

MMRF ANNUAL REPORT 2014



We are curing cancer **NOW**

Walter Capone
President and CEO
Multiple Myeloma Research Foundation



Dear Friends,

2014 was a year of ground-breaking advancements and attainment of new milestones by the MMRF/C that are accelerating the transformation of the treatment landscape for multiple myeloma.

We are at a critical point in time where data and technology converge in ways that will undoubtedly accelerate cures. The focus of the MMRF Campaign is to raise \$100M in funding to fuel data and diagnostics, create world class networks of expertise, build collaborative platforms and analytic tools and drive new treatments to the clinic with more urgency than ever before.

Over the three year duration of the Campaign, the MMRF will leverage unprecedented opportunities with genomic sequencing and clinical “big data” that will accelerate cures for multiple myeloma while serving as a model for other cancers as well.

By working with the best scientists in academia and the pharmaceutical/biotech industry, we

have prioritized three key scientific pillars for changing the landscape in cancer research: the Data Bank, the Learning Network and the Clinic. No other organization has built such a model – an engine to aggregate and integrate high-quality data, share the data with researchers across the globe, build partnerships to drive data analytics and advance the highest priority treatments to make precision medicine a priority.

This is the model that will cure cancer. Like many cancers, multiple myeloma is complex and dynamic and requires an ongoing pipeline of new treatments to address all subtypes. We are committed to finding cures for all myeloma patients.

Sincerely,

A handwritten signature in black ink that reads "Walter M. Capone". The signature is fluid and cursive, with a long horizontal stroke at the end.

Walter Capone
President and CEO
Multiple Myeloma Research Foundation

MMRF 2014 Highlights

Among our 2014 notable accomplishments were:

- A record-high ten new clinical trials launched by the MMRC in 2014
- Public launch of the \$100M MMRF 3-Year Capital Campaign and new MMRF Corporate Website
- A landmark FDA/NCI/FNIH/KOL/Industry consensus meeting on the first ever MM Master Protocol
- CoMMpassSM total enrollment reached 803/1,000 patients with 84 active sites; interim analyses include:
 - Interim Analysis 6 (IA6) released to our Pre-Competitive Consortium (PMIC); comprised of 620 enrolled patients; 363 cases with full sequencing completed
 - Interim Analysis 4 (IA4) was released to the public via the MMRF Researcher Gateway; comprised of 278 enrolled patients and 95 cases with full sequencing
- The data included the first comparisons of molecular changes that occurred at diagnosis and at points of progression from the same patient
- An analysis of the CoMMpass genomics data was presented in December, 2014 at the Annual Meeting of the American Society of Hematology (ASH) in San Francisco. The complete sequencing data from these patients identified new genomic changes, including many that may be associated with disease onset.
- An exciting new collaboration was initiated with Gene Networks Science (GNS) for analysis of CoMMpass data focused on novel pathway, biomarker and disease model discovery/development

In October 2014, the MMRF held the first planning meeting for a Multiple Myeloma Master Protocol involving the US Food and Drug Administration (FDA), National Cancer Institute (NCI), Foundation for the National Institutes of Health (FNIH), key investigators, and industry partners in Washington,

D.C., bringing the promise of precision medicine by matching patients to treatments based on their molecular alterations. At this pivotal meeting, an agreement was obtained to begin finalizing the MM Master Protocol details and to engage industry and government for funding and drug

access. This outcome was attained within six months of an initial FDA meeting in April on the concept of launching a novel, multi-center, multi-arm, molecularly targeted clinical trial in relapsed multiple myeloma patients to accelerate targeted drugs for myeloma patients.

“It is important for those who support the MMRF to know that they are helping to develop the next generation of young bright scientists into the field of multiple myeloma.”

