successful, then we all succeed."

Exhibit 1 Estimated New Diagnoses and Deaths from Selected Cancers, 2014

Cancer	Diagnoses	Deaths
Breast	235,030	40,430
Colon	96,830	50,310
Leukemia	52,380	24,090
Lung & bronchus	224,210	159,260
Lymphoma	79,990	20,170
Melanoma (skin)	76,100	9,710
Myeloma (MM)	24,050	11,090
Pancreas	46,420	39,590
Prostate	233,000	29,480

Source: Adapted from American Cancer Society, "Cancer Facts & Figures 2014," 2014, p. 4, http://www.cancer.org/acs/groups/content/@research/documents/webcontent/acspc-042151.pdf, accessed June 2014.

Exhibit 2 Selected Orphan Diseases (numbers are for U.S. patients only), 2013

	Number of Patients	New Diagnoses (Annual)	Survival Rates/Life Expectancy	Organs/Systems Impacted
ALS	20,000-30,000	5,000	3 to 5 years	Neurological disease
Cystic fibrosis	30,000	1,000	Early 40s (median)	Lungs and pancreas

Source: Compiled by casewriter from the National Institute of Neurological Disorders and Stroke, "Amyotrophic Lateral Sclerosis (ALS) Fact Sheet," updated December 30, 2013, http://www.ninds.nih.gov/disorders/amyotrophiclateralsclerosis/detail_ALS.htm, accessed January 2014; and Cystic Fibrosis Foundation, "About Cystic Fibrosis," http://www.cff.org/aboutcf/, accessed January 2014.

Exhibit 3 MMRF Board of Directors

W. Dana LaForge, Chairman	Partner, Brera Capital Partners, LLC.
Kenneth Anderson, MD	Chief, division of hematologic neoplasia; director, Jerome Lipper Multiple Myeloma Center; Dana-Farber Cancer Institute.
Karen E. Andrews	Senior vice president, legal and business affairs, Hachette Book Group.
William K. Bowes, Jr.	Founding partner, US Venture Partners.
Kathy Giusti	Founder and CEO, MMRF and MMRC.
Stephen Grand	President and partner, Grand-Sakwa Properties.
Eugene P. Grisanti	Retired chairman, president and CEO, International Flavors & Fragrances, Inc.
David L. Lucchino	Co-founder, Semprus BioSciences.
Joel S. Marcus	Chairman, CEO, and founder, Alexandria Real Estate Equities, Inc.
Lori Tauber Marcus	Senior vice president and chief marketing officer, The Children's Place.
Gerald McDougall	Partner, PricewaterhouseCoopers.
William S. McKiernan	Former Chair and CEO, CyberSource Corporation.
Chris A. McWilton	President, U.S. Markets, MasterCard Worldwide.
Mike Mortimer	Executive vice president and chief administrative officer, Quintiles Transnational Corp.
Charles B. Ortner	Senior Partner, Proskauer Rose LLP.
David R. Parkinson, MD	President and CEO, Nodality, Inc.
Marie Pinizzotto, MD	President and CEO, Carol A. Ammon Foundation and president of Drug Safety Solutions LLC.
Michael Reinert	Partner, Sports and Entertainment, Fox Rothschild LLP.
Meryl Zausner	CFO, Novartis Corporation

Source: Multiple Myeloma Research Foundation, "Leadership: Board of Directors," http://www.themmrf.org/about-the-mmrf/leadership/board-of-directors.html, accessed November 2013.

Exhibit 4 Leadership Council

"Our Leadership Council is comprised of prominent, high-profile business leaders who generously lend their expertise and resources to support out mission."

Joseph M. Hogan	Former CEO, the ABB Group.
Lester B. Knight	Founding and managing partner, RoundTable Healthcare Partners.
Philip J. Purcell	President, Continental Investors.
Robert Wolf	Former president and COO, UBS Investment Bank; chairman and CEO, UBS Group Americas; member of UBS Group Executive Board; member of UBS Group Managing Board.

Source: Multiple Myeloma Research Foundation, "Leadership: Leadership Council," http://www.themmrf.org/about-the-mmrf/leadership/leadership-council.html, accessed November 2013.

