

MMRF Annual Report 2015





In December 2015, we experienced something truly remarkable. In the span of 15 days, three new multiple myeloma drugs were approved by the FDA – a first for any cancer.

Just 10 short years ago, there were limited drug-treatment therapies for multiple myeloma patients. Today there are now 10 therapeutic agents – giving patients more treatment options than ever before.

Having been on the front lines in the development of these new drugs, all of us at the MMRF are incredibly gratified to see such dramatic progress being made. Yet we know that multiple myeloma is still incurable and that we must push every day to develop new therapies.

Generating, analyzing and sharing large quantities of high-quality patient data is central to driving the discoveries that can lead to novel, life-saving treatment options. This lies at the heart of the MMRF Precision Medicine Model.

Indeed, our goal of doctors providing a specific treatment based on a patient's genetic makeup is within sight. We achieved yet another notable accomplishment when we enrolled our 1,000th patient in the landmark MMRF CoMMpass StudySM – the largest, most comprehensive genomic data set in all of myeloma – and second largest

in all of cancer. More than 90 cancer research centers worldwide are now taking advantage of this data.

Getting a large and fragmented cancer research community to embrace this new idea of open and “democratic” access to data is complex and requires leadership. With the continued help of our incredible and passionate community of supporters and partners, I’m confident that the perseverance and progress that drove the results we’ve achieved to date will produce even more breakthroughs. At this transformational time in cancer research, we are in it together, and we couldn’t be more appreciative of your support.

Sincerely,

A handwritten signature in blue ink that reads "Paul Giusti". The signature is stylized and fluid.

Paul Giusti
President and Chief Executive Officer
Multiple Myeloma Research Foundation

WE ACCELERATE CURES

The MMRF is dedicated to finding innovative ways to fight cancer. Our mission is to accelerate cures and save lives.

Before the MMRF, there were no new treatments in the past decade. Today, myeloma has seen more drugs approved than any other cancer.

We have built an end-to-end system in precision medicine that collects a wide range of patient data, encourages the open sharing of data, and accelerates clinical trials to make treatments available faster.

We are working in partnership with today's leading companies and research institutions to develop molecularly targeted, immune and novel therapies, to get the right treatment to the right patient, at the right time.

Because the MMRF was founded by a patient, our system was built around the patient. It is the reason we are able to deliver better results, faster and more efficiently.

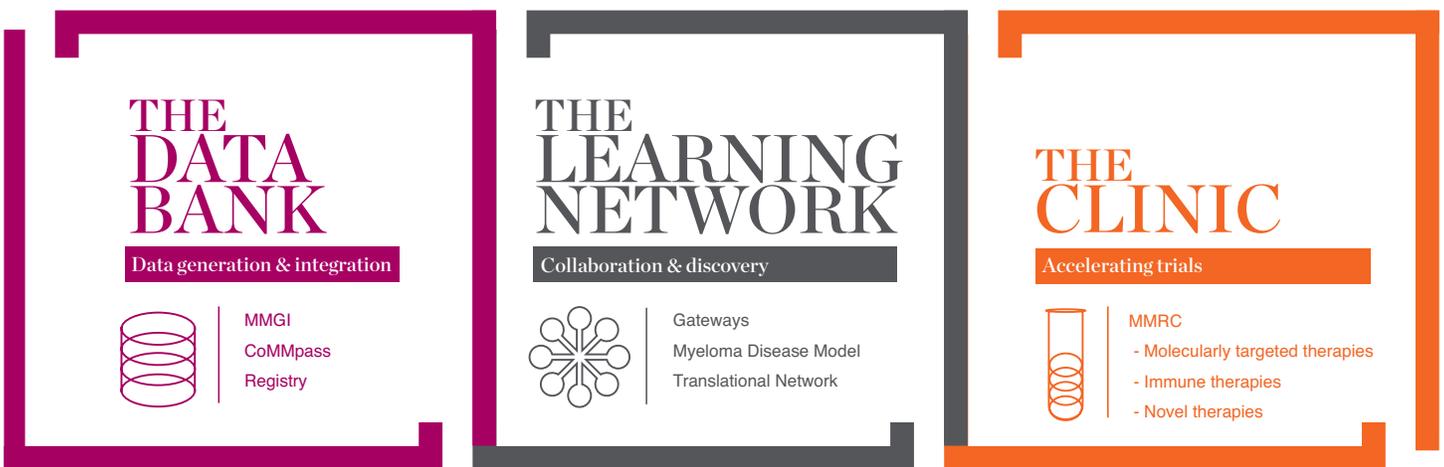
themmrf.org

MMRF 2015 Highlights

An unprecedented four multiple myeloma treatments were approved this year:

