

# GlycoMimetics

CANCER  
**PROGRESS**  
*by Defined Health*

Cancer Progress by Defined Health  
New York, NY | March 8-9, 2016

**DefinedHealth**  
unconventional insight



# GlycoMimetics Corporate Overview

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## Management Team

- ◆ Rachel K. King, CEO
  - ◆ John L. Magnani, Ph.D., VP and Chief Scientific Officer
  - ◆ Helen Thackray, MD, FAAP, VP and Chief Medical Officer
  - ◆ Brian Hahn, Chief Financial Officer
  - ◆ Armand Girard, VP Corporate Development
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## About Us

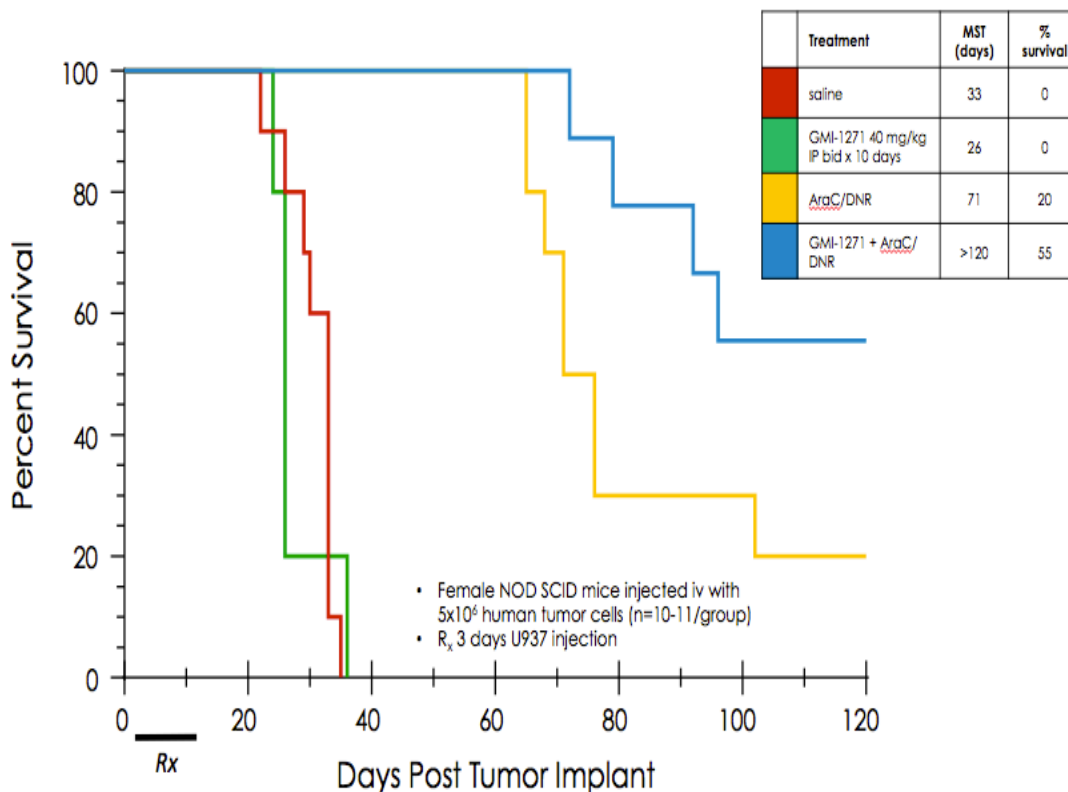
- ◆ Clinical-stage biotechnology company that utilizes pioneering glycobiology platform to develop treatments for specialty diseases
  - ◆ Area of expertise: Disruption of cellular adhesion targets within the vasculature and bone marrow microenvironment
  - ◆ Publicly traded on NASDAQ - GLYC
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## News

- ◆ Phase III program with Rivipansel, a pan-selectin antagonist, for Sickle Cell Anemia vaso-occlusive crisis initiated in 2015
  - ◆ Phase 2 data “Best of ASH”
  - ◆ First-ever SPA agreed with FDA
  - ◆ Worldwide License with Pfizer executed in 2011
- ◆ GMI-1271 - Phase I/II trial in AML ongoing
  - ◆ Expansion of clinical program into immuno-oncology
- ◆ GMI-1359 - IND for hematologic malignancies mid-year

