

MULTIPLE MYELOMA RESEARCH FOUNDATION **ANNUAL REPORT** **2017**



The MMRF is in the top 1% of nonprofits:



Advancing Treatment Options for Patients

The MMRF Precision Medicine Model brings new treatments to patients faster. We do this with the power of big data, data sharing, and applying the best new science and technology to analyze that data.

As a patient-founded organization, we know how urgent these goals are. That is why we raise \$45M per year to accelerate precision medicine and fuel a pipeline of potentially life-extending drugs. With support from investors, we continue to advance our pipeline of therapies at the pace necessary to save lives.

We run our business with the same efficiency that we expect of our partners and investors. The MMRF has received a “Best in America” Seal of Excellence from the Independent Charities of America, and an “A+” rating from the American Institute of Philanthropy, earned Charity Navigator’s four-star rating for 13 years. We consistently outperform our peers in fiscal responsibility, accountability, and transparency. We direct nearly 90% of our total budget to research and related programming.

It is our vision to ensure that every patient
has what they need to prevent or defeat
multiple myeloma.





We are unstoppable in our pursuit of a cure.

Together with research and medical industry partners, The Multiple Myeloma Research Foundation (MMRF) fuels more studies, supports more trials, helps to develop more treatments, introduces more medicines, and continues to have a far greater impact than any other cancer research foundation.

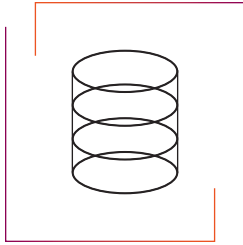
Our strong foundation and proven network is poised to engage the best new science and technology to accelerate development of treatments for patients—as quickly as possible. As a patient-founded organization, we know patients have no time to waste. Which is why we'd like you to be a part of this transformative moment.

MMRF Vision: Patient-powered collaboration

We see a world where every person has precisely what they need to prevent or defeat multiple myeloma, where the entire community collaborates seamlessly from beginning to end, with ever-increasing momentum towards a cure.

MMRF Precision Medicine Model

Our unique model removes barriers to cancer breakthroughs. The MMRF has the only end-to-end solution in cancer research. It is based on three interrelated pillars: The Patient Data Bank, The Learning Network, and The Clinic. Applying our innovative model to precision medicine—getting patients the right treatment at the right time—puts the promise of a cure within reach.



The Patient Data Bank: Data Generation and Integration

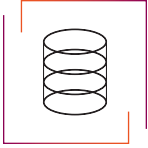
Because multiple myeloma is different for each patient, a critical mass of data is required to optimize treatments and continue the advancement of precision medicine. Collaborating with best-in-class partners like the Translational Genomics Research Institute (TGen) and the Broad Institute, we are uncovering deeper insights into the disease's biology that reveal important new drug targets and predictive markers of drug response and clinical outcome.

Today, with more than \$40M committed, the MMRF CoMMpass StudySM has the largest genomic data set of any cancer and is widely considered one of the most groundbreaking initiatives in the field of cancer research. Notably, all CoMMpass data is placed in the public domain, available to researchers across the world.

The MMRF CoMMpass StudySM Analysis – The Latest Discoveries

Data from the CoMMpass Study continues to deliver information of critical importance to patients, as evidenced by the latest insights reported at the 59th American Society of Hematology (ASH) Annual Meeting and Exposition in San Diego in December 2017. The MMRF was part of more than 30 presentations, 26 of them a direct result of CoMMpass.

- **Computer Model used to Predict High-risk Disease and Stem Cell Transplant Survival:** CoMMpass Study investigators along with researchers from Gene Network Sciences (GNS) examined CoMMpass data, together with data from other studies using a unique computer model. Results from this analysis have identified a pathway that may identify high-risk myeloma and characteristics of patients with long progression-free survival after stem cell transplants.
- **FGFR3 Mutations Suggest High-Risk Myeloma:** Genetic analyses of patients in the CoMMpass Study showed that a mutation in the FGFR3 gene leads to more serious disease. These results support the use of drugs that inhibit FGFR3 as a treatment for high-risk myeloma patients. This trial is one of the first precision medicine trials in multiple myeloma and one of three precision medicine trials now open in the MMRC.
- **New Insights on Stem Cell Transplant and Maintenance Therapy:** An analysis of CoMMpass data showed that patients who have a stem cell transplant (SCT) within one year of diagnosis delay progression longer than patients who have SCT later. In addition, patients who have a SCT have a longer progression-free period than patients who have no transplant at all. Finally, there was no difference in progression delay or overall survival in patients who had treatment after SCT versus patients who had no treatment following transplant.



