

# Canaccord's 42nd Annual Growth Conference Aptose Corporate Presentation

August 2022



PRECISION ONCOLOGY FOR  
THERAPIES OF TOMORROW

NASDAQ: **APTO**  
TSX: **APS**

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# Aptose Biosciences (NASDAQ: APTO)

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## Building a pipeline of “Best in Class” targeted therapies to serve cancer patients with hematologic malignancies

- Precision therapeutics designed to provide **single agent efficacy** and to be used in combination with conventional anti-cancer therapies and other targeted therapies
- Targeting key drivers of disease in cancer cells without overlapping toxicities to provide **efficacy with safety to improve the quality of life for cancer patients**

## Investor highlights

- **Experienced leadership team** with deep expertise in kinase inhibitors & orphan hematologic diseases
- Clinical stage oncology company with **two highly differentiated myeloid kinase inhibitors**

### HM43239 clinically de-risked lead precision medicine as primary value driver

- Safely achieved single agent Complete Remissions (CRs) in R/R AML patients
- CRs delivered in multiple genetically-defined AML target populations
- Response rates 40%+ in AML population that may support **single agent accelerated path**
- Combination studies planned to position as a **preferred agent for broad commercial use**

### Meaningful near-term upside with value-driving clinical updates and milestones through 2022 and 2023

- Value potential: Market Cap. \$75M, \$294M June 2021; 52-Week range: high \$3.13, low \$0.73



# Aptose Biosciences: Clinical Stage Pipeline of Differentiated Myeloid Kinase Inhibitors

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## **HM43239** oral myeloid kinase inhibitor clinically validated for R/R AML patients

<b>Clinically Safe &amp; Effective</b>	<b>25-44% ORR</b> in Phase 1/2 Trial with <b>CRs in multiple genetically-defined AML target populations</b>
<b>Near-term Value Creation</b>	Expansion Trials begin 2022 as passage into Registrational Studies planned for 2023
<b>Orphan and Fast Track</b>	Designations earned with impressive clinical responses across AML populations
<b>Broad Market Opportunities</b>	Across R/R and front line, fit and unfit, induction and maintenance therapies

## **LUXEPTINIB (CG-806)** dual lymphoid and myeloid kinase inhibitor

<b>High Value Targets</b>	B-cell cancers, AML/MDS and inflammation: BTK, FLT3, LCK, LYN, Others
<b>Activity in Ill Patients</b>	Difficult to treat R/R B-cell lymphoma/CLL and R/R AML patients
<b>Improved Formulation</b>	G3 formulation being explored to reduce drug substance and increase plasma exposure



# Aptose Leadership Team: Multifaceted Expertise in Therapeutic Development



**Rafael Bejar, MD, PhD**

Sr. VP & Chief Medical Officer



**William G. Rice, PhD**

Chairman, President & Chief Executive Officer



**Fletcher Payne**

Sr. VP & Chief Financial Officer



**Philippe Ledru**

Sr. VP & Chief Commercial Officer



# Aptose SAB: Distinguished Opinion Leaders with Deep Oncology Expertise

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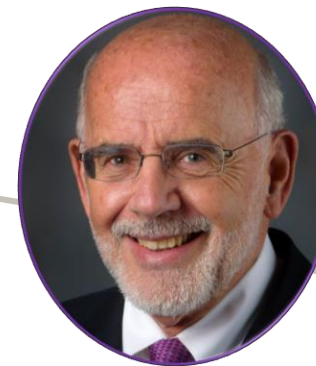
**Daniel Von Hoff, MD, FACP**

Former President of AACR  
Board Member of ASCO  
Former Presidential Cancer Advisory Board  
Physician in Chief, TGen  
Medical Director of Research for McKesson Specialty Health  
Chief Scientific Officer for US Oncology Research  
Professor of Medicine, Mayo Clinic Scottsdale



**Brian J. Druker, MD**

Pioneer in the field of precision medicine  
Key Role in development of Gleevec - the first targeted kinase inhibitor for cancer  
Member, National Academy of Medicine, National Academy of Sciences & American Academy of Arts & Sciences  
Winner of Karnofsky Award, Lasker Award, Japan Prize in Healthcare and Medical Technology, Tang Prize in Biopharmaceutical Science, Sjöberg Prize  
Leader of Inter-institutional Beat AML Initiative



**Michael Andreeff, MD, PhD**

Renowned hematology specialist  
Professor of Medicine  
Paul and Mary Haas Chair in Genetics  
Chief, Section of Molecular Hematology and Therapy  
MD Anderson Cancer Center  
Expert in AML and other hematologic malignancies  
Expert in drug resistance and drug mechanisms







## HM43239 “239”

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Oral, Daily, Myeloid Kinase Inhibitor for  
Genetically-Defined AML Target Populations

# AML in the US: Estimated 20,240 new cases and 11,400 deaths in 2021

## Continued Unmet Need for More Effective and Safe Therapies

Epidemiology	 US (2021)	 EU5 (2020)	 Japan (2021)	 China (2020)
Leukemia Incidence <sup>3</sup>	61,090 <sup>1</sup>	51,820 <sup>3</sup>	14,600 <sup>7</sup>	85,400
<b>AML Incidence</b>	<b>20,240<sup>2</sup></b>	16,580 <sup>3a</sup>	6,570 <sup>7c</sup>	31,430 <sup>3b</sup>
5-Year Prevalence (Leukemia) (2020) <sup>3</sup>	187,560	152,230	41,280	241,750
<b>Mortality (Leukemia)</b>	<b>11,400 (AML)<sup>2</sup></b>	31,690	8,700 <sup>7</sup>	61,690



