



Myeloma: Opportunities & Challenges in Moving Toward a Cure



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Moderator

Mike Rice, MS, MBA, Senior Consultant, Defined Health

Panelists

- *Rafael Amado, MD*, Chief Medical Officer, Adaptimmune
- *Kenneth C. Anderson, MD*, Kraft Family Professor of Medicine, Harvard Medical School; Director, Jerome Lipper Multiple Myeloma Center, Dana-Farber Cancer Institute
- *Louis J. DeGennaro, PhD*, President & CEO, The Leukemia & Lymphoma Society
- *Stanley R. Frankel, MD, FACP*, Corporate VP, Head, Immuno-Oncology Clinical R&D, Celgene
- *Anne Quinn Young, MPH, VP*, Development & Strategic Partnerships, Multiple Myeloma Research Foundation

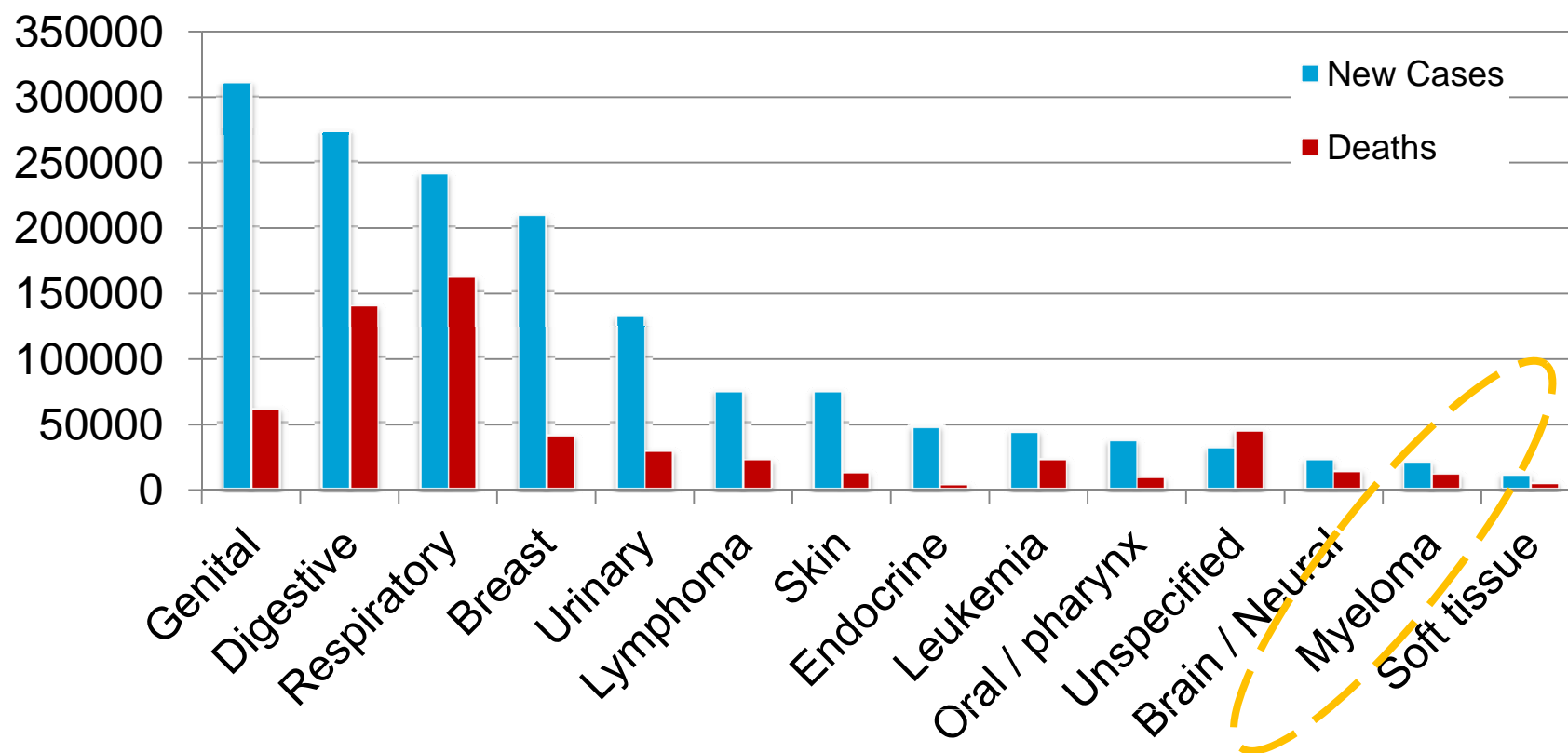
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Multiple Myeloma is a Relatively Low Incidence Oncology Indication

