

# MOLECULIN

September 2024  
Investor Presentation



# Disclaimer



All statements contained herein other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “expect,” and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. Our potential to sustain our relationship with MD Anderson revolves around the continued collaboration and capitalizing on intellectual property resulting from sponsored research. The feasibility and promptness of our clinical trials are influenced by regulatory stipulations from entities like the US Food & Drug Administration (FDA) and their global counterparts. As such, all of our trials, including the MIRACLE trial, are subject to timely, future filings with and feedback, allowance, approvals, etc. from the FDA and their global counterparts. The implications of global events, such as the conflict in Ukraine, the COVID-19 pandemic, and prevalent supply chain challenges, play a role in our forward-looking statements. Additionally, our ongoing need for financing, fueling our clinical trial and product development initiatives, securing regulatory approvals in essential markets, and sourcing cost-effective drug solutions are core to our forward-looking statements. Furthermore, our commitments concerning intellectual property licenses, the potential efficacy of our drug candidates, market reception, potential product liabilities, and the emerging competitive landscape are also fundamental to our forward-looking statements. Any reference related to cardiotoxicity or the lack thereof concerning Annamycin is based on our expert’s opinion as detailed in our filings, from time to time, with the SEC. Our dependencies on third-party manufacturers, strategies for establishing business collaborations, the defense of our intellectual property rights, our plans for fostering company growth, and the imperative to retain key executive personnel also guide our projections. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties, and assumptions, the future events and trends discussed in this presentation may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. More detailed information about Moleculin is set forth in our filings with the Securities and Exchange Commission. Investors and security holders are urged to read these documents free of charge on the SEC’s website at <http://www.sec.gov>. Data related to currently active trials of Moleculin are preliminary and subject to change until a final Clinical Study Report is published.

# Our Team



Walter V. Klemm

Founder, President, CEO and Chairman



Donald Picker, PhD

Chief Scientific Officer



Jonathan P. Foster

Executive VP & Chief Financial Officer



Dr. John Paul Waymack

Senior Chief Medical Officer



Sandra Silberman, MD, PhD

Chief Medical Officer – New Products



Robert Shepard, MD, FACP

Medical Advisor



7 FDA approvals

2 Big Pharma exits

14 Moleculin clinical trials

\$0.5 Million in recent Management Investment

200 Years of drug development experience





# MOLECULIN

DIAGNOSIS

## Core Management Beliefs on Annamycin Positioning:

Safer and more effective than currently prescribed anthracyclines

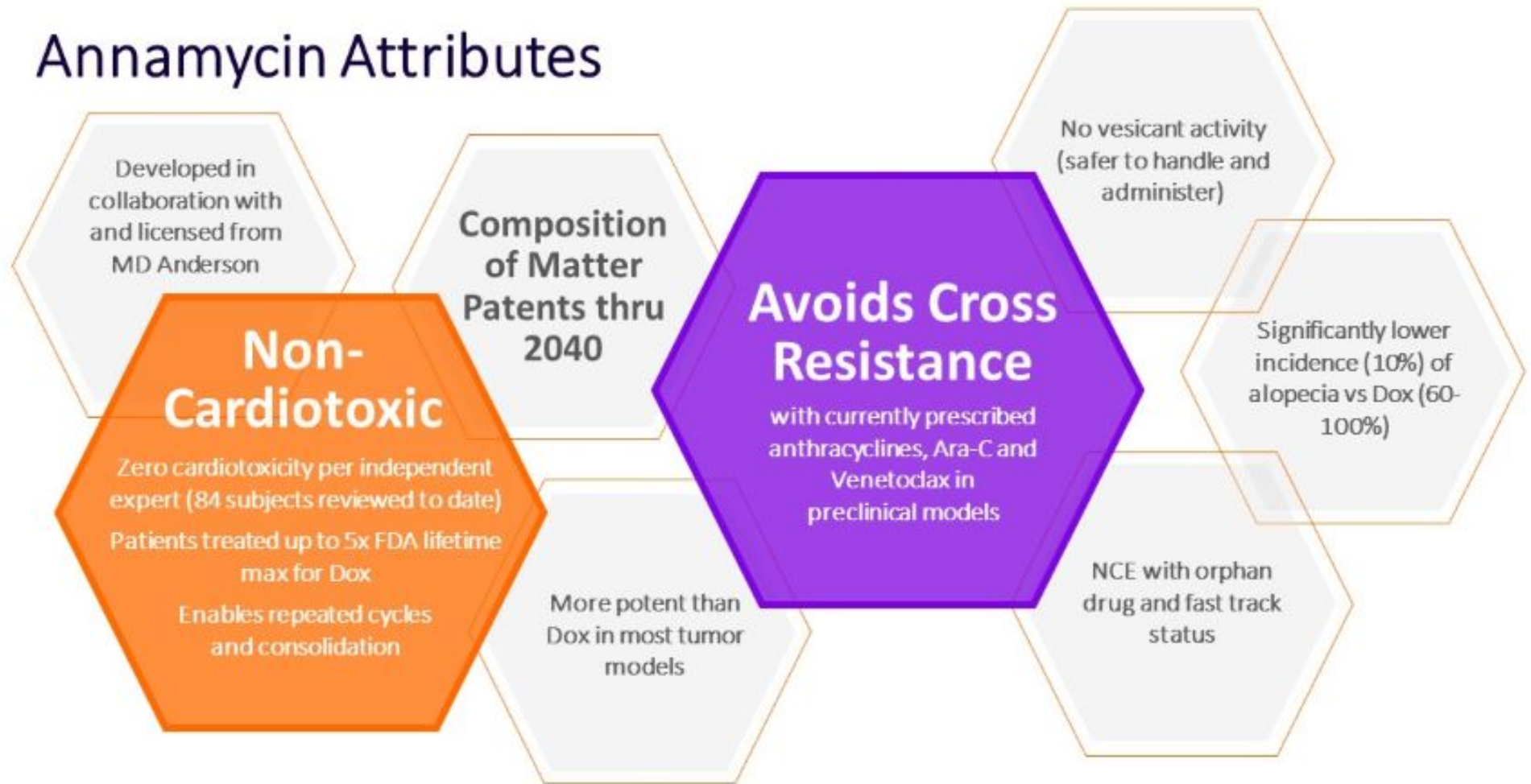
Non-cardiotoxic and avoids cross resistance with dox, Ara-C and Venetoclax

