GlycoMimetics

GMI-1271 Update
December 19, 2017

Innovation Today, Healing Tomorrow.

GlycoMimetics, Inc.

Forward-Looking Statements

To the extent that statements contained in this presentation are not descriptions of historical facts regarding GlycoMimetics, Inc. ("GlycoMimetics," "we," "us," or "our"), they are forward-looking statements reflecting management's current beliefs and expectations. Forward-looking statements are subject to known and unknown risks, uncertainties, and other factors that may cause our or our industry's actual results, levels of activity, performance, or achievements to be materially different from those anticipated by such statements. You can identify forward-looking statements by terminology such as "may," "will," "should," "expects," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "intends," or "continue," or the negative of these terms or other comparable terminology. Forward-looking statements contained in this presentation include, but are not limited to, statements regarding: (i) the conduct of the ongoing Phase 3 clinical trial of GMI-1070 by Pfizer Inc. ("Pfizer"); (ii) the timing of additional clinical trials for our other drug candidates; (iii) the timing of receipt of clinical data for our drug candidates; (iv) our expectations regarding the potential safety, efficacy, or clinical utility of our drug candidates; (v) the size of patient populations targeted by drug candidates we or our collaborators develop and market adoption of our potential drugs by physicians and patients; (vi) the likelihood and timing of regulatory filings and approvals; and (vii) and our cash needs and potential royalties and milestone payments under license and collaboration agreements.

Various factors may cause differences between our expectations and actual results, including unexpected safety or efficacy data, unexpected side effects observed during preclinical studies or in clinical trials, lower than expected enrollment rates in clinical trials, changes in expected or existing competition, changes in the regulatory environment for our drug candidates, failure of our collaborators to support or advance our collaborations or drug candidates, our need for future capital, the inability to protect our intellectual property, and the risk that we become a party to unexpected litigation or other disputes. For a further description of the risks associated with forward-looking statements, as well as other risks facing GlycoMimetics, please see the risk factors described in the Company's Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission on March1, 2017, as well as other reports we file with the U.S. Securities and Exchange Commission from time to time, including those factors discussed under the caption "Risk Factors" in such filings. Forward-looking statements speak only as of the date of this presentation, and GlycoMimetics undertakes no obligation to update or revise these statements, except as may be required by law.

An Exciting Portfolio of Product Candidates

Compound	Therapeutic Area	Discovery	Pre-Clinical	Ph 1	Ph 2	Ph 3	Registration	Partner
Selectins								
Rivipansel (Pan-selectin Inhibitor)	Sickle Cell Anemia Vaso-occlusive Crisis							Pfizer
GMI-1271 and Follow-ons (E-selectin Inhibitor)	Acute Myelogenous Leukemia							--
	Multiple Myeloma							--
	Various Tumor Types & Inflammatory Diseases							--
GMI-1359 (E-selectin & CXCR4 Inhibitor)	Various Tumor Types							--
Galectins								
Galectin-3 Inhibitor	Fibrosis & Oncology							--
Undisclosed Galectin Inhibitor	Various Tumor Types							--

GlycoMimetics, Inc.

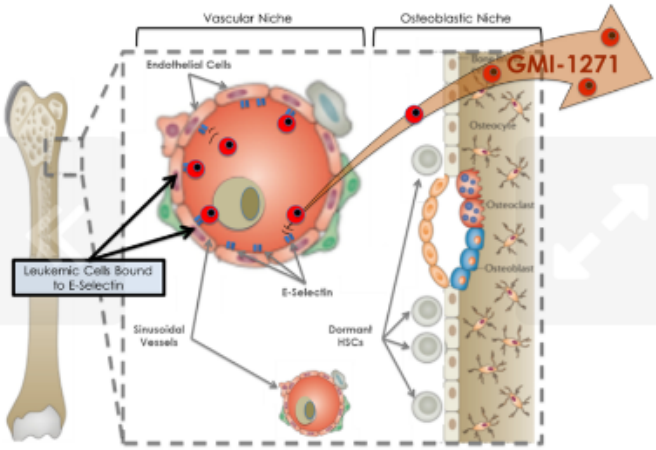
GMI-1271 improves efficacy and safety of chemotherapy in R/R and newly diagnosed older patients with AML: results of a Phase 1/2 study

