



2020 MMRF IMPACT REPORT



MULTIPLE MYELOMA
Research Foundation

2020 YEAR IN REVIEW



It is my immense pleasure to have taken the lead at the MMRF during such a critical time in our organization's history. It is no surprise that 2020 was an unprecedented year for the MMRF and the patient community we serve due to the COVID-19 pandemic. However, despite the obstacles our organization and so many others faced, we have stayed focused on advancing critical initiatives as part of our strategic plan. We also recognize that when confronted with adversity, organizations have unique opportunities to challenge the status quo and guide future strategy and vision.

This impact report outlines all the incredible things we were able to accomplish in 2020. The entire MMRF team shares renewed energy, a sense of urgency, and optimism around the work we do for each and every multiple myeloma patient, and we look forward to sharing our progress with you below.

Thank you all for your continued support of our organization. I hope you will review this report to learn more about all the breakthroughs you have already made possible.

Kind regards,

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

Michael Andreini
President and CEO

A CATALYST FOR A CURE



15
drugs
FDA approved



90+
clinical trials
opened



3x
patient life
expectancy



First
to sequence the
myeloma genome



More than
500M
invested in research



First
to launch at home
liquid biopsy



40%
improvement in
5-year survival rate



First
platform clinical trial:
MMRC MyDRUGSM

WORKING SMARTER

Breakthroughs in immunotherapy

IMMUNE ATLAS

As precision medicine in the myeloma field continues to evolve, the ability to determine the best treatment for each and every patient will hinge not only on identifying genomic subtypes but also on defining each patient's immune subtype. The Immune Atlas Initiative is a first step in mapping each patient's immune system and identifying immune subtypes.

In the initiative's first phase, each of the five partner institutions received the same set of patient bone marrow samples from the MMRF tissue bank. These samples were collected as part of the MMRF CoMMpassSM Study and used to map the genomic landscape for myeloma as part of CoMMpass.

Researchers at each institution analyzed the samples using their own methods, with the new goal of analyzing different immune cell populations in patients with long remissions (slow progressors) versus patients with short remissions (fast progressors). The researchers shared all their results with each other for comparison and aggregated analysis. Going forward, the MMRF will work with Immune Atlas partners to develop a common set of protocols and reagents for use in myeloma immune analysis.

In the fall of 2020, the Immune Atlas partners began an immune analysis of CoMMpass patient samples using high-dimensional single-cell RNA sequencing (scRNAseq) and mass cytometry (CyTOF) assays to create an immune profile for each CoMMpass patient. These immune profiles were combined with genomic and clinical profiles for each patient to provide a comprehensive picture of multiple myeloma and immunity for each patient's specific disease subtype. Over time, we will add additional immune profiling technologies to the Immune Atlas protocol and expand our knowledge of immunity in multiple myeloma.



The MMRF plays a critical role in bringing together all the main actors in myeloma research – from the academic community to those in the pharmaceutical industry – to work together to determine how best to integrate and solve immune-based therapies in developing better treatments for our patients.



David E. Avigan MD
Beth Israel Deaconess Medical Center

IMMUNE ATLAS INITIATIVE PARTNERS



PLATFORM TRIALS

Clinical trials give patients access to the most cutting-edge therapies – critically important for those who have exhausted all other treatment options. Two groundbreaking trials are MyDRUGSM (2019) and MyCheckpointSM (2020).

MyDRUGSM (Myeloma Developing Regimens Using Genomics) – The first platform study in myeloma that evaluates targeted therapies against specific genomic alterations is continuing to enroll patients across all study arms. Two new sub-protocols were added to include Xpovio (Selinexor) and Blenrep (belantamab mafodotin) for patients who do not have actionable mutations from genomic sequencing.

MyCheckpointSM – The MMRF opened the first sites in the novel MyCheckpoint immune therapy platform trial, the first of its kind in multiple myeloma. These agents do not attack the tumor directly; they are monoclonal antibodies that block “immune checkpoints” – molecules on the surface of cells that can turn off T-cell immune responses. When the checkpoint is blocked, T-cells are



re-activated and able to destroy myeloma cells. Through MyCheckpoint, myeloma patients who have exhausted all other treatment options will be treated with a checkpoint inhibitor directed against either LAG-3 or TIGIT, combined with Pomalyst and dexamethasone. After a delayed start due to the pandemic, all sites are now open and enrolling patients to the study.