**Deadly and heterogeneous cancer with 5-year survival rate at diagnosis of approx. 29%**

**Relapsed AML patients have a median life expectancy of < 6 months\* with approved therapies**

**Need more effective & better tolerated targeted agents**

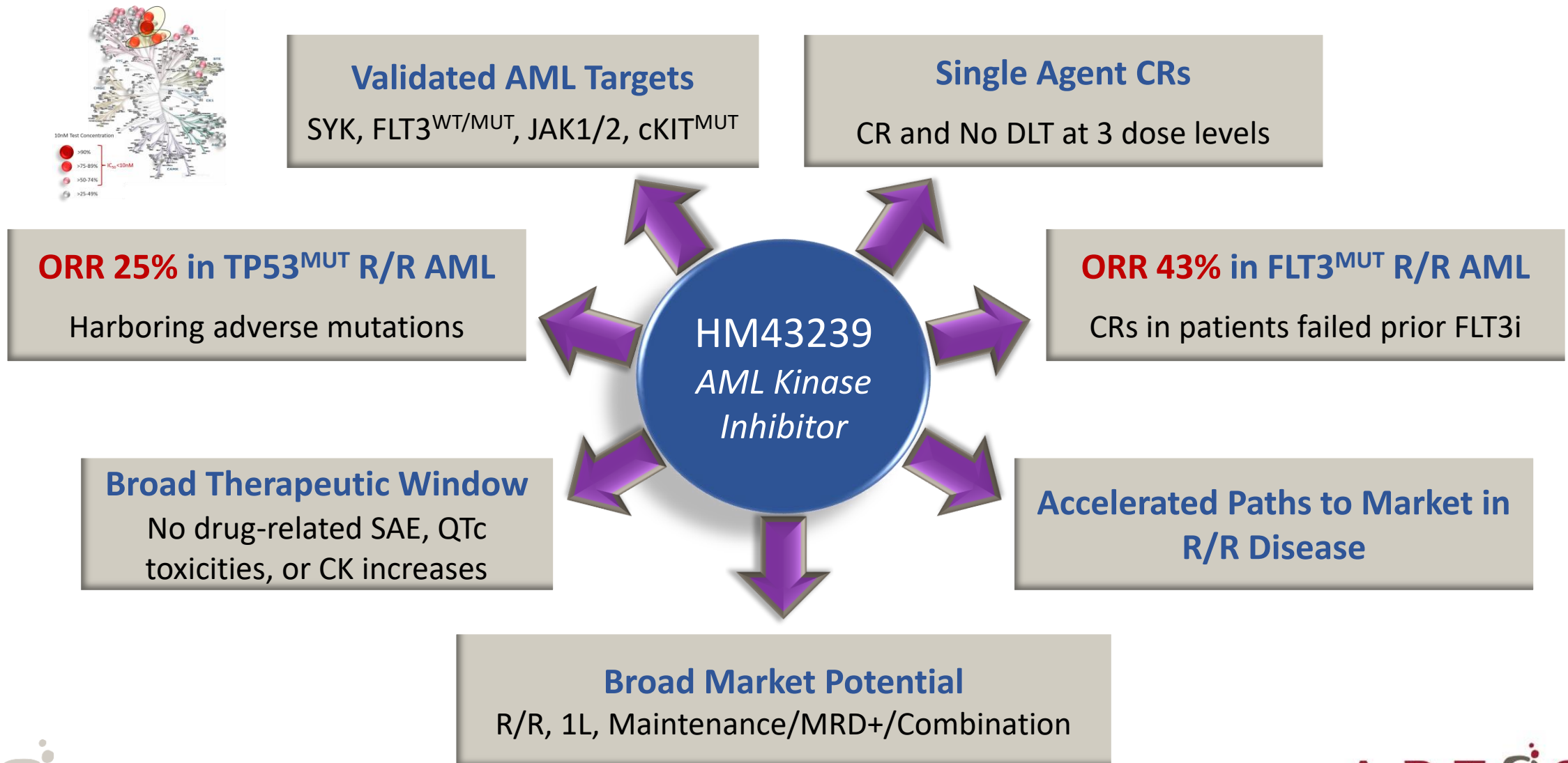
- **DURABILITY** to achieve **lasting remissions** and **extend meaningful/quality life**
- **SAFETY** for **maintenance / MRD+ therapy** and for drug **combination therapy**
- **BREADTH** to **better treat R/R AML patients** and **overcome resistance** to current agents

**Safe and effective agents expected to expand AML market and command significant market share**



# HM43239 Effective and Well Tolerated Targeted Agent

## Proven Broad Clinical Activity in AML Patients to Treat Significant Unmet Needs



# Emerging Clinical Data Support HM43239 as Potential Superior Therapy

# HM43239 Phase 1/2 Study in R/R AML: Ongoing Dose Escalation & Dose Exploration

PART A : DOSE ESCALATION (18 Pts Dosed)			PART B : DOSE EXPLORATION (39 Pts Dosed)		
Cohort 6	200 mg QD	Ongoing			
Cohort 5	160 mg QD	Completed	➡	160 mg QD	14 Treated → 20 Planned
Cohort 4	120 mg QD	Completed	➡	120 mg QD	16 Treated → 20 Planned
Cohort 3	80 mg QD	Completed	➡	80 mg QD	20 Treated
Cohort 2	40 mg QD	Completed	➡	40 mg QD	Beginning
Cohort 1	20 mg QD	Completed			



**Favorable safety profile:** No drug related SAE or death and no observed relation between delta-QTc throughout the trial. And no DLT through 160 mg dose level.