Daniel J. DeAngelo, Brian A. Jonas, Jane L. Liesveld,
Dale L. Bixby, Anjali S. Advani, Paula Marlton,
Michael E. O'Dwyer, John L. Magnani,
Helen M. Thackray, Pamela S. Becker

Dana-Farber Cancer Institute, Boston, MA; UC Davis Comprehensive Cancer Center, Sacramento, CA; U of Rochester School of Medicine and Dentistry, Rochester, NY; University of Michigan, Ann Arbor, MI; Taussig Cancer Institute, Cleveland Clinic, Cleveland, OH; Princess Alexandra Hospital, University of Queensland School of Medicine, Brisbane, Australia; National University of Ireland Galway, Galway, Ireland; GlycoMimetics, Rockville, MD; University of Washington/Fred Hutchinson Cancer Research Center, Seattle, WA

Abstract 894; ASH 2017. December 11, 2017; Atlanta, GA

GMI-1271, and E-selectin Antagonist, Disrupts Relationship Between Tumor Cells and Bone Marrow Microenvironment



E-selectin –

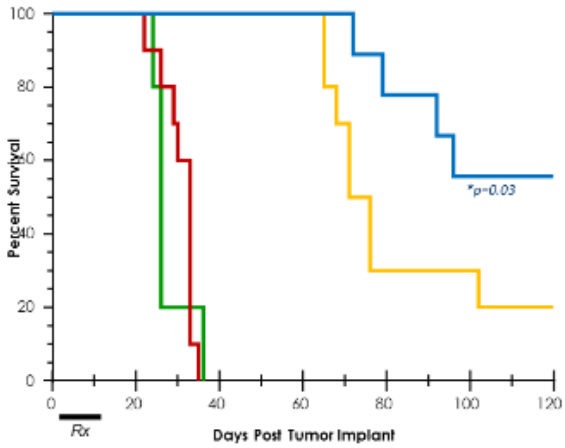
- Constitutively expressed in the bone marrow microvasculature
- Binds to E-selectin ligand on AML cells
- Promotes cell-adhesion-mediated drug resistance (CAMDR) of leukemic cell

GMI-1271, an E-selectin antagonist –

- Inhibits activation of cancer survival pathways (e.g. NF-κB), disrupting CAMDR within bone marrow micro-environment
- Protects normal HSCs by enhancing quiescence and ability for self-renewal
- Reduces chemotherapy-associated mucositis

GMI-1271 in Combination with Chemotherapy Prolongs Survival in AML Tumor Models

Prolonged Survival Replicated in 4 Separate AML *in vivo* Models (e.g. syngeneic, xenogeneic and patient blasts)

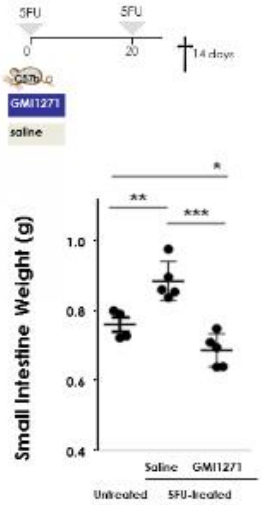


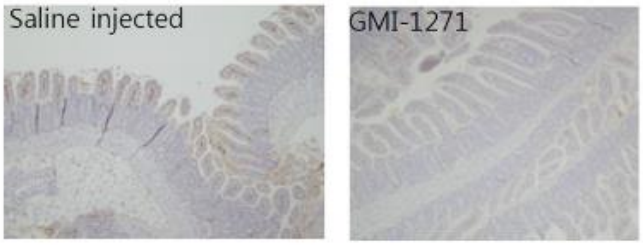
	Treatment	Survival (days)	% survival (day 120)
	Saline	33	0
	GMI-1271 IP 40 mg/kg bid x 10 days	26	0
	AraC/DNR	71	20
	GMI-1271 + AraC/DNR	>120	55

- Female NOD SCID mice injected iv with $5x10^4$ human tumor cells (n=10-11/group)
- R_x 3 days U937 injection

*Winkler IG et al. Blood 2016, 128:2823. Chien S, et al. Blood 2012;120:4092.

