

XIITH INTERNATIONAL MYELOMA WORKSHOP

MAY 2, 2011



MMRF®

Multiple Myeloma
Research Foundation

Powerful Thinking Advances the Cure™

OVERVIEW

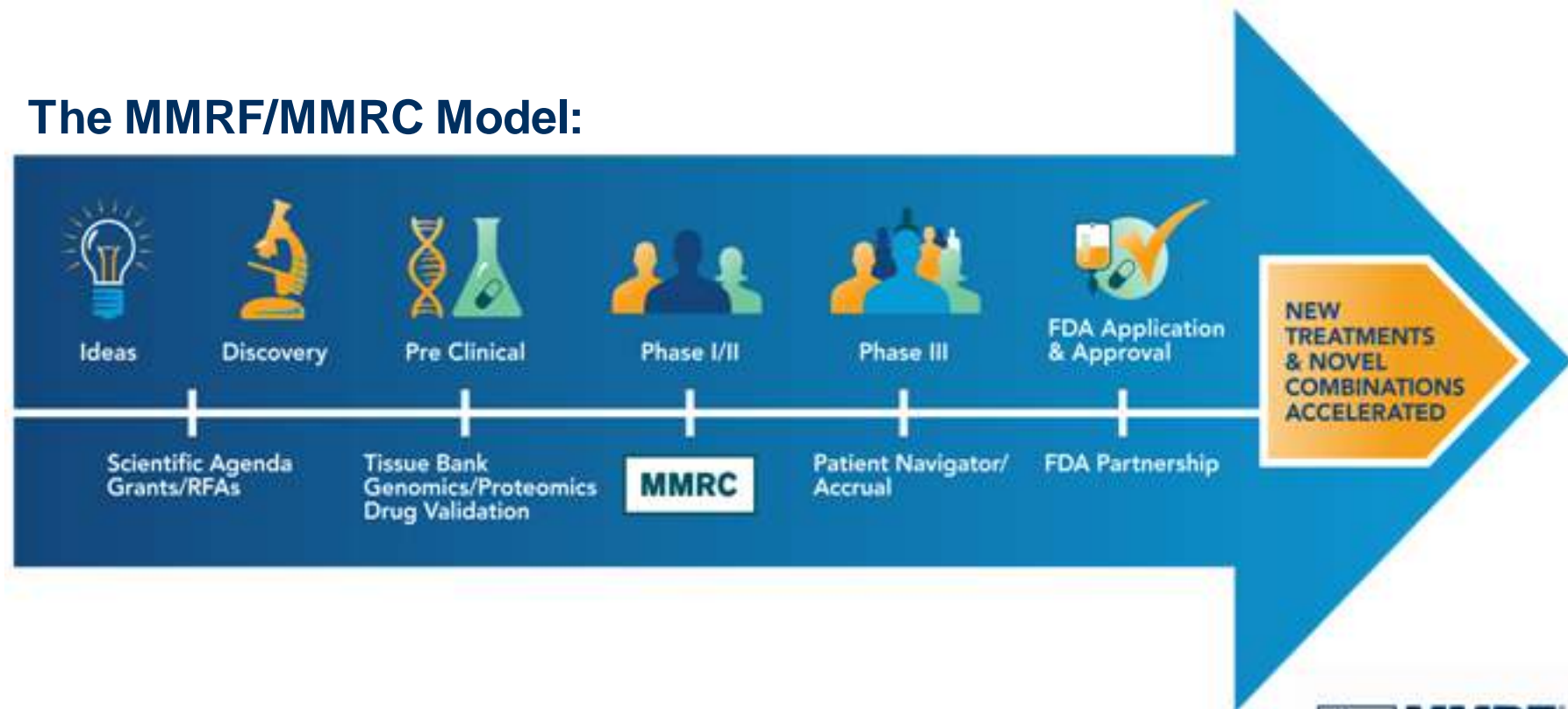
The MMRF mission is to accelerate drug development and find a cure.



MODEL

Together, the MMRF and MMRC serve as an end-to-end solution in drug discovery and development.

The MMRF/MMRC Model:






FUNDING

The MMRF is the world's #1 non-profit funder of myeloma research, having funded 300+ grants to 80+ institutions and companies worldwide.

