Insights from Heme Malignancies: Making Breakthrough Conventional and Unconventional Therapies Accessible Beyond Niche Blood Cancer Patients

Cancer Progress
New York, NY | May 7 - 8, 2019

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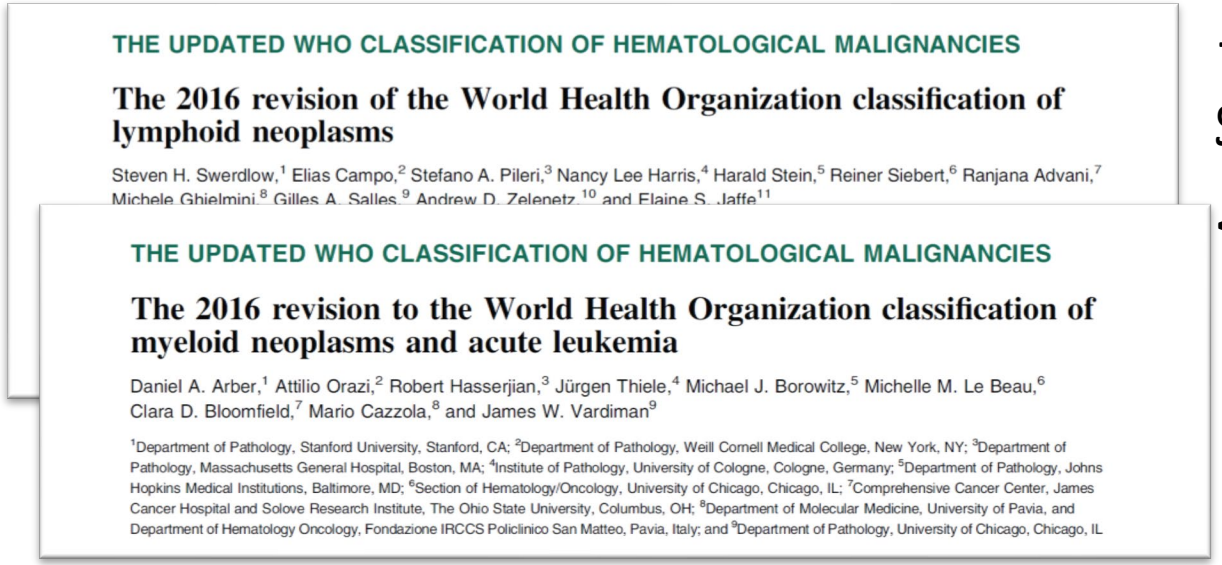
Moderator: **Michael C. Rice**, Principal, Cello Health BioConsulting

Panelists:

- **Lee Greenberger**, PhD, VP and Chief Scientific Officer, The Leukemia & Lymphoma Society
- **Chris Bowden**, MD, CMO, Agios Pharmaceuticals
- **Dan Shoemaker**, PhD, CSO, Fate Therapeutics
- **Vatnak Vat-Ho**, Vice President Business Development, Affimed

Only ~10% of New Cancer Diagnoses are for Heme Malignancies – Very Heterogeneous Groups of Rare Neoplastic Disorders

- ~175 clinically distinct diseases (according to the 2016 WHO classification)
- Combined newly diagnosed cases of blood cancers contributes only about 1/10 of solid tumors.
- Prevalence of individuals living with, or a history of blood cancer is increasing as long-term remission and cures are achieved!



THE UPDATED WHO CLASSIFICATION OF HEMATOLOGICAL MALIGNANCIES

The 2016 revision of the World Health Organization classification of lymphoid neoplasms

Steven H. Swerdlow,[1] Elias Campo,[2] Stefano A. Pileri,[3] Nancy Lee Harris,[4] Harald Stein,[5] Reiner Siebert,[6] Ranjana Advani,[7] Michele Ghielmini,[8] Gilles A. Salles,[9] Andrew D. Zelenetz,[10] and Elaine S. Jaffe[11]