**Dose Exploration continues across several cohorts:** currently enrolling patients at 120 mg and 160 mg dose levels and plan to explore 40 mg dose level

# HM43239 Safety and Efficacy Data

## Broad Therapeutic Window as a Single Agent in R/R AML Patients

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- **Safety Profile Favorable to Date**

- No drug related SAE, deaths, or AE of elevated CK (creatine kinase)
- No drug related AE of QT prolongation – No observed relation between  $\Delta QT_c$  and dose
- No DLT up to 160 mg and one DLT of muscle weakness (not rhabdomyolysis) at 200 mg

- **Demonstrated Efficacy Across a Diverse Set of R/R AML Patients**

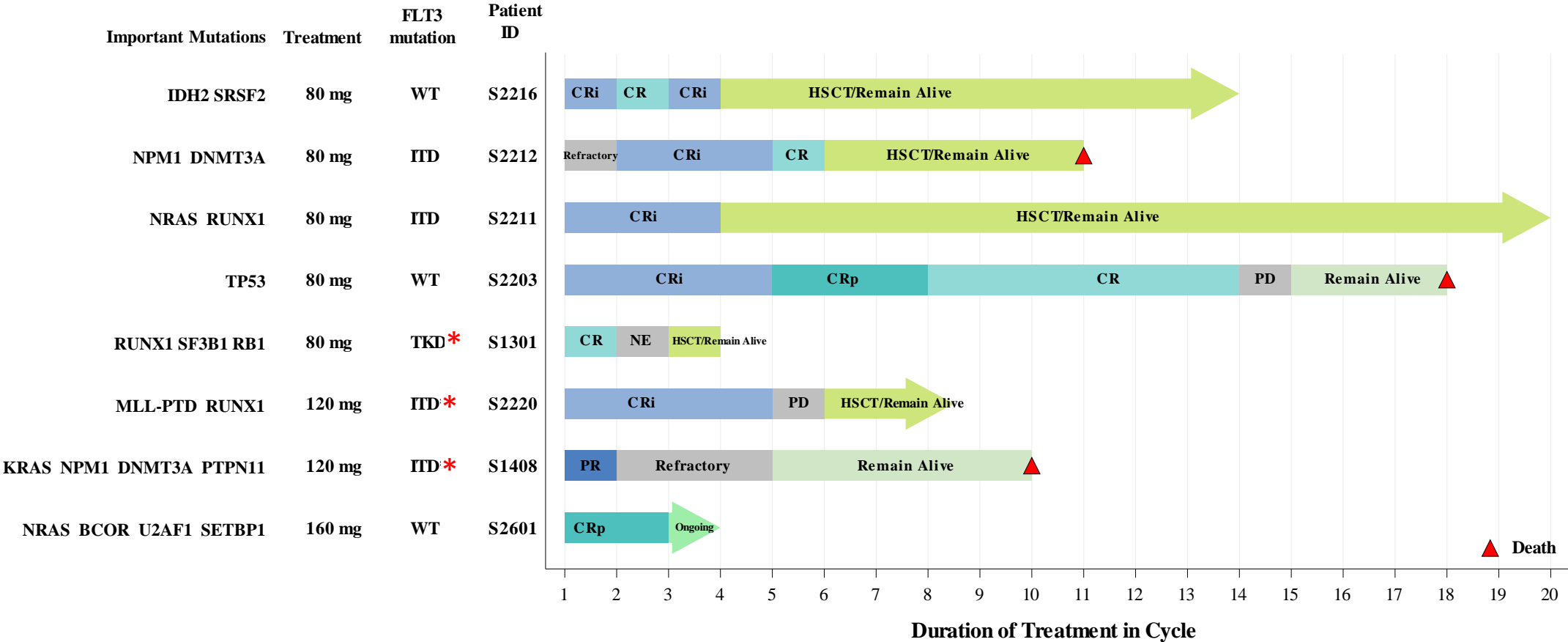
- **CRc in AML with Adverse Mutations (FLT3<sup>WT</sup>)** incl. TP53-Mutant and Complex Karyotype)
- **CRc in FLT3-Mutant AML (Fast Track)** incl. Prior Failure of Other FLT3 Inhibitors

- **Identified a Broad Therapeutic Window**

- **Safely achieved efficacy at 3 separate dose levels** (80 mg, 120 mg, 160 mg) with no DLT
- Demonstrated **broad therapeutic range** across safe dose levels
- Safety profile supports **combination therapy with other agents**