- **Darzalex®** (daratumumab) is the first monoclonal antibody to be approved in multiple myeloma. It targets a common protein found on myeloma cells, CD38, and exerts an attack directly on the cell.
- **Empliciti™** (elotuzumab) is the second monoclonal antibody approved for myeloma. It targets a myeloma-unique surface protein, SLAMF7.
- **Ninlaro®** (ixazomib) is the first oral proteasome inhibitor (PI) to be approved by the FDA.
- **Farydak®** (panobinostat) is the first approved myeloma therapy in a class of medicines known as histone deacetylase inhibitors. Farydak also inhibits the growth of new blood vessels needed to fuel tumors.

MMRF Precision Medicine Model



The Data Bank

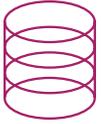
It all starts with data, so we created ways to generate it, gather it, decode it, and track it over time. The MMRF Data Bank is a goldmine of longitudinal genomic and clinical data.

The Learning Network

By pushing our valuable data to the public domain and creating incentives for academia and industry to share their learnings (instead of safeguarding them until they are published), we accelerate research and discovery.

The Clinic

Data and Learning produce treatments to be tested. Our own clinical network, the Multiple Myeloma Research Consortium, helps us speed promising new therapies to trial, and the patients who need them.



THE DATA BANK

MMRF CoMMpass StudySM

The MMRF CoMMpass Study (Clinical Outcomes in Multiple Myeloma to Personal Assessment of Genetic Profiles) is the cornerstone of our Precision Medicine Initiative, and is now yielding extraordinary insights into new targets for drug development as well as new ideas on how to identify and treat high-risk patients. Launched in 2011, it is the first large-scale, longitudinal study in multiple myeloma. It is a \$40 million effort funded by the MMRF in conjunction with our pharmaceutical partners.

In 2015, the CoMMpass Study reached its target enrollment of 1,000 multiple myeloma patients, a remarkable achievement for a relatively rare disease, and a testament to the commitment of patients to help advance the study of multiple myeloma. Baseline characteristics of the CoMMpass population show broad representation and are characteristic of myeloma patients in the general population. The average age was 64 years and the majority of participants were males of non-Hispanic/non-Latino descent. Approximately 16% of CoMMpass patients self-reported as African American, an important feature, given the higher incidence of persons of African descent in the myeloma population compared to the general population.

The MMRF CoMMpass Study involves an active assessment schedule, including bone marrow samples when first diagnosed, to establish a baseline, then again at response to treatment, and at relapse. Each patient is followed for up to eight years and their genomic and clinical data is analyzed every six months.

“The MMRF CoMMpass Study is the single most important thing going on in the multiple myeloma world.”



– David Siegel, MD, PhD
Chief of Multiple Myeloma
Hackensack University Medical Center

Current Findings:

- Preliminary findings demonstrate improved progression-free survival with triplet therapy versus doublet therapy.
- Research also indicates improved progression-free survival with triplet therapy followed by stem cell transplant compared to triplet therapy alone.
- Integrative analyses using CoMMpass data will help identify patients at greater risk of progression at diagnosis and optimize their treatment from the beginning of their disease course.
- CoMMpass is helping to confirm and expand the list of genes that are altered and likely play a role in myeloma.
- CoMMpass highlights the importance of understanding the form of the disease (clones) present in a patient at the time a new treatment is considered.
- By looking closely at the molecular changes happening at disease progression, one can identify potential new therapeutic options.

Launched the MMRF Molecular Profiling Initiative (MPI)

This initiative provides Clinical Grade sequencing for patients who relapse or become refractory to existing drugs. This sequencing offers essential guidance in identifying appropriate clinical trials or approved treatments that may immediately benefit this group of patients.



THE LEARNING NETWORK

Partnership with GNS Healthcare

The MMRF entered its second year of a collaboration with GNS Healthcare, a leading data analytics company that applies causal machine learning technology to match health interventions to individual patients. Using data generated by CoMMpass, the MMRF/GNS Healthcare partnership, also termed the Myeloma Disease Model, is identifying potential drivers of clinical outcomes and their associated molecular pathways.

Translational Network

The MMRF recently established a transformative MMRF Translational Network of Excellence, which is focused on the most promising research on novel preclinical models for new targets and drug validation, immune

biology, immune therapeutics, and minimal residual disease in myeloma and myeloma-related diseases. This groundbreaking initiative has been made possible due to decade-long efforts by the MMRF to generate assets and create a highly integrated clinical consortium. Participating centers include Mayo Clinic, University Health Network (Toronto); University of Navarra; Mount Sinai; MD Anderson; UCSF; and Dana-Farber.