GMI-1271 Protects against Chemotherapy Induced Mucosal Injury



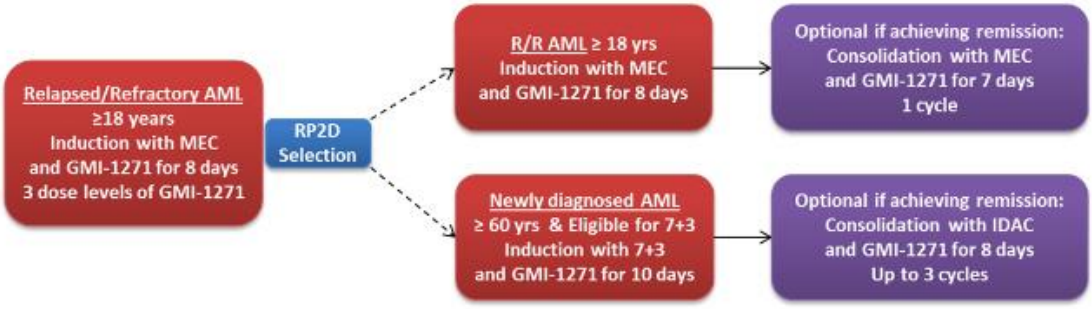


F4/80 macrophage staining on murine small intestine.

- Absence or blockade of E-selectin significantly reduced intestinal mucositis and therapy-induced weight loss.

*Winkler et al. Blood. 2014;122:21.

Phase 1/2 Study Schema



MEC Induction: mitoxantrone (10 mg/m²/d IV), etoposide (100 mg/m²/d IV), cytarabine (1000 mg/m²/d IV over 60 mins) for 5 days
MEC Consolidation: same, for 4 days

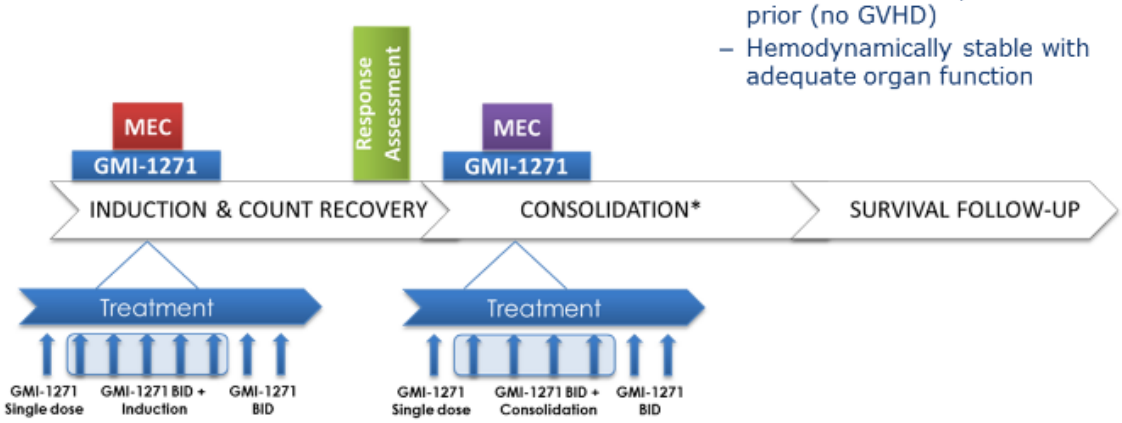
7+3 Induction: cytarabine (200 mg/m² continuous infusion x 7d), idarubicin (12 mg/m² x 3d). Day 15 optional re-induction of 5+2 of same
IDAC Consolidation: cytarabine (2 g/m²/d IV over 3 hrs QD for 5d), OR cytarabine (1.5 g/m²/dose IV over 3 hrs, BID QOD for 6 doses)