Fills an unmet need for more than half of AML patients

Delivers more than double the CR rate of any approved treatment for r/r AML

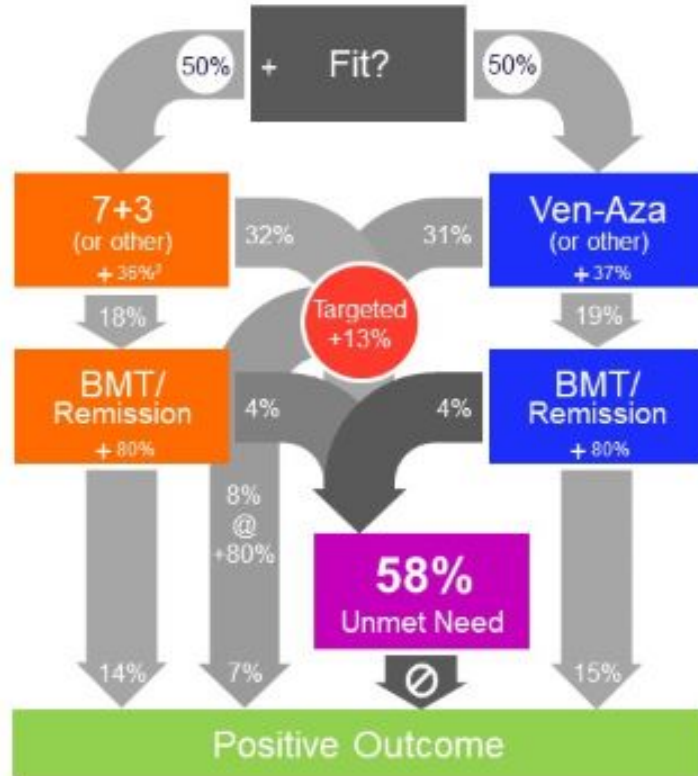
Potential uses extend far beyond AML

# Annamycin Attributes



Notes: 1) Current Cardiology Review, Anthracycline Cardiotoxicity: Prevalence, Pathogenesis and Treatment, Maria Volkova and Raymond Russel III, Referenced from Cancer. 2009 Jun 1;97(11):2869-79. "Congestive heart failure in patients treated with doxorubicin: a retrospective analysis of three trials". Swain SM, Whaley FS, Ewer MS., PMID: 12767102; 2) Preliminary clinical studies from Moleculin; data subject to change; 3) Refer to Form 10K for FYE 2023 for discussion on latest subject with an increase in troponins and our Expert's opinion.

# Approved Therapies are Successful for Only ~40% of AML Patients<sup>1</sup>



	Daunorubicin (+ Ara-C)	Venetoclax (+ Azacitidine)	Targeted Therapy
Regimen	7+3	Ven-Aza	Gene-Targeted
Sub-population	Fit Patients (50%)	Unfit Elderly (50%)	55%-65% Eligible
Durable CR%	36% <sup>2</sup>	37%	~21%
All AML benefit <sup>3</sup>	14%	15%	7%
Critical characteristics	Cardiotox; de novo/acquired resistance	Still leaves significant unmet need	Limited to certain gene mutations

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Notes: 1. Management estimates; 2. Net of relapse, Management estimate, varies depending on study; 3. % of AML population sustaining durable positive outcome via BMT or remission, allowance made for other therapies where appropriate in the percentages shown