The Community GatewaySM

There are currently 3,068 active participants in the MMRF Community Gateway. This gateway provides an online community where patients can share their journey as active participants with other patients, researchers, industry and clinicians.

MMRF Research Grants Summary 2015

MMRF Senior Research Awards Fund Important Discoveries

Recipient	Institution	Title
Nizar Bahlis, MD, Associate Professor	University of Calgary Calgary <i>Alberta, Canada</i>	CRL4a-CRBN E3 ligase hypermorphic and neomorphic repurposing in myeloma
Esteban Ballestar, PhD	Bellvitge Biomedical Research Institute <i>Barcelona, Spain</i>	Epigenomics of Myeloma Plasma Cell, Osteoblast and Osteoclast Interactions
Alan Lichtenstein, MD	Brentwood Biomedical Research Institute <i>Los Angeles, CA</i>	Regulation of c-myc translation by hnRNPA1
Robert Orłowski, MD, PhD	University of Texas, MD Anderson Cancer Center <i>Houston, TX</i>	Validating Novel Approaches Targeting High-risk Deletion 17p Myeloma
Fenghuang Zhan, MD, PhD, Professor	University of Iowa <i>Iowa City, IA</i>	NEK2 plays a critical role in myeloma minimal residual disease

The MMRC activated four new clinical trials bringing our portfolio total to 18 available for patient enrollment.

- Smoldering Multiple Myeloma trial investigating the effects of Darzalex® (daratumumab), an anti-CD38 antibody in SMM patients was activated sponsored by Janssen Boitech, Inc. The drug was approved in 2015 and works by harnessing the body's immune system to destroy myeloma tumor cells.
- SMM trial was opened at Dana-Farbar. The study investigates adding a novel biological therapy, Emluciti (elotuzumab), into the treatment regimen of Revlimid® (lenalidomide) and dexamethasone. The goal of the study is to identify the role of early intervention with this novel drug combination to prevent the progression in patients with high-risk SMM to active myeloma.
- First trial opened on a class of drugs known as immune checkpoint inhibitors. Phase 1 study of MPDL3208A from Genentech, which is an anti-PDL-1 immune checkpoint antibody, is being given to early relapsed patients as a single agent to patients who have residual disease after autologous stem cell transplant.
- The MMRC is also exploring novel agent combinations such as the previously reported company-sponsored trial with the company Karyopharm Therapeutics. In this study, the novel agent selinexor is being tested in combination with low-dose dexamethasone in highly refractory patients.

Patient and Professional Education

Webinars and CME Programs



The MMRF provides web-based education to help people living with multiple myeloma learn about the latest advances in treatments and clinical research. Access updates from major medical meetings and other timely information to understand the important progress in multiple myeloma today for patients and healthcare providers. Last year we hosted three CME programs with a total of 1,000 participants.

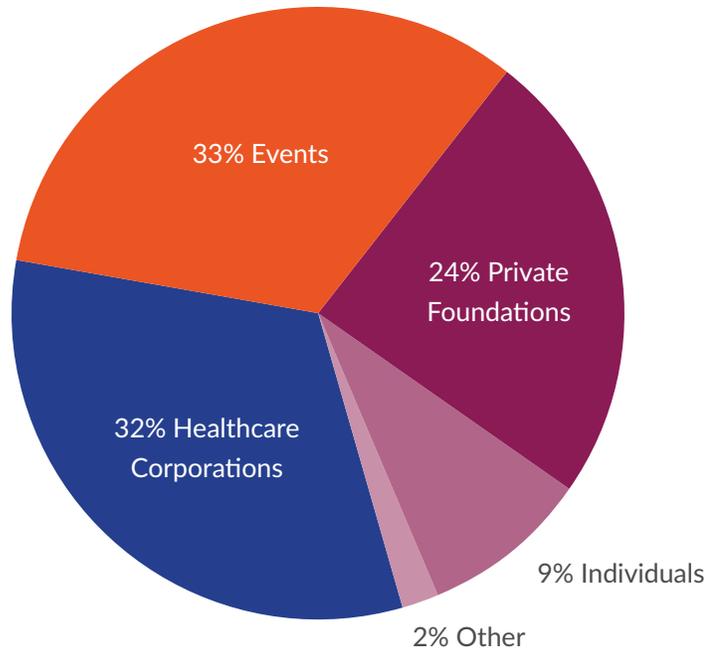
Patient Summits



The MMRF also provides education programs for people who are living with multiple myeloma. In 2015, we held patient summits in New York, Atlanta, Boston, Chicago and Seattle. Over 1,000 patients, family members, caregivers and healthcare professional attended these events.

