

CASE FOR SUPPORT – What’s a Cure Worth to You?

THE ROAD TO CURES ...

The power of the *patient* has opened the floodgates ...

The **MMRF** has created a **whole new system** that redefines multiple myeloma treatment—empowers patients, fosters research and expedites the search for cures ...

Now we’re putting it in play ... It’s time to make history!

The **MMRF** has launched a series of historic initiatives using Big Data to augment **Precision Medicine**. Thanks to our new **MMRF Community Gateway**, myeloma patients will now access information and interaction with their own treatment like never before. And thanks to our **MMRF Researcher Gateway**, scientists around the world can rapidly pool their knowledge and data to collaboratively work toward finding new treatments ... and seeking cures.

Join us in this historic initiative ... Help us extend lives ...

What’s a cure worth to you?

“All I care about is finding a cure in my lifetime and that is why I get out of bed in the morning living with this fatal disease. I am all about creating business solutions for these scientific problems.”

Kathy Giusti
Founder and CEO / The MMRF & MMRC

A VISION THAT WORKS ...

There is no known cure for Multiple Myeloma—a blood cancer with a very complex makeup at the molecular level. In the United States alone there are 20,000 new cases diagnosed each year. Before the **MMRF** was founded, life expectancy for patients was a mere three years or less. Worse, at that time there were absolutely no new drugs in the research pipeline—not one inkling of hope to offer thousands upon thousands of patients who were powerless to do anything but wait until their disease progressed to its final stage.

The system was broken. Drug trials bogged down in the mire of red tape. A disconnect between industry and academia made collaboration rare, despite the vital necessity of bridging that gap. Funds

for many studies were not available, and the specific genomic data so important to conducting real investigations into treatments was simply nonexistent.

Then, in 1998, the **MMRF** was founded by a newly diagnosed patient named Kathy Giusti. A savvy, Harvard-educated pharmaceutical professional with a strong corporate background, Kathy made an unerring commitment to do all she could to see drug trials expedited, research abetted and treatment options expanded.

Over the past 15 years, the **MMRF** has created a vision that works. We've refused to take 'No' for an answer. Instead, we've channeled our energy and resources into fueling those proven avenues that net results. We've forged relationships throughout government, industry and academia in an effort to create the vital alliances needed to expedite trials of new drugs. We've not only supported senior researchers in their efforts to discover cures, but have done all we can to encourage burgeoning fellows to aim their attention and intellect on myeloma research. Further, we've embraced new technologies and scientific discoveries to aid our core mission, bringing Genomic Sequencing, Big Data and Real-Time Communication into play with our continuing efforts to advance research. Finally, we've shown cancer patients themselves just how vital their role is, and how future drugs and cures are contingent on their participation in the process.

Now, drawing on our unique history as a trusted flagship in myeloma treatment, the **MMRF** has created its groundbreaking Gateways ...

The **MMRF Patient Gateway** allows people instant access to personalized information about their disease and all the new treatment options as they become available. Along with the chance to be part of a burgeoning social network of myeloma patients, the **MMRF Patient Gateway** is an opportunity to gain ownership over one's unique medical condition and recovery. The molecular specifics of one patient's condition can instantly connect them with all relevant data, including information on new trials. This allows a patient the chance to stay on top of treatment developments, consider recommendations and guidance when it comes to doctors and centers, and enjoy being part of a community of like-minded individuals who understand the personal battles being fought.

The **MMRF Researcher Gateway** is a priceless portal of information that goes directly from patients to the people trying to find new treatments and cures. A consolidated reservoir of actual data on individual patients' medical make-up, the **MMRF Researcher Gateway** gives scientists and scholars from throughout the world instant access to the information they need to do their job. The results are exponential. Now professional collaboration can become second-nature, as any and all scientific breakthroughs become instantly accessible for everyone, enabling researchers of all variety to immediately build upon, augment and expand the valuable work of others toward the common goal of understanding myeloma and creating cures.

Now one patient's story can speak to millions.