# HM43239 Diversity of Genetically-Defined R/R AML Patients Who Achieved a Clinical Response to Date in Phase 1/2 Study



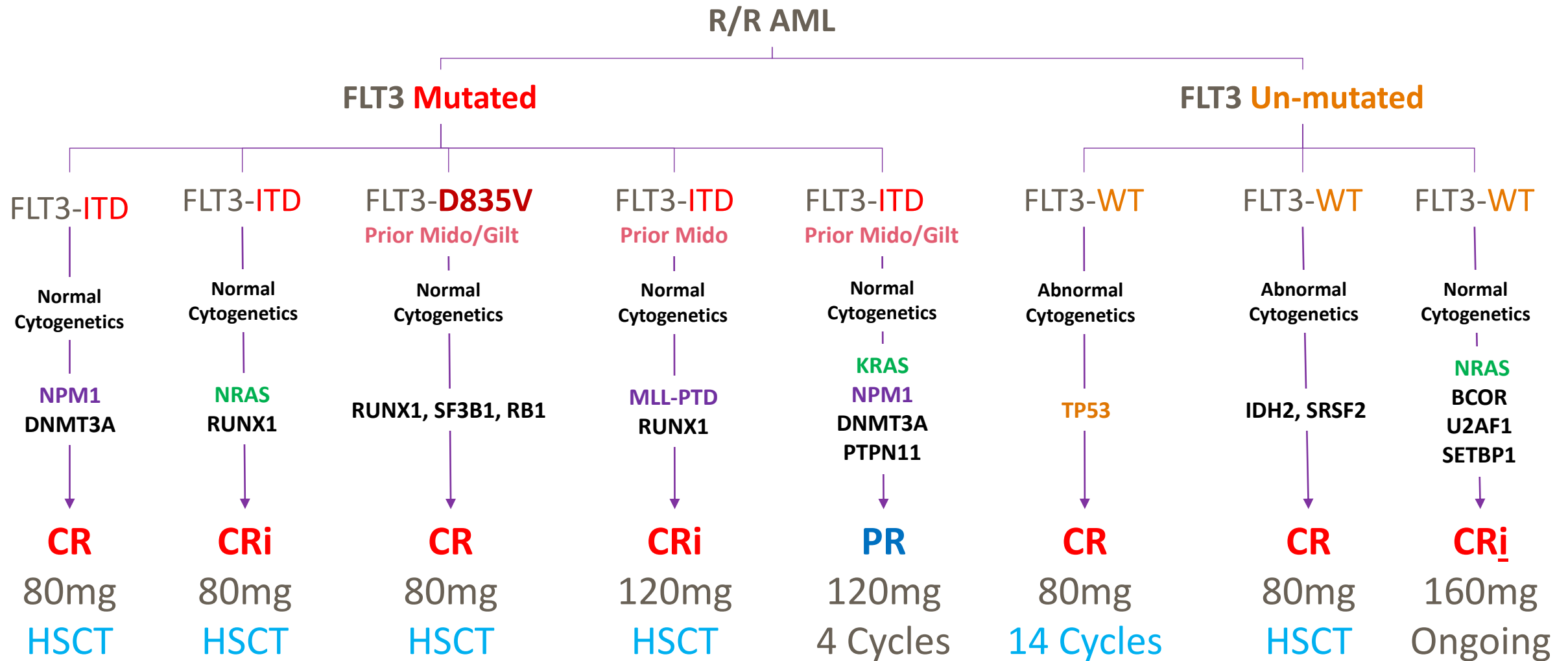
Abbreviation: CR, complete response; CRi, complete response with incomplete hematologic recovery; CRp, complete response with incomplete platelet recovery; HSCT, hematopoietic stem cell transplantation; NE, not evaluable; PD, progressive disease; PR, partial remission.  
Note: 'Ongoing' means treatment is still ongoing; 'Remain Alive' indicates patients' status in follow-up after treatment termination; The right arrow at the end of horizontal bar indicates patients are still on study, whereas without the right arrow indicates patients discontinued from study.  
Note: Each response assessed at a regular visit is considered to have started 1 cycle before the assessment; however the start of the response is considered the integer part of (study day/28) if the response occurred at the End of Treatment visit.

\*Indicates patients who received prior FLT3 inhibitors, including gilteritinib and/or midostaurin.

Data as of July 14, 2022

# HM43239 AML Patients with Best Clinical Responses to Date

## Observed 7 CRc and 1 PR in Diverse and Challenging Patient Populations



# HM43239 Overall Response Rate (CRc + PR)

## 7 CRc and 1 PR to Date in Phase 1 as a Single Agent in R/R AML Patients

Mutation Status	All Patients			Evaluable Patients		
	N = 45 Patients	Number Responders	Response Rate	N = 41 Patients	Number Responders	Response Rate
FLT3+	20	4CRc   1PR	25%	19	4CRc   1PR	26.3%
<b>FLT3+/prior FLT3i</b>	<b>7</b>	<b>3</b>	<b>42.9% (CRc + PR) 28.6% (CRc only)</b>	<b>7</b>	<b>3</b>	<b>42.9% (CRc + PR) 28.6% (CRc only)</b>
FLT3-WT	25	3	12%	22	3	13.6%
<b>TP53+</b>	<b>4</b>	<b>1</b>	<b>25%</b>	<b>3</b>	<b>1</b>	<b>33.3%</b>

### Overall Response Rate for “All Patients” and “Evaluable Patients” Receiving ≥ 80mg HM43239

- Findings represent a snapshot in time: The reported safety, tolerability, PK, PD and efficacy findings reported herein represent the data available may change as additional patients are assessed and more data are collected.
- “Evaluable Patients” removes those non-evaluable patients who did not have a response evaluation and had no other evidence indicating refractory disease in the peripheral blood.
- Most CRc patients went to HSCT and cannot be evaluated for transfusion independence assessment.