Exhibit 5 MMRF Revenue, 1999-2004 (in $US millions)

	1999	2000	2001	2002	2003	2004
Revenue	2.8	4.2	5.4	6.0	8.7	12.1

Source: Multiple Myeloma Research Foundation, "Annual Report 2006," p. 14, http://www.themmrf.org/about-the-mmrf/financial-reports/, accessed November 2013.

Exhibit 6 The National Cancer Institute's (NCI) Funding for MM Research, 2007-2011

	2007	2008	2009	2010	2011
NCI's total budget (in billions)	$4.8	$4.8	$5.0	$5.1	$5.1
NCI's funding for Myeloma research (in millions)	$32.3	$41.5	$45.2	$48.5	$54.9

Source: National Cancer Institute at the National Institutes of Health, "A Snapshot of Myeloma," March 22, 2013, http://www.cancer.gov/researchandfunding/snapshots/myeloma, accessed November 2013.

Exhibit 7 MMRF Patient Database

	2012	2013	2014 Goal
Total Records	313,000	356,480	375,000
% donors	15%	13%	18%
% patient donors	9%	8%	15%
% patient spouse/caregiver donors	14%	15%	20%

Source: Company documents.

Exhibit 8 Select MMRC Leadership

Executive Committee

Kathy Giusti	Founder and CEO
Walter Capone	President, MMRF
Beverly L. Harrison	Vice president of clinical development, MMRF
Anne Quinn Young	Vice president of development and strategic partnerships, MMRF

Steering Committee

Kenneth Anderson, MD	Chief, division of hematologic neoplasia; director, Jerome Lipper Multiple Myeloma Center; Dana-Farber Cancer Institute.
Jesus Berdeja, MD	Director, myeloma research at the Sarah Cannon Research Institute.
Rafael Fonseca, MD	Getz family professor of cancer; chair, department of medicine; associate director, Center for Individualized Medicine.
Kathy Giusti	Founder and CEO, MMRF and MMRC.
Shaji Kumar, MD	Associate professor of medicine and consultant, Mayo Clinic.
Andrzej Jakubowiak, PhD, MD	Professor of medicine and director of the MM program, University of Chicago.
Sagar Lonial, MD	Professor of medicine, Emory University.
Donna Reece, BA, FRCPC, MD	Associate professor and director of the program for MM and related diseases at Princess Margaret Hospital/University of Toronto.
Paul Richardson, MD	Clinical director, Jerome Lipper Multiple Myeloma Center, Dana-Farber Cancer Institute; Associate professor of medicine, Harvard Medical School.
Ravi Vij, MD	Associate professor of medicine, Washington University.

Source: Multiple Myeloma Research Consortium, "Steering Committee," http://themmrc.org/about/leadership/steering-committee/, accessed January 2014; and Multiple Myeloma Research Consortium, "Executive Committee," http://www.themmrf.org/about-the-mmrf/leadership/executive-committee.html, accessed June 2014.

Exhibit 9 MMRF Financials, 2006-2013 (all years ending December 31; in $US)