GMI-1271: 5, 10, or 20 mg/kg q12hrs in Phase 1. RP2D was 10 mg/kg

ClinicalTrials.gov identifier: NCT02306291

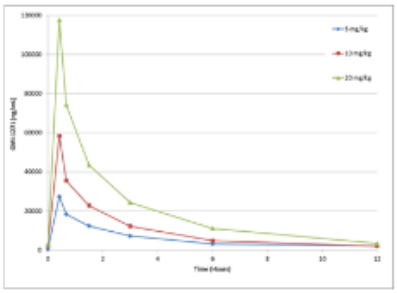
Phase 1/2 Study Schema – R/R Patients

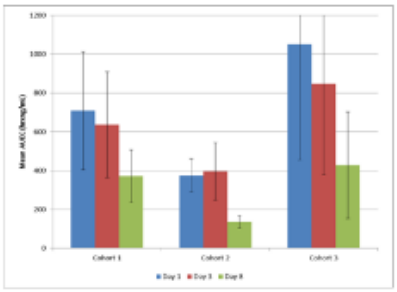
- Eligible patients –
  - ≥18 years old
  - **Primary refractory AML**, ≤2 prior inductions (one with anthracyclines)
  - OR **in first or second relapse**
  - HSCT was allowed, >4 months prior (no GVHD)
  - Hemodynamically stable with adequate organ function



*Amended to include 1 cycle of consolidation in Phase 2

Phase 1 – R/R Patients





Outcome, n (%)	Phase 1 N=19
Response	
CR/CRi	9 (47)
CR	8 (42)
All-Cause Mortality 60 days	2 (11)

Safety/DLT Assessment:

- No Dose Limiting Toxicities were seen
- No deaths occurred during treatment phase (44 days)

RP2D Selection:

- PK was dose proportional
- On-target effect seen at all dose levels
- Similar responses seen at all dose levels
- Dose level 2 (10 mg/kg) gave optimal exposure above IC50 and below toxicology testing limits

Demographics – R/R Patients

	Phase 1 N=19	Phase 2 N=47	Total N=66
Age, median (range)	51 (26-77)	61 (27-84)	59 (26-84)
Refractory, n (%)	**7 (37)**	**15 (32)**	**22 (33)**
Relapsed All, n (%)	12 (63)	32 (68)	44 (67)
Duration of prior remission <6 mos	**5 (26)**	**17 (36)**	**22 (33)**
Prior Therapies			
HSCT	4 (21)	7 (15)	11 (17)
≥2 Induction Regimens	6 (32)	16 (34)	22 (33)
ELN Risk Category			
Intermediate	2 (11)	9 (19)	11 (17)
Adverse	**11 (58)**	**22 (47)**	**33 (50)**
Unknown	4 (21)	11 (23)	15 (23)
Mutations			
FLT3-ITD	0	3 (6)	3 (5)
TP53 mutation; del (17p)	1 (5)	3 (6)	4 (6)

Response Data – R/R Patients

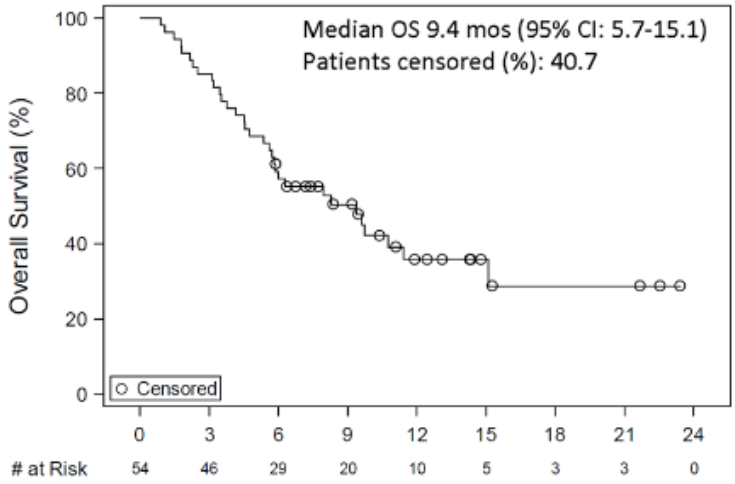
Outcomes, n (%)	Phase 1 N=19	Phase 2 N=47	Total N=66	RP2D N=54
CR/CRi	**9 (47)**	**18 (38)**	**27 (41)**	**23 (43)**
CR	8 (42)	14 (30)	22 (33)	19 (35)
ORR (CR/CRi/MLFS/PR)	10 (53)	22 (47)	32 (48)	27 (50)
Mortality, All-Cause				
30 days	0	1 (2)	1 (2)	1 (2)
60 days	**2 (11)**	**4 (9)**	**6 (9)**	**5 (9)**
Outcomes by Subgroup (CR/CRi Rate and %)				
Primary Refractory	4/7 (57)	4/15 (27)	8/22 (36)	**5/17 (29)**
Relapsed (all)	5/12 (42)	14/32 (44)	19/44 (43)	18/37 (49)
Duration, prior remission <6 mos	1/5 (20)	5/17 (29)	6/22 (27)	**6/19 (32)**
Duration, prior remission ≥24 mos	3/3 (100)	3/4 (75)	6/7 (86)	6/7 (86)