- 26,850 MM patients diagnosed each year in the US (~100k Prevalence)
- ~230k new diagnoses each year WW



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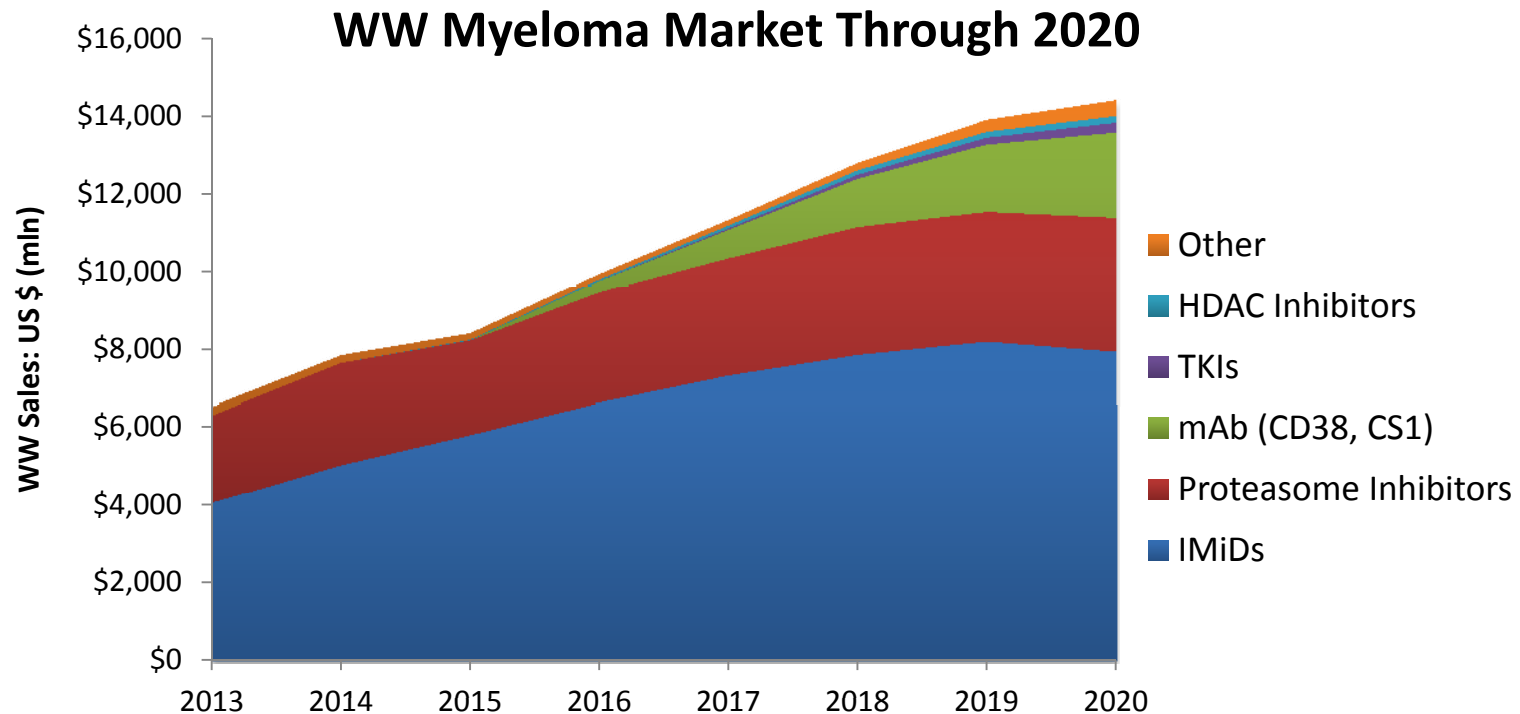
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Yet, Myeloma is Projected to Become One of the Largest Oncology Markets

- \$8B market in 2015, significant growth through 2020 reaching \$14B (11% CAGR)
- IMiDs serve as the treatment backbone in most MM Settings, account for roughly half of sales in MM