Abbreviation: CR, complete remission; CRc, composite complete remission; CRp, complete remission with incomplete platelet recovery; CRi, complete remission with incomplete hematological recovery; PR, partial remission.  
Note: efficacy evaluable patients include all patients with at least 80% drug compliance during Cycle 1 or who had reported a DLT during Cycle 1, and who reported relevant data for efficacy interpretation such as bone marrow assessment, CBC counts, reason for treatment termination.

<sup>[1]</sup> Overall response includes CRc and PR.

<sup>[2]</sup> CRc includes CR, CRh, CRp and CRi.

<sup>[3]</sup> The reported prior FLT3 inhibitors include gilteritinib, midostaurin and sorafenib.

Data as of July 14, 2022

# HM43239 Potential for Accelerated Path Supported by Expansion & Registration Trials

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## Ongoing Dose Escalation/Dose Exploration Phase 1/2 Trial in R/R AML Patients

- Continue **Exploration of Highly Adverse Molecular Genotypes** (TP53-Mutated, etc.) for **Potential Fast Track Designations**
- Continue Dose Exploration at 40 mg, 120 mg and 160 mg to **Deliver Response Rate Updates & Rolling News Flow**

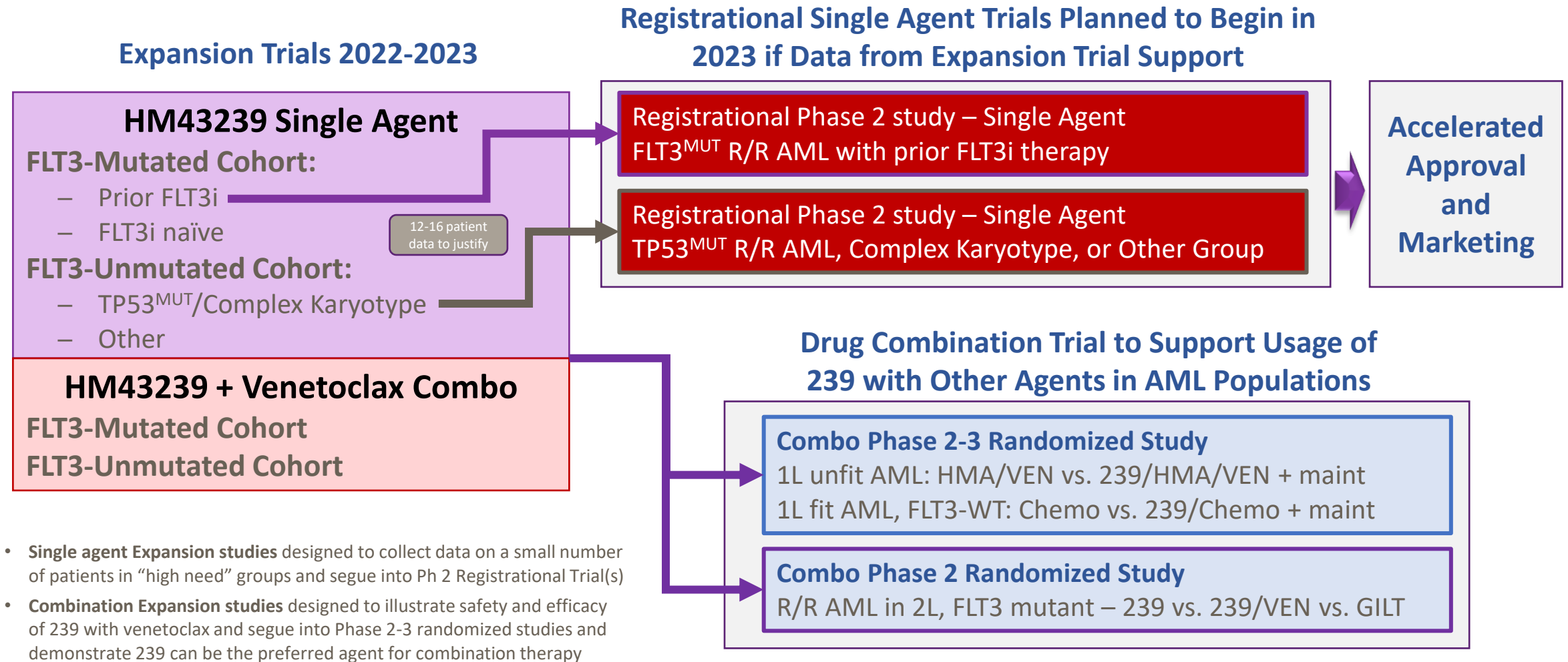
## Doses and Patient Populations have been Selected for Expansion Trials