A HISTORY WELL-MADE

In 1996, when Kathy Giusti was diagnosed with multiple myeloma, she assumed help was on the way. Despite the shocking news that she would likely die before her one-year-old daughter even reached kindergarten, Kathy's professional background led her to believe there were suitable drugs in development that could aid her condition and perhaps one day bring a cure to light.

The more Kathy investigated her options for treatment, however, the more shocked she was to discover how little was available for myeloma patients. But rather than sit and serve out what amounted to a death sentence, Kathy—with the help of her twin sister Karen Andrews—decided to make things happen ...

The MMRF: In 1998 the **MMRF** was formed, thanks in large part to a dedicated group of friends and supporters, who became **MMRF's** advisors, board members and councilors to the cause. At that time one main goal of the **MMRF** was to award funding for research to senior investigators, as well as many junior fellows in an effort to encourage future dedication to myeloma research. Since that time we've distributed more than 340 grants totaling well over \$100 million. We've given funds to more than 130 research institutions throughout the world, along with 11 biotech and pharmaceutical companies.

But perhaps more importantly, the **MMRF** took the lead to become a *trusted third-party* in myeloma cancer. It didn't take long for us to learn that the biggest obstacle to driving trials to the clinics was a lack of unity. We saw that if we were going to break new ground in myeloma treatment, we needed to get all the players on the same page.

The **MMRF** worked aggressively to forge relationships with pharmaceutical companies, hospitals, and of course patients. We then leveraged those relationships to motivate changes in the system. We imparted a real sense of urgency into trials, and got drugs moving through the pipeline faster than ever before—60 percent faster than industry standard in Phase I and Phase II trials—because we truly know the value of time. The **MMRF** began a series of educational roundtables that brought a new climate of cooperation to professionals in both academia and industry, and began empowering patients with knowledge and an understanding of what opportunities may be available. We also established a Patient Support Call Center to provide tangible guidance from trained professionals.

The MMRC: The work we did in our first six years showed us that there was a vital need for a dedicated tissue bank for myeloma research. In order to orchestrate such an endeavor—and to help further bridge the gap between academic and industry research professionals—we founded the **Multiple Myeloma Research Consortium (MMRC)** in 2004. This is an expansive network of 16 leading academic research centers working as one clinical network to conduct its own Phase I and Phase II clinical trials of the most promising myeloma compounds and combination therapies. We've directly supported 70 clinical trials and pre-clinical studies to date and more are on the way ...

One of the keys to this innovative model was the **MMRC Tissue Bank**—the first and largest bio-bank of its kind to enable researchers to conduct unprecedented genomics

studies. For the first time ever, we began collecting the much-needed cellular samples of patients in large numbers, giving opportunities for research that were unheard of up until that time. As a trusted third party in myeloma, the **MMRF** has been able to encourage and educate patients on the value of generously making their unique data available to the research community.

The MMGI: Expanding on the value of genomics research, in 2006 we took our next step forward with the co-creation of the **Multiple Myeloma Genomics Initiative (MMGI)**—the most comprehensive program ever to focus solely on myeloma. Incorporating new technologies, in 2009 the **MMGI** became the first group to successfully map the myeloma genome in its entirety, laying the foundation for pursuit of personalized treatment aimed at targeting a patient’s specific genomic profile. The important value of **Precision Medicine** became readily apparent to us, and we began widening our mission scope to focus on what we believe is the future of cancer research.

CoMMpass: Our Tissue Bank was expanded in 2011 under our landmark **CoMMpass** study. With over 250 patients currently enrolled, this groundbreaking initiative continues to collect molecular data from myeloma patients *over time*. The myeloma disease is capable of morphing. The data collected through **CoMMpass** gives not just a cellular snapshot of a patient, but a moving history of their progress. It enables researchers to see—for the first time—the progression and changes in the disease, as well as response to various treatments based on an individual’s genomic profile. This effort exponentially increases the number of opportunities for **Precision Medicine**, enabling physicians to tailor treatment to individuals based on their genomic makeup.

Thanks to the encouragement of the **MMRF**, the 50 institutions that are participating in this study have all consented to give up intellectual property rights to the data that’s being collected, allowing this wealth of information to remain in the public domain to insure that everyone can make use of it. This is an astounding feat, for it represents the first time such an extensive body of relevant myeloma research will have ever been assembled for cooperative use.