	2006	2007	2008	2009	2010	2011	2012	2013
Support and revenue								
Contributions	$3,921,368	$4,319,668	$7,001,152	$5,381,449	$8,188,625	$10,681,787	$9,887,802	$9,273,518
Private foundation grants	5,285,924	9,883,022	9,936,269	9,602,091	10,215,460	7,795,430	8,250,701	6,676,708
Fee for service	NA	NA	NA	NA	NA	1,426,530	5,006,210	810,010
Federal grant support	498,567	465,846	475,796	459,060	392,759	330,345	-	-
In-kind contribution	75,696	13,695	24,404	98,755	64,054	214,324	344,623	150,521
Special events								
Special events support	5,164,480	7,874,323	5,264,974	6,648,033	7,177,154	8,188,580	8,695,871	8,960,970
Less special events expenses	(1,201,954)	(1,808,862)	(1,780,555)	(1,498,976)	(1,913,200)	(2,236,976)	(2,621,075)	2,609,606
Investment return	272.896	239,915	32,692	143,589	204,947	131,944	308,598	66,306
Total support and revenue	**14,016,977**	**20,987,607**	**20,954,732**	**20,834,001**	**24,329,799**	**26,531,964**	**29,872,730**	**23,370,427**
Expenses								
Program								
Research	10,500,381	14,156,123	13,815,646	12,908,973	13,757,372	16,516,780	20,227,722	14,542,375
Education	1,028,770	2,286,439	1,666,752	1,679,015	2,178,744	2,753,263	2,087,083	1,962,185
Awareness	1,010,334	489,049	364,599	249,420	407,546	757,249	805,611	1,201,531
Other program expenses	855,538	1,074,625	1,206,472	1,301,115	1,770,253	2,743,483	2,640,914	2,705,654
Total program expenses	**13,395,023**	**18,006,236**	**17,053,469**	**16,138,523**	**18,113,945**	**22,770,775**	**25,761,330**	**20,411,745**
Supporting services								
Management and general	425,301	526,719	552,453	598,423	534,624	551,236	673,250	550,709
Fundraising	598,129	839,041	1,781,766	1,355,668	1,556,511	1,864,404	2,181,259	2,354,842
Total supporting services	**1,023,430**	**1,365,760**	**2,334,219**	**1,954,091**	**2,091,135**	**2,415,640**	**2,854,509**	**2,905,551**
Total expenses	**14,418,453**	**19,371,996**	**19,387,688**	**18,092,614**	**20,205,080**	**25,186,415**	**28,615,839**	**23,317.296**
Change in net assets	**(401,476)**	**1,615,611**	**1,567,044**	**2,741,387**	**4,124,719**	**1,345,549**	**1,256,891**	**53,131**
Net assets, beginning of year	**3,564,332**	**3,162,856**	**4,778,467**	**6,345,511**	**9,086,898**	**13,211,617**	**14,557,166**	**15,814,057**
Net assets, end of year	**$3,162,856**	**$4,778,467**	**$6,345,511**	**$9,086,898**	**$13,211,617**	**$14,557,166**	**$15,814,057**	**15,867,188**

Source: Data for 2013 is from company documents. All other financial data is from the Multiple Myeloma Research Foundation's annual reports, including: "Donor Progress Report 2012," p. 6; "Donor Progress Report 2010," p. 18; "Annual Report 2008," p. 19; "Annual Report 2006," p. 15. All were accessed in November 2013, and reports for 2012, 2010, 2008, and 2006 are available at: Multiple Myeloma Research Foundation, "Financial Reports," http://www.themmrf.org/about-the-mmrf/financial-reports/.

Exhibit 10 Sources of Funding for Select Years, 2006-2012

	2006	2008	2010	2012
Events	37.5%	23%	27%	27%
Healthcare corporations	15.5%	19%	22%	38%
Individuals	12%	12%	9%	8%
Interest	2%	NA	NA	NA
Major gifts & private foundations	29%	44%	39%	25%
Other	4%	2%	3%	2%

Source: Multiple Myeloma Research Foundation, "Donor Progress Report 2012," p. 5; Multiple Myeloma Research Foundation, "Donor Progress Report 2010," p. 17; Multiple Myeloma Research Foundation, "Annual Report 2008," p. 18; Multiple Myeloma Research Foundation, "Annual Report 2006," p. 14. All accessed November 2013, and reports for 2012, 2010, 2008, and 2006 are available at: Multiple Myeloma Research Foundation, "Financial Reports," http://www.themmrf.org/about-the-mmrf/financial-reports/.

Note: From 2008 onwards, the major gifts and private foundations category only counted private foundations.

Exhibit 11 Drugs for MM

Drug	Company	Approval Date (for MM)	Purpose
Velcade	Millennium: The Takeda Oncology Company	2003	Proteasome inhibitor
Thalomid	Celgene	2006	Immunomodulating agent
Revlimid	Celgene	2006	Immunomodulating agent
Doxil	Janssen Pharmaceuticals Inc.	2007	Anthracyclines
Kyprolis	Onyx Pharmaceuticals	2012	Proteasome inhibitor
Pomalyst	Celgene	2013	Immunomodulating agent