- **Three Safe and Efficacious Doses Identified**
  - **120 mg** planned as **Primary** Single Agent Expansion Dose with **80 mg** and **160 mg** as Bracketing Doses
- **Expansion with FLT3 Mutated R/R AML Population** (Fast Track Designation)
  - Includes FLT3+/Prior FLT3i Failure target population for potential accelerated approval
  - Includes broader FLT3-mutated population to support full approval trials in FLT3-mutated AML
  - Plan single agent to begin 2H2022 and combination (239+Ven) to begin thereafter
- **Expansion with FLT3-Unmutated R/R AML Population** (with Adverse Mutations)
  - Including TP53-Mutated target population for potential accelerated approval
  - Includes broader population to support full approval trials in NPM1/MLL, RUNX1-DNMT3A-Ras and other populations
  - Plan Single Agent to begin 2H2022 and Combination (239+Ven) to begin thereafter
- **Expansion Includes Broader Populations to support full approval : NPM1/MLL, RUNX1-DNMT3A-RAS, Broad FLT3+ and Others**

## Expansion Trials in AML Patients Serve as Segue to Registrational Trials

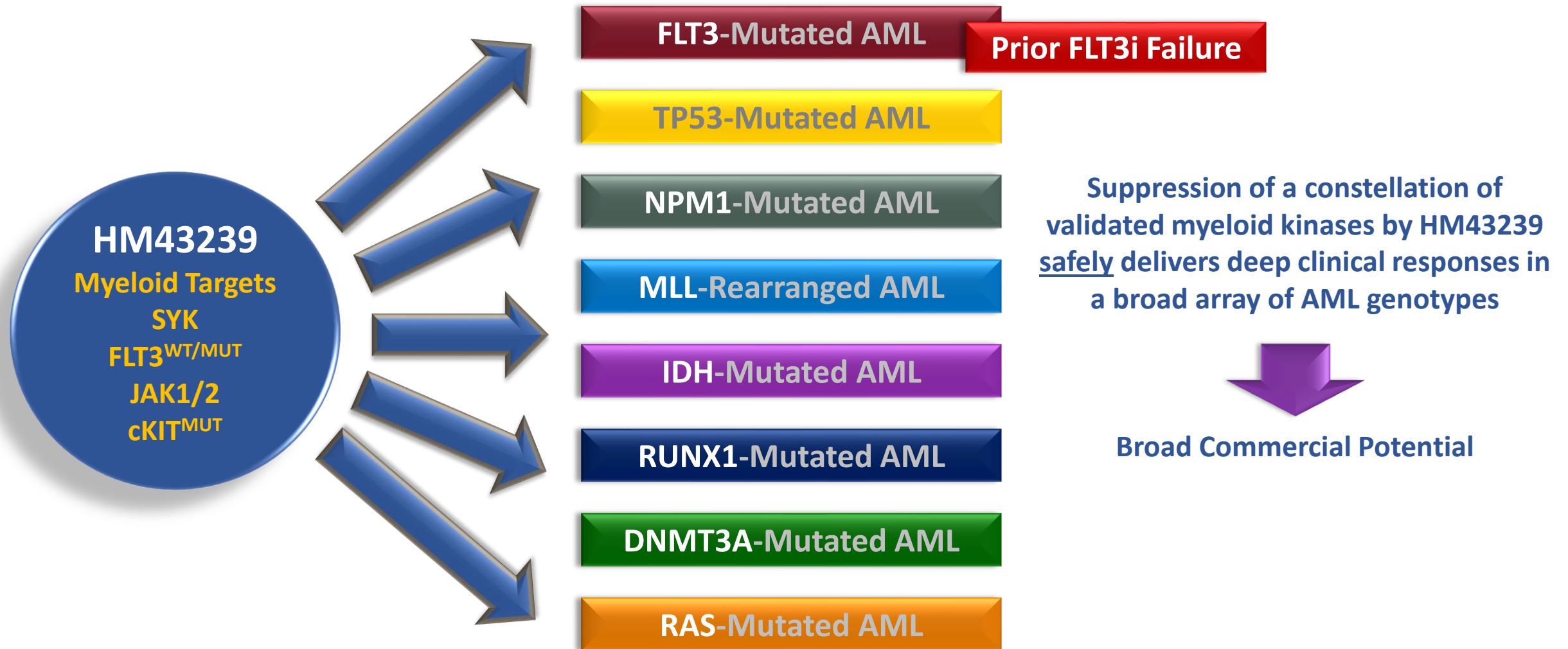


# HM43239 Global Dose Expansion Trial Planned to Support Phase 2 Registrational Trials for Accelerated Approval and Drug Combination Trials for Broad Commercialization