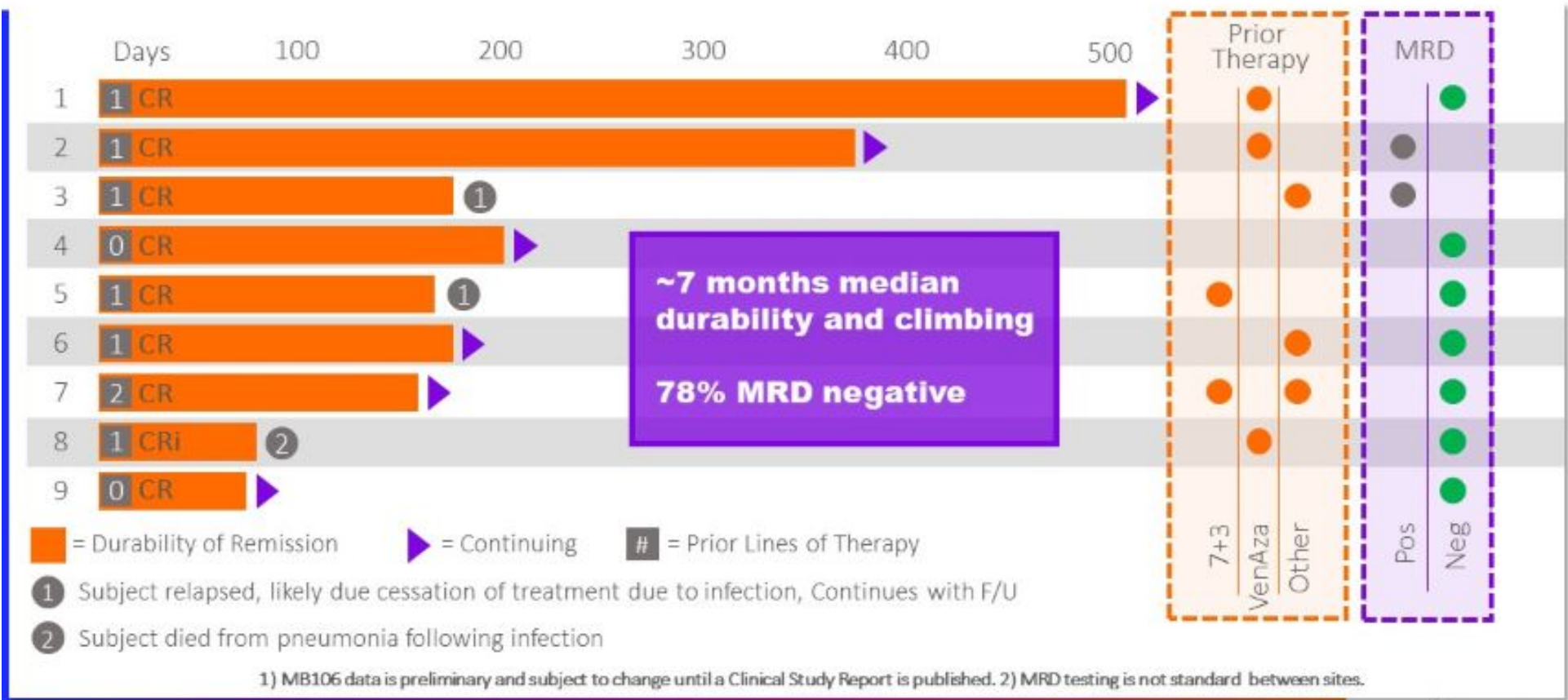
# MB-106 (Annamycin + Ara-C (AnnAraC); n=22)

Line of Therapy	All Lines (1 <sup>st</sup> – 7 <sup>th</sup> )	1 <sup>st</sup> Line	2 <sup>nd</sup> Line	2 <sup>nd</sup> & 3 <sup>rd</sup> Line Combined
Subjects Evaluable To Date	22	4	<b>10</b>	14
Subjects Evaluable Not Dosed Per Protocol	2	0	<b>1</b>	1
Median Age - Years (Range)	67.5 (19-78)	56.5 (19-69)	<b>71 (53 - 78)</b>	69.5 (53-78)
Complete Remission (CR)	8 (36%)	2 (50%)	<b>5 (50%)</b>	6 (43%)
Complete Remission Composite (CRc)	9 (41%)	2 (50%)	<b>6 (60%)</b>	7 (50%)
Partial Response (PR)	2	0	<b>1</b>	2
CRc Relapsed To Date	3	0	<b>3</b>	3
BMT To Date	2	0	<b>1</b>	2

**Median CRc Durability = ~7 Months and Climbing**

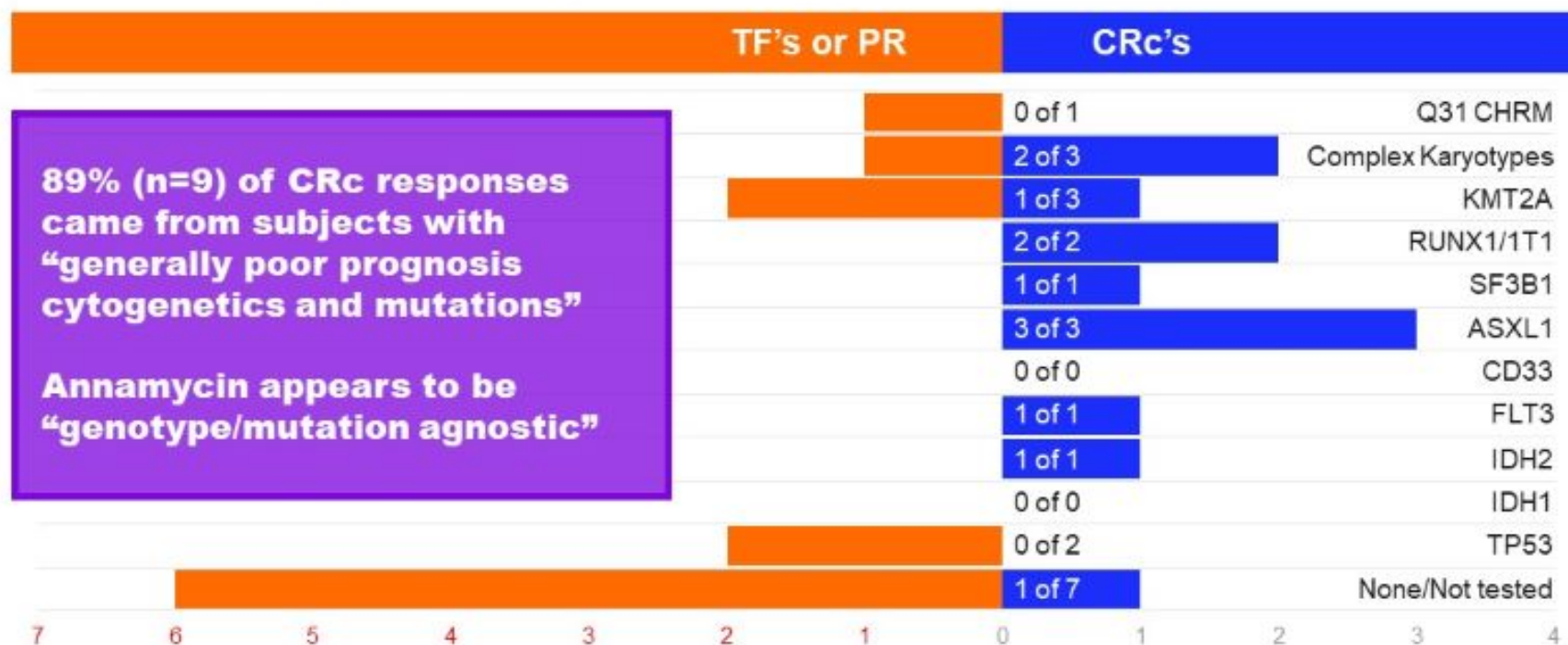
Notes: 1) Data from MB-106 are for intent to treat subjects who had efficacy determined (n=22); 2) Data from MB-106 are preliminary and subject to change; and 3) Relapses include 1 death due to pneumonia (unrelated to drug).