# GMI-1271: E-Selectin Antagonist That Disrupts Relationship Between Tumor Cells and BM Microenvironment

## GMI-1271 in Combination with AraC/DNR in U937 AML Tumor Model



### Preclinical data with GMI-1271

- Prevents trafficking of tumor cells to the bone marrow
- Disrupts cell adhesion-mediated drug resistance (CAMDR) within bone marrow microenvironment
- Inhibits activation of cancer survival pathways (e.g. Wnt)
- Protects normal HSCs from chemotherapy by enhancing quiescence and ability for self-renewal

# Phase I / II Open-Label Study in AML

- **Study Design**

- Dose Escalation: up to 4 dose levels (5, 10, 20 & 40 mg/kg)
  - Relapsed/refractory AML (MEC)
- Dose Expansion: up to 50 patients
  - Up to 25 R/R AML (MEC)
  - Up to 25 newly diagnosed AML (Ara-C & idarubicin)

- **Endpoints:** Safety, PK, Biomarkers & Efficacy (ORR)

- **Lead Investigator:** Dan DeAngelo, MD – Dana Farber Cancer Institute

**GlycoMimetics, Inc.** [Previous Release](#)

03/02/2016

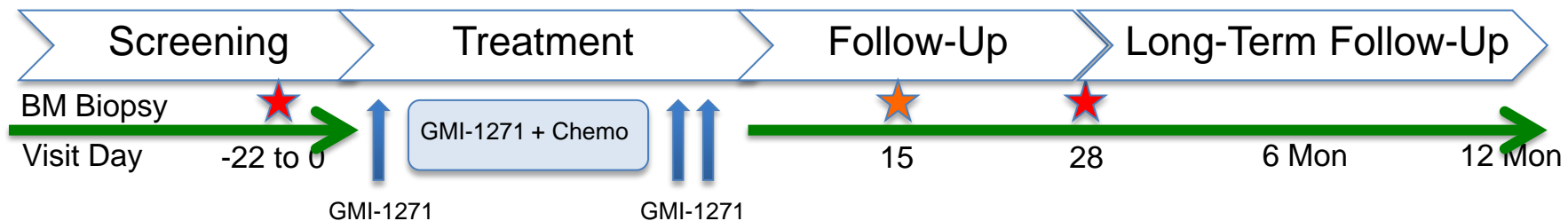
### GlycoMimetics Announces Initial Clinical Data from Phase 1 Portion of Clinical Trial of GMI-1271 in AML Patients

*- Encouraging Anti-Leukemic Activity Observed in Patients with Advanced AML; Overall Response Rate of 62% in Initial Cohort of 13 Patients*

ROCKVILLE, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ: GLYC) today announced that of the first 13 evaluable patients in its clinical trial of GMI-1271 in combination with chemotherapy in patients with relapsed/refractory acute myeloid leukemia (AML), investigators have observed clinical responses in eight patients, for an overall response rate of 62%. Of the eight objective responses, seven patients achieved a complete response (CR), with the eighth patient achieving complete response but with an incomplete blood count recovery (CRI). GMI-1271 was also well tolerated in this group of 13 patients. The detailed findings have been submitted to a major scientific meeting.

"We are encouraged by the responses seen so far in patients in this trial," said Daniel J. DeAngelo, M.D., Ph.D., Director of Clinical and Translational Research of the Adult Leukemia Program at the Dana-Farber Cancer Institute and the Principal Investigator on the trial. "Patients with AML have such unsatisfactory treatment options that it is important to continue enrolling patients into this trial to characterize the full potential of GMI-1271."

"I am pleased to see the favorable safety profile and early clinical activity of GMI-1271 in these patients," said Helen Thackray, M.D., Chief Medical Officer of GlycoMimetics. "We believe that by selectively disrupting cell-adhesion-mediated drug resistance mechanisms within the bone marrow, GMI-1271 may significantly enhance the efficacy of chemotherapy, without adding incremental toxicity. We look forward to presenting the complete safety, pharmacokinetics, pharmacodynamics and anti-tumor activity, including durability of CRs, at an upcoming scientific meeting."

