

# IOM PM ROUNDTABLE MMRF UPDATE

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# MMRF MODEL OVERVIEW

The MMRF is the largest private funder of multiple myeloma research raising over \$190M to-date through a proven, collaborative model.



Powerful Thinking Advances the Cure<sup>SM</sup>

- Founded in 1998
- Funds worldwide research
- Builds myeloma community
- Partners with MMRC



Powerful Collaboration Accelerates Results<sup>SM</sup>

- Founded in 2004
- Supports 16 institutions
- Conducts tissue banking
- Facilitates Phase I/II trials

# MULTIPLE MYELOMA CHALLENGE: 1998

Finding the cure for the 2<sup>nd</sup> most common blood cancer.

**3 years**

average survival

**0**

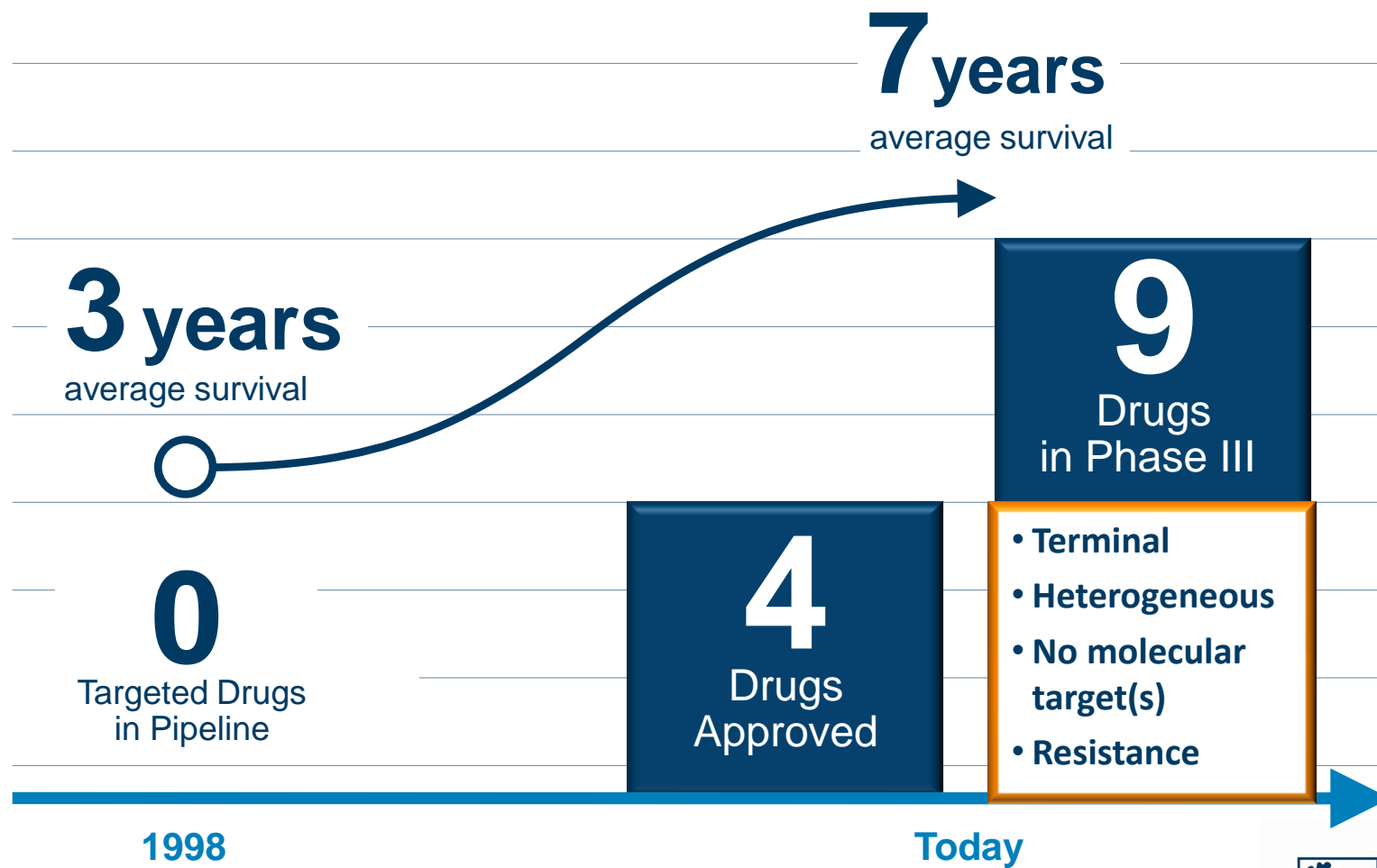
Targeted Drugs  
in Pipeline

**1998**

- US/Global Prevalence: 64,000/102,000
- US/Global Incidence: 21,700/32,000\*
- US/Global Deaths: 10,710/14,000\*
- US demographics: Older adults, African Americans, predominantly male