RESEARCH FELLOW AWARDS FOR 2020

The MMRF’s Research Fellow Award Program is available to investigators at not-for-profit academic institutions in the United States and abroad. Researchers must hold a PhD, MD, or equivalent degree and be at the post-doctorate, clinical fellow, or junior faculty level. With awards ranging from \$100,000 to \$1 million, we advance innovative research that seeks to learn more about myeloma’s biology and identify potential biomarkers for drug development. At the same time, our grants ensure that expert researchers continue to apply their talents to accelerating cures for myeloma. To date, the MMRF has funded more than 400 research grants at over 140 research institutions around the globe.

- **Ben Derman, MD** (University of Chicago): MRD (Minimal Residual Disease) to Guide Maintenance Treatment Discontinuation
- **Sweta Kambhampati, MD** (University of California San Francisco): Phase II Study of Daratumumab/ Azacytidine/Dexamethasone in Relapsed Myeloma
- **Kylee Maclachlan, MD, PhD** (Memorial Sloan Kettering Cancer Center): Defining Genomic Instability in Multiple Myeloma
- **Romanos Pistofidis Sklavenitis, MD** (Dana-Farber Cancer Institute/Broad Institute): Investigating Stem-like Myeloma Cells as Drivers of Tumor Progression
- **Paola Storti, PhD** (Università di Parma): The role of the bone marrow microenvironment in myeloma progression

WORKING FASTER

Accelerating progress with the Myeloma Investment Fund®



As the first and only mission-driven, self-sustaining, scalable venture philanthropy fund focused on multiple myeloma, the **Myeloma Investment Fund®** (MIF) strives to accelerate the MMRF's mission to deliver transformative treatment options to every patient by bringing the most promising companies, clinical assets, and technologies to multiple myeloma.



This collaboration with the MMRF and MIF will be invaluable in helping us advance the clinical development of our universal allogeneic G-NK cell therapy.



Guy DiPierro
Founder and Chief Executive Officer
Indapta Therapeutics

The MIF continues to accelerate research and attract promising new companies to the field of myeloma. New investments made in 2020 include Indapta Therapeutics, a San Francisco-based biotech developing an allogeneic Natural Killer (NK) cell therapy for the treatment of multiple myeloma, and Abcuro, Inc., a Newton, MA-based biotech company that is developing a new immune checkpoint therapy for the treatment of autoimmune diseases and cancer, including myeloma.

Since its inception, the MIF has developed a robust portfolio of six innovative companies and provided valuable strategic guidance to accelerate their clinical programs.



Newton, MA

The MIF's investment supports the ongoing development of Abcuro's pre-clinical therapies, which are directed against a novel immune checkpoint target, KLRG1. Blocking KLRG1 signaling may augment a patient's immune system by activating their Natural Killer (NK) and T-cells against tumors.



San Francisco, CA

Indapta's universal allogeneic G-NK cell therapy treats multiple types of difficult-to-treat hematologic cancers and solid tumors. It promises to help address the limitations of currently available autologous T-cell therapies.

WORKING TOWARD A CURE

Democratizing data with the MMRF CureCloud®

Launched in July 2020, the **MMRF CureCloud®** (CureCloud) is a novel research study designed to democratize myeloma care and advance smarter treatment strategies for every multiple myeloma patient – regardless of where they receive treatment.

At its core it is a groundbreaking direct-to-patient research initiative, where patients can enroll online and gain access to the first at-home genomics testing program in myeloma. At no cost to them, participating patients and their physicians receive personalized reports to guide clinical decisions, which offer actionable guidance and insights to help guide every patient towards remission.

By taking part in the CureCloud, patients are not only working toward better answers for themselves; they are

also driving the entire myeloma community toward precision medicine. The MMRF is sharing this data widely, aiming to provide access to every physician and patient – including those who are not enrolled in the CureCloud to inform smarter treatment decisions and optimize patient outcomes.

As we move forward in 2021, our team is focused on completing the remaining components of our CureCloud platform including building out the data visualization modules that will provide contextual information for patients and their clinicians to optimize patient care by looking at aggregated data from similar patient cohorts. To date, 402 patients are fully enrolled in CureCloud, with a goal of 750 enrollments by the end of 2021.