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MM Will Yield 3 of the Top 15 Oncology Blockbusters – Including Celgene's Top Selling *Revlimid*

2020 Top 15 Cancer Drugs (\$70B)				
Product	Company	Class	2020 Revenue	Patent Expiry
Revlimid	Celgene	IMiD	\$10,183	Mar 2022
Opdivo	BMS	PD-1 MAb	\$9,276	Dec 2030
Avastin	Roche	VEGF MAb	\$6,514	Jul 2019
Imbruvica	Janssen, AbbVie	BTK Inhibitor	\$5,877	Nov 2027
Herceptin	Roche	Her2 MAb	\$5,805	Jun 2019
Xtandi	Astellas Pharma	Other cytostatics	\$5,198	Aug 2027
Rituxan	Roche	CD20 MAb	\$5,190	Dec 2018
Keytruda	Merck & Co	PD-1 MAb	\$4,311	May 2029
Perjeta	Roche	Her2 MAb	\$3,969	Jun 2025
Ibrance	Pfizer	CDK 4/6 Inhibitor	\$3,252	Jan 2023
Atezolizumab	Roche	PD-L1 MAb	\$2,529	-
Tasigna	Novartis	Abl/c-Kit Inhibitor	\$2,378	Jul 2023
Pomalyst	Celgene	IMiD	\$2,130	Jun 2025
Kyprolis	Amgen	Proteasome Inhibitor	\$1,960	Jun 2026
Jakafi	Incyte	Other cytostatics	\$1,868	Dec 2027

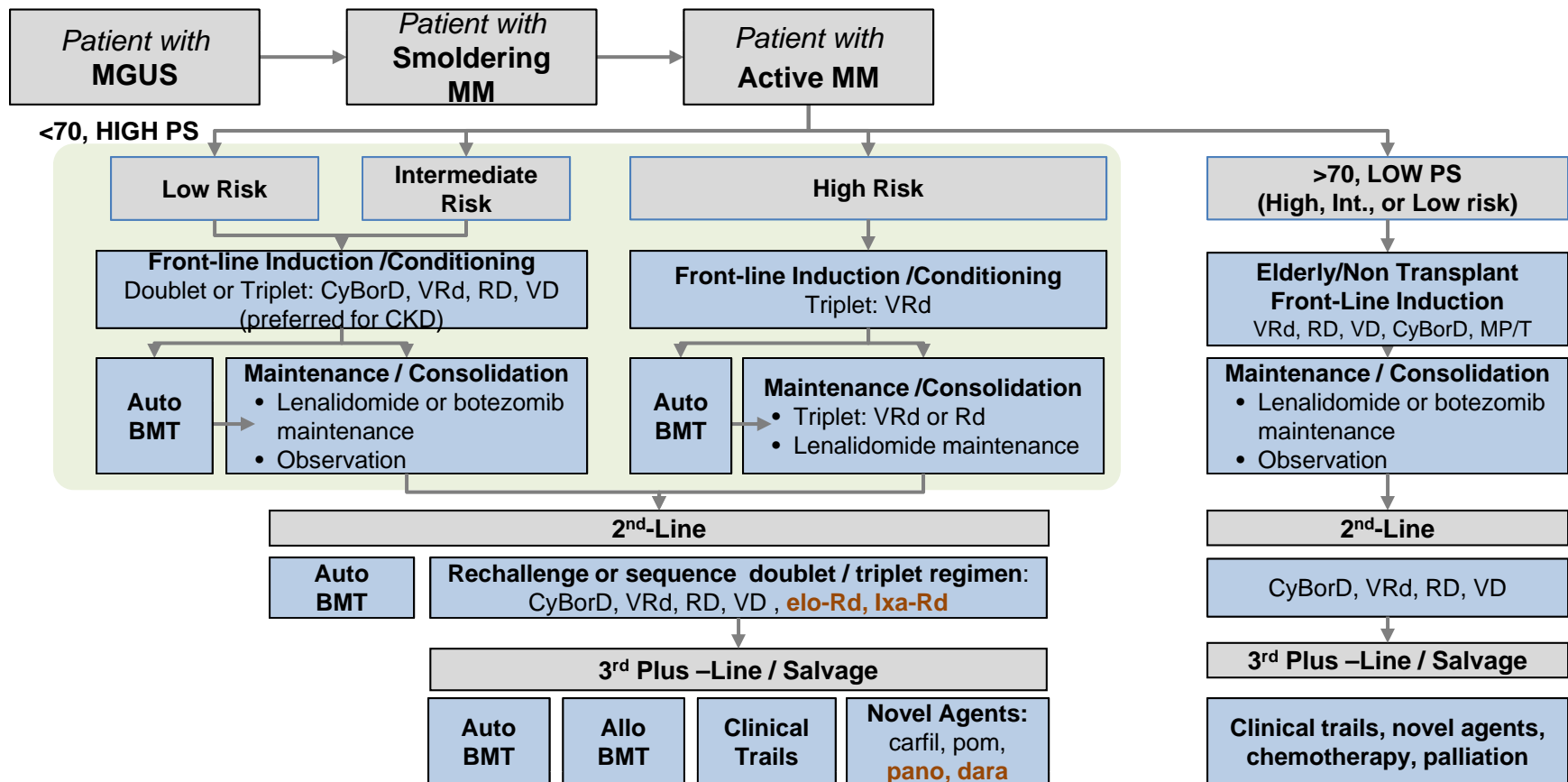
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MM Treatment is Rapidly Evolving With Three New Drug Classes, And Expanding Indications



