

Bridging the Chasm Between Microscope and Marketplace



Partnering for Cures | A **FasterCures** Meeting

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MMRF Personalized Medicine Initiative

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MMRFSM
Multiple Myeloma
Research Foundation

Powerful Thinking Advances the CureSM

Background



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The MMRF accelerates new treatments by identifying barriers and building business solutions to solve scientific problems.

1998

No Awareness/Funding

Built Community
Funded Research

2003

Orphan Disease

Tissue Bank
MMRC

Heterogeneous Disease

Genomics

2008

Segmented Disease

Personalized Medicine
Combinations

Crowded Market

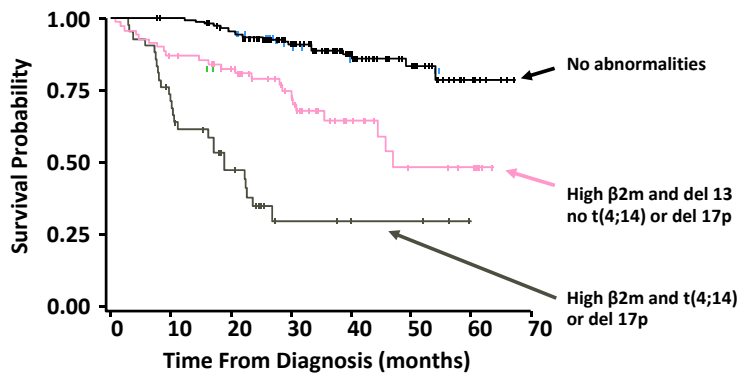
De-risk with Industry

2013

Need

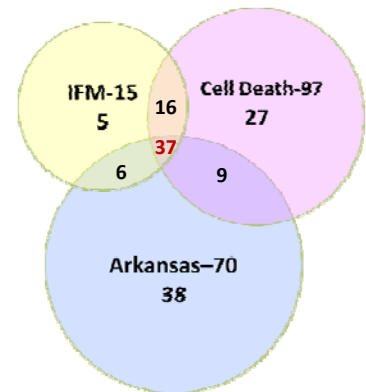
The MMRF must improve overall survival of this fatal cancer by providing objective data and testing hypotheses in the clinic.

Variations in Survival According for Specific Subtypes



Avet-Loiseau H et al. *Blood* 109(8):3489-3495, 2007

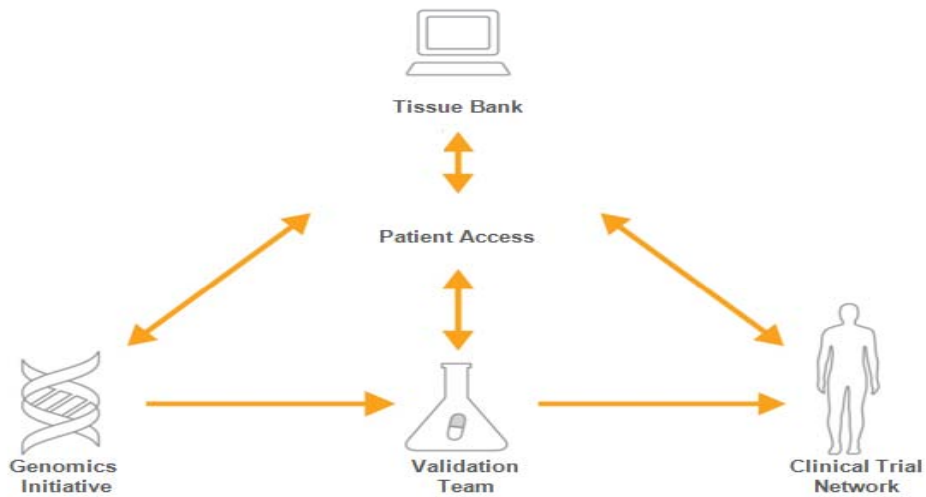
Minimal Overlap in Gene Signatures



Clin Cancer Res. Author manuscript; available in PMC 2010 September 15.

Platform

The MMRF has built a strong platform to succeed in personalized medicine.



Strategic plan



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Working with PWC, the MMRF developed its five-year strategic plan in personalized medicine. Current focus is to strengthen the platform.

Strategic Plan

- Benchmarking
- 1:1 Interviews
- Expert Roundtable
- Operating Plan

Platform Advancements

- Tissue Bank Evolution
- Community Expansion
- Patient Empowerment
- Bioinformatics Platform

Key Success Factors

- Leadership
- Technical Expertise
- Partner Selection
- Donor and Industry Funding

Programs



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While building the platform, hiring and raising funds, the MMRF team is moving forward on program development.