CoMMpass Overview

- The MMRF CoMMpass StudySM has enrolled 1150 patients from 90 sites worldwide
- Each patient is checked every six months for eight years
- The average age of a CoMMpass patient is 64
- 66% are male
- 77% of participants are Caucasian, 17% are African American, 2% are Asian and 5% are “other”

Molecular Profiling Initiative: Update

2016 marked the beginning of a partnership with the University of Michigan to complete clinical-grade sequencing for 500 relapsed patients to match them to precision trials based on the results.

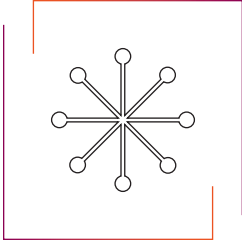
The purpose of the Molecular Profiling Initiative (MPI) is to provide timely Clinical Laboratory Improvement Amendments (CLIA) genomic sequencing information to multiple myeloma patients and their doctors, allowing them to make informed treatment decisions and identify potential treatment options, including clinical trials, based on a patient’s genomic alterations.

The MMRF Molecular Profiling Initiative is part of the MMRF’s broader effort to accelerate research toward a cure, and will contribute to our goal of improving the understanding of the disease and its impact on each individual patient. De-identified data from the MPI will complement and augment existing datasets from the MMRF Multiple Myeloma Genomic Initiative (MMGI) and the MMRF CoMMpass Study.

As of December 2017, enrollment includes over 250 patients. The study is open at 18 MMRC sites with one site pending approval.

“The Molecular Profiling Initiative has been tremendously useful in our center, which gets a large number of challenging patient referrals with relapsed and refractory myeloma. We are using both the DNA mutational data as well as RNA expression of ‘outlier’ genes to make treatment decisions.”

SAMIR PAREKH, MBBS
The Mount Sinai Health System
New York, NY



The Learning Network: Collaboration and Discovery

We have made it a priority to share MMRF data with the public, accelerating research and discovery.

- The **MMRF Researcher Gateway** is a revolutionary patient information ecosystem that makes key genomic and clinical data accessible to all scientists and clinicians. Researchers from around the world can use the data to form new hypotheses, driving discoveries in multiple myeloma
- **Myeloma Disease Modeling**: through a partnership with GNS Healthcare, we are applying leading-edge computer models and analytics to uncover disease pathways, biomarkers, and novel targets
- The **Translational Network of Excellence** unites the best in class academic medical centers to establish state-of-the-art, pre-clinical models and collaboratively validate new emerging targets

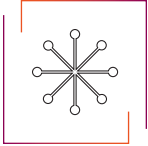
Continued open access to the latest information from the CoMMpass study, the most comprehensive genomic data set in cancer today, ensures the fastest possible development of new precision treatments for myeloma patients and may ultimately lead to the discovery of a cure.

This year, the MMRF contributed the largest genomic data set of any cancer to the National Cancer Institute's Genomic Data Commons (GDC). The GDC allows researchers to browse the data and make discoveries within the GDC environment, and then download only the subset of the data that is useful for their research.

“The MMRF has proven itself a leader in scientific innovation. ...[A]s a leader in data sharing, they have donated their data to the GDC with the goal of improving the lives of their patients and cancer patients worldwide.”