# Durability, MRD, Prior Therapies





# MB-106 Response by Genotype and Mutation



Note – n=20; Some subjects had multiple mutations or abnormalities, hence totals of treatment failures (TF), partial remissions (PR) or composite complete remissions (CRc) do not equal totals for each response category – TF's/PR's, or CRc's; Data are anecdotal only and not intended to indicate statistical significance. Not all mutations/subjects were tested.

# Clinical History Supporting Activity in Leukemia

Trial	Callisto <sup>1</sup>	MB-105	MB-106 <sup>2</sup>	Total of 3 Trials
Subjects at RP2D	8	5	22	Total of 35 R/R AML/ALL subjects treated at over 10 unique sites with 15 (43%) achieving CRc
R/R AML/ALL	ALL	AML	AML	
Prior Lines of therapy (median)	1-5 (2)	2-8 (4)	0-5 (1)	
Regimen	Single Agent	Single Agent	Combination	
# of Sites	4 (US)	5 (EU)	7 (EU)	
Complete Remission Composite (CRc)	3 (38%)	3 (40%)	9 (41%)	

Notes: 1) Wetzler, et al, Phase I/II Trial of Nanomolecular Liposomal Annamycin in Adult Patients With Relapsed/Refractory Acute Lymphoblastic Leukemia, Clinical Lymphoma, Myeloma & Leukemia, Vol. 13, No. 4, 430-4, 2013; 2) Data from MB-106 are preliminary and subject to change; and 3) Relapses include 1 death due to pneumonia (unrelated to drug).