The creation and continuation of the **CoMMpass** study is pivotal to the work the **MMRF** has begun. The greater part of our mission rests with generating this data in order to facilitate research aimed at hopefully finding cures through **Precision Medicine**. By harvesting the fruits of the **CoMMpass** study, we can learn what medications and compounds work on which strains of the disease. By fueling this vital research, we’re driving new compounds and options to the clinic faster than ever before.

MMRF Community Gateway & MMRF Researcher Gateway: Having discovered the utter value of **Precision Medicine**—the future of cancer treatment—in 2013 the **MMRF** unveiled its Gateway projects.

The **MMRF Community Gateway** affords patients the chance to join a global network allowing them to tailor their treatment to their unique myeloma subtype. A veritable social network for myeloma patients, the **MMRF Community Gateway** provides instant access to up-to-date information on trials, treatments, hospitals and much more. **Precision Medicine** means not only discovering individualized treatment, but *giving patients the opportunity to have ownership over their own recovery ...*

Knowing what we do about the tremendous value of cooperative research among professionals, the **MMRF Researcher Gateway** fosters shared data throughout the world. This portal of bioinformatics utilizing individual myeloma patients' histories and related studies instantaneously brings scientists into collaboration, allowing them to drive forward with new hypotheses. Work being conducted around the globe toward finding cures for myeloma—and even other cancers—can be delivered into the hands of other experts in various and even disparate disciplines, all with the aim of creating new treatments and expediting research toward finding cures.

What began with one patient's refusal to accept the status quo has led to a remarkable push to expedite new trials and treatments for myeloma, *and* incorporate Big Data and cutting-edge technologies to finally make **Precision Medicine** a reality in myeloma.

"We are finally sharing all of this data, building team science, where everybody's looking at the data and getting new ideas and getting targeted trials to the clinics so much faster. That's what patients need. That's Precision Medicine in action."

Kathy Guisti
Founder and CEO / The MMRF & MMRC

NEW VISION ... NEW NETWORK ... NEW OPPORTUNITIES!

Quite simply, new drugs can't come to market if research can't create them!

But in order for researchers to discover the compounds to effectively treat myeloma, **they need access to the right data.**

Myeloma is a complicated disease. At the molecular level it has many subtypes, which will differ from patient to patient. Further, we've begun to see how different subtypes react differently to treatment based on the genomic profile of each individual patient. This is why two patients with the same disease can have vastly different reactions to the same medication. Researchers see it happening, but haven't yet been able to discover the formulas necessary to change it.

Part of unraveling this riddle rests with getting the relevant data in the hands of the experts. That's why the **MMRF Community Gateway** and the **MMRF Researcher Gateway** are so pivotal to the future of treatment for myeloma, and other cancers as well.

Some of the very drugs that the MMRF has played a role in bringing to market have been used in the treatment of 30 other types of cancer. Both Kyprolis (carfilzomib) and Pomalyst (pomalidomide)—which the MMRF and the MMRC played extremely important roles in quickly pushing through to approval—have been tried in the treatment of ((XXXX, OOOOO and XXXXX)) among other cancers.

We're helping to recreate the entire world of cancer research through the first open-access platforms for myeloma—the **MMRF Researcher Gateway** and the **MMRF Community Gateway**. Fueled by the **CoMMpass** study—and operating in tandem—this historic vision combines cutting-edge technology and Big Data with the proven commitment of the **MMRF** to drive treatments faster than ever before.

The **MMRF** is unique in its role as a trusted third-party. Building on a colossal consortium of experience, expertise and enthusiasm, we're driving treatment down roads never traveled before.

PROVE IT!

Did you know that many trials being conducted at any one time are sponsored by the **MMRC**? Are you aware that a wide variety of myeloma-related research programs and investigations around the world have been funded by **MMRC** researcher grants?

Since its inception, the **MMRF** has shown a laser-like focus on accelerating myeloma drug development. We've aided in expediting six different myeloma drugs through the research pipeline to FDA approval—Velcade, Revlimid, Thalomid, Doxil, Kryprolis and Pomalyst. Most recently, the **MMRF** and **MMRC** were instrumental in giving key clinical trial support to our member institutions for Kryprolis (carfilzomib) and Pomalyst (pomalidomide), bringing these to market as fast as was possible.