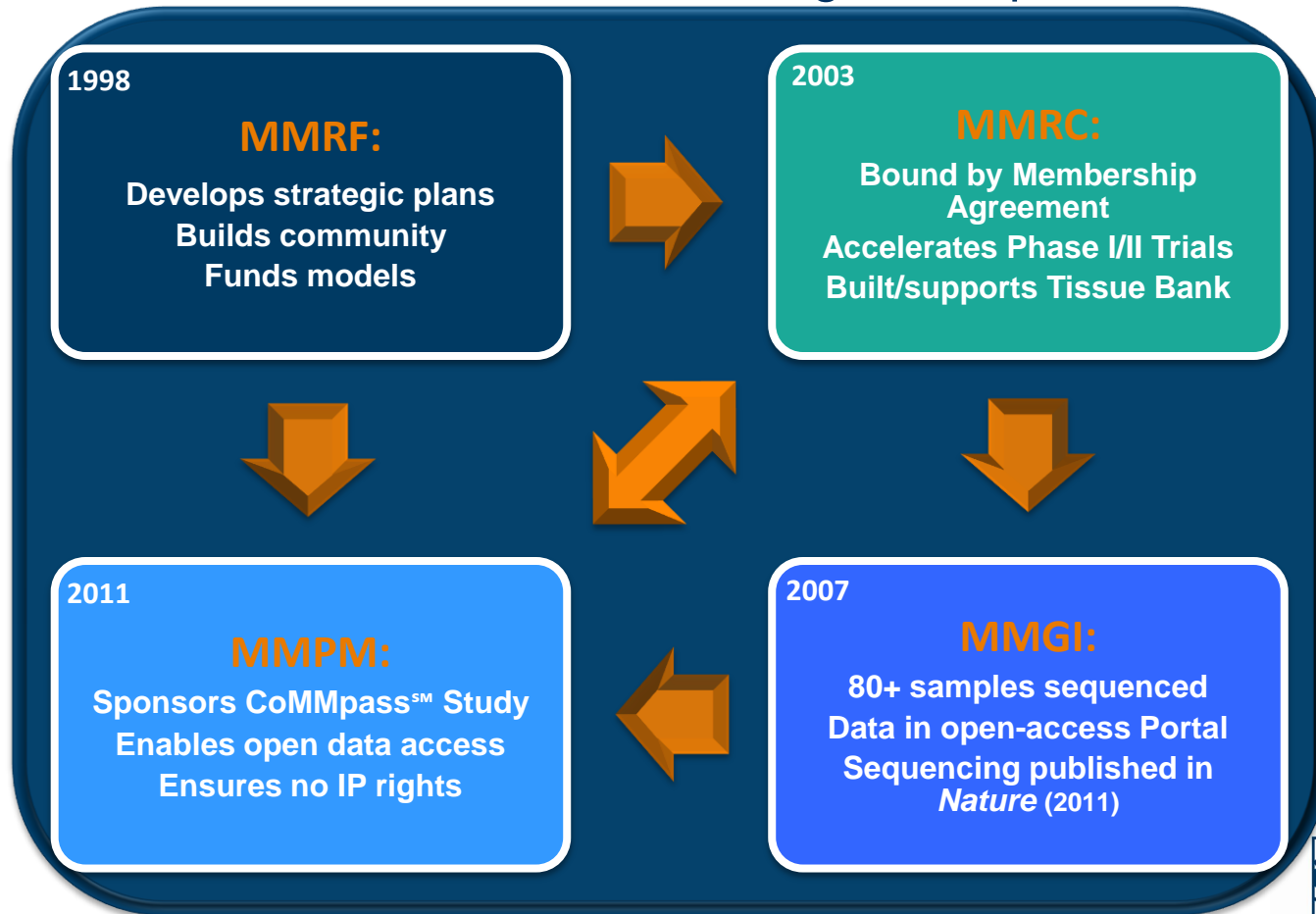


# MULTIPLE MYELOMA CHALLENGE: 2012



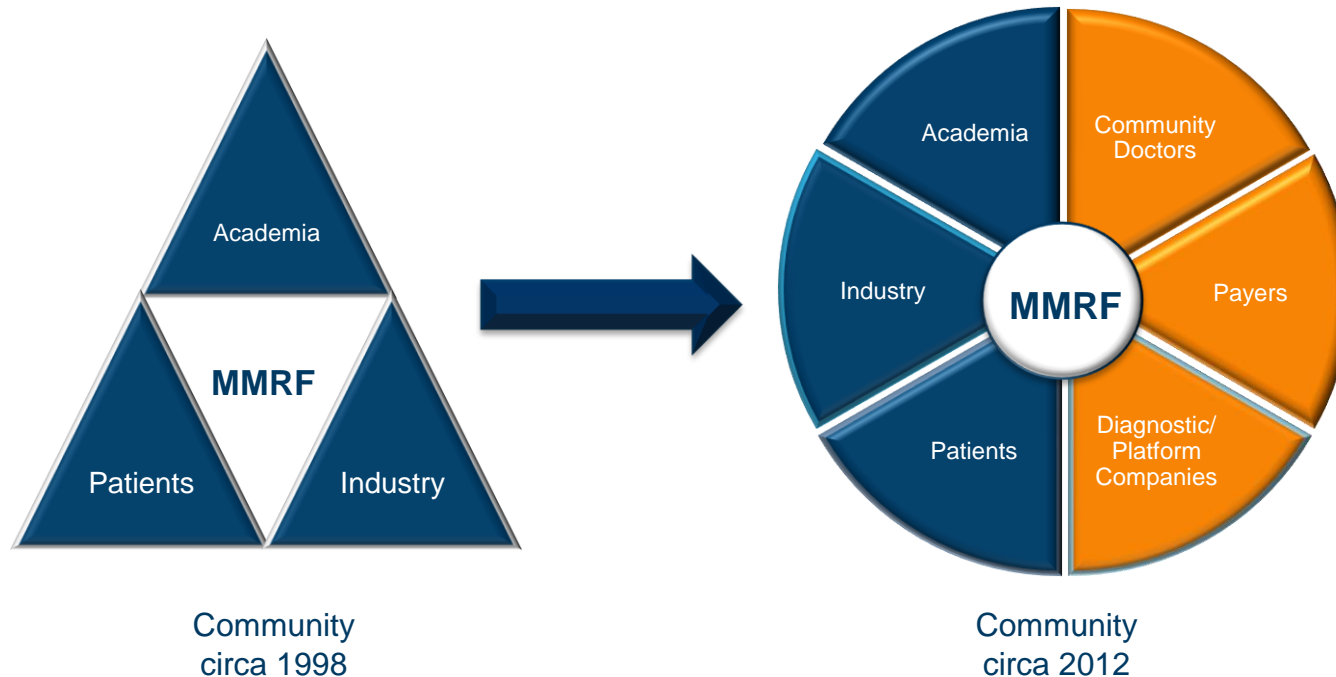
# MMRF MODEL

The MMRF continues to lead by building and maintaining increasingly complex collaborative models to drive drug development.



# MMRF COMMUNITY

The MMRF is continuing to expand its programs in multiple myeloma therapy as the range of stakeholders in the field grows more complex.



# MMRC

The MMRC integrates 16 leading academic and community to bank tissue and speed Phase I/II clinical trials; metrics are tracked.