NCCN; DH primary research and analysis

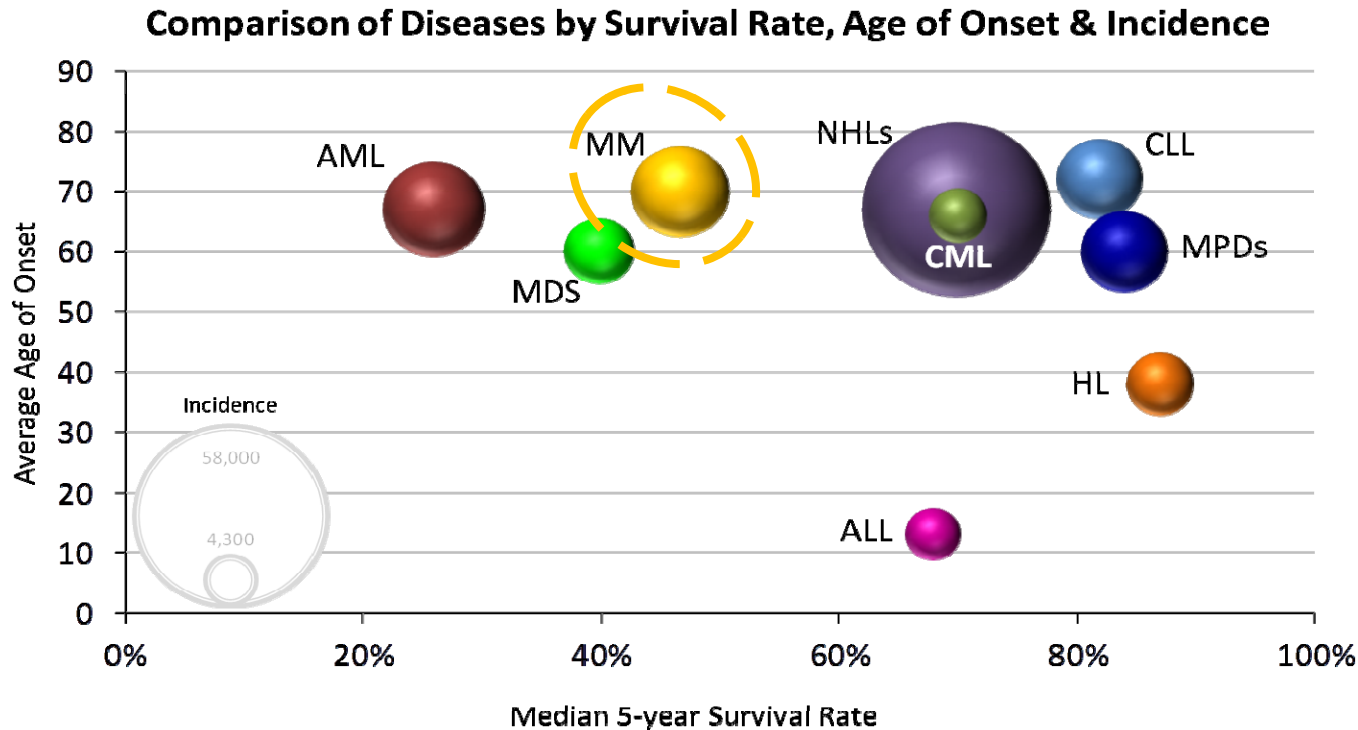
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However, MM Remains a Relatively Poor-Prognosis Among the Blood Cancers