Common Grade 3/4 Adverse Events – R/R Patients

Adverse Event Type	Phase 1 N=19	Phase 2 N=47	Total N=66	RP2D N=54
Cardiac	**1 (5)**	**5 (11)**	**6 (9)**	**5 (9)**
Colitis	**2 (11)**	**0**	**2 (3)**	**1 (2)**
GI	**4 (21)**	**3 (6)**	**7 (11)**	**4 (7)**
Hepatic	**0**	**3 (6)**	**3 (5)**	**3 (6)**
Infectious	**16 (84)**	**34 (72)**	**50 (76)**	**39 (72)**
Bacteraemia	2 (11)	6 (13)	8 (12)	8 (15)
Febrile neutropenia	6 (32)	25 (53)	31 (47)	27 (50)
Sepsis	6 (32)	6 (13)	**12 (18)**	**8 (15)**
Oral Mucositis Events				
Grades 1/2, n (%)	5 (26)	9 (19)	14 (21)	9 (17)
Grades 3/4, n (%)	**1 (5)**	**1 (2)**	**2 (3)**	**1 (2)**

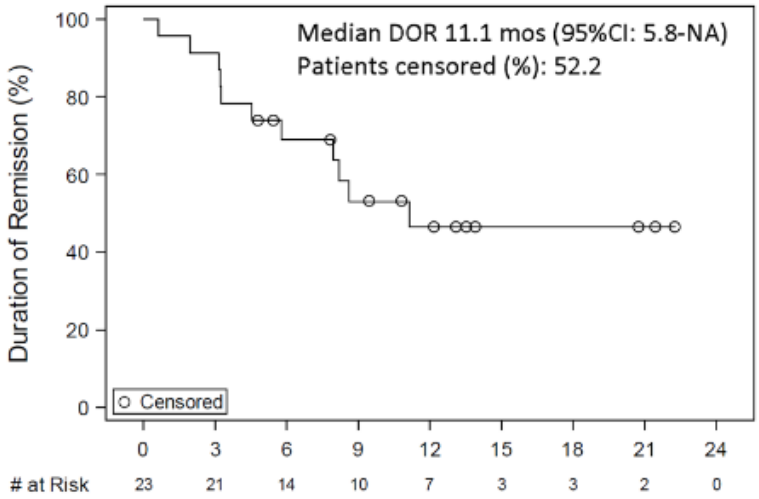
*AE grade definitions follow CTCAE v4.03.

Overall Survival – R/R Patients



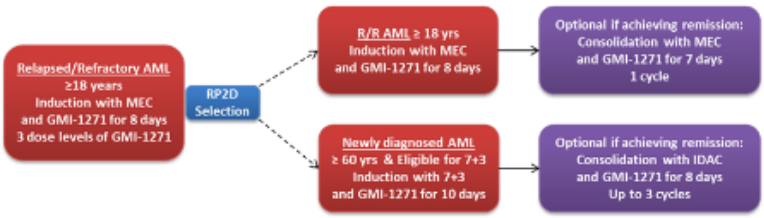
- RP2D analysis shown
- Median follow up is 6.6 months
- Censored at last known follow-up
- 16/66 (24%) have proceeded to HSCT