## COMMUNITY CENTERS



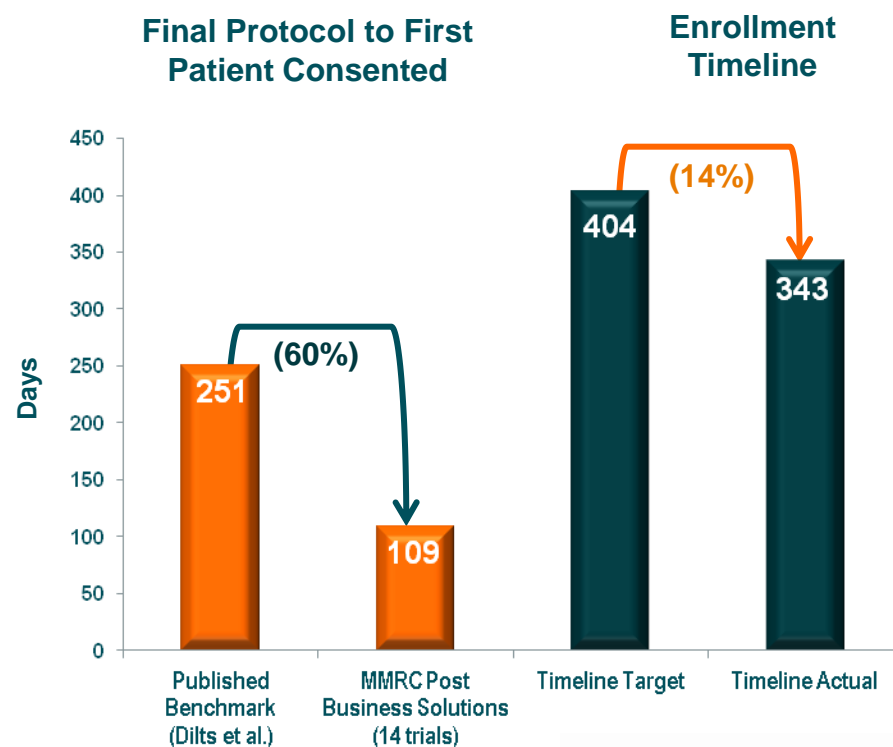
## MMRC Metrics

Operations  
Trial Enrollment  
PI Engagement

# MMRC RESULTS

The MMRC has partnered with industry to open 37 trials with 20 novel agents, and sped the time to trial opening by 60% and enrollment by 14%.

Novel Compounds/Drugs Evaluated	
ARRY-520	PD0332991
AT 7519	Perifosine
Carfilzomib	Pomalidomide
Elotuzumab	SF1126*
Ganetespib	Siltuximab
GSK2110183	Tipifarnib*
INK128	TKI258
MLN8237	Torisel®*
NPI-0052	Treanda®
Panobinostat	Zolinza®

