

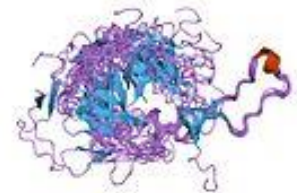
Annual Report 2012 & 2013

In 2012 the Foundation faced a critical question: do we continue to focus exclusively on activities related to funding research or expand our mission to include activities such as education of patients and doctors. To tackle this issue we sought input from the patient, researcher, biotech and advocacy communities to see what they think the role we play could be in the world of MPNs.

The feedback we received encouraged us to accelerate our research plan and put a spotlight on the need for further MPN research. As a consequence, in 2013 we undertook a plan to award four grants through the MF Challenge, a joint venture between MPNRF and the Leukemia & Lymphoma Society, but to otherwise begin building a war chest with which we would aggressively pursue new research projects with.

To decide where to focus our efforts, in late 2013 we convened thought leaders from academia and industry for a meeting in which the topic would be singular: with the discoveries made in myeloproliferative research in general, and myelofibrosis specifically, what would the next advances towards better treatments or a cure come from? Leading up to the meeting the news came out that one of our researchers – Dr. Robert Kralovics from Vienna – had been one of two labs to confirm the discovery of a new genetic mutation (CALR) which accounts for nearly all the JAK2 negative cases of MPN.

Picture of the CALR Mutation



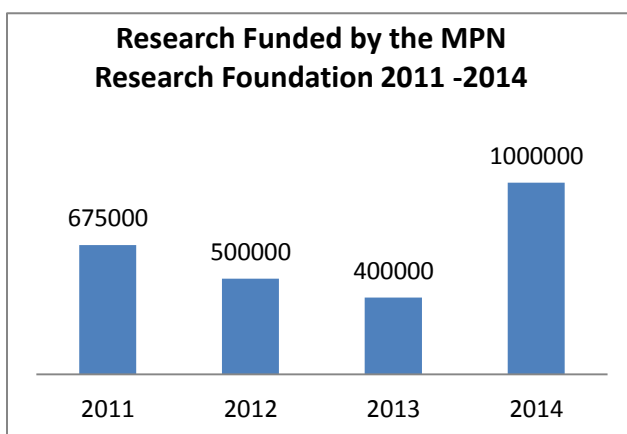
What came out of the meeting – which included presentations from MPNRF grantees – were five focus areas for our next call for research proposals:

- 1. Fibrosis and the Bone Marrow Niche.** New or continued effort to discover the factor(s) that induce(s) fibrosis of the bone marrow, and to identify opportunities to arrest and reverse this fibrosis. Of particular interest are the interactions between the MPN stem cell and the bone marrow/microenvironment niche which contributes to fibrosis, extramedullary hematopoiesis, or MPN cell survival.
- 2. CALR Mutation.** The recent discovery of the Calreticulin gene (CALR) exon 9 mutations in the majority of JAK2-negative patients with chronic MPNs opens new avenues for translational research. In addition to its potential for new druggable targets, basic understanding of the mechanisms and consequences of CALR mutations in MPNs will be needed to develop novel rational therapeutic agents for these disorders.
- 3. Reducing MPN Allele Burden.** In a subset of MPN patients, interferon has shown the ability to reduce the mutant allele burden, be it JAK2 V617F or other disease alleles which mark the disease clone. Recent studies have suggested that other agents might have anti-clonal effects in a subset of patients, which have the potential to indicate disease modifying activity.

4. Selective JAK2 inhibition. Development of a strategy for selectively targeting the mutant JAK2 protein to reduce toxicities associated with inhibiting wild-type JAK2. Additionally, approaches to target pathways downstream of JAK2 are sought.

5. Immunotherapy. Basic research to investigate the potential for control of marrow fibrosis and suppression of aberrant MF clones by immunotherapy or immune manipulation. It is well understood that immunotherapeutic approaches have clinical therapeutic value at least a subset of MPNs. CML and other MPNs, for example, can be effectively treated with interferon, and in some cases of CML, cures are possible using adoptive T-cell transplantation.

