

# Precision Medicine in Myeloma

## Multiple Myeloma Research Foundation

For the Consideration of

DATE



**MMRF**<sup>®</sup>  
Multiple Myeloma  
Research Foundation

Powerful Thinking Advances the Cure<sup>SM</sup>

(Replace in all blue banners: **ACCELERATING CURES THROUGH PRECISION MEDICINE**)

## BEING PRECISE MEANS FASTER CURES

### Multiple Myeloma Research Foundation (MMRF)

#### REQUEST FOR FUNDING

It is our privilege to respectfully submit to \_\_\_\_\_ our proposal to consider a contribution to the development of Precision Medicine approaches at the Multiple Myeloma Research Foundation (MMRF). We are at the forefront of developing more-effective lifesaving treatments, extending the lifespan of myeloma patients, and changing the world of cancer research as we know it. Your philanthropic investment will help us accelerate cures for patients living with multiple myeloma and cancers of similar biology.

#### The MMRF – RESEARCH PIONEERS IN MYELOMA

Each year, 20,000 new cases of multiple myeloma, an uncommon and incurable blood cancer that originates in the bone marrow, are diagnosed in the United States. The good news is, promising new treatments continue to be developed through extensive research and substantial improvements in therapies have been made in the past decade thanks to the MMRF and its partners.

With a laser focus on accelerating drug development in myeloma, the MMRF has built a collaborative myeloma community, playing a role in delivering the next generation of treatments to patients as quickly as possible. Building critical mass of research efforts and data are paramount to making progress in an uncommon, highly heterogeneous disease such as multiple myeloma. Working with world-class, like-minded academic and industry partners to speed up the time of new discoveries and new therapies are being developed to deliver more cutting-edge treatment options for patients.

But, even with these advancements, the natural history of myeloma is often relapse following treatment. Therefore, it is imperative that we continue our research to understand the differences of myeloma subtypes and how we can treat patients with the most effective targeted therapies at their first point of intervention. This is the world of precision medicine in action.

The MMRF is in the top 1% of Nonprofits:



(Insert in blue banner: **ACCELERATING CURES THROUGH PRECISION MEDICINE**)

## WHERE WE ARE TODAY

When the MMRF was founded in 1998, patients with multiple myeloma had little hope. There was very little research on the disease. The same drugs to treat the disease had been used for several decades, dating back to the 1960's, with no new innovations on the horizon. As a result, patients had a life expectancy of only three years. Since then, the MMRF has done revolutionary work with researchers, clinicians and partners in the academia, biotech and pharmaceutical industries, bringing hope of a better future for patients and dramatically changing the treatment landscape.

Today, the MMRF has become the world's number-one private funder of multiple myeloma research, injecting more than \$225 million directly into research and related programming since its inception. The MMRF has been instrumental in launching six new FDA-approved myeloma drugs that have not only doubled the life expectancy among patients, but are also being tested or used in more than 30 other cancers.

In order to construct collaborative models to build critical mass in myeloma research and put speed into clinical trials with a diverse portfolio of investigational agents, in 2004 the MMRF launched the Multiple Myeloma Research Consortium (MMRC). The MMRC is now 16 centers strong, fielding more than 46 Phase I and II clinical trials of 24 drugs since its inception. Notably, trials within the MMRC launch 60% faster—approximately 100 days sooner than other trials at similar centers—an unparalleled achievement in cancer research.

In addition, The MMRF created the first and largest multicenter tissue bank, establishing guidelines for tissue collection and processing for molecular profiling and making it possible for researchers to conduct first-of-their-kind genomics and correlative science studies associated with MMRC clinical trials.

As a natural progression to developing the MMRC and the tissue bank, in 2006 the MMRF launched the Multiple Myeloma Genomics Initiative (MMGI)—a comprehensive program that mapped the multiple myeloma genome in its entirety. The MMGI has expanded its genome sequencing from 38 published patient samples to 250 completed. Together, with comprehensive genomic profiling, this is the largest dataset in myeloma captured on a public portal and viewed by more than 1,200 registered researchers.