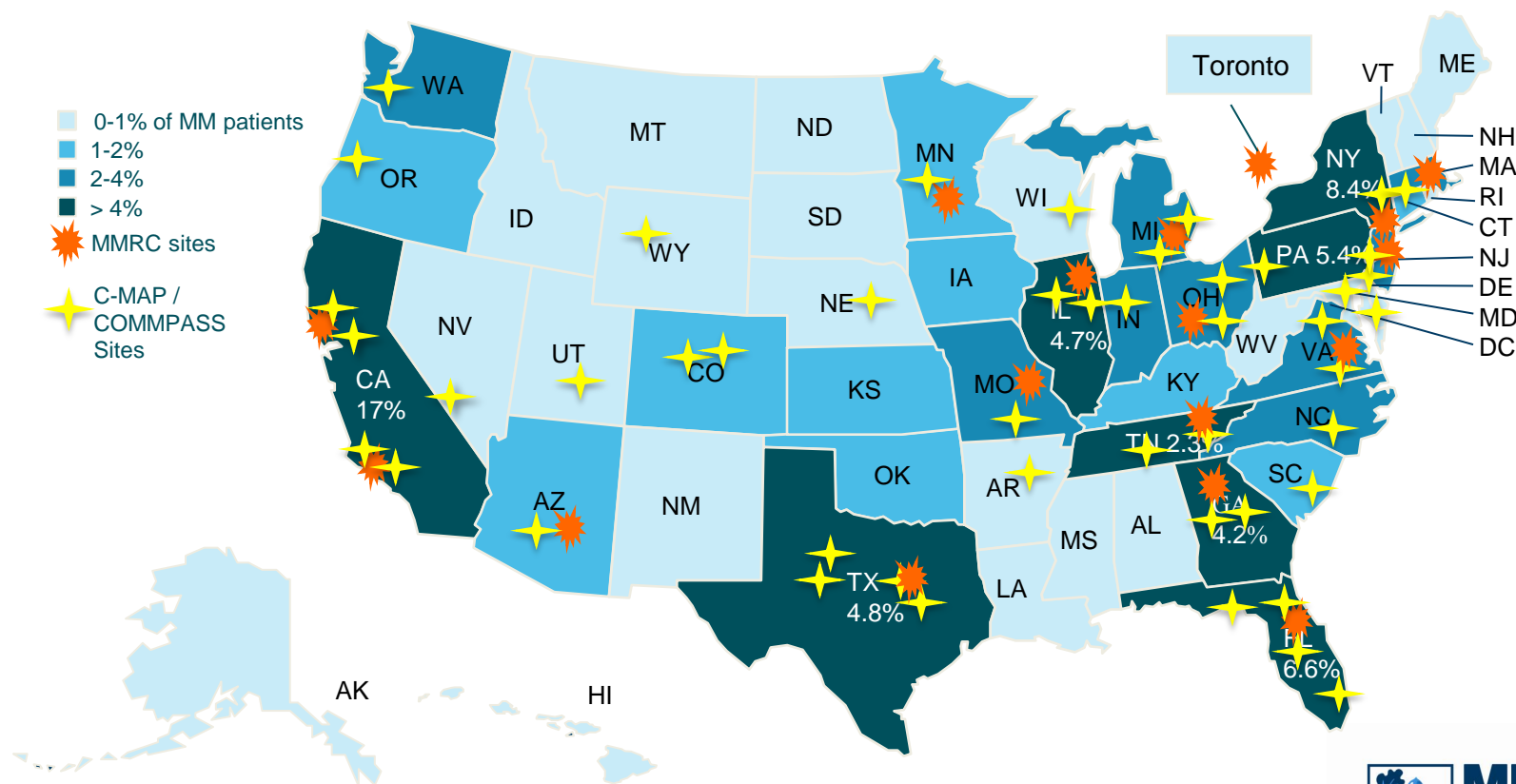


\*On registration track



# MMRF EXPANSION

New MMRF programs like C-MAP and CoMMpass have expanded clinical reach beyond MMRC sites.



Source: NCI SEER database, MMRC SAGE patient database, MMRC knowledge

# MMGI

The MM Genomics Initiative (MMGI) is the most comprehensive analysis of myeloma using cutting-edge technologies.

## Partners



## Results

3,500 samples in MMRC Tissue Bank

GEP, aCGH, DNA methylation,  
DNA sequencing on 250 samples

Open access data portal (1,000+ users)