Data for Prevention – slowing multiple myeloma’s progression through the CureCloud

The MMRF strives to give patients at every stage of the disease their best chance at a longer, healthier life, including those diagnosed with smoldering multiple myeloma (SMM), an early-stage form of multiple myeloma where observation is standard of care for most patients. In late 2020, the MMRF expanded CureCloud enrollment parameters to accept patients with active myeloma, including those diagnosed with SMM.



In a new innovative initiative with our partners at the Dana-Farber Cancer Institute, the MMRF is working to amass the largest dataset of SMM patients and aggregate the data within the CureCloud. Just as it did with active myeloma patients, our research will empower us to better understand this early condition, stratify risk for progression, and discover promising new treatment options. Armed with this information, we will be able to drive toward precise, data-driven, proactive treatment approaches that slow and, hopefully, prevent disease progression.

EMPOWERING PATIENTS IN THEIR JOURNEY

PATIENT EDUCATION

Through our Patient Education programming, we are working directly with patients and caregivers to ensure they have the tools and information they need to feel empowered in navigating this disease.

2020 Patient Education programming:

We created four (4) new High Impact Topic videos, or HITs, in 2020. HITs are videos that use engaging animations and narration to present information on topics of importance to myeloma patients and their caregivers.

We held four (4) free Expert Sessions. Our Expert Sessions feature discussion and insights into key clinical advancements and hot topics in multiple myeloma, including a Q&A with myeloma experts.

We held six (6) Patient Summits and twelve (12) Webinars that provide further guidance in understanding multiple myeloma with an in-depth look at lab work, supportive care, smoldering myeloma, and other timely topics.

We produced a new patient brochure on Autologous Stem Cell Transplant (ASCT) and updated our patient tool kits.

PATIENT NAVIGATION CENTER

PNC Numbers

The Patient Navigation Center (PNC) is a space for multiple myeloma patients to connect with patient navigators — who are professionals specializing in oncology — for guidance, information, and support. You can connect with a patient navigator via phone, email, or online web form. Whenever you have a question, our patient navigators are here to help.

2020 by the numbers:

Over 3,000 patient/caregiver cases were handled by our PNC nurses for the myeloma community in 2020; 1,193 Patient Education Toolkits were delivered to newly diagnosed patients.

In 2020, the PNC maintained a 5/5 survey score from those we serviced.



The MMRF fills me with hope and promise. Sixteen years later, I am still here fighting, and I will not stop until we find a cure.



Carmen Phaneuf
Patient

FINANCIALS

19,048
supporters

\$27.1MM
raised

\$24MM
spent on research
and related
programming

Thank you for making this all possible!

ABOUT THE MULTIPLE MYELOMA RESEARCH FOUNDATION (MMRF)

Our Promise to Our Donors: Impact, Transparency, and Results

PLATINUM

GuideStar 2020
Seal of Transparency.

“BEST IN AMERICA”

Seal of Excellence from
the Independent Charities
of America.

“A+” RATING

from the American
Institute of Philanthropy.

16TH YEAR

with four stars
(highest rating) from
Charity Navigator.

A pioneer in precision medicine, the Multiple Myeloma Research Foundation (MMRF) seeks to accelerate a cure for all multiple myeloma patients by relentlessly pursuing innovations that accelerate the development of precision treatments for cancer. Founded in 1998 by Kathy Giusti, a multiple myeloma patient, and her twin sister Karen Andrews as a 501(c)(3) nonprofit organization, the MMRF has created the business model around cancer from data to analytics to the clinic. The MMRF identifies barriers and then finds the solutions to overcome them, bringing in the best partners and aligning incentives in the industry to drive better outcomes for patients.

Since its inception, the organization has collected thousands of samples and tissues, opened nearly 100 trials, helped bring 15 FDA-approved therapies to market, and built CoMMpass, the single largest genomic dataset for any cancer. Today, the MMRF is building on its legacy in genomics and is expanding into immunotherapy, as the combination of these two fields will be critical to making precision medicine possible for all patients. The MMRF has raised nearly \$500 million and directs nearly 90% of the total funds to research and related programs. To learn more, visit www.themmr.org.

OUR MISSION: **CURE MULTIPLE MYELOMA**

The Multiple Myeloma Research Foundation (MMRF) drives discoveries for new treatments, accelerates groundbreaking clinical trials, and fuels the most robust data-driven initiatives in cancer research. Our goal is to find a cure for each and every patient diagnosed with multiple myeloma.



Multiple Myeloma Research Foundation

383 Main Ave., 5th Floor

Norwalk, CT 06851

TheMMRF.org