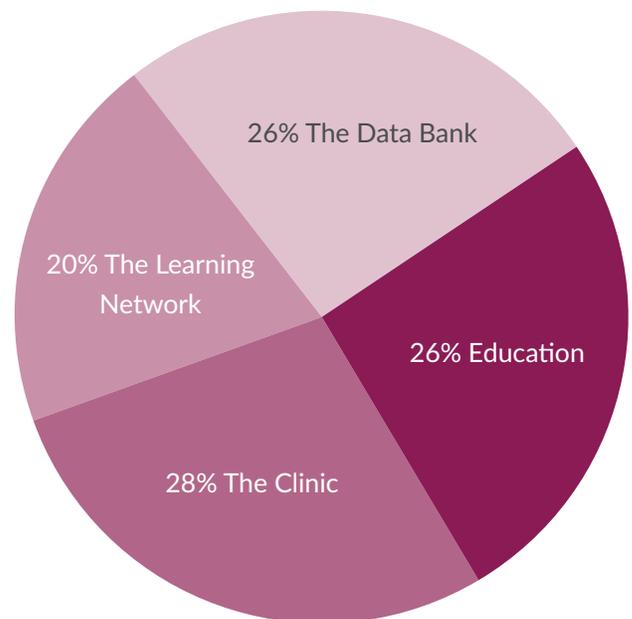
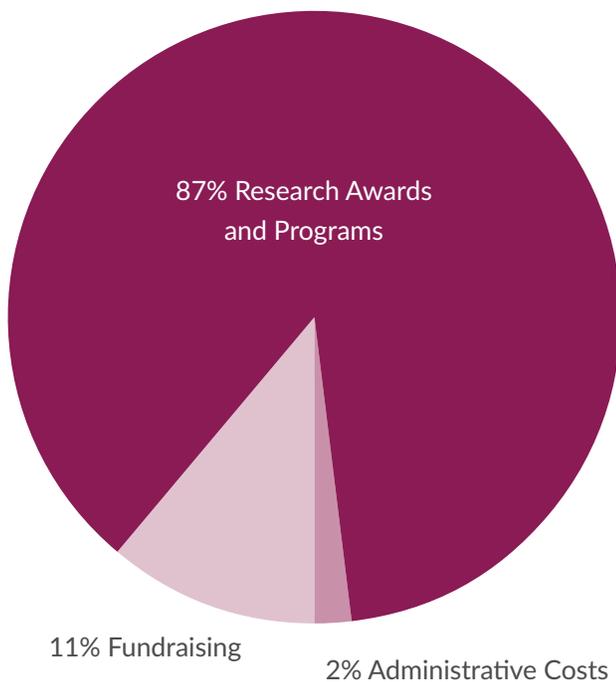
Financial Summary

MMRF 2015 Source of Funds*



*Based on gross revenue

MMRF 2015 Spending Allocations



Multiple Myeloma Research Foundation, Inc

Statements of Activities (Audited) – Years Ended December 31, 2014 and December 31, 2015

Support and Revenue	2014	2015
Contributions	\$10,216,080	\$10,430,576
Private foundation grants	7,850,301	7,542,882
Fee for service	878,000	2,948,004
In-kind contribution	210,000	146,377
Special Events		
Special events support	\$10,261,158	\$10,578,021
Less special events expenses	(2,927,000)	(3,926,485)
Investment return	99,730	71,422
Total support and revenue	\$26,588,269	\$27,790,797
Expenses		
Program		
Research	\$16,657,964	\$19,002,925
Education	3,352,633	3,935,287
Awareness	2,323,773	2,806,094
Total program Expenses	\$22,334,370	\$25,744,306
Supporting services		
Management and general	\$707,470	\$625,834
Fundraising	2,895,145	3,245,605
Total supporting services	3,602,615	3,871,443
Total expenses	\$25,936,985	\$29,615,749
Change in net assets	\$651,284	(\$1,824,952)
Net assets, beginning of year	18,112,434	18,763,718
Net assets, end of year	18,763,718	16,938,766

**At the end of 2014, the Multiple Myeloma Research Consortium was merged into the Multiple Myeloma Research Foundation.*

2015 Corporate Information

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Robert Wolf

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