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- Acceleron
 - Aileron
 - Astex
 - Constellation
 - Epizyme
 - Karyopharm
 - MannKind
 - Semafore
 - Synta
 - University of Alabama at Birmingham
 - University of Arizona
 - University of Arkansas
 - University of Bari
 - Barts and the London School of Medicine
 - Beth Israel Deaconess Medical Center
 - Boston VA Research Institute
 - Free University of Brussels
 - California Institute of Technology
 - University of Calgary
 - University of California, Davis
 - University of California, Irvine
 - University of California, LA
 - University of California, San Diego
 - Cancer Center University of Texas, San Antonio
 - Centro de Investigacion del Cancer
 - University of Chicago
 - Colorado State University
 - Columbia University
 - Cornell University
 - Dana-Farber Cancer Institute
 - Dartmouth Medical School
 - Duke University
 - Emory University
 - University of Florida
 - Fondazione Centro San Raffaele
 - del Monte Tabor
 - Fred Hutchinson Cancer Research Center
 - Universiteit Gent
 - Georgetown University
 - Hackensack University
 - Hadassah Medical Organization
 - Harvard Medical School
 - Herlev University Hospital
 - The Hospital for Sick Children
 - Institute for Myeloma and Bone Cancer Research
 - Imperial College of Science, Technology and Medicine
 - Indiana University
 - The University of Iowa
 - John Hopkins University
 - Karolinska Hospital
 - Leiden University Medical Center
 - University of Maryland, Baltimore
 - Massachusetts General Hospital
 - Massachusetts Institute of Technology
 - Mater Medical Research Institute
 - Mayo Clinic
 - Medical College of Georgia Research Institute
 - Memorial Sloan-Kettering Cancer Center
 - University of Miami
 - University of Michigan
 - University of Minnesota
 - University of Missouri
 - H. Lee Moffitt Cancer Center
 - Monash University
 - Mount Sinai School of Medicine
 - NCI
 - New York University School of Medicine
 - University of North Carolina-Chapel Hill
 - North Shore-LIJ Hospital
 - Northwestern University
 - University of Oslo
 - Ospedale Molinette
 - Ohio State University
 - University of Oklahoma
 - University of Oxford
 - University of Pennsylvania
 - University of Pittsburgh
 - Princess Margaret Hospital
 - Queen Mary, University of London
 - Roswell Park Cancer Institute
 - Royal Prince Alfred Hospital
 - St. Vincent's Institute of Medical Research
 - Sanford Burnham Medical Research Institute
 - University of Sheffield Medical School
 - South Hampton University
 - University of Southern California
 - Stanford University
 - SUNY-Downstate Medical Center
 - Tel Aviv University
 - Temple University
 - University of Tennessee
 - University of Texas-MD Anderson Cancer Center
 - TGen
 - Toronto General Research Institute
 - University of Torino
 - Tulane University Health Sciences Center
 - University Health Network
 - University Hospital, Rotterdam
 - Universitair Medisch Centrum
 - Uppsala University
 - University of Utah
 - University Medical Center Utrecht
 - The VA Pittsburgh Healthcare System
 - UCLA-West LA VA Medical Center
 - Van Andel Research Institute
 - Virginia Commonwealth University
 - The Walter and Eliza Hall Institute of Medical Research
 - Universitat Würzburg
 - University of Wisconsin
 - Yale University

GENOMICS

The 5-year, \$12M MMRF Genomics Initiative yielded the most comprehensive genomics analysis in myeloma to date.

Partners	Results
  	<p>3000 samples in Tissue Bank</p> <p>250 comprehensively analyzed with aCGH and GEP</p> <p>80 sequenced via WGS/WES</p> <p>Epigenetic targets</p> <p>Mutant BRAF/Activated Ras</p>

www.myelomagenomics.org



VALIDATION

The MMRF has supported the preclinical validation of more than 70 compounds and combination therapies.

MMRF-Funded Agents			
Plitidepsin *	Tubacin	NPI-1387	Bcl9 peptides
Temsirolimus**	Vorinostat*+	CPS 11	HYD1
Tanespimycin*	BX471	SU5402	PRLX93936
NPI-0052**	BMS-345541	PD173074	TCH-013
Perifosine**	PKC412*	Sorafenib*	Anti-b2M Abs
Panobinostat**	NVP-ADW742	17-DMAG	RAP-011
SCIO-469*	NVP-AEW541	AT-101	ACE-011*
SGN-40*	RAD001*	PTK-787*	Pyrimidinetriones
Mapatumumab*	NVP-AAL881	BMS354825	AZ PI3K inhibitor
Bortezomib**	PK 11195	ABT-737*	P5091
Lenalidomide**	Carfilzomib**	AT7519**	Withaferin A
Tipifarnib**	GRN163L	BH3 stapled peptides	SF1126**
INK128**	TG02	Clioquinol*	Anti-CD138 fusions
Pomalidomide**	Bendamustine+	MLN8237**	Flavopiridol
PD0332991**	Elotuzumab+	TKI-258**	Largazole
Anti-CD38	GX15-70	LLPZA	Picropodophyllin
DMAPT	100 NA, RO-H		

* Compounds studied in the clinic

+Compounds in clinical trials in MMRC

INDUSTRY PARTNERS

The MMRF/C collaborates with more than 25 industry partners, in addition to academic partners, and will continue to strengthen those partnerships.