THE UPDATED WHO CLASSIFICATION OF HEMATOLOGICAL MALIGNANCIES

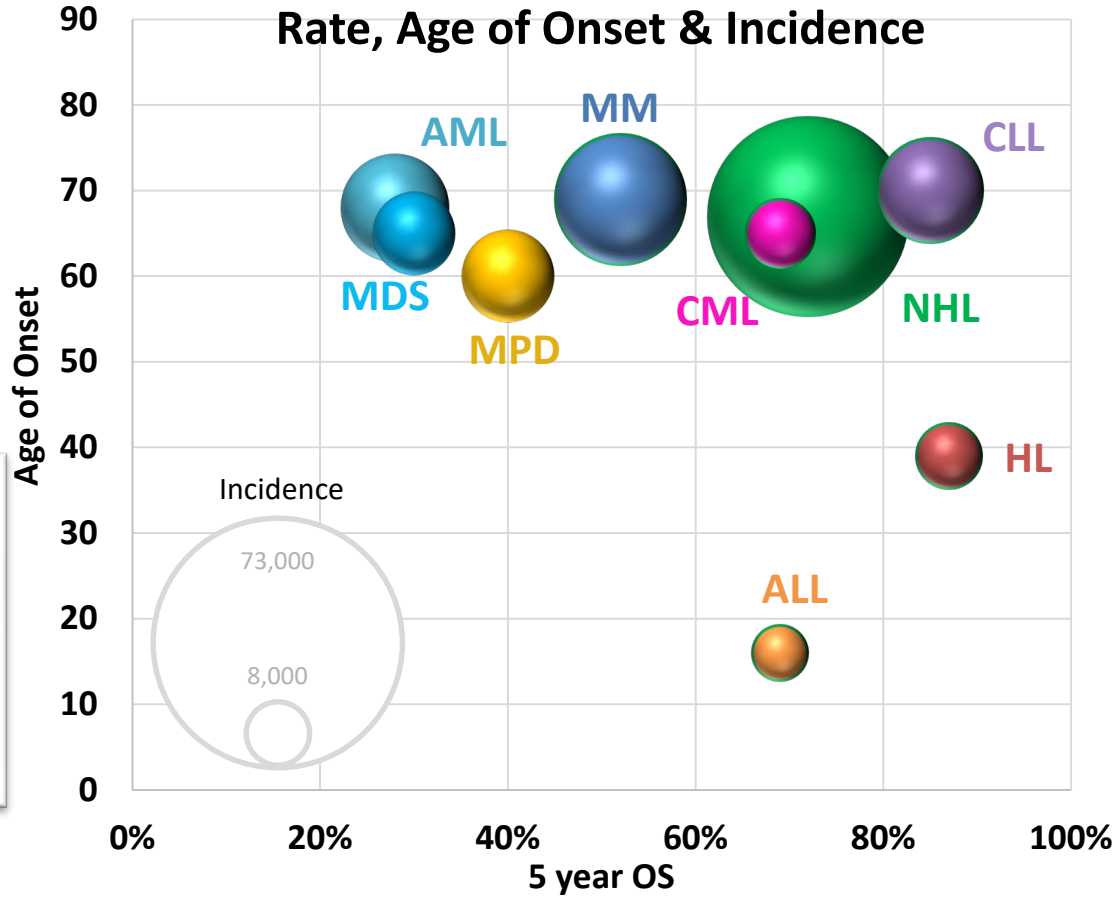
The 2016 revision to the World Health Organization classification of myeloid neoplasms and acute leukemia

Daniel A. Arber,[1] Attilio Orazi,[2] Robert Hasserjian,[3] Jürgen Thiele,[4] Michael J. Borowitz,[5] Michelle M. Le Beau,[6] Clara D. Bloomfield,[7] Mario Cazzola,[8] and James W. Vardiman[9]

[1]Department of Pathology, Stanford University, Stanford, CA; [2]Department of Pathology, Weill Cornell Medical College, New York, NY; [3]Department of Pathology, Massachusetts General Hospital, Boston, MA; [4]Institute of Pathology, University of Cologne, Cologne, Germany; [5]Department of Pathology, Johns Hopkins Medical Institutions, Baltimore, MD; [6]Section of Hematology/Oncology, University of Chicago, Chicago, IL; [7]Comprehensive Cancer Center, James Cancer Hospital and Solove Research Institute, The Ohio State University, Columbus, OH; [8]Department of Molecular Medicine, University of Pavia, and Department of Hematology Oncology, Fondazione IRCCS Policlinico San Matteo, Pavia, Italy; and [9]Department of Pathology, University of Chicago, Chicago, IL

