

Multiple Myeloma Research Foundation Annual Report 2013



This is how cures are found.

Letter from the President and CEO



Dear Friends,

In 2013, the Multiple Myeloma Research Foundation (MMRF) experienced one of its most ambitious and innovative years to date as it launched a revolution in precision medicine for multiple myeloma patients. This evolutionary next step in the organization's history harnessed the power of big data, open access, and a worldwide network – working towards a goal of precisely tailoring therapies to each patient's individual molecular profile.

The MMRF also joined the myeloma community in celebrating the historic FDA approval of Pomalyst® (pomalidomide) from Celgene Corporation, an IMiD (in the same class as Revlimid®) as a treatment for myeloma patients with relapsed and refractory multiple myeloma – marking the second accelerated approval for myeloma in less than a year. The Multiple Myeloma Research Consortium (MMRC) collaborated closely with Celgene for more than five years to accelerate early clinical trials that led to Pomalyst's approval. The approval of Pomalyst is important because it offers even patients whose disease no longer responded to available treatments a new and much-needed option.

In 2013, the MMRC opened six new trials and has a record number of trials in the start-up process. As a result, at least seven trials are anticipated to start in the first half of 2014 alone, with as many as four trials anticipated to start in the second half of 2014.

Since the first patient enrolled in 2011, the MMRF CoMMpass Study continues to revolutionize longitudinal genomic and clinical data collection to identify molecular segments and variations in multiple myeloma patients. The study is designed to profile multiple myeloma in a way that no other myeloma trial has attempted – by comprehensively characterizing the disease in over 1,000 newly diagnosed patients and obtaining their clinical and genomic information over a course of 10 years.

To maximize the MMRF CoMMpass Study and adding speed to drug discovery and drug development, the MMRF launched two open-access gateways in September 2013: The MMRF Researcher Gateway and the MMRF CoMMunity Gateway.

Letter Continued

- MMRF Researcher Gateway—allows the open sharing of CoMMpass data with the scientific community, providing doctors and researchers worldwide with some of the most extensive genomic sequencing and 'big data' in oncology on an ongoing basis. All inventions and discoveries based on this data are dedicated to the public domain – nothing can be patented. The most brilliant minds will have open access to the data and they will work collaboratively to speed up new and even more effective therapies for patients leading to potential cures for many subtypes.
- MMRF CoMMunity Gateway—empowers myeloma patients to connect to other patients like themselves, share their own clinical data with the myeloma community and take action by joining clinical trials with treatment opportunities specific to their type of multiple myeloma.

In continuing our dedication to responsible financial stewardship, the MMRF was gratified to receive a four star rating for the 11th consecutive time by Charity Navigator, the highest acknowledgement possible for financial responsibility and fiscal management. This honor reconfirms what we already know: that the MMRF demonstrates every day the exceptional intensity of its ongoing commitment to its patients and its donors.

As transformational as 2013 was for fast-tracking new treatments for myeloma patients, 2014 is shaping up to be even more remarkable in terms of breakthroughs and the record number of clinical trials opening. We believe curing multiple myeloma remains a possibility in our lifetime. There is much more to do and with your support we can continue our journey of lifesaving work.

Thank you for the progress that you continue to make possible.

Sincerely,

A handwritten signature in blue ink that reads "Walter M. Capone". The signature is fluid and cursive, with the first letters of the first and last names being capitalized and prominent.

Walter M. Capone
President and CEO
MMRF

Highlights

Your generosity is the catalyst that has raised more than \$250 million to benefit the MMRF—allowing us to accelerate research for this small and uncommon disease. We have a history of producing results and speeding up treatments that have extended the lives of myeloma patients the world over.