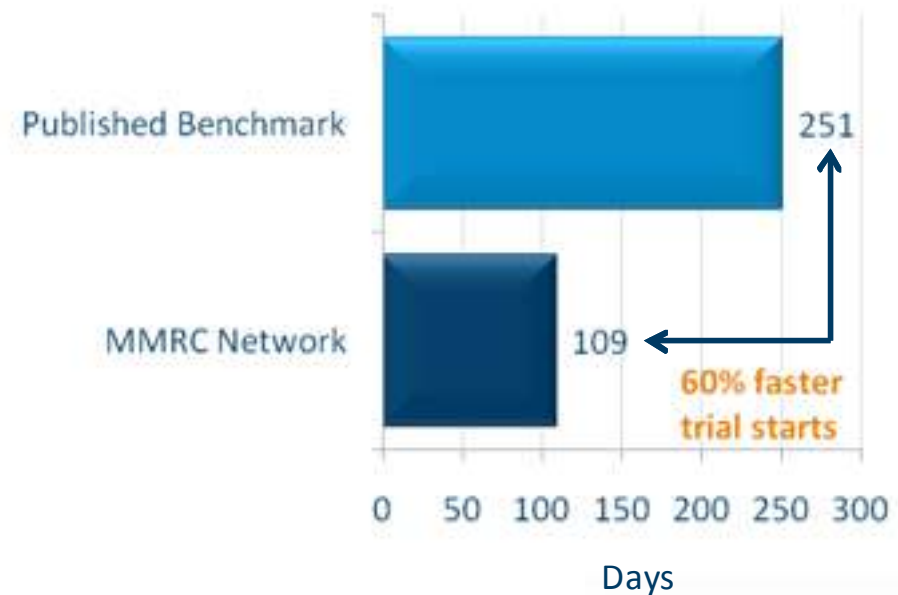


MMRC RESULTS

The MMRC has partnered with industry to open 30 trials with 18 next-generation agents, and accelerated the timeline to trial opening by 60%*.

Compounds/Drugs Evaluated	
ARRY-520	Perifosine
AT 7519	Pomalidomide
Carfilzomib	SF1126
Elotuzumab	Siltuximab
INK128	Tipifarnib
MLN8237	TKI258
NPI-0052	Torisel®
Panobinostat	Treanda®
PD0332991	Zolinza®

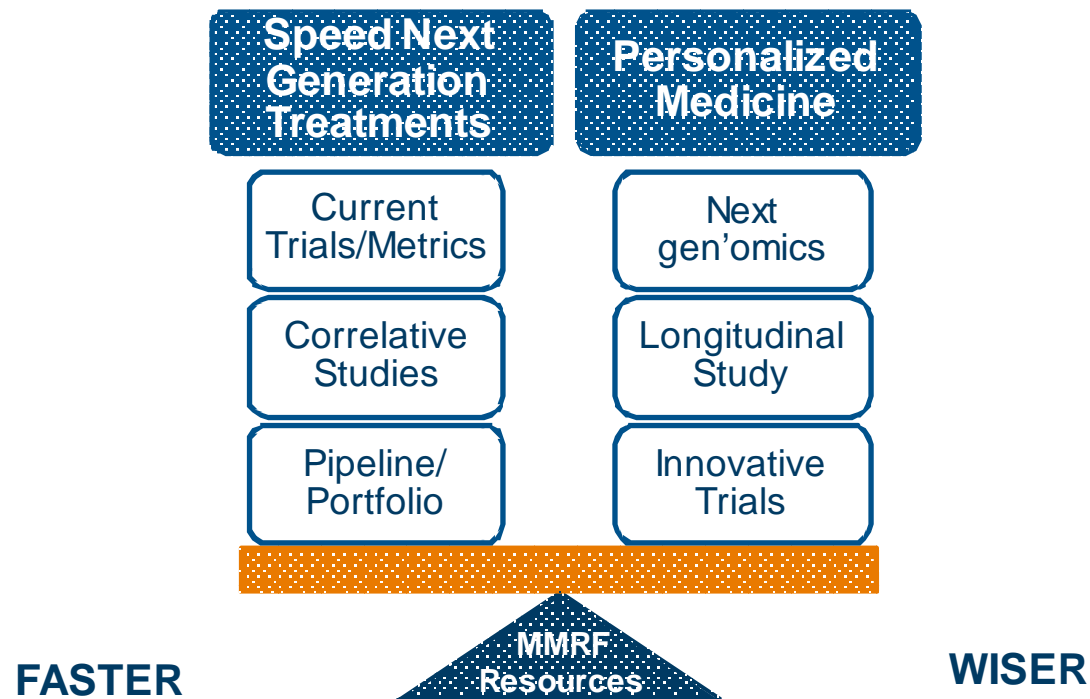
Final Protocol to First Patient Consented



*(Reference: Dilts, 2008)

MOVING FORWARD

The MMRF/C will balance its focus on accelerating drug development with personalizing therapy based on genomic profiling.



COLLABORATION

“In the long history of humankind (and animal kind, too), those who learned to collaborate and improvise most effectively have prevailed.”

– Charles Darwin



Thank You.

Word cloud containing various expressions of gratitude in multiple languages: arigato, sas, takk, obrigado, saqelun, dank u, toda, eijharisto, danke, grazie, tack, hvala, tak, danke, arigato, and merci.