https://link.springer.com/content/pdf/10.1007%2F978-3-642-16483-5_2615.pdf

Comparison of Diseases by Survival Rate, Age of Onset & Incidence



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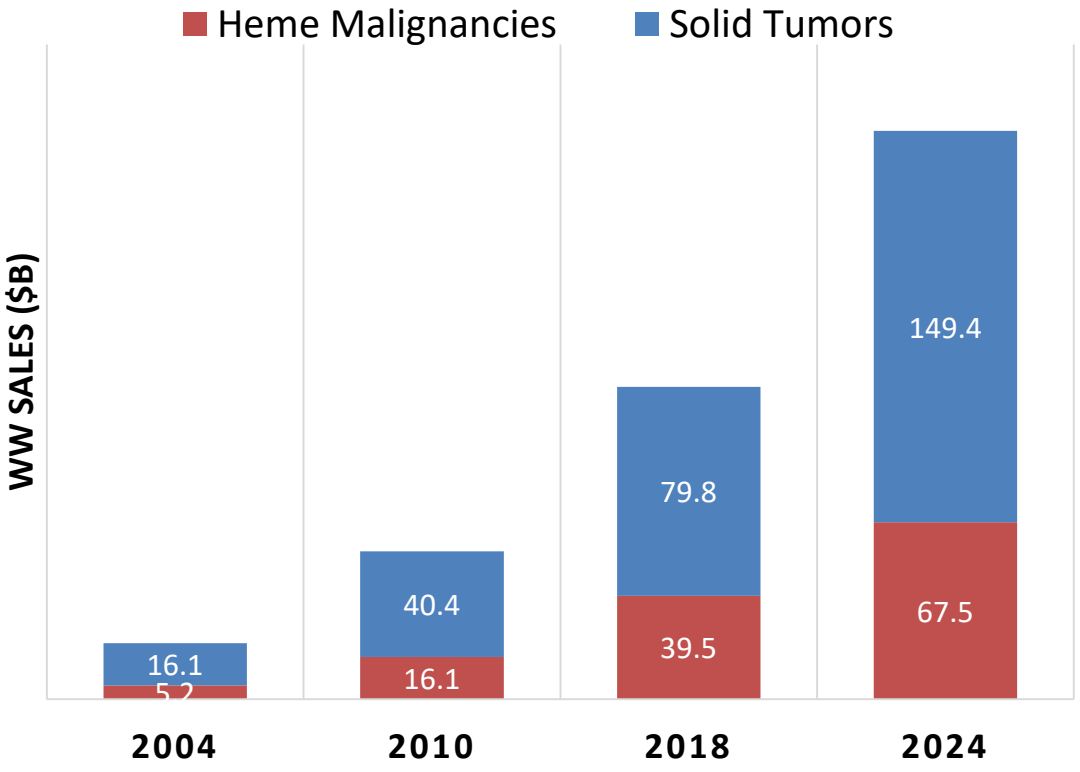
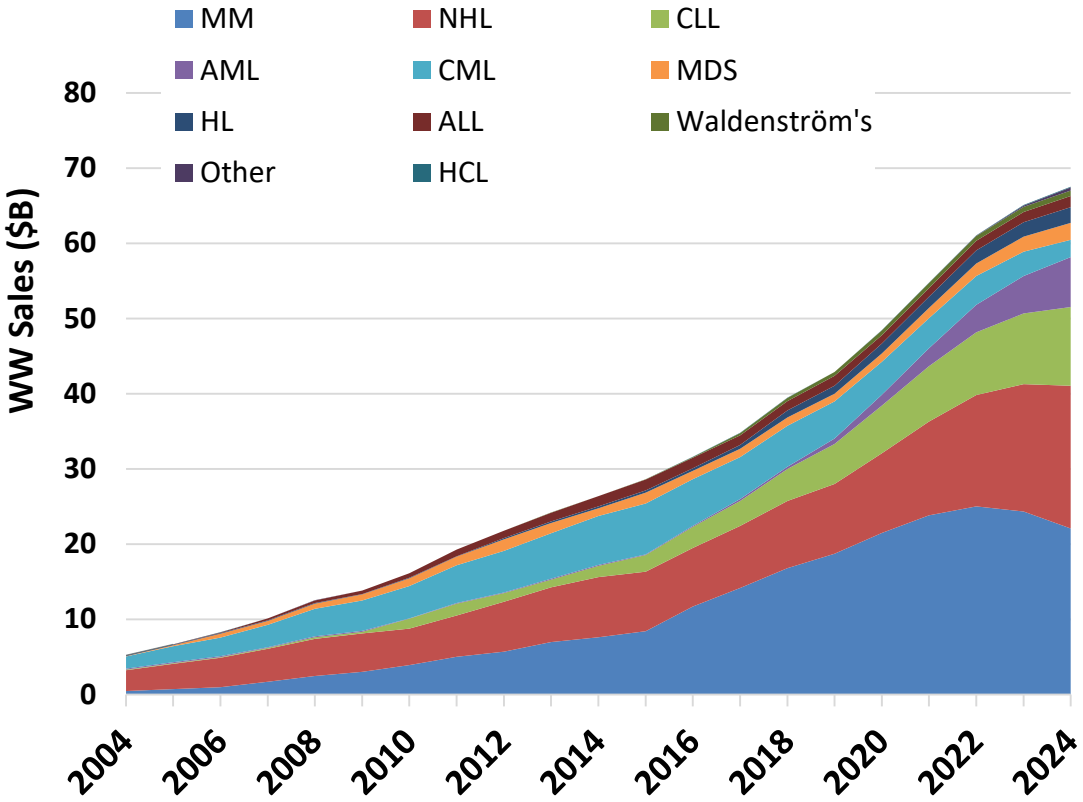
Blood Cancers Comprise ~33% of Current Total Oncology Market – Expected to Grow to ~$67.5B by 2024 (CAGR, 14%)