- 75-85% of patients are symptomatic, warranting medical treatment
- Relative to other hematological malignancies, the mortality risk is high in MM, and >11,000 deaths occur from MM each year in the US



SEER; DH Analysis

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Unmet Needs of Myeloma Patients Serve as Opportunities for Continued Impact in MM

Myeloma remains incurable

- Approved agents do not effectively target molecular drivers of disease progression
- RVd (lenalidomide, bortezomib and dexamethasone) induce remissions, but is rarely curative.
- The prognosis for others such as high-risk and relapse/refractory patients remains grim.

Curative therapies far off

- Immunotherapy (CARTs, anti-PD1 mAbs) promises more durable responses, but still in early days.
- Advances are being made in the use of transplantation (e.g. allogeneic SCT, T-cell depletion with DLI add-back), but complex, center-specific protocols are generally not exportable to the broader community.
- Selective targeting of malignant stem cell populations remains an important but as yet unachievable goal.

Empirical treatment decisions

- Few (if any) tools to select which patients are most likely to respond to certain drug classes. One cannot even predict which patients will respond to newly-approved mAbs since their targets are somewhat broadly expressed.
- Absence of minimal residual disease (MRD) following transplant is emerging as a possible surrogate for response durability but has yet to be adopted by the broader clinical or regulatory community.
- Cytogenetics and gene expression profiling are not routinely incorporated in patient stratification and therapy decisions.

Rising cost of therapy

- 3- and 4-drug regimens composed of multiple branded drugs are becoming increasingly expensive, particularly in the US where payers have historically not been equipped to effectively negotiate (e.g. unlike single-payer systems).
- Biomarker-guided treatment strategies and generic competition can only address this so much, at least in the near term.

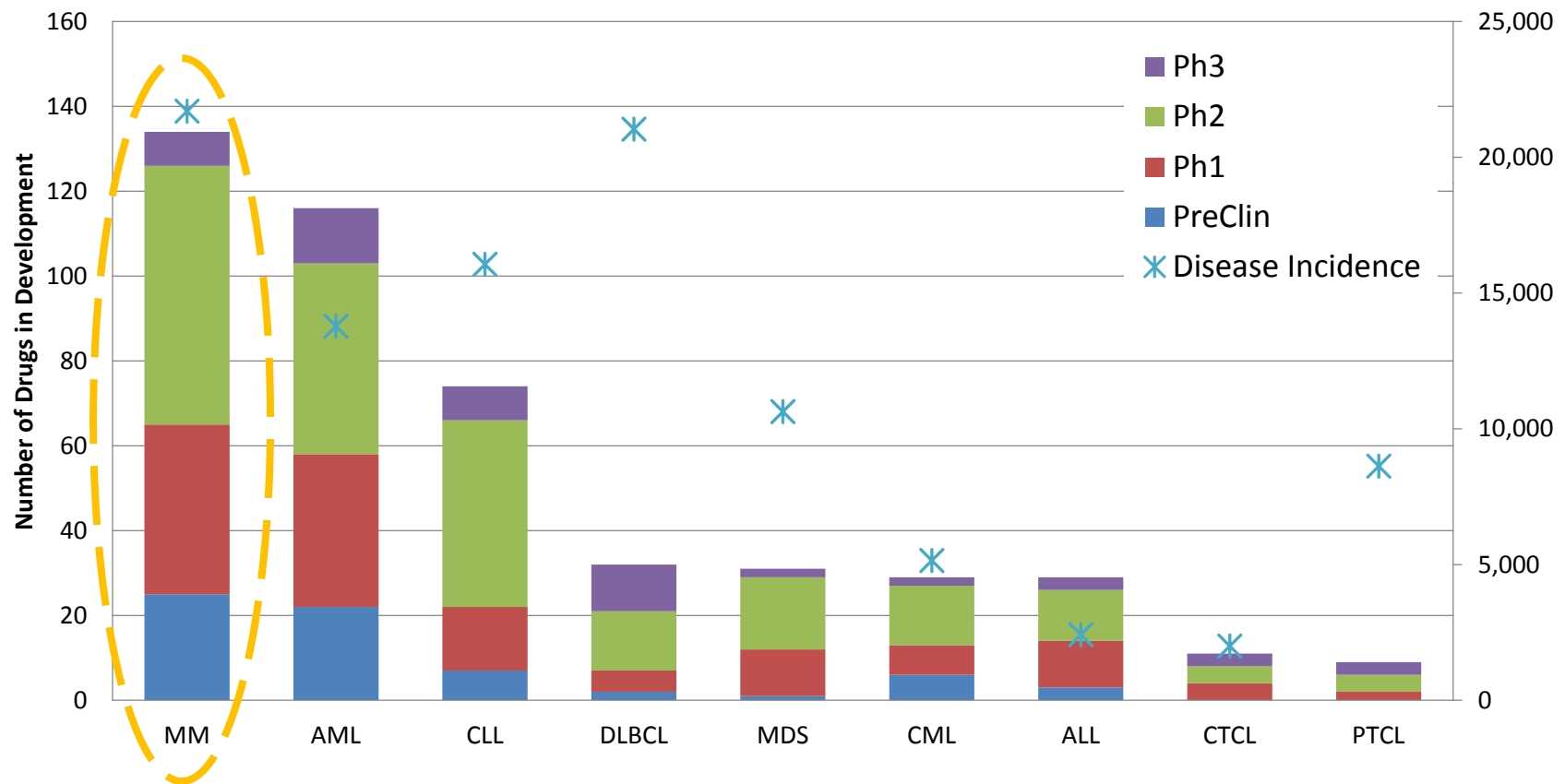
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MM has the Most Competitive Pipeline Among the Heme Malignancies