In 2013, we:

- Helped accelerate the approval process of Pomalyst® by the FDA for the treatment of patients with relapsed and refractory multiple myeloma who no longer responded to available therapies. The MMRC entered into collaboration with Celgene and bolstered the clinical development of Pomalyst, facilitating the Phase I and Phase II clinical trials that provided the basis for the approval.
- Strengthened the MMRC pipeline by starting up or finalizing negotiations to open more trials than in 2012—the fruits of which will mostly be seen in 2014.
- The MMRC launched the following important trials: SAR + Len +Dex, ARRY 520 / Kyprolis, and ABT199 + Velcade + Dex.
- Completed more than 425 patients enrolled in the MMRF CoMMpass Study.
- Interim Analysis 5 completed: 420 patients with clinical data; 198 patients sequenced: 190 with full (whole genome, whole exome and RNA) sequencing, seven with partial profiling and one relapse patient sample completed.
- The MMRF Researcher Gateway and the patient-centric MMRF CoMMunity Gateway drove broader participation from the community – from researchers and patients alike – to speed the process of bringing novel and precision medicine to patients.
- Welcomed new international research partners from Spain to the MMRF CoMMpass Study.
- Received our 11th consecutive 4-star rating—the highest rating possible—from Charity Navigator, the nation's premier independent evaluator of charitable organizations, a distinction only one percent of nonprofits have received. We also received an A+ rating from CharityWatch.



“CoMMpass data will help us identify patient sub-types that respond in a particular way to specific treatment regimens. Our hope is these treatments will offer the ability to effectively manage myeloma long-term.”

*Sundar Jagannath, MD
Mount Sinai Hospital, NY*

Highlights Continued

- Provided MMRF educational materials to 10,000 myeloma patients and caregivers annually. In 2013, the MMRF Patient Support Center was launched – providing complimentary direct access to myeloma nurse specialists to guide patients through their myeloma journeys, help them better understand their treatment options and access to clinical trial information.
- Funded \$775,000 in Senior Research Awards and Fellow Awards, as well as a \$1.5 million multi-institutional, international program to further understand epigenetics in myeloma biology and treatment options.

MMRF SENIOR RESEARCH AWARDS

MMRF Senior Research Awards fund cutting-edge basic science and pre-clinical research focused on accelerating the development of novel treatments.

Aaron Rapoport, M.D.

University of Maryland
Supported by the Holway Family

Sherie Morrison, Ph.D.

University of California, Los Angeles
Supported by the Assink Family

MMRF FELLOW AWARDS

MMRF Fellows Awards support innovative research conducted by talented young investigators working under the supervision of a senior research mentor. The MMRF acknowledges generous contributions from Amgen, Celgene, Genentech, Millennium, and Onyx in support of the five awards.

Nikolay Sushin, Ph.D.

Harvard Medical School

Barbara Muz, Ph.D.

Washington University

Chakra Chaulagain, M.D.

Tufts Medical Center

Leslie Robertson, Ph.D.

University of California, San Diego

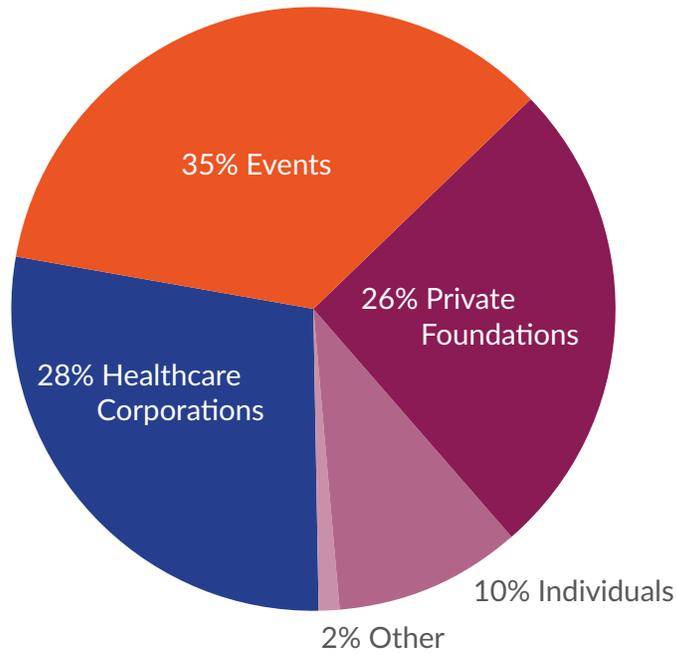
“With the help of the MMRF, I participated in a clinical trial of Pomalyst (pomalidomide) and achieved a near complete response. Thanks to the efforts of the MMRF and their partners, the newly FDA-approved Pomalyst is now available for patients like me that needed new treatment options.”