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But more must be done. The MMRF must continue to invest in early stage research and increase the number of more selective drugs in clinical trials. We must reinforce our efforts to bring new drugs to approval stages using our track record of efficiency and speed as a trusted third party.

We also realize because of the extreme heterogeneity of this cancer, we must build our scientific knowledge of the disease, how it progresses, and why individuals respond differently to specific therapeutics. Combining that scientific knowledge allows us to identify more precise approaches toward disease management and a cure.

## THE FUTURE IS NOW

Where are we going? We know extending life is no longer good enough. Finding cures is the ultimate goal. The good news is we know how to get there; we drafted our initial Precision Medicine plan two years ago and we continue to execute as quickly as possible against the plan. The MMRF Precision Medicine plan focuses on big data, open access and a worldwide network.

The CoMMpass Study, launched by the MMRF in 2011, follows 1,000 patients from initial diagnosis through their course of treatment, over a minimum of 5 years; conducting sequential tissue samplings to identify how their molecular profile affects his or her clinical progression and individual response to treatment. This robust dataset is unique in that it ties clinical and genomic data together in a longitudinal effort.

Using the MMRF Researcher Gateway, scientists can look now at 'big data' of different groups of patients based on their disease characteristics, molecular and genomic alterations. Because myeloma is a heterogeneous disease we continue to aggregate not only CoMMpass and MMGI data but also world wide datasets because we need all of this data in order to make sense of the many myeloma subtypes. Simply put, the more data we have, the more precise we can be at finding cures.

The MMRF Community Gateway is a unique informational and social forum aimed at fostering precision care, giving patients access to information to help guide their choices and be a part of micro-communities focused on their particular sub-segment. Patients can target the trials and treatment opportunities relevant to their particular disease makeup. They can interact with other patients who share aspects of their unique condition and perhaps best of all, come to truly understand what drives their treatment and how they can play a vital role in hopefully finding cures.

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## PRECISION MEDICINE IN ACTION

The MMRF is leading the charge in bringing precision medicine trials to myeloma patients which involves coupling established clinical and pathological indexes with state-of-the-art molecular profiling to create diagnostic, prognostic, and therapeutic strategies precisely tailored to each patient's requirement — hence the term “precision medicine.” We are harnessing the wealth of data available from our new models and overcoming previous pitfalls that held cancer research back.

With extraordinary data becoming available, the MMRF works with the best scientists to identify new targets and clinical approaches. While CoMMpass and the MMGI generate data, the MMRF Gateways rapidly push it out to the world. The proposed clinical trials will be spearheaded by the MMRC with patients identified in the MMRF Community Gateway. The MMRF Community Gateway will work side by side with academia, industry and the FDA to drive efficient and rapid trials through the system.

The MMRF is setting the standard for the new transformative model of Precision Medicine for myeloma and developing treatment strategies to target molecular alterations.

## PHILANTHROPIC OPPORTUNITY

We believe Precision Medicine is the answer to finding cures for multiple myeloma and we're providing the vision, leadership, research, partnerships and communications platforms needed to bring urgency and speed to this endeavor, but significant investment is needed to generate the kind of results and impact we know is possible for myeloma and other cancer patients.

We have the proven track record to accelerate the pace of drug discovery and development and we are positioned to make Precision Medicine a reality for patients, which will lead to cures in our lifetime. By contributing to these goals, a select group of donors will play a vital role in rapidly accelerating critical advancements in the field of myeloma cancer research, extending patient lives and helping us find cures.

We respectfully ask the \_\_\_\_\_ to support the MMRF with a philanthropic commitment \$\_\_\_\_\_ in support of Multiple Myeloma Precision Medicine (MMPM). Your gift will represent an important life saving investment in support of our continued efforts to push the frontiers of science and bring leading edge treatments to patients in the fastest ways possible.

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We would be honored if you will consider supporting the MMRF in this ongoing critical effort to help patients living with this disease. Every second and every dollar counts. Just ask a multiple myeloma patient.

Thank you for your consideration and know we can't continue to change the way cancer research is accomplished without you.

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