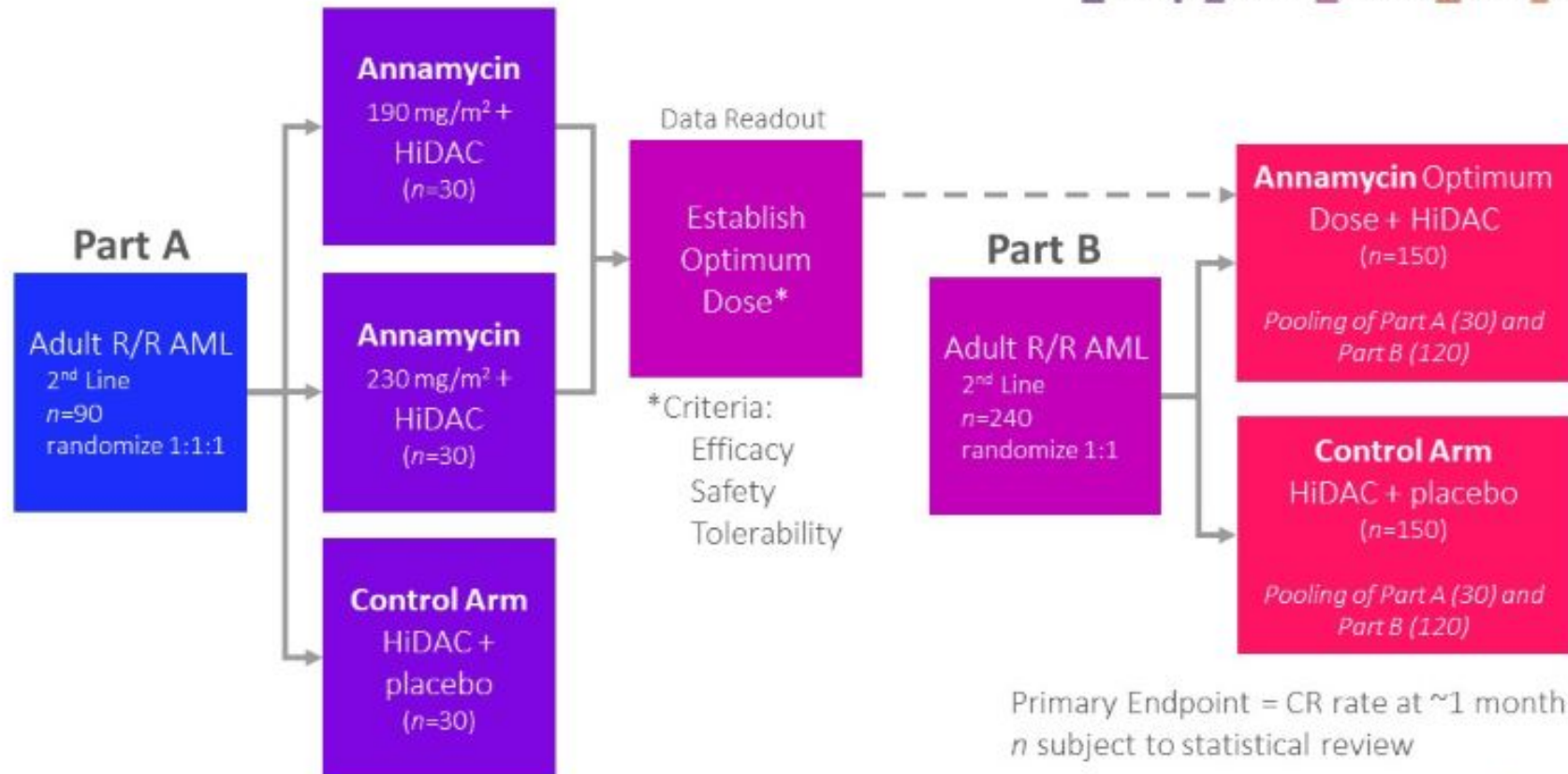
# miRACLE

Moleculin R/R AML AnnAraC Clinical Evaluation

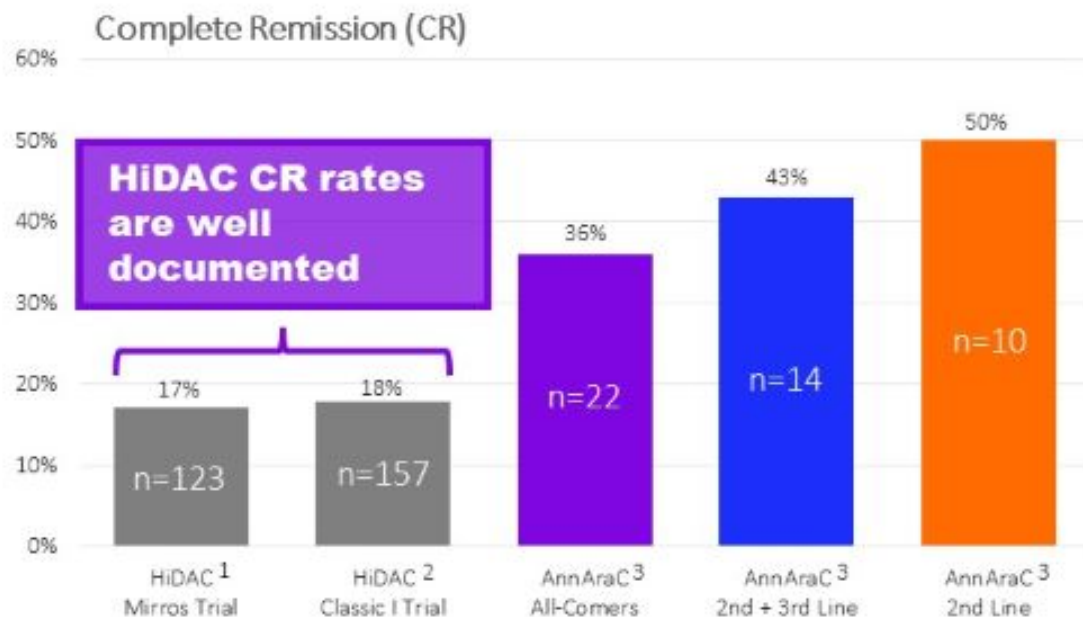
**Phase 3 pivotal clinical trial designed to seek accelerated new drug approval for Annamycin in the treatment of relapsed or refractory acute myeloid leukemia. Targeted to begin recruiting in 1Q2025.**