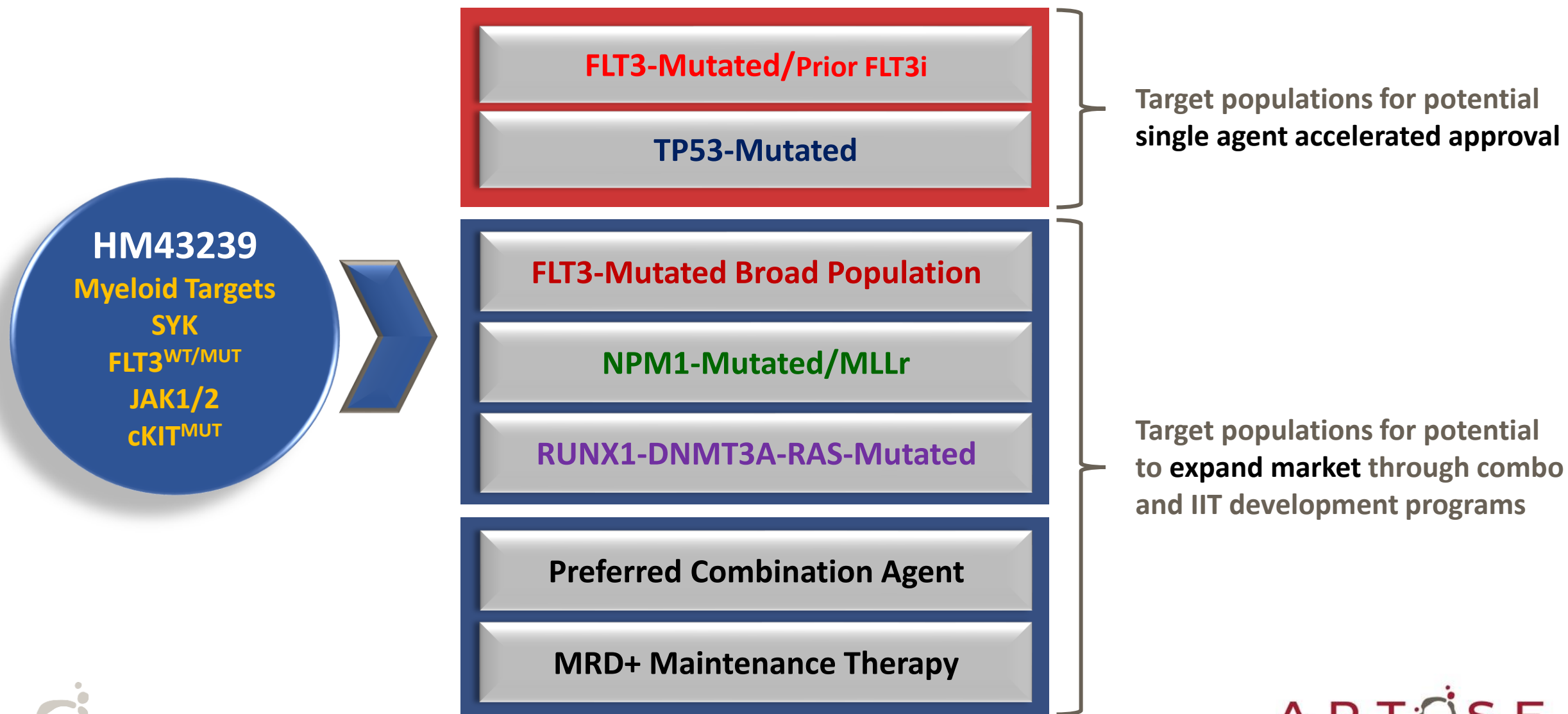
# Clinical Development Plan Sets the Stage for Broad Commercial Success

# HM43239: Unlike Any Other Targeted Agent for AML Safely Delivers CRs in Multiple Genetically Defined AML Target Patients



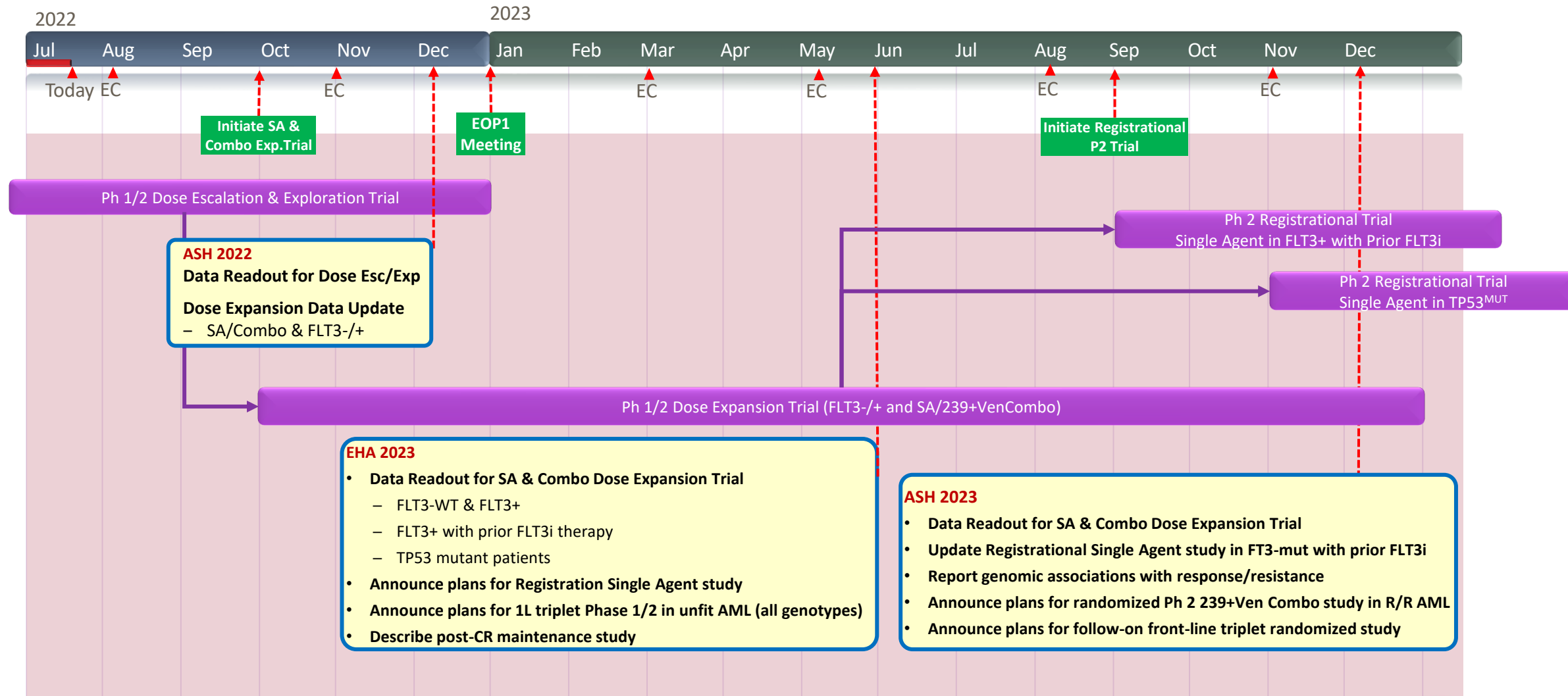
# HM43239: Positioned for Accelerated Approval & Traditional Development