Remission Duration – R/R Patients

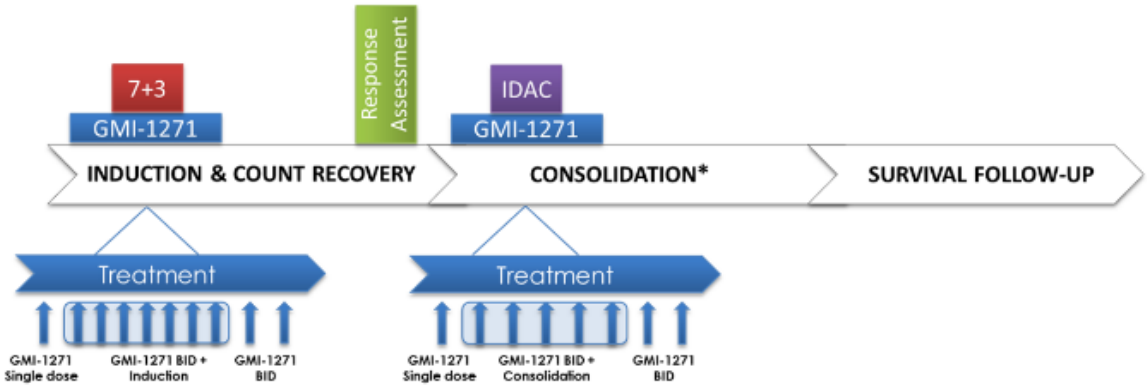


- RP2D analysis shown
- Median follow up is 6.6 months
- Censored at last known follow-up

Phase 1/2 Study Schema – Older Newly Diagnosed Patients



- Eligible patients –
  - **Patients ≥60 years with newly diagnosed AML**
  - Prior treatment for MDS, CMML was allowed
  - ECOG 0-2
  - Hemodynamically stable with adequate organ function



*Amended to include up to 3 cycles of consolidation in Phase 2

Demographics – Older Newly Diagnosed Patients

	N=25
Age, median (range)	67 (60-79)
Newly diagnosed, All	
de novo	12 (48)
Secondary AML	**13 (52)**
ELN Risk Category	
Favorable	3 (12)
Intermediate	7 (28)
Adverse	**12 (48)**
Unknown	3 (12)

Clinical Outcomes – Older Newly Diagnosed Patients

Outcome, n (%)	N=25
CR/CRi	**17 (68)**
CR	13 (52)
ORR (CR/CRi/MLFS)	20 (80)
Mortality, All-Cause	
30 days	2 (8)
60 days	**3 (12)**
Outcomes by Subgroup (CR/CRi rate and %)	
AML Type	
de novo	9/12 (75)
Secondary AML	**8/13 (62)**
ELN Risk Category	
Favorable risk	3/3 (100)
Intermediate risk	4/7 (57)
Adverse risk	8/12 (67)

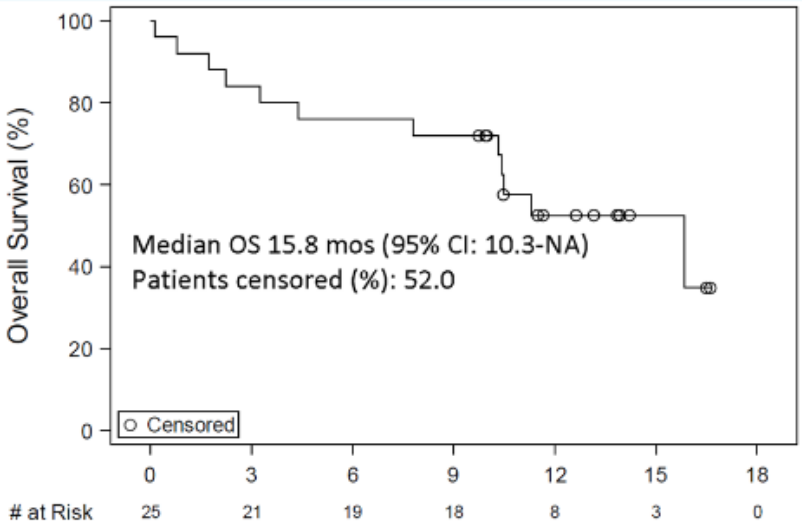
Common Grade 3/4 Adverse Events – Older Newly Diagnosed Patients

Adverse Event Type	N=25 Evaluable
Colitis	3 (12)
Infectious	19 (76)
Febrile neutropenia	17 (68)
Pneumonia	3 (12)
Sepsis	4 (16)
Rash	3 (12)
Respiratory	7 (28)