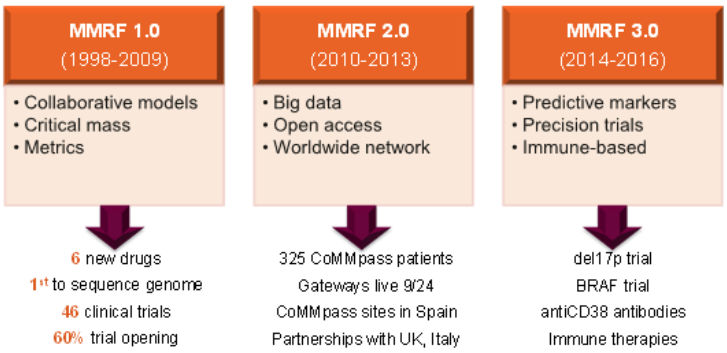
Source: Compiled by casewriter from Millennium: The Takeda Oncology Company, "Corporate Fact Sheet," January 2009, http://www.millennium.com/PDF/MillenniumCorporateFactSheet.pdf, accessed January 2014; "Multiple Myeloma Overview," American Cancer Society, revised February 13, 2013, http://www.cancer.org/acs/groups/cid/documents/webcontent/003065-pdf.pdf, pp.12-13, accessed November 2013; U.S. Food and Drug Administration, Drugs@FDA, "Thalomid," http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.Overview&DrugName=THALOMID&CFID=21187535&CFTOKEN=177f3fa8c4d89b1-55DC9CE1-C150-8FB7-692955C78BFC06BA, accessed January 2014; U.S. Food and Drug Administration, Drugs@FDA, "Revlimid," http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.DrugDetails, accessed January 2014; U.S. Food and Drug Administration, Drugs@FDA, "Pomalyst," http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.DrugDetails, accessed January 2014; U.S. Food and Drug Administration, Drugs@FDA, "Kyprolis," http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm?fuseaction=Search.DrugDetails, accessed January 2014; Multiple Myeloma Research Foundation, "Relapsed/Refractory Patients: Treatment Options – Doxil," http://www.themmrf.org/living-with-multiple-myeloma/relapsed-refractory-patients/treatment-options/doxil.html, accessed January 2014; approval dates for Thalomid and Revlimid are from case interviews.

Exhibit 11 Drugs for MM (continued)

Drug	MMRF's/MMRC's Role In Drug's Approval
Thalomid	Helped enroll patients in clinical trials; educational/awareness building programs for patients and healthcare providers; and educated the community (patients, caregivers, health care providers) about maximizing the drug's effectiveness and minimizing/managing toxicities.
Velcade	Helped enroll patients in clinical trials; educational/awareness building programs for patients and healthcare providers; and educated the community (patients, caregivers, health care providers) about maximizing the drug's effectiveness and minimizing/managing toxicities. Worked closely with Millennium on the drug's launch (including some marketing functions) to account for doctor's and patient's unfamiliarity with the drug.
Revlimid	(Same as **Thalomid**)
Doxil	(Same as **Thalomid**)
Kyprolis	Facilitated early Phase I clinical trials and three Phase II trials with Kyprolis within the MMRC network, including the trial that supported its approval by the FDA, the original Phase II trial, and the Phase II trial in patients who had relapsed after one to three prior therapies; provided Onyx access to the MMRC network of clinical experts, secured trial investigators, supplied centralized contracts, provided guidance on site selection and protocol design, and accrued patients to trials; raised awareness and drove enrollment to ASPIRE, Onyx's Phase III clinical trial with Kyprolis and educated the MM community on emerging data from clinical trials; partnered with Onyx on the Carfilzomib Myeloma Access Program (C-MAP), an EAP that made carfilzomib available to hundreds of eligible patients in the U.S. before FDA approval.
Pomalyst	Facilitated the Phase I and Phase II clinical trials that provided the basis for the accelerated approval, which included assistance with protocol development and Institutional Review Board submissions; drove rapid patient enrollment to the Phase I and Phase II registration trials, enrolling 100% of patients in the Phase I trial and more than 80% in the Phase II trial, completing enrollment in less than two years and exceeding timeline expectations by several months; provided dedicated site management and guidance on key safety and efficacy issues throughout the Phase I and Phase II registration trials.

Source: Company documents.

Exhibit 12 The MMRF's "Three Versions"



Source: Company documents.

Endnotes

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