Whole-genome and whole-exome  
sequencing

- 38 published in *Nature* 2011
- 80+ complete
- 250 target total

## Confirmed findings in MM

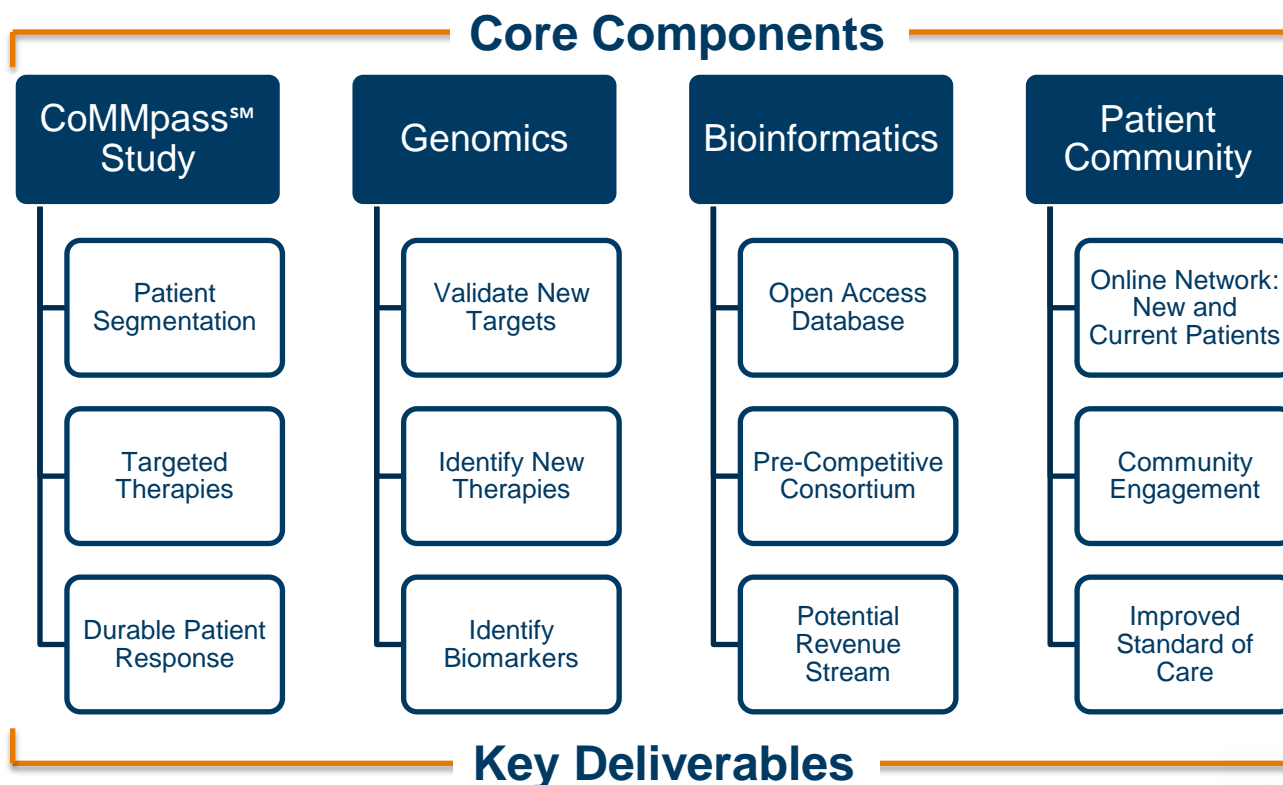
- NF-κB
- KRAS/NRAS
- p53
- Cyclin D1

## Novel findings in MM

- BRAF (melanoma)
- FAM46C
- DIS3

[www.myelomagenomics.org](http://www.myelomagenomics.org)

The MMRF Personalized Medicine program links research, clinical and community activities to move toward tailored therapeutic approaches.



# CoMMpass<sup>SM</sup> OVERVIEW

The CoMMpass Study is a core element of the PM Program to identify patient segments based on molecular profiling.

## Target Enrollment:

>1,000 newly diagnosed patients

## Clinical Data and Tissue Profiling

## Duration:

~8 years (3 year enrollment plus 5 year F/U)

## North America and EU Expansion:

Target 50-75 Centers

Pilot 25 centers Year 1

## Core Molecular Tests

Flow cytometry & BRAF mutations\*

RNA sequencing expression analysis

Whole exome DNA sequencing

Whole genome chromosome analysis

Cytospin slides for FISH

*\* Performed by Spectrum Health - Physician will be provided with results from these assays*



# CoMMpass<sup>SM</sup> GENOMICS

The MMRF CoMMpass trial brings state of the art next-generation sequencing to MM patients at both community and academic centers.

	Patients can obtain w/o physician	Community Clinic Availability	Academic Center Availability	MMRF CoMMpass Academic & Community Centers
Exome Normal Tissue Sequencing	•	*		Yes
MM tumor purification and storage	No	No		Yes
MM tumor exome sequencing	No	No	+/-	Yes
MM tumor RNA sequencing	No	No	+/-	Yes
MM tumor Flow Cytometry and FISH	No	+/-	+/-	Yes

**Legend:** • yes, paid by patient

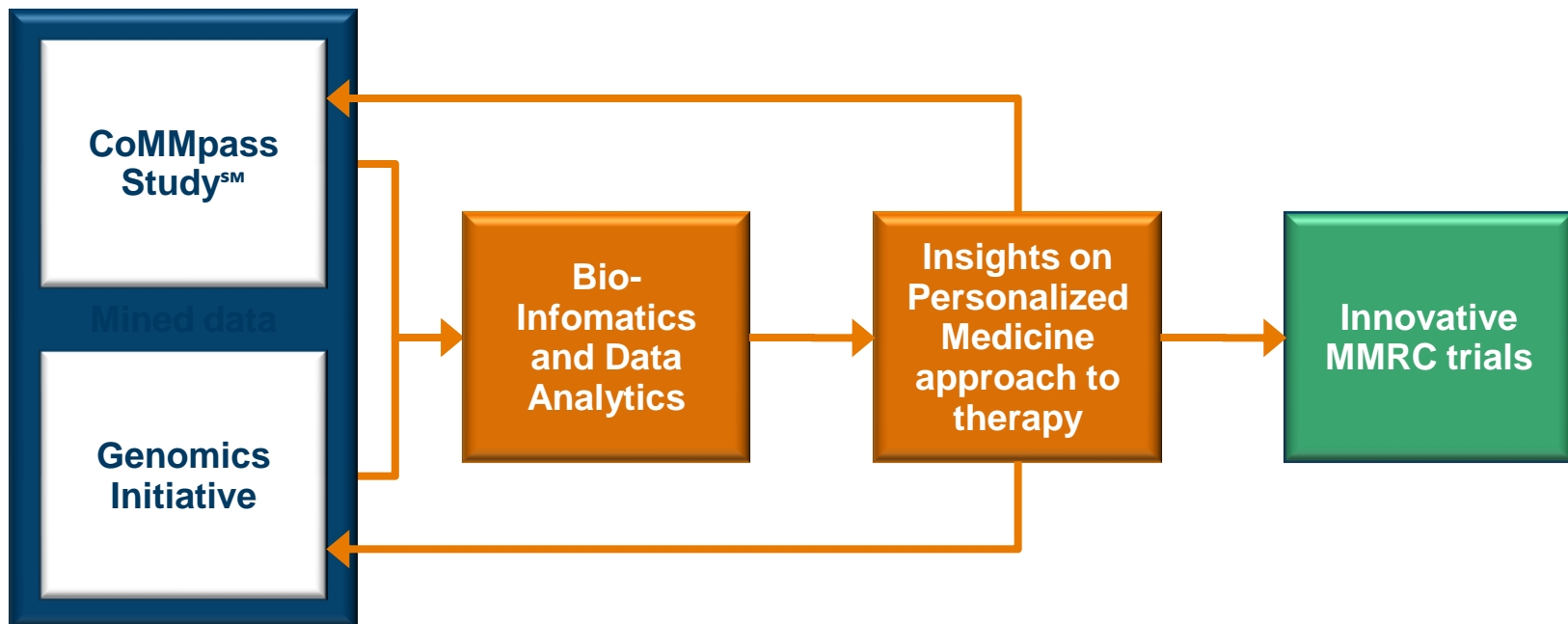
▲ may be paid as part of research program