Oral Mucositis Events	
Grades 1/2	5 (20)
Grades 3/4	**0**

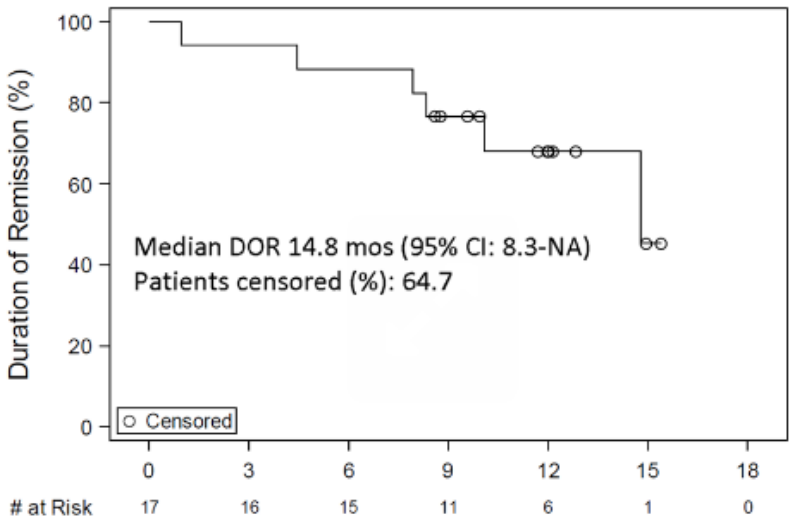
*AE grade definitions follow CTCAE v4.03.

Overall Survival – Older Newly Diagnosed Patients



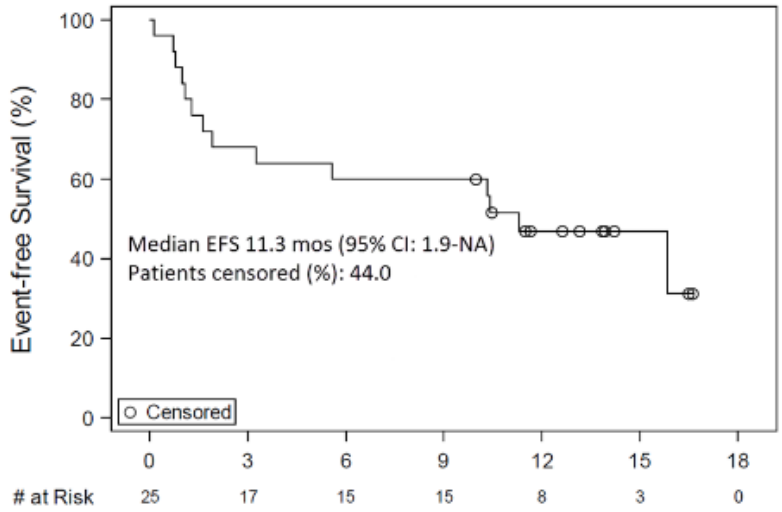
- Censored at last known follow-up
- Median follow up is 10.5 months
- 10/25 (40%) have proceeded to HSCT

Remission Duration – Older Newly Diagnosed Patients



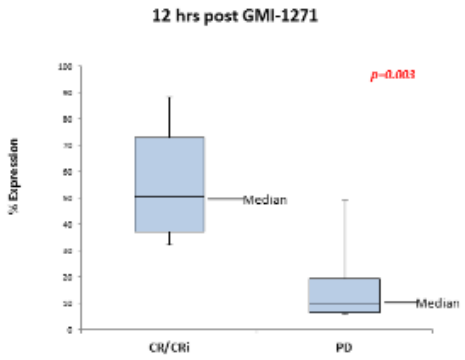
- Censored at last known follow-up
- Median follow up is 10.5 months

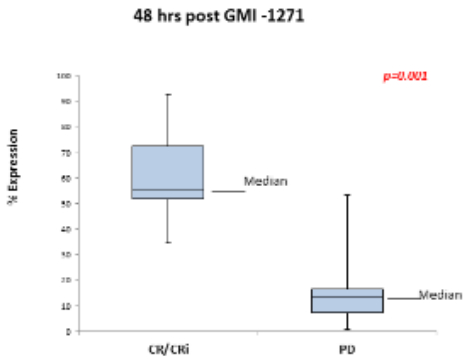
Event Free Survival – Older Newly Diagnosed Patients



- Censored at last known follow-up
- Median follow up is 10.5 months

Functional E-selectin Binding by Peripheral Blasts Predicts Response to Therapy with GMI-1271 in R/R Patients





- E-selectin ligand is detectable on blasts of all patients
- Proportion of baseline bone marrow blasts with detectable E-sel ligand is higher in relapsed than refractory patients (p=0.006) and trends higher in sAML than in de novo AML (p=0.13)
- Functional E-selectin binding by circulating blasts (shown) is higher for those achieving remission