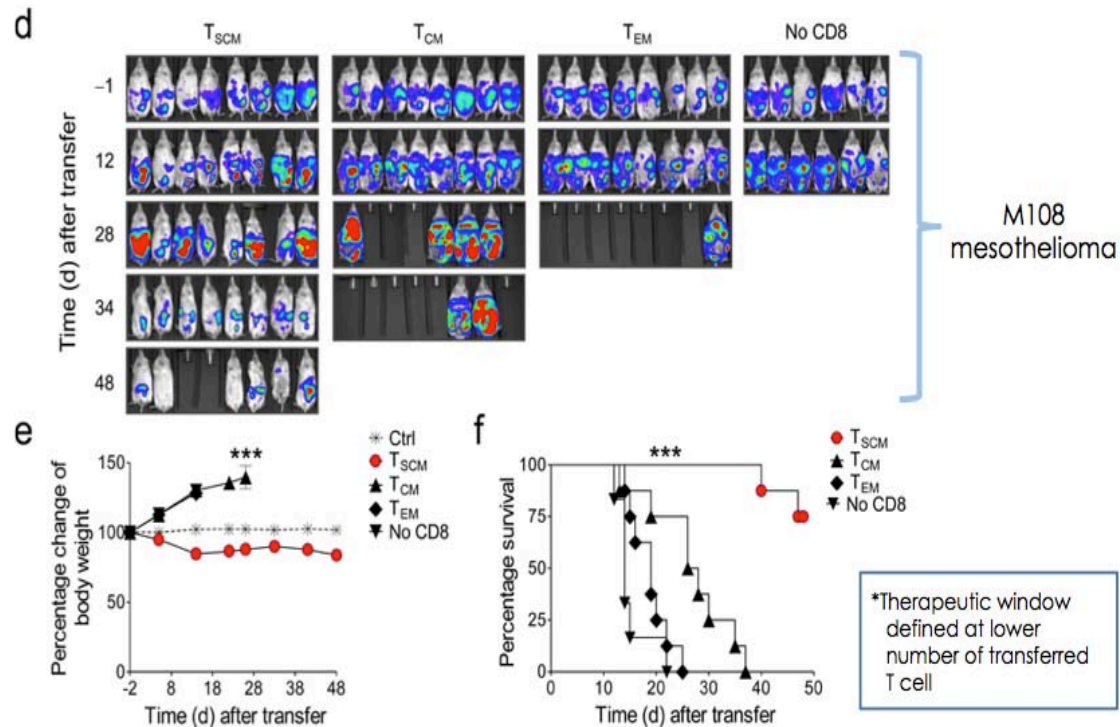


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# Enhanced Responses with More Primitive T-Cells Strongly Supported in the Literature

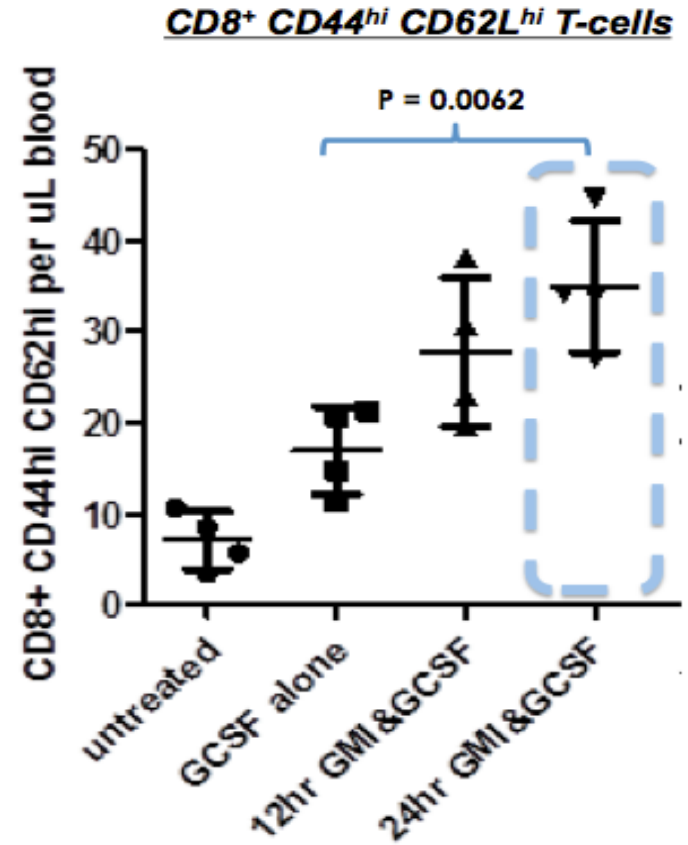
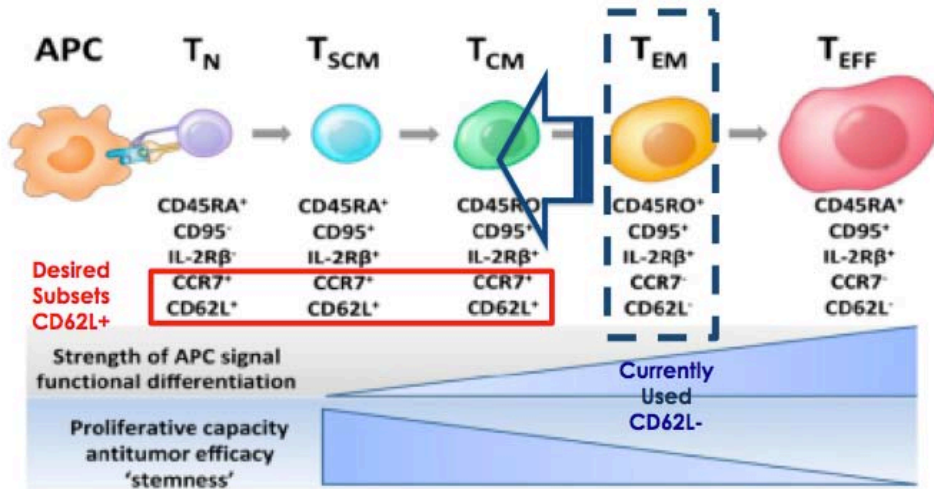


