Gamida Cell Presents Data from Two Key Development Programs at the 2017 ASH Annual Meeting

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— Final results from the company's phase I/II study of NiCord® demonstrate important potential as a universal transplantation solution for patients with high-risk blood cancers —

— Preclinical study of NAM-NK Cells supports utility as an immunotherapeutic modality for treating cancer —

JERUSALEM and CAMBRIDGE, Mass., December 11, 2017 – Gamida Cell, a leading cellular and immune therapeutics company, today announced final results from the phase I/II trial evaluating NiCord, a product derived from cord blood stem cells, as a stand-alone graft to treat patients with high-risk hematologic malignancies. The study met its primary endpoint, demonstrating rapid neutrophil engraftment with manageable side effects. The company also presented preclinical data for the advancement of natural killer cells (NK cells) as an immunotherapeutic modality for patients with cancer. Both studies were presented today at the annual meeting of the American Society of Hematology (ASH) in Atlanta, GA.

"We are enthusiastic about the data presented today at ASH, including the final results of our NiCord phase I/II study, which support the basis for our global phase III trial of NiCord versus standard unmanipulated cord blood transplantation (UCBT), currently enrolling patients with hematologic malignancies," said Julian Adams, Ph.D., chairman and chief executive officer of Gamida Cell. "We look forward to further evaluating the potential of NiCord to serve as the graft of choice for the thousands of patients with no matched donor in need of a transplant every year, as well as continuing to progress our other pipeline programs."

Final Data from Phase I/II Trial of NiCord.
The multicenter phase I/II study evaluated the safety and efficacy of NiCord as a stand-alone graft in 36 patients with high-risk hematologic malignancies, with a primary endpoint of time to neutrophil engraftment following transplantation. Despite varying blood cancer diagnoses and preparative conditioning regimens across patients across centers, improved results were seen in the majority of study participants treated with NiCord.

Final results of the study include the following:

- Participants transplanted with NiCord had rapid and durable engraftment of neutrophils and platelets, as well as prompt immune reconstitution:
  - Median time to neutrophil engraftment was 11 days (95% CI: 9-13 days);
  - Median time to platelet engraftment was 34 days (95% CI: 32-42 days).
- Results from the study participants were compared to a database of matched patients from the Center for International Blood and Marrow Transplant Research (CIBMTR). According to the CIBMTR data, patients who received UCBT had a median time to neutrophil engraftment of 21 days and a median time to platelet engraftment of 46 days.

NiCord demonstrated an acceptable safety profile, with moderate/severe chronic graft vs. host disease (cGvHD) in 9.8% of patients at one year following transplantation. By day 100, 20.2% of participants experienced grade 2-3 bacterial or grade 3 fungal infections.

"Historically, transplantation with cord blood has been limited due to slow engraftment time in patients. We are looking to address this gap, and this study demonstrated rapid and sustained engraftment in study participants by utilizing technology to expand the number of stem cord blood cells in a culture," said Mitchell Horwitz, M.D., principal investigator, co-study chair and professor of medicine at the Duke Cancer Institute. "These phase I/II data demonstrate the potential to make stem cell transplants accessible to a greater number of patients who do not have a matched donor."

Preclinical Data from NAM-NK Cell Program
Proof-of-concept data on the application of the company's proprietary NAM technology to healthy donor natural killer cells (NK cells) as a potential immunotherapeutic approach to treating cancer were highlighted in an oral presentation.

"The use of NK cells as a modality for immunotherapy has been limited by impaired functionality of adoptively transferred NK cells in patients," said Ronit Simantov, M.D., chief medical officer at Gamida Cell. "We are encouraged by the study results, which demonstrated persistence and proliferation of NAM-NK cells in pre-clinical in vivo models and describe a reliable, scalable culture model for the expansion of functional donor NK cells aimed at clinical use."

The analysis, which combines data from multiple preclinical studies, validates the approach and is the basis for an investigator-sponsored, phase I clinical trial of NAM-NK Cells in patients with relapsed/refractory multiple myeloma or CD20-positive non-Hodgkin lymphoma.

About NiCord
NiCord, the company's lead clinical program, is under development as a universal bone marrow transplant solution for patients with high-risk hematologic malignancies. NiCord has demonstrated improved efficacy over unmanipulated cord blood, including fewer bacterial and fungal infections and a reduction in duration of hospital stays. NiCord has been granted breakthrough status by the U.S. Food and Drug Administration, making it the first bone marrow transplant alternative to receive this designation. It has also received U.S. and EU orphan drug designation. The ongoing phase III study is evaluating NiCord as a curative treatment for patients with leukemia and lymphoma who have been indicated for an allogeneic stem cell transplant. For more information on NiCord clinical trials, please visit www.clinicaltrials.gov.

About NAM-NK Cells
Gamida Cell expanded the capabilities of its NAM technology to utilize NK cells to create an immunotherapy to treat patients with refractor blood cancers and solid tumors. Through expansion of highly functional NK cells using NAM technology, NAM-NK Cells can be used to harness the immune system to attack cancer. NAM-NK Cell is under phase I development (NCT03019666) in patients with relapsed or refractory B-cell lymphoma and multiple myeloma.

About Gamida Cell
Gamida Cell is a leader in cellular and immune therapeutics dedicated to treating patients with cancer and rare genetic diseases. The company is building a diverse pipeline based on its proprietary NAM technology platform to deliver transformative medicines to patients in need of new treatment options. To learn more about Gamida Cell, including current clinical studies, please visit gamida-cell.com and on Twitter, LinkedIn and Facebook.

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