Pipeline Volume vs. Market Size

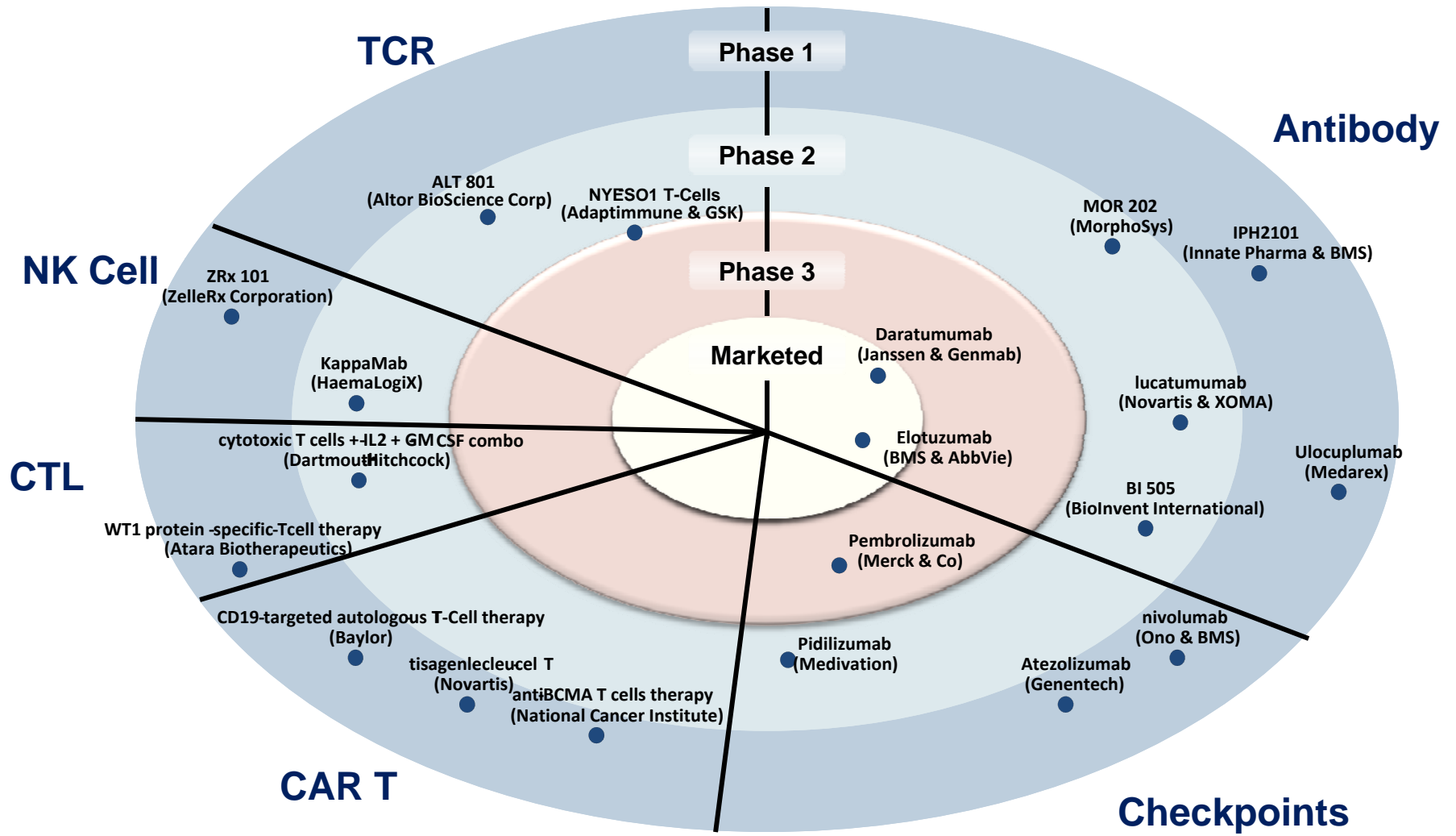


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While Advances in IO Trails Other Cancers, Recent Progress in MM Fuels Investment



Non-profits are Addressing Gaps in MM Research, Patient Education, and Providing Access to Therapy



Headquarters	White Plains, NY	Norwalk, CT	North Hollywood, CA
Yr. Founded	1949	1998	1990
Leadership	Chairman: James Davis CEO: Louis DeGennaro	Chairman: Kathy Giusti CEO: Paul Giusti	Chairman: Brian Durie President: Susie Novis
Total Revenue	\$299.7M	\$26.6M	\$11.0M