LOUIS M. STAUDT, MD, PHD

*Co-Chief, Lymphoid Malignancies Branch, Center for Cancer Research,
National Cancer Institute, National Institutes of Health*

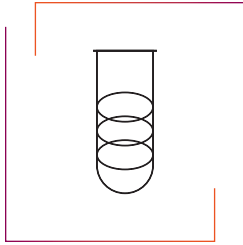


The MMRF Answer Fund

The Multiple Myeloma Research Foundation (MMRF) is investing \$5 million over the next three years in a new initiative titled **The MMRF Answer Fund**, a multifaceted effort addressing important questions facing the multiple myeloma community and advancing precision medicine. The Answer Fund will leverage data from the landmark MMRF CoMMpass StudySM, which is the largest collection of genomic data of any cancer, tracking more than 1,100 myeloma patients over eight years.

Phase I of the Answer Fund project will support research around high-risk myeloma. One of the most urgent questions facing the multiple myeloma community is how to define and treat high-risk patients.

Phase II of the Answer Fund involves a crowdsourcing effort which includes the entire myeloma community. Patients, health care providers, and researchers were asked to provide their opinion on which question in myeloma treatment is the most pressing to answer immediately.



The Clinic: Accelerating Trials

A first-in-class worldwide network

Our ultimate goal is to put life-saving treatments into the hands of patients as quickly as possible. We are doing that through innovative collaboration among cancer centers that produces unprecedented results. To date, the MMRC has conducted 76 clinical trials, 24 of which are currently ongoing.



25 leading centers worldwide



76 clinical trials



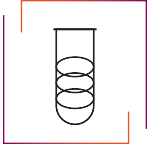
35 new treatments for multiple myeloma

2017 ACTIVE CLINICAL TRIALS

| | NOVEL AGENTS/MECHANISMS | ANTIBODIES & IMMUNE | PIS & IMIDS | MOLECULARLY TARGETED |
|--------------------|--------------------------------------------------------------------------------------------------------------------------------------------------------|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------|--------------------------------------------------------------|-------------------------------------------------------------------------------------------------------------------|
| RELAPSED OR R/R | <p>ACE011</p> <p>Ibrutinib/Kyprolis</p> <p>Ruxolitinib/Kyprolis (Car-Jak)</p> <p>Venetoclax/Ninlaro</p> <p>Selinexor/Dex</p> <p>Selinexor/Kyprolis</p> | <p>Isatuximab/Kyprolis</p> <p>Empliciti/Pomalyst/Dex</p> <p>Rev/Dex/Empliciti +/- Cyclophos (Amyloidosis)</p> <p>Empliciti/Pomalyst/Velcade/Dex</p> <p>Isatuximab</p> | <p>Pomalyst/Kyprolis/Dex</p> <p>Pomalyst/Ninlaro/EMD/PCL</p> | <p>Idasanutline/Ixa 17p deleted</p> <p>PINR Biomarker-driven</p> <p>Dabrafenib/Trametinib</p> <p>JNJ-42756493</p> |
| NEW DX./TRANSPLANT | <p>Empliciti/Rev/Velcade/Dex</p> | <p>Empliciti/Kyprolis/Rev/Dex</p> | <p>Ixazomib/Revlimid/Dex Transplant*</p> | |
| SMM | | <p>Empliciti/Rev/Dex</p> | | |

| KEY | Phase 1 | Phase 1/2 | Phase 2 |
|-----|---------|-----------|---------|
|-----|---------|-----------|---------|

*There are two similar but distinct studies led by Washington University and the University of Chicago.



Clinical Trial Highlights

Revlimid®/Dexamethasone/Empliciti™ for Patients with Relapsed AL Amyloidosis

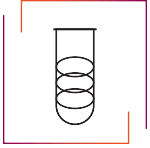
Amyloidosis is a serious complication of multiple myeloma that affects up to 30% of myeloma patients at some point. This year, the MMRC has added an amyloidosis trial to their trial lineup for the first time ever! This trial is studying how well linalidomide, dexamethasone, and Empliciti with or without Cytoxan work in treating patients with primary amyloidosis that has come back after a period of improvement. These are chemotherapy drugs that work in different ways to stop the growth of cancer cells by killing them or stopping them from dividing or spread.