*John Carpten, PhD
Deputy Director of Basic Science
Translational Genomics Institute (TGEN)
MMRF Research Fellow Mentor*



MMRF Research Grants Summary 2014

MMRF Senior Research Award

Recipient	Institution	Title
Qing Yi	Cleveland Clinic, Cleveland, OH	Role of osteoclasts in immunosuppression in myeloma
Kari Hemminki	German Cancer Research Center, Heidelberg, Germany	Genomic changes in plasma cell transformation to myeloma in vivo
David Toczyski	University of California, San Francisco, CA	Identifying Ubiquitin ligase substrates important for myeloma progression
Stephen Nutt	Walter and Eliza Hall Institute of Medical Research, Victoria, Australia	How do BLIMP1/PRDM1 and XBP1 act as tumor suppressors in multiple myeloma
Mike Chapman	University of Cambridge, UK	A proteomic approach to discover novel monoclonal antibody targets

MMRF Research Fellow Award

Recipient	Institution	Title
Kim Chan Chung	University Health Network, Toronto, Ontario, Canada	Towards small molecule GRK6 inhibitors as therapeutics for multiple myeloma
John Simmons	National Cancer Institute, Washington, D.C.	A systems approach for identifying potential drug combinations in myeloma
Jens Lohr	Dana Farber Cancer Institute Boston, MA	Determining the mutational profile of circulating multiple myeloma cells
Eric Smith	Memorial Sloan Kettering Cancer Center, NYC	Chimeric antigen receptor modified T cells for multiple myeloma
Mohit Verma	Jackson Labs, Bar Harbor, ME	Development of preclinical humanized mouse model of multiple myeloma
Teresa Ezponda-Itoizl	University of Navarra, Spain	Epigenetic deregulation by loss of the histone demethylase UTX in multiple myeloma

The MMRF acknowledges generous contributions from Amgen, Bristol-Myers Squibb, Celgene, Genentech, Novartis, Onyx, and Takeda Oncology in support of the awards.

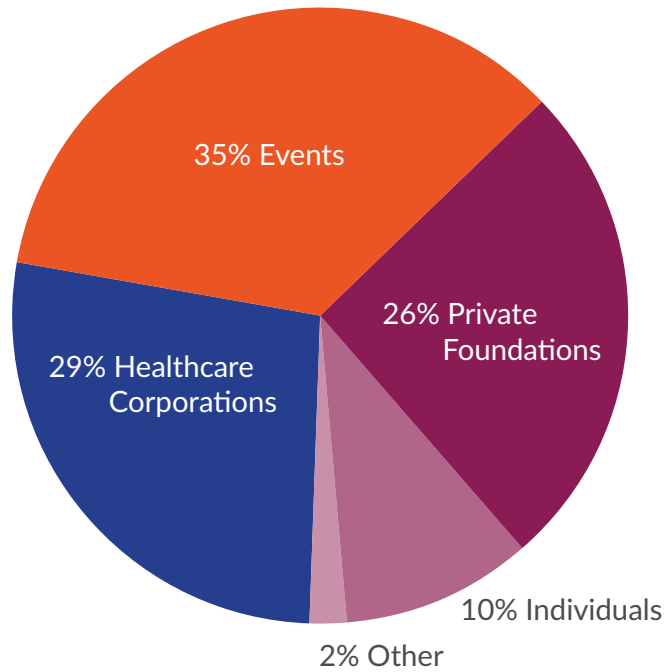
As the MMRF Moves Forward

The MMRF will continue to accelerate the development of new drug therapies and therapeutic approaches for molecular targets associated with the development and progression of multiple myeloma over the next several months.

In 2015, the MMRF plans to open at least 8-10 clinical trials. Among those trials starting this year are the first for patients with specific molecular alterations including p53 (del17p).