ONCOLOGY SALES BY HEME MALIGNANCIES VS SOLID TUMORS



EvaluatePharma; CHBC Analysis

HEME MALGNANCY SALES BY INDICATION (2004 - 2024)



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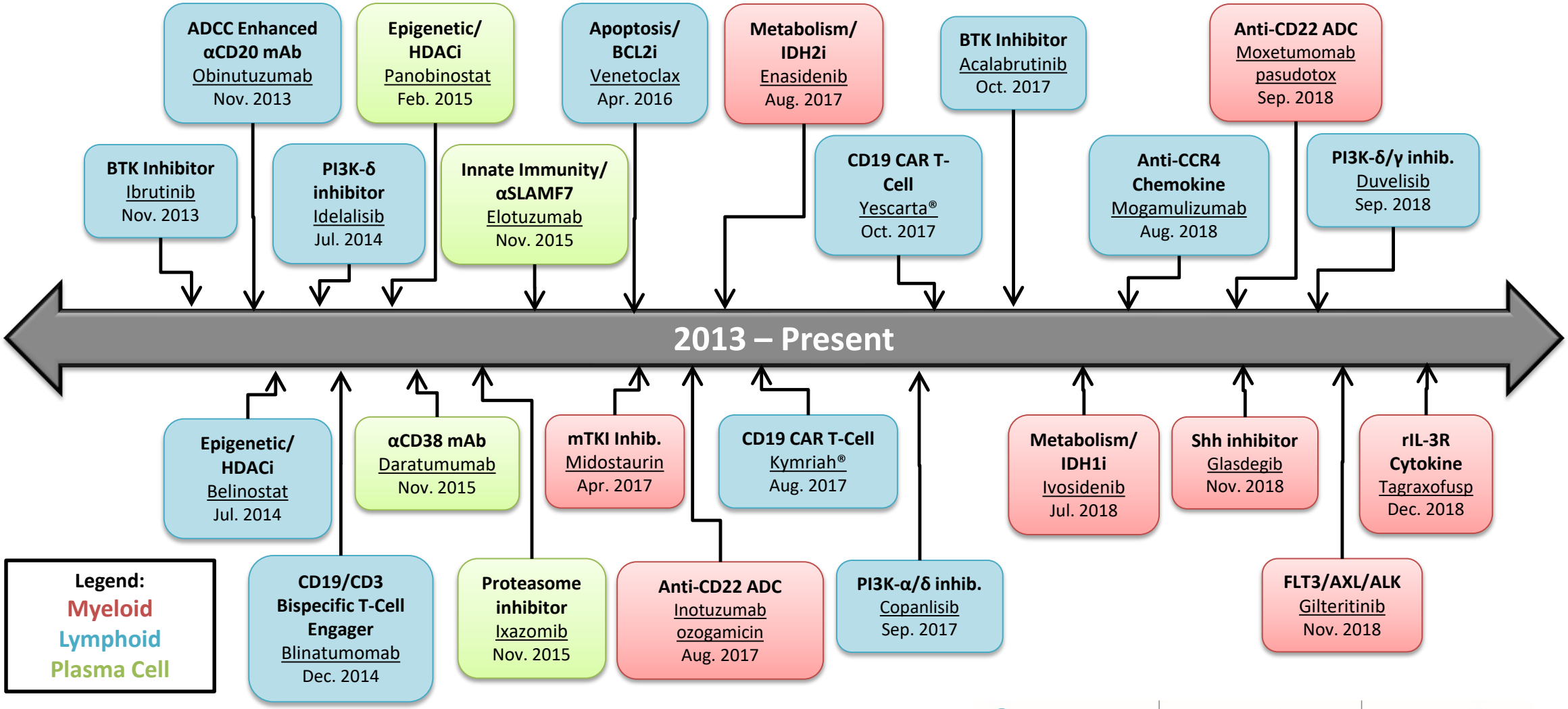
In The Past Five Years, Rare Blood Cancers Have Been a Hotbed of Innovation for First-in-Class Drug Approvals (2013 – Present)