Tafinlar and/or Mekinist in Patients with Relapsed and/or Refractory Multiple Myeloma and BRAF/NRAS/KRAS Mutations

This pilot is evaluating two drugs for the first time in myeloma. Patients with multiple myeloma whose tumors have a mutation in the BRAF, NRAS, or KRAS genes will be given one of three treatments – dabrafenib plus trametinib, dabrafenib alone, or trametinib alone. These mutations were first documented in multiple myeloma through research done by the MMRF in our MMGI and CoMMpass studies. Dabrafenib and trametinib work to block the BRAF, NRAS, and KRAS mutant genes from contributing to the spread of myeloma.

FGFR3 Inhibitor JNJ-42756493 with Dexamethasone for the Treatment of Relapsed or Refractory Multiple Myeloma

This study aims to determine how effective investigational drug JNJ-42756493 is when given together with dexamethasone. The study is looking at two groups of patients. The first group have tumors with a mutation in a gene called Fibroblast Growth Factor Receptor-3 (FGFR3), which may be involved in the growth of myeloma. The second group of patients has tumors containing a normal FGFR3 gene. JNJ-42756493 has shown effectiveness against tumors with mutant FGFR3 and may boost the effectiveness of dexamethasone for these patients.



The MMRF Immunotherapy Initiative

In 2017 we launched a three-year, \$15 million **Immunotherapy Initiative**, aimed at identifying which myeloma patients might respond to immunotherapy treatments. The Initiative is centered around the creation of Networks of Excellence, which will bring together top researchers from different institutions for collaborative work in three areas:

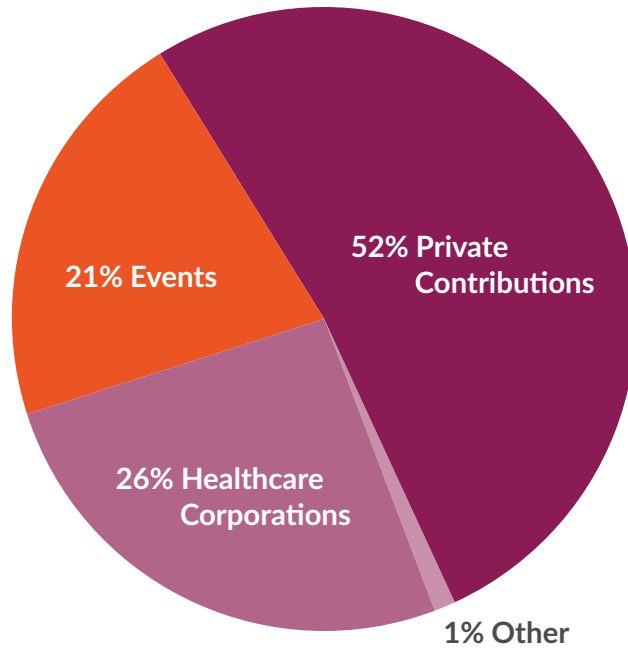
- Creating new tests to identify patients likely to respond to immunotherapy
- Identifying mechanisms of resistance to immunotherapeutic treatments
- Establishing clinical trials of new immunotherapy treatments for myeloma

The MMRF Prevention Project

Last year the MMRF launched the first ever research program solely dedicated to the early detection and prevention of multiple myeloma. A multimillion dollar gift from the Perelman Family Foundation provided seed funding for the groundbreaking **Perelman Family Foundation Early Disease Translational Research Program**, part of the **MMRF Prevention Project**, to speed efforts toward early detection, delayed disease progression, and eventually, ultimately, prevention of this incurable disease. Through partnerships with six leading researchers, we will focus on:

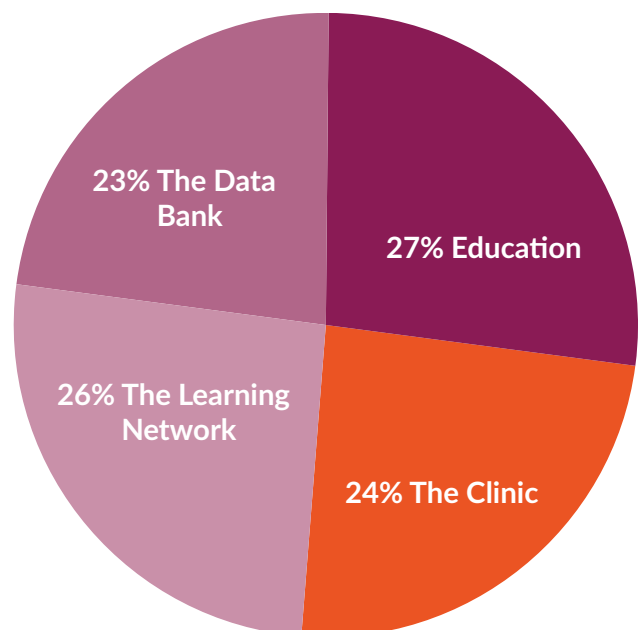
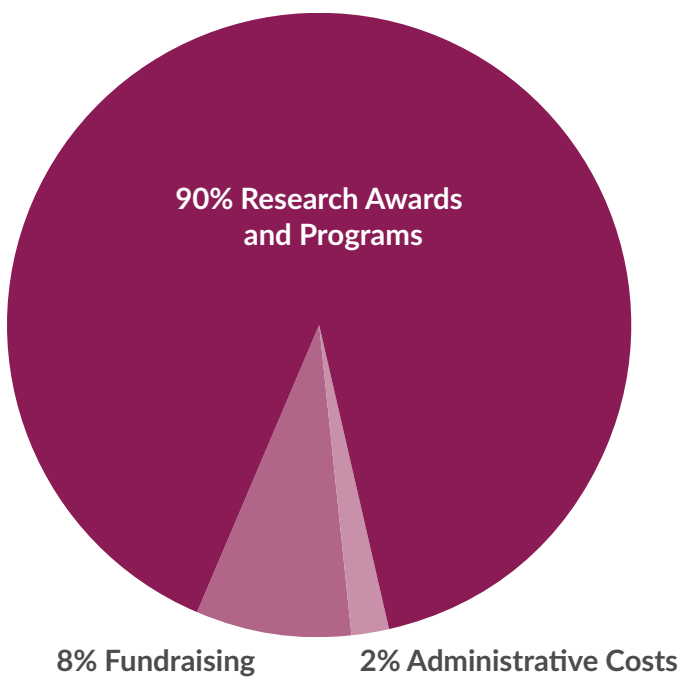
- Better understanding of genomic determinants of early disease progression
- Impact of micro-environmental factors on early disease progression
- Enhancing tumor immunity in MGUS/SMM
- Building out the smoldering myeloma data set and trials.

MMRF 2017 Source of Funds*



*Based on gross revenue

MMRF 2017 Spending Allocations



Multiple Myeloma Research Foundation, Inc.

Statements of Activities (Audited) – Years Ended December 31, 2016 and December 31, 2017

| Support and Revenue | 2016 | 2017 |
|--------------------------------------|---------------------|---------------------|
| Contributions | \$25,964,322 | \$34,274,956 |
| Fee for service | 2,377,395 | 3,005,435 |
| In-kind contribution | 394,136 | 294,932 |
| Royalties | - | 1,997,388 |
| Special Events | | |
| Special events support | \$11,513,204 | \$10,526,860 |
| Less special events expenses | (4,064,371) | (4,559,448) |
| Investment return | 186,583 | 426,247 |
| Total support and revenue | \$36,331,269 | \$45,966,370 |
| Expenses | | |
| Program | | |
| Research | \$16,419,485 | \$20,316,447 |
| Education | 4,259,707 | 4,618,557 |
| Awareness | 3,946,527 | 2,871,963 |
| Total program Expenses | \$24,625,719 | \$27,806,967 |
| Supporting services | | |
| Management and general | \$711,765 | \$481,104 |
| Fundraising | 2,185,536 | 2,543,459 |
| Total supporting services | 2,897,291 | 3,024,563 |
| Total expenses | \$27,523,010 | \$30,831,530 |
| Change in net assets | 8,808,259 | 15,134,840 |
| Net assets, beginning of year | 16,938,766 | 25,747,025 |
| Net assets, end of year | \$25,747,025 | \$40,881,865 |

2017 Corporate Information

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MULTIPLE MYELOMA RESEARCH FOUNDATION

OUR MISSION

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Thank you for supporting the MMRF
in accelerating cures and saving lives.



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