## Broad Commercial Opportunities >\$1 billion in Multiple AML Target Populations





# HM43239 Potential Timelines of Value Driving Milestones



# HM43239 Clinically Validated, Once Daily, Oral Myeloid Kinase Inhibitor

## Confidence of Clinical Investigators and KOLs

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### Targets Constellation of Kinases Important in AML

- Potent inhibitor of myeloid kinases SYK, FLT3<sup>WT/MUT</sup>, JAK1/2 and mutant forms of c-KIT associated with transformation and resistance
- Potential to treat genetically defined AML patients across multiple lines of therapy & populations
- Safety & efficacy foretell significant market potential for R/R, 1L, FLT3-/+, Fit/Unfit AML populations



### Clinical Validation Supports Path of Rapid Development for Breadth of AML Patients

- **FLT3-Mutated Patients**
  - CRc in patients who failed prior FLT3 inhibitors
  - CRc in patients with ITD and TKD mutated FLT3
  - FDA Fast Track received for FLT3<sup>MUT</sup> R/R AML
- **FLT3-Unmutated Patients**
  - CRc in genetically-defined patients with specific mutations: NPM1, MLL, TP53, DNMT3A, N/KRAS, IDH2, U2AF1, RUNX1, Others
- **Broad Therapeutic Window**
  - Well tolerated across three active & safe doses
- **Preferred Agent Profile for Combination Therapy**



### Program Goals Supporting Rapid Development

- *Explore Molecular Subgroups* for Potential Fast Track Designations
- *Single Agent Expansion Trial (239)* planned 2H2022
- *Combo Expansion Trial (239+Ven)* planned 2H2022
- *Registrational Ph2 study(ies)* planned 2023 from Expansions
- **Broad commercialization goals** supported by clinical development in diverse patient populations

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- Targeting key drivers of disease in cancer cells without overlapping toxicities to provide **efficacy with safety to improve the quality of life for cancer patients**

## Investor highlights

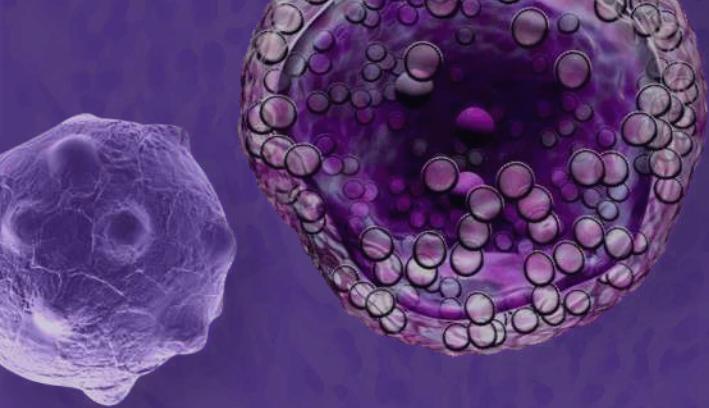
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- CRs delivered in multiple genetically-defined AML target populations
- Response rates 40%+ in AML population that may support **single agent accelerated path**
- Clinical development progressing toward single agent and combination therapy
- Positioned to become a preferred agent for broad commercial use

## Meaningful near-term upside with value-driving clinical updates and milestones through 2022 and 2023

- Value potential: Market Cap \$75M (O/S 92M), \$294M June 2021; 52-Week range: high \$3.13, low \$0.73



We thank our partners, investigators, and investors for helping us bring novel drugs to patients with the greatest need.



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