\* Insurance reimbursement is evolving

+/- ordered by a limited number of centers

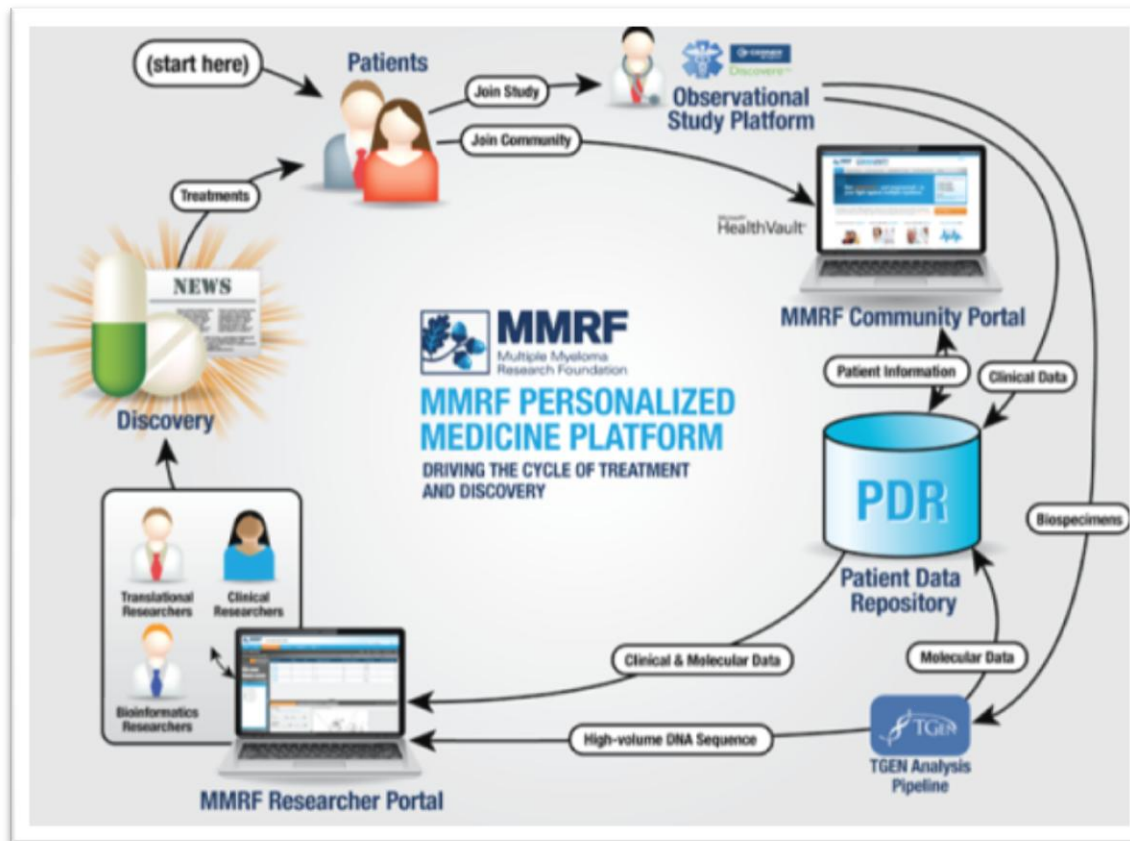
# MMPM MODEL

As new data emerge from CoMMpass<sup>SM</sup>, the MMRC will serve as the go-to place for novel, hypothesis-driven trials.



# MMPM INFORMATICS PLATFORM

The MMPM Informatics Platform is an integrated data system linking researchers, industry, and patients to accelerate scientific advances.