Over the past 15 years, the **MMRF** has invested more than \$225 million in research and drug development. We've advanced 46 clinical trials of more than 70 new compounds and combination approaches, with another six to eight expected by the end of 2013. Our trials are 60 percent faster than oncology's industry standard—meaning they arrive approximately 100 days sooner.

But what really makes the **MMRF** such a crucial instrument in combating myeloma is the remarkable network we've been able to create. We've forged a coalition of scientists, academic institutions, hospitals, doctors, pharmaceutical companies and patients. As an objective but passionate third party, the **MMRF** stands at the center of this coalition. By getting everyone on the same page and drawing on everyone's unique strengths and purposes, we're unifying efforts to keep the focus on what's most important—trying to find cures and realizing new treatments faster.

Numbers to Live By ...

(((STATS & CHARTS – T/K)))

FUELING THE FIGHT

The work of the **MMRF** over these past 15 years has brought us to this very historic juncture. We have imagined, designed, created and now constructed the mechanisms to drive **Precision Medicine** and Big Data research into the future. But now we need the fuel to fire up the engines and get these vehicles moving forward.

This is why we've launched a \$100 million campaign. The time has arrived to make our stand and engage the tools we've created to change history.

Bringing our historic CoMMpass study to final fruition alone will require approximately \$40 million. These funds allow the 50 cancer centers we're utilizing for this initiative to fuel our **MMRF Researcher Gateway** with the raw genomic data necessary to find answers. Without that data, there is nothing to investigate—nothing to explore.

If cures are going to be found, it will start with collecting pristine clinical and genomic data together over time.

It's important to know that more than 90 percent of the **MMRF** funds go directly to research and related programming. Out of thousands of charitable organizations, we're the only one to have received combined recognition that includes a 4-star rating from Charity Navigator, an A+ from the American Institutes of Philanthropy, compliance with the Better Business Bureau's standards for charity accountability, and approval from the National Cancer Institute for its research grants program.

We're the real deal ...

We want to find cures ...

We need your help to do it!

HELP MAKE HISTORY

The **MMRF** has embarked on a historic three-year, \$100-Million Campaign.

With the official launch of our **MMRF Researcher Gateway** and **MMRF Community Gateway**, the **MMRF** kicks off a drive to raise the necessary funds to fuel our new efforts. The initiatives are in place—we're moving and gaining speed! But in order to sustain it—in order to smash through the walls that have been blocking progress in myeloma, and cancer research in general—it's imperative that the **MMRF** receive adequate funding.

We need you ... And you know what's at stake!

There are different ways to help. Opportunities abound to bring your involvement to action.

- **CoMMpass** is a colossal endeavor that must receive adequate support in order to function. An estimated \$40 million will be needed over the next eight years in order to bring the study successfully to conclusion.
- **Clinical Funding** remains a cornerstone of the **MMRF**, and Precision Trials and Umbrella Trials through the **MMRC** are always in vital need of your support. **((FIGURES T/K))**
- **Biotech Awards** can make a tremendous difference in helping advance research. **((FIGURES T/K))**
- **New Technology** is also key. A range of cutting-edge developments must be supported in order to continue our work. **((FIGURES T/K))**
- **Epigenetics ((DETAIL, FIGURES T/K))**
- **Immuno-therapeutics** presents a new frontier in the research of myeloma. As we move forward with our mission, this is a key area that will garner more and more attention. **((DETAILS, FIGURES T/K))**
- **Venture Funding** is an ongoing need in order to stay on top of new developments. **((FIGURES T/K))**

The **MMRF** and **MMRC** have been there ...

Throughout the past 15 years, ((the greater number of)) drug trials have been funded by the **MMRF** and **MMRC**. We've also been there funding frontline researchers around the globe.

Our lifesaving contributions have made a difference ...

But our contributions wouldn't exist at all without *your* contributions!

Help let one patient's story speak to millions ...

What's a cure worth to you?