Gamida Cell to Present Data from NiCord® Programs at the 2018 BMT Tandem Meetings

February 21, 2018

– NiCord demonstrated rapid and durable engraftment in patients with high-risk hematologic malignancies –

– 13 patients with sickle cell disease transplanted with NiCord and engrafted at median seven days –

CAMBRIDGE, Mass., February 20, 2018 – Gamida Cell, a leading cellular and immune therapeutics company, today announced two presentations from its NiCord programs in high-risk hematologic malignancies and sickle cell disease (SCD) at the 2018 BMT Tandem Meetings, held from February 21 – 25 at the Salt Palace Convention Center in Salt Lake City, UT. Details for the presentations are as follows:

- *NiCord Single Unit Expanded Umbilical Cord Blood Transplantation (UCBT): Final Results of a Multicenter Phase I/II Trial;* Mitchell Horwitz, M.D., Session G: Alternative Donor / Immune Reconstitution, Feb. 23, 2018 at 10:30 a.m. MT in Ballroom I
- *A Novel Therapy for Sickle Cell Disease (SCD): Co-Transplantation of NiCord [Ex-Vivo Expanded Umbilical Cord Blood (UCB) Progenitor Cells with Nicotinamide] and an Unmanipulated Unrelated UCB Graft Leads to Successful Engraftment and Cure of Severe SCD;* Suhag H. Parikh, M.D., Poster Session 1: Cord Blood, Feb. 21, 2018 at 6:45 p.m. MT in Hall E

"We are pleased with the results of the data being presented at the BMT Tandem Meetings, which highlight the promise of NiCord as a potential graft for transplantation in multiple patient populations," said Ronit Simantov, M.D., chief medical officer of Gamida Cell. "These data are the basis of our phase III study that is currently enrolling patients with high-risk hematologic malignancies, and support our future plans to evaluate NiCord as a stand-alone graft in patients with sickle cell disease."

Phase I/II Data Evaluating NiCord in High-Risk Hematologic Malignancies
The multicenter phase I/II study, led by Mitchell Horwitz, M.D., co-study chair and professor of medicine at the Duke Cancer Institute, evaluated the safety and efficacy of NiCord as a stand-alone graft in 36 patients with high-risk hematologic malignancies. The study met its primary endpoint of time to neutrophil engraftment; participants experienced rapid and durable neutrophil engraftment at a median of 11 days (95%CI: 9-13 days), representing an improvement over historical standard cord blood transplantation as well as compared to a database of patients from the Center for International Blood and Marrow Transplant Research (median 21 days, 95%CI: 20-23 days). NiCord demonstrated an acceptable safety profile, with moderate/severe chronic graft vs. host disease (GvHD) in 9.8% of patients at one year following transplantation. These data were also presented at the 59th American Society of Hematology (ASH) annual meeting in December 2017.

Phase I/II Data of NiCord in Sickle Cell Disease
The phase I/II study evaluated the safety and efficacy of NiCord in combination with an unmanipulated unrelated umbilical cord blood (UCB) unit in 13 patients with SCD in need of an allogeneic bone marrow transplant (BMT). The primary endpoint of the study was cumulative incidence of neutrophil engraftment following transplantation.

Key results include:

- Rapid engraftment was observed in all 13 patients at a median of seven days (range: 6-20 days)
- 11 of 13 (84.6%) patients were alive at a median follow-up of 22 months (range: 1-63 months)
- Eight participants experienced grade II-IV acute GvHD and two experienced extensive chronic GvHD. Two patient deaths were observed due to secondary graft failure and severe GvHD respectively
- The nine patients with long-term follow-up achieved transfusion independence with normal hemoglobin profile and no active GvHD

"Allogeneic bone marrow transplantation is a potentially curative treatment for sickle cell disease, but patients eligible for a transplant face significant challenges finding a donor match. We initiated this study to evaluate if partially matched, unrelated double cord transplantation of NiCord and unmanipulated cord blood could serve as an effective alternative graft and successfully overcome the engraftment barrier in this difficult to engraft patient population," said Dr. Parikh, study investigator and associate professor of pediatrics at the Duke Cancer Institute. "We are pleased with the results of this patient group, which showed sustained engraftment despite using partially matched UCB, as well as resolution of SCD-related symptoms in all nine study participants with long-term follow-up. Further research will focus on decreasing the risk of graft vs. host disease."

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. A phase III study evaluating NiCord in patients with leukemia and lymphoma is ongoing in the United States, Europe and Asia (NCT02730299). For more information on NiCord clinical trials, please visit www.clinicaltrials.gov.

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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