# Adaptive Trial Design



# The Bar for Approval is Low



**Annamycin  
NDA to be  
based on CR  
rate in 2<sup>nd</sup>  
line subjects  
at ~1 month**

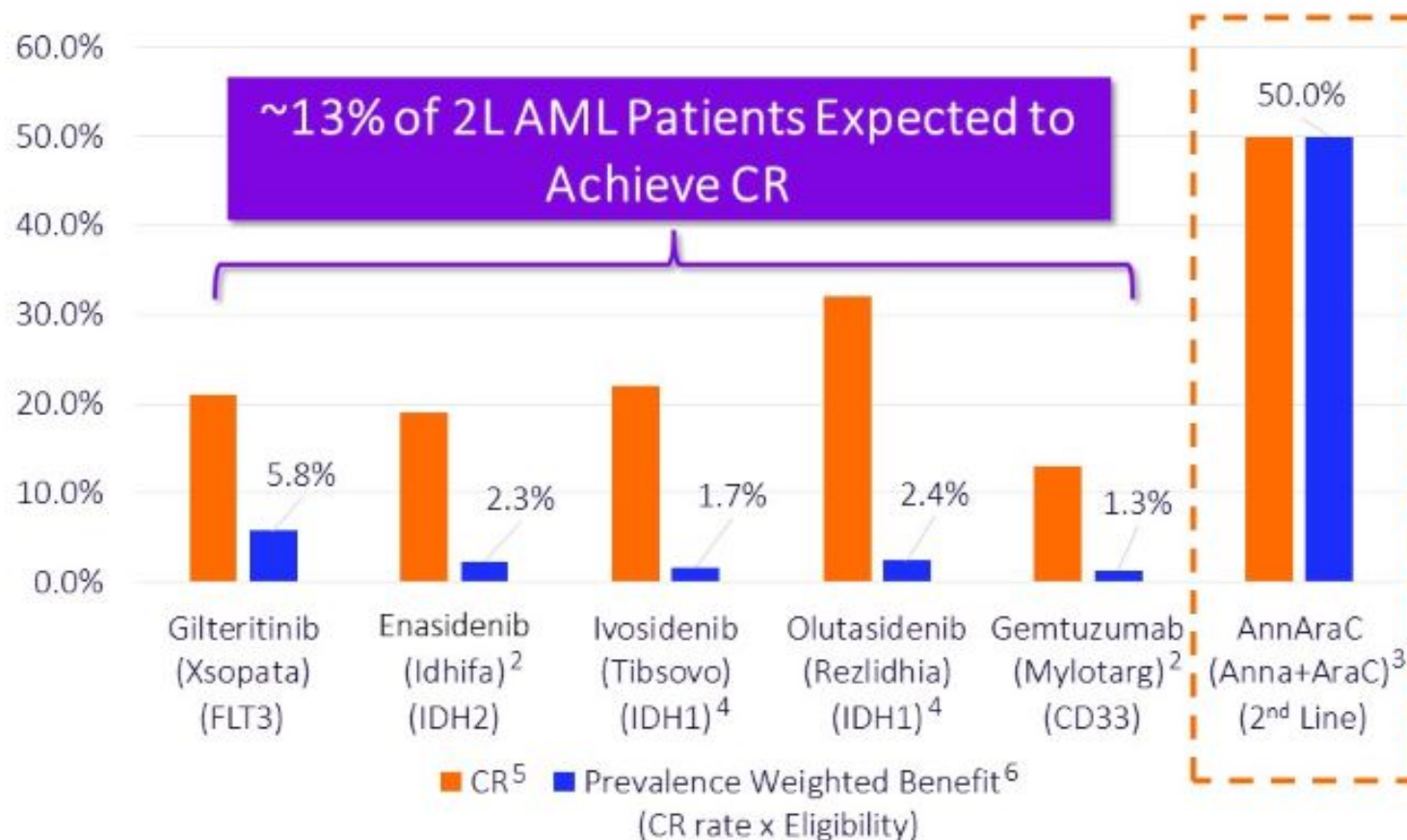
1 – Mirros Trial, 81% 2<sup>nd</sup> line patients, Konopleva et al, Blood Advances, 26 July 2022, Volume 6, Number 14; 2 – Classic I Trial, Faderl et al, J Clin Oncol, July 2012, Volume 30, Number 20; 3 – MB-106 trial, 50% CR rate for 2<sup>nd</sup> line patients (n=10), 43% CR rate for 2<sup>nd</sup> + 3<sup>rd</sup> line patients (n=14), and 36% CR rate for all-comers (1<sup>st</sup> through 7<sup>th</sup> line, n=22)

# Estimated Regulatory Timeline





# AnnAraC Should Increase 2nd Line AML CRs 2-Fold or More



# Potential Asset Value

	Approved			Phase 2 Complete			
	1 <sup>st</sup> Line		2 <sup>nd</sup> Line				
	Jazz	AbbVie	Servier	Kura <sup>1</sup>	Syndax <sup>1</sup>	JNJ <sup>1</sup>	Moleculin
	Vyxeos	Ven-Aza	Idhifa/Tibsovo	Ziftomenib	Revumenib	617	Annamycin
N	153	286	199/174	20	57	17	10
CR	<b>38%</b>	<b>37%</b>	<b>19%/25%</b>	<b>35%</b>	<b>18%</b>	<b>24%</b>	<b>50%</b>
CRC	48%	64%	23%/33%	40%	25%	47%	60%
AML Population	50%	50%	15-23%	30% <sup>2</sup>	24% <sup>2</sup>	30% <sup>2</sup>	60%
Revenue <sup>3</sup>	\$128M	\$2B	~\$150M				
Valuation	<b>\$1.5B</b>	<b>N/A</b>	<b>\$2B</b>	<b>~\$1.5B</b>	<b>~\$1.9B</b>	<b>N/A</b>	<b>~\$.015B</b>
	<b>Exit<sup>4</sup></b> (Acquisition of Celator, 2016)		<b>Exit<sup>5</sup></b> (Acquisition of Agios, 2021)	<b>Market Cap<sup>6</sup></b>	<b>Market Cap<sup>6</sup></b>		<b>Market Cap<sup>6</sup></b>

1. All three are pursuing essentially the same patient population; best overall performance from either NPM1 mutation or KMT2A rearrangement cohorts; 2. Limited to 2<sup>nd</sup> Line due to low CRC performance; 3. Jazz and AbbVie revenue per SEC disclosure, Servier revenue per Management estimate based on Agios revenue disclosure for Tibsovo sales and Idhifa royalties; 4. Company press release - <https://investor.jazzpharma.com/news-releases/news-release-details/jazz-pharmaceuticals-and-celator-pharmaceuticals-announce>; 5. Company press release - [https://servier.com/wp-content/uploads/2022/11/servier-completes-acquisition-agios-oncology-business\\_PR.pdf](https://servier.com/wp-content/uploads/2022/11/servier-completes-acquisition-agios-oncology-business_PR.pdf); 6. As of April 11, 2024, calculation of Share Price multiplied by Shares Outstanding