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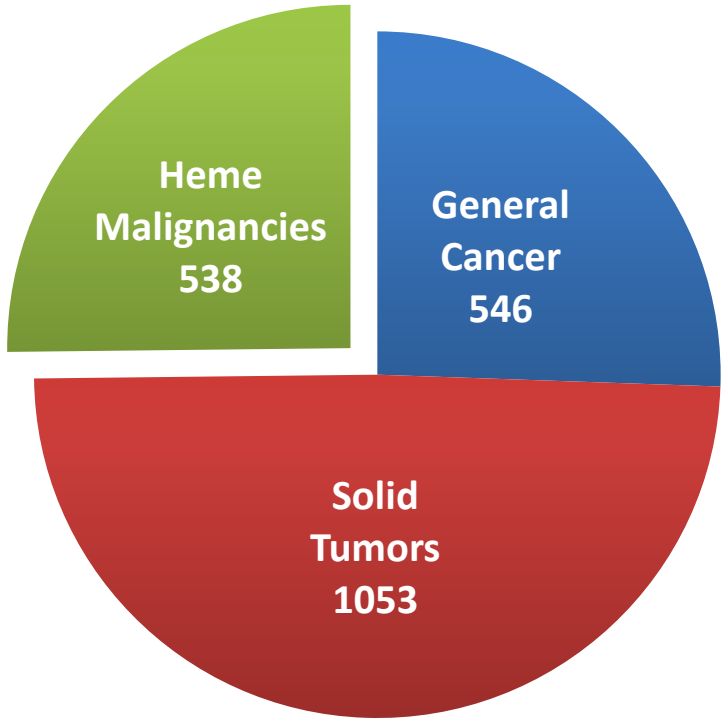
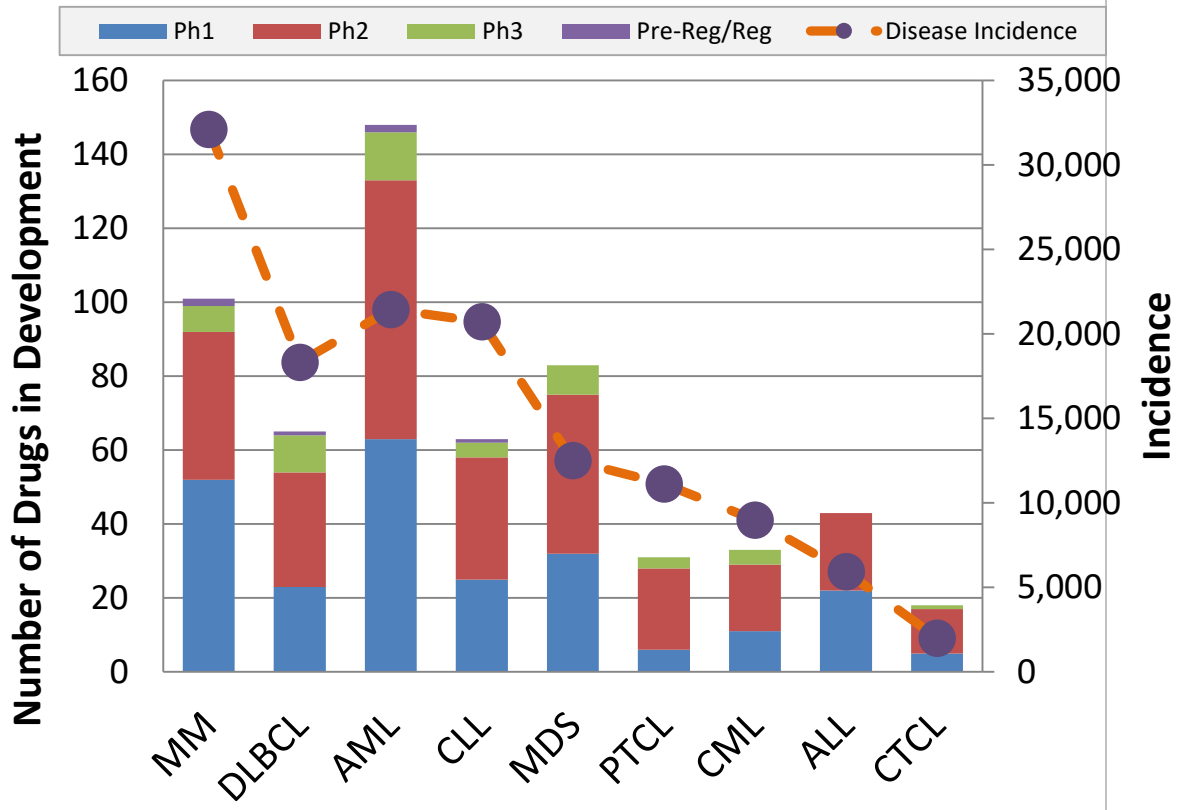
~1/4 of Total Oncology Pipeline Is Focused on Blood Cancers – 70% of Which are in Phase 1 – Phase 2 Developments in the US