In addition to re-committing to our focus on funding the best MPN research, we made the strategic decision to fund fewer grants in 2012 and 2013 so that we could amplify our efforts on the MPNs in 2014 with renewed energy. As a result, in 2014 we will be funding \$1 million in targeted MPN research, the largest annual amount we have ever committed to. This will be distributed among 10 projects which passed two review sessions by experts in the field who looked not only at scientific merit, but likelihood of accomplishing their goals.



Matched to our rigorous scientific standards is our commitment to using our donors' funds as responsibly as possible. Particularly because we are largely funded by patients with ET, PV, and MF, who contribute to the Foundation so that they can see their money used only for MPN research. For that reason we cap administrative overhead on all grants at 8%.

As we amped up our grant program, we also built new and stronger ties to others working in the MPN and rare disease community. In 2013 we joined OVAC (One Voice Against Cancer) Coalition for lobby days aimed at increasing the amount of cancer research funding at the federal level. We also continue to share information on clinical trials, educational opportunities and support groups to our large database of MPN patients, family, friends, hematologists and researchers.

Our mandate is to fund research, but also to be a connector within the MPN community so that patients can easily access what they need to manage their ET, PV, and MF until we can find the cure we are all waiting for.

THE MPN RESEARCH FOUNDATION

STATEMENT OF ACTIVITIES

YEARS ENDED NOVEMBER 30, 2012 AND 2011

	2012			2011		
	Unrestricted	Temporarily Restricted	Totals	Unrestricted	Temporarily Restricted	Totals
REVENUES AND OTHER SUPPORT						
Contributions	534,896	640,429	1,175,325	1,024,773	283,550	1,308,323
Investment income	4,647		4,647	7,452		7,452
Net assets released from restrictions	801,018	(801,018)		314,421	(314,421)	
Total Revenues and Other Support	1,340,560	(160,589)	1,179,971	1,346,646	(30,871)	1,315,775
EXPENSES						
Program	1,389,867	-	1,389,867	1,081,636	-	1,081,636
General and administrative	152,922	-	152,922	140,307	-	140,307
Fundraising	160,619	-	160,619	153,269	-	153,269
Total Expenses	1,703,408	-	1,703,408	1,375,212	-	1,375,212
CHANGE IN NET ASSETS	(362,847)	(160,589)	(523,436)	(28,566)	(30,871)	(59,437)
NET ASSETS -						
Beginning of year	390,297	420,048	810,345	418,863	450,919	869,782
End of Year	27,450	259,459	286,909	390,297	420,048	810,345

THE MPN RESEARCH FOUNDATION

STATEMENT OF ACTIVITIES

YEARS ENDED NOVEMBER 30, 2013 AND 2012

	2013			2012		
	Unrestricted	Temporarily Restricted	Totals	Unrestricted	Temporarily Restricted	Totals
REVENUES AND OTHER SUPPORT						
Contributions	994,517	660,146	1,654,663	536,253	640,429	1,176,682
Investment income	3,652		3,652	4,647		4,647
Net assets released from restrictions	<u>592,218</u>	<u>(592,218)</u>	<u>-</u>	<u>820,443</u>	<u>(820,443)</u>	<u>-</u>
Total Revenues and Other Support	<u>1,590,387</u>	<u>67,928</u>	<u>1,658,315</u>	<u>1,361,343</u>	<u>(180,014)</u>	<u>1,181,329</u>
EXPENSES						
Program	627,755		627,755	1,391,226	-	1,391,226
General and administrative	126,472		126,472	152,921	-	152,921
Fundraising	<u>184,273</u>	<u>-</u>	<u>184,273</u>	<u>160,619</u>	<u>-</u>	<u>160,619</u>
Total Expenses	<u>938,500</u>	<u>-</u>	<u>938,500</u>	<u>1,704,766</u>	<u>-</u>	<u>1,704,766</u>
CHANGE IN NET ASSETS	651,887	67,928	719,815	(343,423)	(180,014)	(523,437)
NET ASSETS -						
Beginning of year	46,874	240,034	286,908	390,297	420,048	810,345
End of Year	698,761	307,962	1,006,723	46,874	240,034	286,908