# The Full Annamycin Opportunity<sup>1</sup>

Potential for Significant Upside



1 - Per management's estimates; 2 - Preliminary and subject to final clinical study report



# Global Network



All technology licensed from MD Anderson Cancer Center (MDACC)



Supports continuing preclinical research on our technology at MDACC close to \$1M per year



Active contractors in US, EU and Asia for drug production and distribution as well as for clinical trial management



Past & current externally funded trials – MD Anderson Cancer Center; Emory University, Aflac Cancer & Blood Disorders Center, Children's Healthcare of Atlanta; Northwestern University (NIH & BrainUp); Madame Curie Institute (Poland), and others in discussion



# Cash to Fund Operations into the First Quarter of 2025<sup>1</sup>

Nasdaq: MBRX



~\$15M Cash Balance<sup>2</sup>



~\$14.4M Market Cap<sup>3</sup>



~5.6M Shares Fully Diluted Outstanding<sup>4</sup>



~58K – Month of August Avg. Daily Trading Volume<sup>5</sup>

Notes: 1: Based on Management's current expectations and planned increase in development activities and cash on hand as of June 30, 2024 plus cash raised in August 2024; 2: Cash on hand as of June 30, 2024, plus cash raised in August 2024; 3: As of August 30, 2024, and using fully diluted shares outstanding w/ price per share = \$2.55 per share x (229,506 pre-funded warrants + 2.31M common stock o/s); 4: Fully diluted common stock outstanding as of August 30, 2024; 5: August 2024 daily trading volume.

# Upcoming Milestones



PROGRAM	MILESTONE	ESTIMATED TIME OF ACHIEVEMENT
<p style="text-align: center;">Annamycin AML</p>	Begin contracting with MIRACLE trial sites	2H 2024
	First subject treated in MIRACLE trial	1Q 2025
	Recruitment Update (n=~45)	4Q 2025
	Data Readout (n=90) unblinded and Optimum Dose set for MIRACLE trial	Mid 2026
	Begin enrollment of 3 <sup>rd</sup> line subjects in MIRACLE2	2H 2026
	Enrollment ends for 2 <sup>nd</sup> line subjects	2027
	Final data for 2 <sup>nd</sup> line subjects; NDA submission	2028
<p style="text-align: center;">Annamycin STS Lung Mets</p>	Final MB-107 Data Readout	2025
	Identify Next Phase of Development / Pivotal Program	2025