*Barry Samson, MD
Diagnosed with multiple myeloma in 2002*



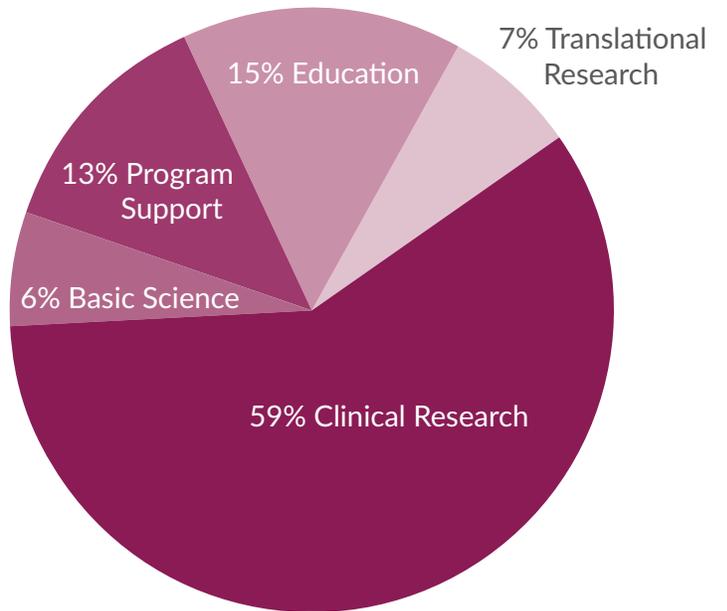
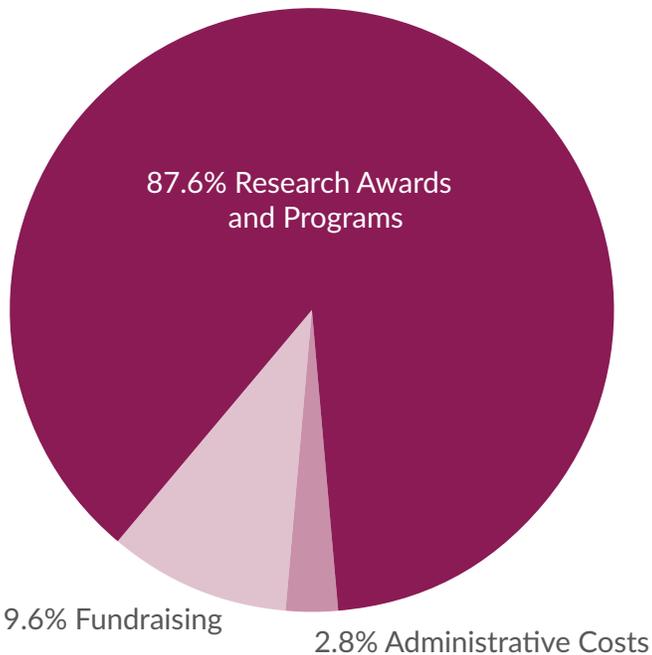
Financial Summary

MMRF 2013 Source of Funds*



*Based on gross revenue

MMRF 2013 Spending Allocations



Multiple Myeloma Research Foundation, Inc

Statements of Activities (Audited) – Years Ended December 31, 2013 and December 31, 2012

Support and Revenue	2013	2012
Contributions	\$9,275,518	9,887,802
Private foundation grants	6,676,708	8,250,701
Fee for service	810,010	5,006,210
In-kind contribution	150,521	344,623
Special Events		
Special events support	8,960,970	8,695,871
Less special events expenses	(2,567,606)	(2,621,075)
Investment return	66,306	308,598
Total support and revenue	23,370,427	29,872,730
Expenses		
Program		
Research	16,571,611	20,227,722
Education	2,367,890	2,087,083
Awareness	1,472,244	805,611
Total program Expenses	20,411,745	25,761,330
Supporting services		
Management and general	550,709	673,250
Fundraising	2,354,842	2,181,259
Total supporting services	2,905,551	2,854,509
Total expenses	23,317,296	28,615,839
Change in net assets	53,131	1,256,891
Net assets, beginning of year	15,814,057	14,557,166
Net assets, end of year	15,867,188	15,814,057

2013 Corporate Information

Leadership Council

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Lester B. Knight
Philip J. Purcell
Robert Wolf

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Multiple Myeloma Research Foundation

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