# MMPM RESEARCHER PORTAL

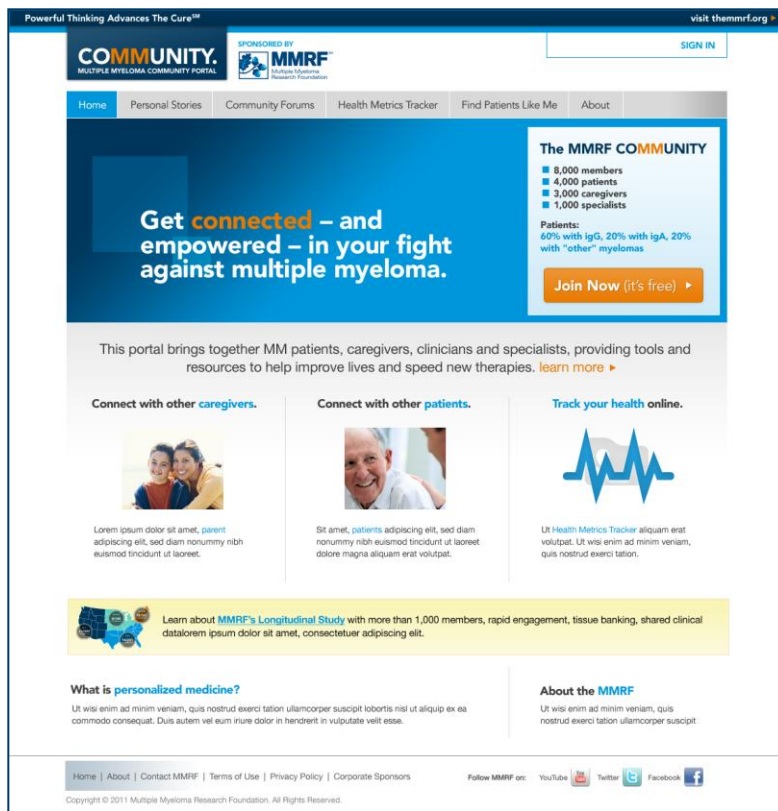
CoMMpass<sup>SM</sup> Study data, available through the Research Portal, will afford access to and socialization of unprecedented, rich data.





# MMPM ON-LINE COMMUNITY

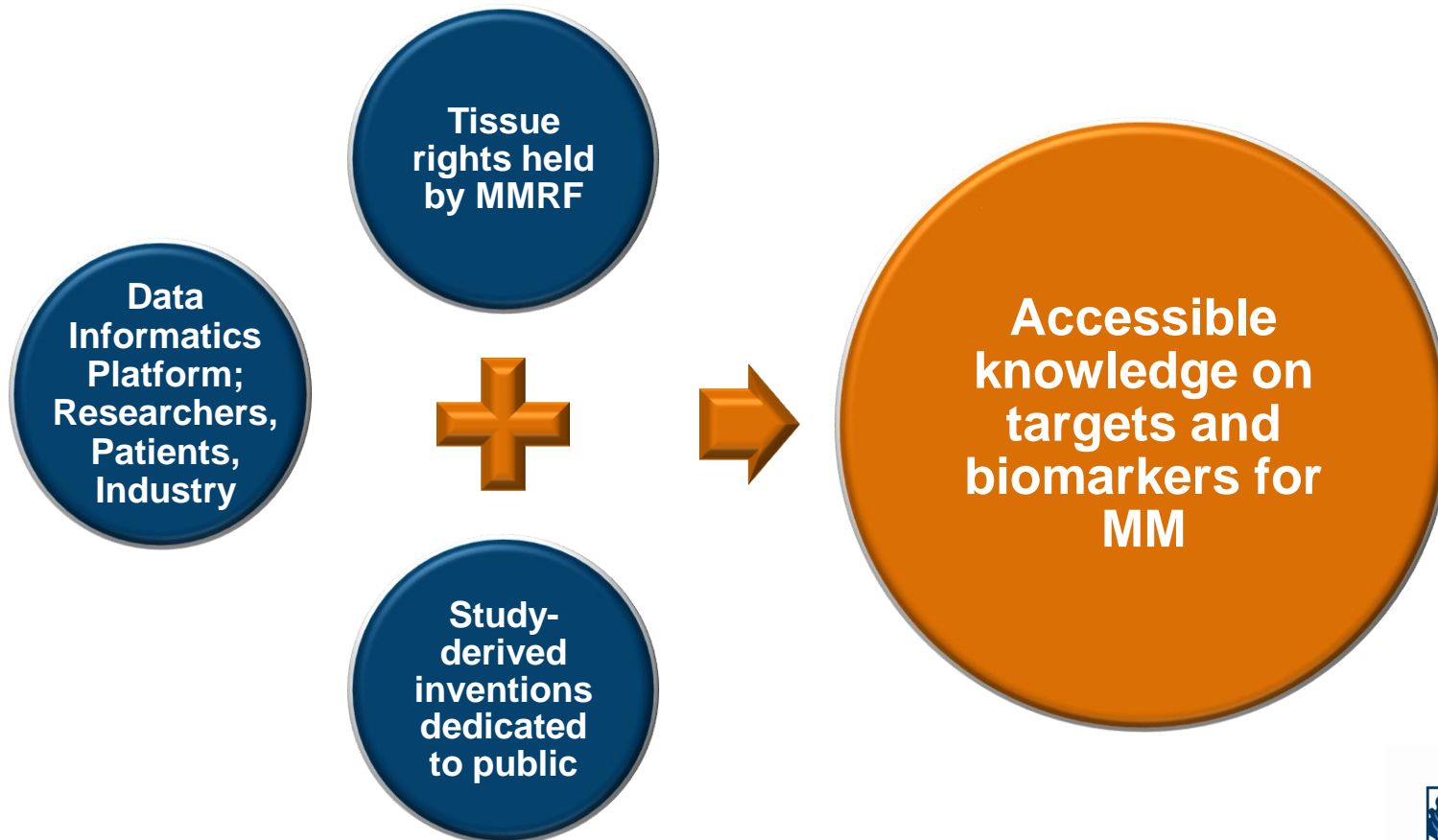
By mobilizing the MM community online, the MMRF aims to accelerate and enable personalized approaches. Key features include:



- Groups based on common molecular profiles
- Ability to connect with similar patients
- Health metrics tracker including treatment history and genetic data
- Ability to manage disease (eg, tools to improve adherence, manage toxicities, etc)
- Access to relevant education materials/data
- Live web discussions hosted by KOLs
- Clinical trial/CoMMpass recruitment tools

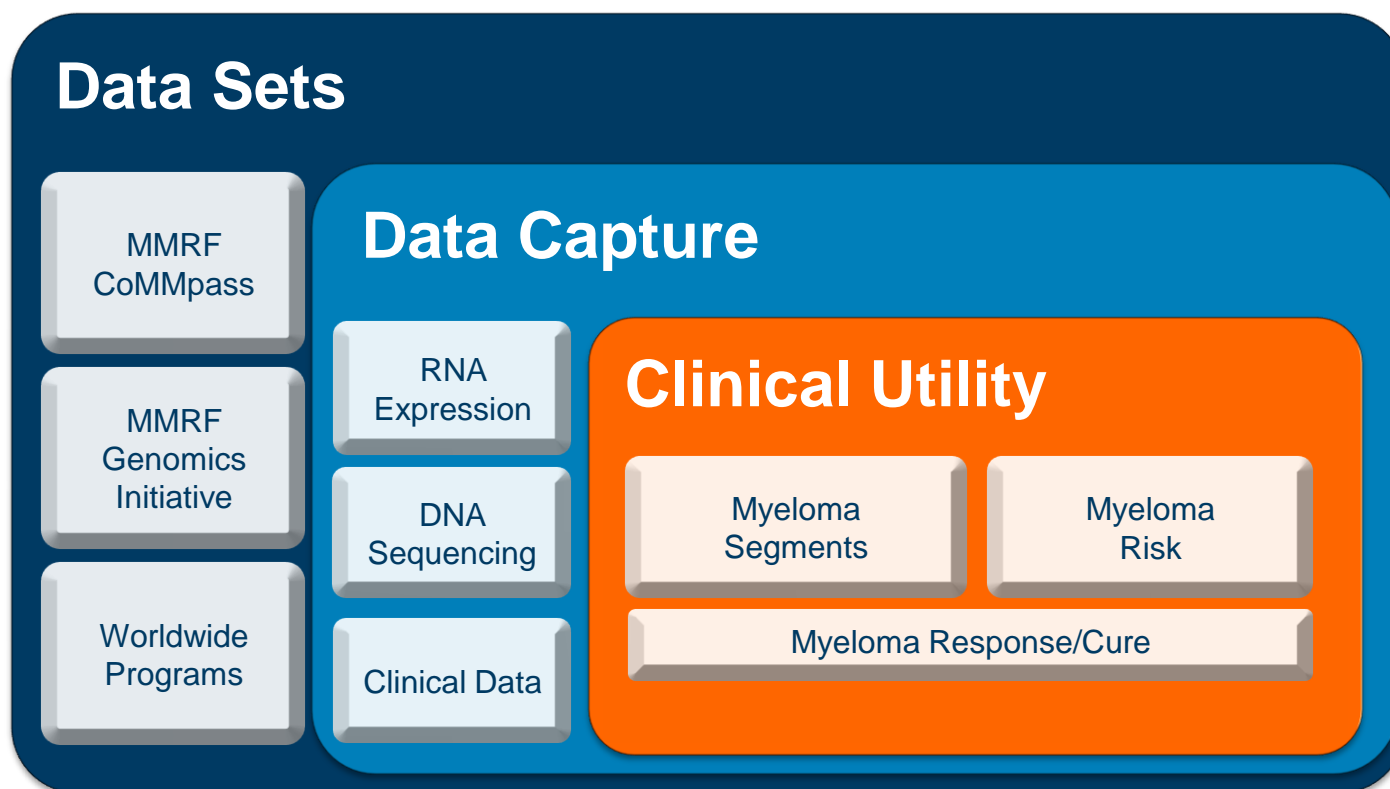
# MMRF AND MMPM MODEL

Through innovative agreements with Industry and Academia, MMRF dedicates CoMMpass<sup>SM</sup> inventions to the public to catalyze scientific discovery.



# FUTURE

The MMRF will build on CoMMpass<sup>SM</sup> and other initiatives to segment the disease, define risk, response, and unmet medical need.



# RESULTS TO DRIVE THE MISSION

From finding more drugs...  
to finding the **right** drugs



# MMRF: ACCELERATING RESULTS

The MMRF and MMRC are improving lives for many patients with debilitating diseases.



Powerful Thinking Advances the Cure<sup>SM</sup>