## Increased Proliferative Capacity, Survival and Antitumor Activity of T<sub>SCM</sub> cells<sup>1</sup>



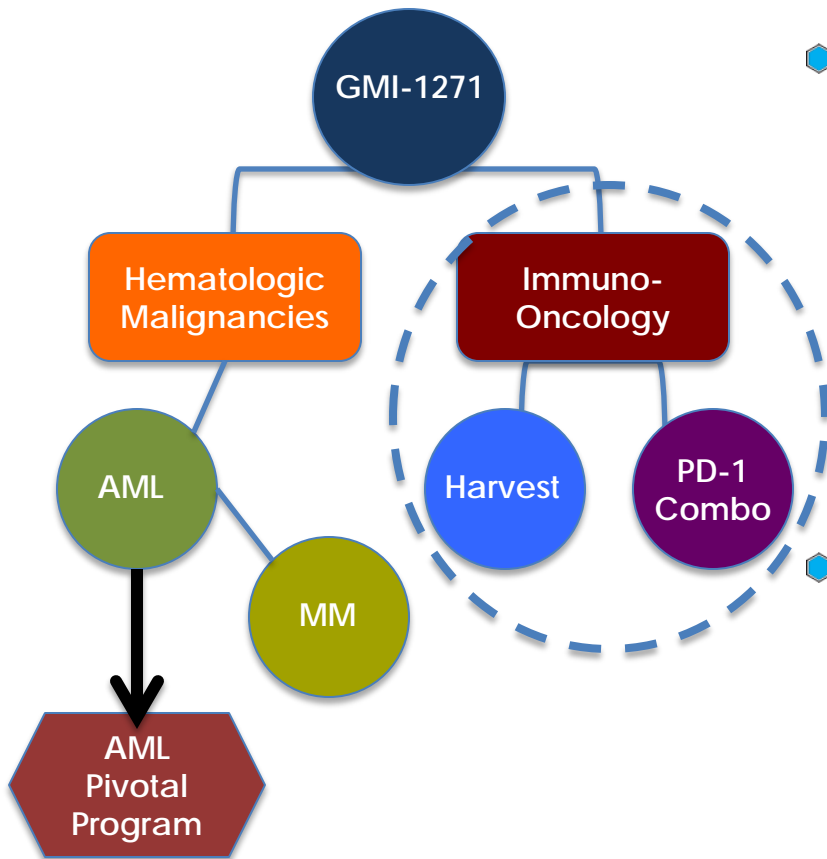
<sup>1</sup> Gattinoni et al., Nat Med. 2011 Sep 18; 17(10): 1290–1297.

# GMI-1271 Primitive T-Cell Enriching Strategy



ASH 2015 - "GMI-1271 significantly improves the in vivo reconstitution potential and regenerative properties of CD8<sup>+</sup> T-cells from the donor blood allowing a valuable source of desired T-cells for use in adoptive immunotherapy and T-cell engineering"

# Strategic Considerations



- ◆ Plans in place to establish POC in Immuno-oncology settings:
  - ◆ What fold increase in harvested  $T_{SCM}$  and  $T_{CM}$  T-cell subsets is clinically relevant?
  - ◆ Will approach for mobilizing primitive T-cell subsets also enhance the efficacy of checkpoint inhibitors?
- ◆ “T-cell enrichment strategy” represents an exciting additional opportunity – but requires expertise outside GMI’s scope
  - ◆ e.g. CAR-T engineering and manufacturing

Pilot studies in humans / collaborations with leading institutions

*How far should GMI take this novel immuno-oncology approach? When is optimal time for partnering?*