Tumor Type and Number of Pipeline Agents by Highest Phase Indication in US

N = 2,137



Pipeline Volume vs. Market Size in US



Clarivate Analytics Cortellis; CHBC Analysis

Hematology Deals Over The Past Year Has Had A Mix Of Modalities, Particularly In Early Stages

Alliance	License / Acq	Class	Indication (Phase)	Upfront	Total	Notes
BMS / Celgene	Acquisition	Multiple therapies (including CAR-T cell therapies)	Multiple	$35B	$74B	Bristol-Myers Squibb acquiring Celgene for $74B, leveraging rights to WW blockbuster drug Revlimid. Deal includes Lisocabtagene maraleucel and pipeline from Juno acquisition in Jan 2018.
Eli Lilly / Loxo Oncology	Acquisition	Small Molecules	Multiple	$7.2B	$7.2B	Eli Lilly acquires Loxo Oncology for $7.2B who develop small molecules to inhibit TRK, BTK, and others.
Jazz Pharma / Codiak Biosciences	WW License	Exosomes	Preclinical	$56M	$1.07B	Codiak grants Jazz exclusive, worldwide rights to develop, manufacture and commercialize exosome candidates against five oncogene targets for hematological malignancies and solid tumors using Codiak's engEx precision engineering platform
Triphase Accelerator / Celgene	Option	Small Molecule	Preclinical	$980M	$40M	Triphase Accelerator grants Celgene an option to acquire TRPH-395 (WDR5 inhibitor) to treat blood cancers including leukemia.
TeneoBio / AbbVie	Option	Monoclonal Antibody	Preclinical	$90M	N/A	TeneoOne will receive an upfront payment of $90 million and will continue developing TNB-383B through Phase 1. AbbVie will hold the exclusive right to acquire TeneoOne.
argenx / Janssen	WW License	Monoclonal Antibody	Phase I/II	$300M	$1.8B	Janssen acquires a WW license for an anti-CD70 monoclonal antibody (cusatuzumab) for AML and high-risk MDS.
Abzena / Tmunity	R&D Collab	CAR-T Cell therapy	Preclinical	N/A	N/A	Abzena will humanize monoclonal antibodies and Tmunity will use them to develop their CAR-T products.
Molecular Templates / Takeda	Collab & Option	Engineered Toxin Bodies	Preclinical	$30M	$663M	Joint development of CD38-targeted engineered toxin bodies (ETBs) for multiple myeloma.
Ono / Fate Therapeutics	Collab & Option	CAR-T Cell therapy	Preclinical	N/A	$1.25B	Using Fate's iPSC platform, two off-the-shelf CAR-T cell therapies will be jointly developed, one for lymphoblastic leukemias, and the other for solid tumors.

BCIQ; Adis R&D Insight; CHBC Analysis.

Questions for Panel

1. What are the most significant advances in heme malignancies in recent years?
2. Why has drug research for blood cancers yielded such a diverse array of treatment options, many vastly improving patient outcomes?
3. Most of the recent approvals have narrowly defined indications and/or issues with patient access, what efforts are underway to improve accessibility?
4. What conventional and IO combination approaches look promising and what are the drawbacks in terms of toxicities and economics?
5. What is the promise and what efforts are being made to expand the potential of these recently approved drug classes and therapeutic technologies to solid tumors?
6. Vice versa: How are innovations in solid tumors impacting management of patients with solid tumors?

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