Mission Statement	To cure leukemia, lymphoma, Hodgkin's disease, and myeloma and improve the quality of life of patients and their families	Helps accelerate the development of next generation multiple myeloma treatments to extend patient's lives, and lead to a cure	Improving the quality of life of myeloma patients while working toward prevention and a cure

LLS, MMRF, IMF websites & annual reports

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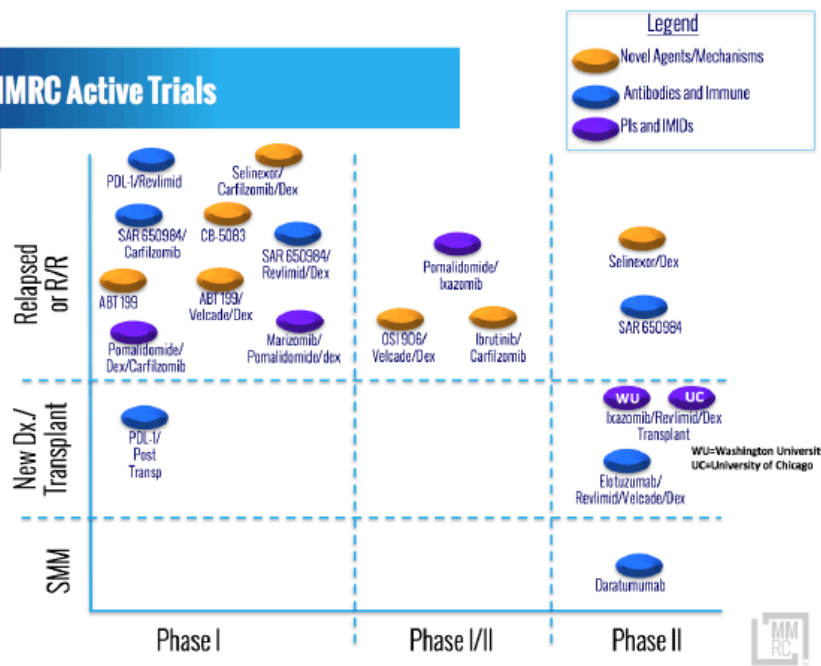
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Patient Advocacy Funding Basic, Translational, and Risk Sharing Clinical Research