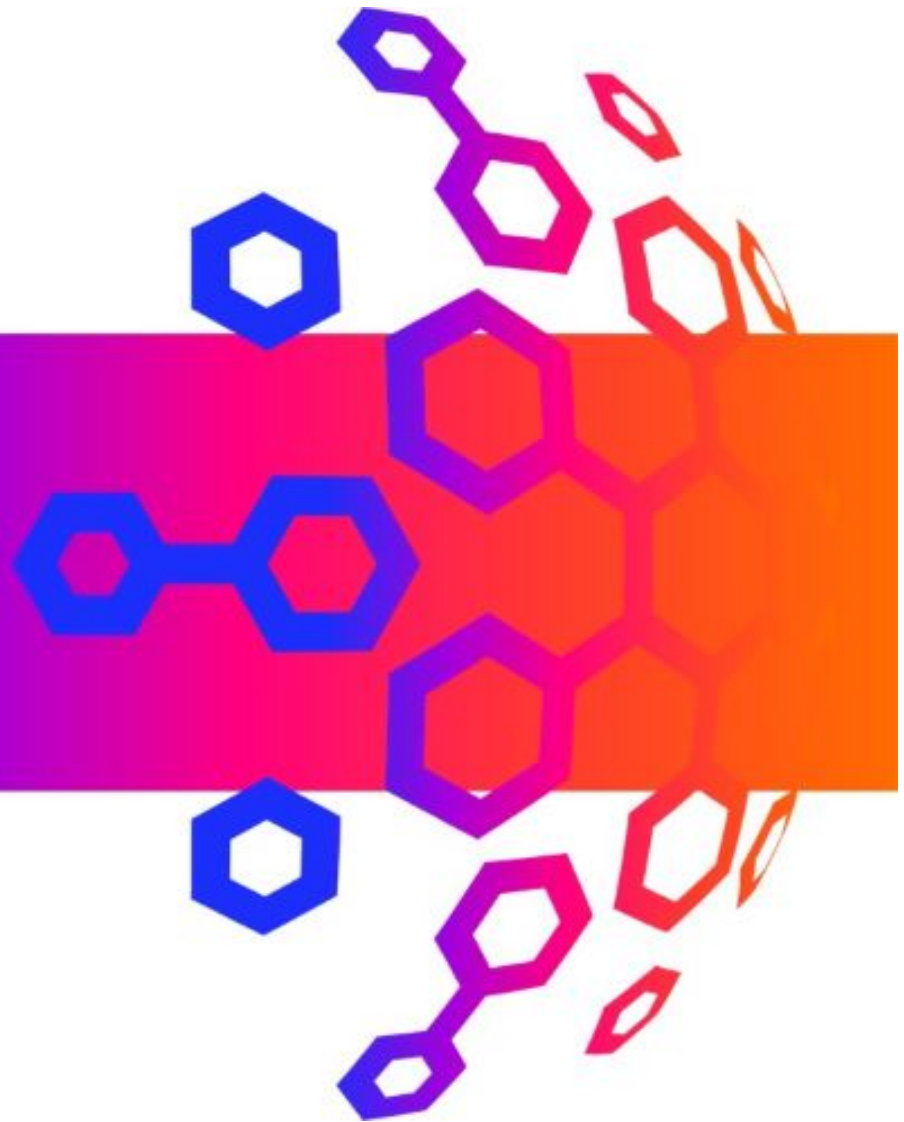


# miRACLE

Moleculin R/R AML AnnAraC Clinical Evaluation

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# Appendix



# Performance of AML Therapies in 2<sup>nd</sup> Line

## CLAVELA: International Randomized Phase III Study of Elacytarabine Versus Investigator Choice in Patients With Relapsed/Refractory Acute Myeloid Leukemia

*Gail J. Roboz, Todd Rosenblat, Martha Arellano, Marco Gobbi, Jessica K. Altman, Pau Montesinos, Casey O'Connell, Scott R. Solomon, Arnaud Pigneux, Norbert Vey, Robert Hills, Tove Flem Jacobsen, Athos Gianella-Borradori, Øivind Foss, Sylvia Vetthusand, and Francis J. Giles*

J Clin Oncol 32:1919-1926. © 2014 by American Society of Clinical Oncology

### Results

There were no significant differences in OS (3.5 v 3.3 months), response rate (CR = 15% v 12%) between the elacytarabine and control arms, respectively. There was no significant difference in OS among any of the investigator's choice regimens.

Study compared Elacytarabine with 7 different NCCN recommended therapies in 381 R/R AML subjects.

### Therapies compared:

- high-dose cytarabine (HiDAC)
- MEC
- FLAG/FLAG-Ida
- low-dose cytarabine
- hypomethylating agents
- hydroxyurea
- supportive care

# AML Clinical History

<b>Phase 1: MB-104</b> MONOTHERAPY 100-120 mg/m <sup>2</sup>	<b>Phase 1/2: MB-105</b> MONOTHERAPY 120-240 mg/m <sup>2</sup>	<b>Phase 1/2: MB-106</b> COMBINATION THERAPY Annamycin + Cytarabine
<ul style="list-style-type: none"> <li>N = 7</li> <li>17% CRi (at suboptimal dosing)</li> <li>Dosing limited by FDA Lifetime Anthracycline Dose (LTMAD)</li> <li>Trial location – US</li> </ul>	<ul style="list-style-type: none"> <li>N = 20</li> <li>Median lines of prior therapy = 4</li> <li>Median age of 240mg/m<sup>2</sup> (RPD2) cohort = 65 years</li> <li>80% ORR in 240mg/m<sup>2</sup> Cohort (N=5)</li> <li>Trial location – Poland</li> </ul>	<ul style="list-style-type: none"> <li>N = 22 all lines (0-6), N = 10 (2<sup>nd</sup> line)</li> <li>All subjects (N=22) 41% CRc (ITT)</li> <li>2nd Line N=10, 60% CRc</li> <li>Prior therapies range 0-10</li> <li>Median age all subjects = 69</li> <li>Trial location – Poland &amp; Italy</li> </ul>
<b>Key Findings</b>		
<ul style="list-style-type: none"> <li>Well-tolerated in the study population</li> <li>Limited to low doses</li> <li>Morphologic leukemia free state was achieved in one subject in the 120mg/m<sup>2</sup> cohort</li> </ul>	<ul style="list-style-type: none"> <li>Positive correlation between response rate and dose</li> </ul>	<ul style="list-style-type: none"> <li>“3+5” therapy</li> <li>Durability: ~7 months and increasing</li> <li>Early evidence of efficacy in patients with previous therapy failures</li> </ul>
<b>Regulatory Significance</b>		
<ul style="list-style-type: none"> <li>Demonstrated safe dosing within FDA-mandated limitations for anthracycline exposure</li> </ul>	<ul style="list-style-type: none"> <li>Demonstrated safe dosing beyond FDA (and EMA) limitations for cumulative anthracycline exposure and early efficacy as single agent</li> </ul>	<ul style="list-style-type: none"> <li>Addition of Cytarabine supported by compelling preclinical data showing improvement over Annamycin monotherapy</li> </ul>



# Technology Portfolio

Program	Indication	Phase 1	Phase 2	Phase 3
<b>Annamycin</b> Next-Gen Anthracycline	R/R AML	Miracle: Adaptive Phase 3 Pivotal initiating		
	STS Lung Mets	2 trials active; alt. dosing; P1B/2		
<b>WP1066</b> STAT3 Inhibitor	Adult GBM	NU IIT with radiation active; P2		
	Pediatric Brain Tumors	Emory IIT P2 planning		
<b>WP1122</b> Anti-metabolite	GBM; Virology	IIT P2 ready		

# Science Advisors



Waldemar Priebe, PhD  
MD Anderson Cancer Center  
Founding Scientist & SAB Chair

Dr. Daniel Von Hoff  
Mayo Clinic



Dr. Martin Tallman  
Northwestern University

Dr. Jorge Cortes  
Augusta University



Hematology Oncology



Dr. Michael Andreef  
MD Anderson Cancer Center

Dr. Giovanni Martinelli  
Bologna University