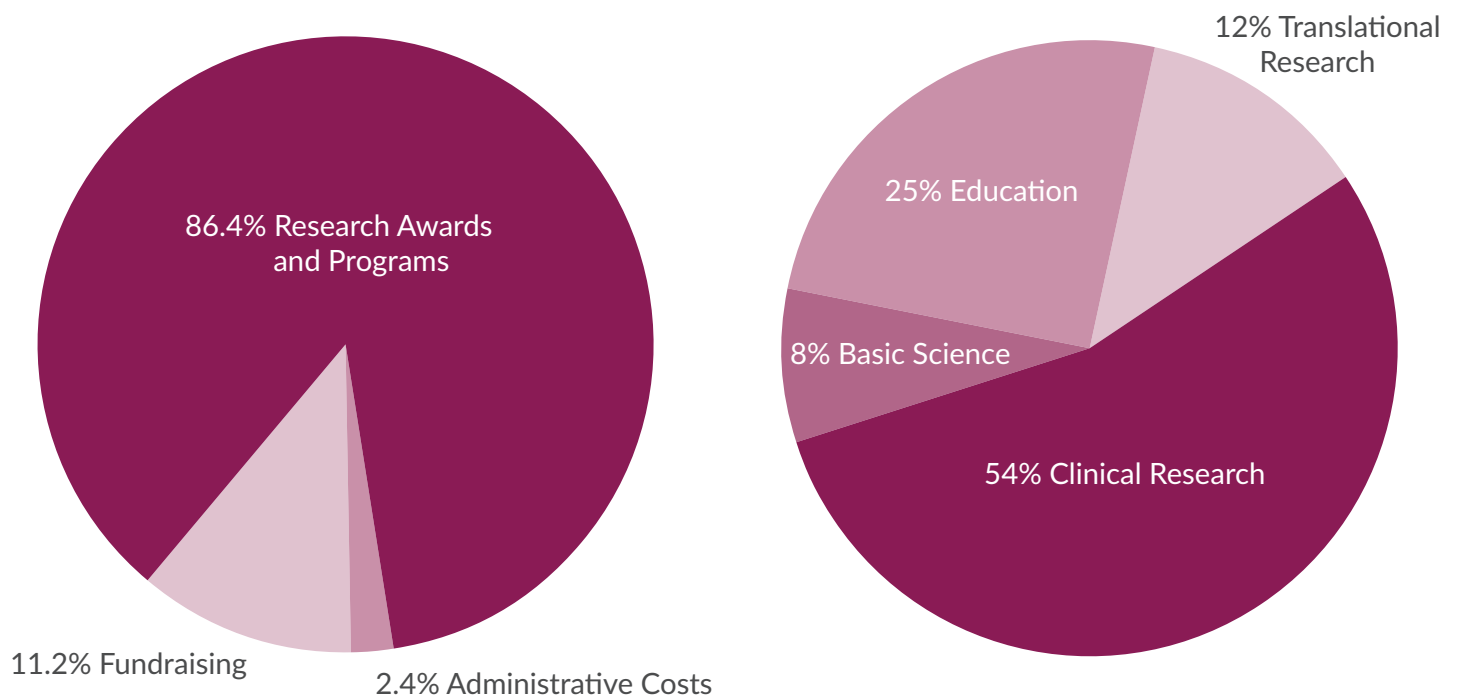
Financial Summary

MMRF 2014 Source of Funds*



*Based on gross revenue

MMRF 2014 Spending Allocations



Multiple Myeloma Research Foundation, Inc

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

Support and Revenue	2014	2013
Contributions	\$10,216,080	\$9,275,518
Private foundation grants	7,850,301	6,676,708
Fee for service	878,000	810,010
In-kind contribution	197,815	150,521
Special Events		
Special events support	10,261,158	8,960,970
Less special events expenses	(2,927,000)	(2,567,606)
Investment return	99,223	66,306
Total support and revenue	26,575,577	23,370,427
Expenses		
Program		
Research	16,721,987	16,571,611
Education	3,352,633	2,367,890
Awareness	2,323,773	1,472,244
Total program Expenses	22,398,393	20,411,745
Supporting services		
Management and general	630,755	550,709
Fundraising	2,895,145	2,354,842
Total supporting services	3,525,900	2,905,551
Total expenses	25,924,293	23,317,296
Change in net assets	651,284	53,131
Net assets, beginning of year	15,867,189	15,814,057
Net assets, end of year	18,763,718	15,867,188

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

2014 Corporate Information

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