MMRC Active Trials



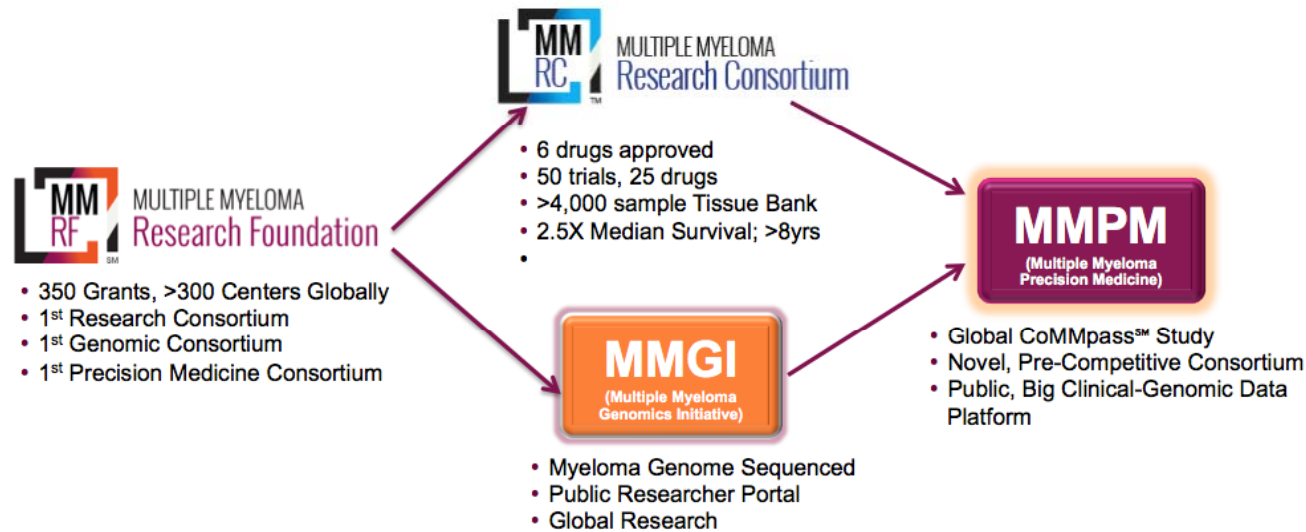
Biotech Accelerator TAP Pipeline

TARGET THERAPY	INDICATION(S)	PRECLINICAL	PHASE I	PHASE II	PHASE III
Apoptosis CPX-351	Secondary AML	Celator			
IL-3R SL-401	BPDN	Stemline			
CD30/CD16A AFM13	Hodgkin Lymphoma	affimed			
CD19 KTE-C19	DLBCL, PMBCL & TFL	Kite Pharma			
Cell Therapy ATR101	Haplo-identical transplant (AML & ALL)	Kiadis			
CD70 ARGX-110	Waldenstrom's Macroglobulinemia	GENX			
HDAC6 ACY-1215/ACY-241	Multiple Myeloma	Acetylon			
CS1/CD138/XBP1 PVX-410 + Revlimid	Smoldering Myeloma	OncoPep			
BET CPI-0610	Lymphoma Multiple Myeloma AML/MDS	Conseration			
CD20/IFNa IGN002	Lymphoma	VALOR			

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Patient Advocacy Seeking Understanding of MM Genetics and Personalized Treatment



- The Multiple Myeloma Genomics Initiative has mapped the full genomes of over 200 MM patients and the results were made widely available to hundreds of researchers worldwide via a public portal.
- The MMRF is also leading the charge toward personalized medicine with the **CoMMpass study**, which aims to recruit 1000 patients and learn how their unique genetic profiles impact the progression of their illness and their response to treatments.


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Patient Advocacy Addressing Increasing Economic Burden of MM




International Myeloma Foundation

We are


What Is the Solution for the Rising Cost of Cancer Drugs?

By [Brian G.M. Durie, MD](#) on March 19, 2015 9:54 AM | [No Comments](#)



What Is the Solution for the Rising Cost of Cancer Drugs?
Dr. Durie's Blog
by Brian G. M. Durie, MD

In the current issue of the [Mayo Clinic Proceedings](#), Hagop Kantarjian, MD and S. Vincent Rajkumar, MD discuss the high cost of cancer drugs and propose some solutions. The high cost of cancer drugs is a major barrier to providing solutions and a chance of implementation. For example, the



LEUKEMIA & LYMPHOMA SOCIETY
fighting blood cancers






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