Conclusions

- GMI-1271, an E-selectin antagonist, can be safely added to MEC salvage chemotherapy as well as standard induction chemotherapy (7+3).
- GMI-1271 when added to chemotherapy demonstrated improvements in important clinical endpoints
  - High remission rates that were durable in both R/R and newly diagnosed AML
  - Low induction mortality as well as low rates of mucositis and sepsis
  - Resultant survival outcomes promising in both populations
- Correlative studies confirm the expression of E-selectin ligand in AML and expression levels can predict response
- Future studies with GMI-1271 are warranted
  - Breakthrough Therapy Designation granted by FDA for R/R population
  - Randomized Phase III trials are being planned for both R/R and elderly untreated AML

GMI-1271 Represents a Significant Market Opportunity Across the Continuum of Care in AML

~45,000 Patients
(Annual Diagnosis Rates in 7MM)

Fit patients eligible for intensive therapy

Less-fit patients eligible for low-intensity therapy

Relapsed / Refractory

Combination of GMI-1271 + Salvage Chemotherapy (e.g. MEC)

+

Newly Diagnosed

Combination of GMI-1271 + 7&3

+

Unfit / Vulnerable

Combination of GMI-1271 + Hypomethylating Agent (e.g. Vidaza)

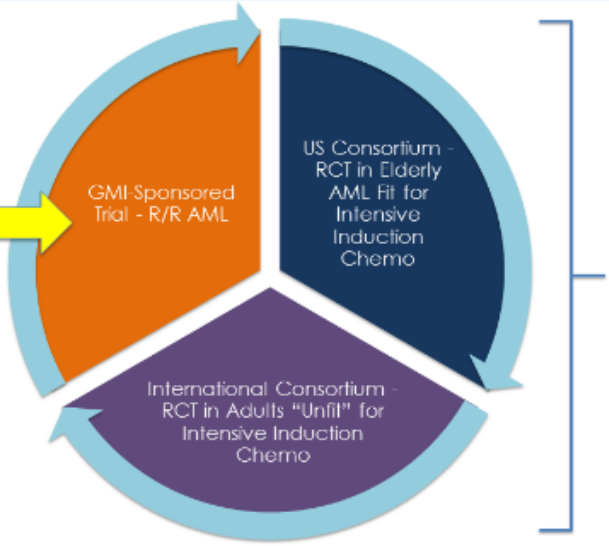
Expanding Commercial Opportunity

American Cancer Society; SEER; GLOBOCAN

GlycoMimetics, Inc.

GMI-1271 Comprehensive Development Approach in AML

Key operational focus for the company with intent to initiate pivotal trial in mid-2018



Collaborations Under Consideration

Goal is to Accelerate the Development of GMI-1271 through Strategic, Geographic Collaborations with Leading Hematology / Oncology Cooperative Groups in the USA and Europe

Next Steps – Development of GMI-1271 in AML

- We continue to have productive engagement with FDA under Breakthrough Therapy Designation.
  - Clinical, CMC, non-clinical issues all being addressed
- Phase 3 study design near final
  - Anticipate providing details in Q1
- Objective remains to initiate Phase 3 in relapsed/refractory disease in mid-2018
- Also anticipate initiating a consortium-funded trial in newly diagnosed patients during 2018.

GlycoMimetics is Positioned for Success

Promising Pipeline	• Rivipansel: Sole "on-demand" treatment in late-stage, registration trial for acute VOC under SPA • GMI-1271: Clinical proof-of-concept in AML; Breakthrough Therapy Designation granted by the FDA • GMI-1359: Simultaneous blockade of CXCR4 & E-Selectin inhibits established pathways of cancer cell trafficking
Significant Revenue Opportunities	• Rivipansel: ~100,000 patients in USA • GMI-1271: > 40,000 AML patients in 7MM
Strong Investment Base	• Cash balance provides runway through key milestones • Top-tier biotech investors
Experienced Team	• Pioneers in the field of glycobiology and small-molecule, therapeutic "mimetics" • Relationships with leading KOLs and oncology networks

GlycoMimetics

GMI-1271 Update
December 19, 2017

Innovation Today, Healing Tomorrow.

GlycoMimetics, Inc.