	2011	2012	2013	2014
Basic Science	Next Gen 'omics Biomarker Development	Epigenetics Diagnostics	Correlative Biomarker Identification	Correlative Biomarker Validation
Clinic	Longitudinal Study (LS) Pilot Community Expansion	LS Launch	LS Expansion Clinical Data	Hypothesis-driven Trials
Community	On-line pilot – t(4; 14), p53	On-line Launch	Trial Accrual	Diagnostic Support

Basic science



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The MMRF's commitment to basic science is now being leveraged toward the clinic.

Partners	Results	Next Steps
 WGS, WES, shRNA	10 genes frequently mutated: 4 known and 6 novel in MM	Next-gen 'omics Retrospective tissue bank analysis
 GEP, CGH, siRNA DNA Methylation	HMTs BRAF and RAS	Epigenetic target validation Biomarker-driven trials
 Multiple Myeloma Research Consortium 2,800 samples	Nature manuscript accepted	Diagnostic development

Clinical



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MMRC assets must be leveraged to support emerging challenges in and opportunities in myeloma.

Questions

- What is high risk?
- Management of Revlimid/Velcade failures
- Approaches for t(4;14), p53, t(14;16)

Approaches

- Tissue bank studies
- Correlative Studies
- Longitudinal Study

Critical Actions

- Expansion: Sarah Cannon, Fairfax, Baylor
- Structure: TB, genomic, clinical and portfolio
- Skill Sets: Data Mgmt, Trial design, Business development

Community



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The MMRF community systems are now being transformed to address patient interest and clinical need.

- 200,000 members
- All comers
- General information
- Fundraising



Website



On-line Community

- 10,000 members segmented
- Patients/researchers/MDs
- Data and trials by segment

- >1,000 members
- Rapid engagement
- Tissue banking
- Clinical data
- Data sharing

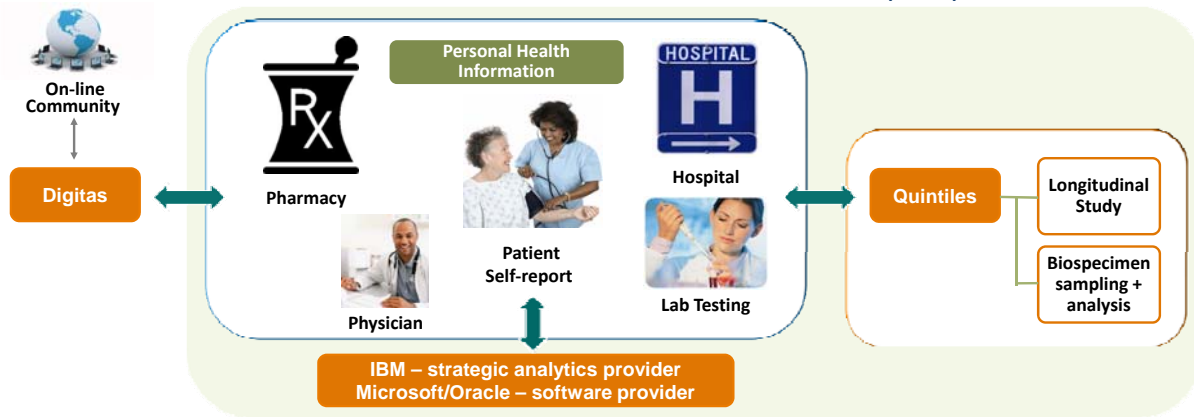


Longitudinal Study

IT Framework

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Integration of and access to complete personal health information is needed for the Personalized Medicine initiative, via multiple partners.



Personalized Medicine Data Ecosystem

Partners



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The MMRF is currently partnering with PWC on the PM strategic plan and Quintiles on the PM operating plan.

Basic Science	Biomarkers/Clinical	Community/IT
Broad	Quintiles	Digitas
TGen	Pharma/Biotech	IT Solutions Provider
Academia	Diagnostics	PHI Provider
Platform Providers	Insurers	Insurers

Investment



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The overall investment in Personalized Medicine is \$35-\$40M over the next five years.

2010

- Strat Plan: PwC
- Next gen 'omics
- Diagnostics
- Market Research
- Community Expansion

TOTAL: \$3M

2011

- Ops Plan: Quintiles
- TB Retrospective
- Epigenetics
- Longitudinal Study Pilot
- On-line Launch

TOTAL: \$8M

2012-2014

- Project Mgmt: Quintiles
- Diagnostics
- Longitudinal Study Expansion
- Hypothesis-driven Clinical Trials
- Standard of care Guidelines

TOTAL: \$8-10M/yr

“It’s very difficult for large organizations to change.
My own advice is to form small organizations that will
change the big ones.”

- Michael Milken
Chairman, FasterCures



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Thank you



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