Gamida Cell to Present Immune Reconstitution Data for its NiCord® Program at the 2018 EBMT Annual Meeting

March 20, 2018

NiCord recipients demonstrated rapid and robust immune reconstitution following transplantation

CAMBRIDGE, Mass., March 19, 2018 – Gamida Cell, a leading cellular and immune therapeutics company, today announced that new data on immune reconstitution (IR) from its phase I/II study of NiCord in high-risk hematologic malignancies will be presented during the 44th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT), held from March 18-21, 2018, in Lisbon, Portugal.

Presentation Details:
Title: Rapid and Robust CD4+ and CD8+ T-, NK-, B- and monocyte cell reconstitution after Nicotinamide-expanded Cord Blood (NiCord) Transplantation
Presenter: Stefan Nierkens, Associate Professor of the Central Immune Monitoring lab and the Boelens/Nierkens research group at the University Medical Centre Utrecht, Utrecht, Netherlands
Session: Oral Session 18, Alternative donors and approaches; Auditorium IV
Date: March 21, 2018, 11:00 a.m. WET

“Along with the clinical data from our phase I/II study of NiCord in patients with hematologic malignancies, the immune reconstitution findings being presented at EBMT provide further evidence for the use of NiCord as a stand-alone graft in bone marrow transplantation,” said Ronit Simantov, M.D., chief medical officer at Gamida Cell. “We look forward to evaluating NiCord in our ongoing phase III study and to advancing our clinical development program.”

The presentation highlights IR findings of a random subgroup of 22 patients from the phase I/II multicenter trial of NiCord as a stand-alone graft after myeloablative therapy in patients with high-risk hematologic malignancies. The primary endpoint was the probability of achieving CD4+ IR (>50×106/L) within the first 100 days, and the secondary endpoints were IR of CD4+, CD8+, monocytes, natural killer (NK) and B-cells during the first year after transplantation. Data were compared to cohorts of adolescent and young adult patients with hematologic malignancies from the University Medical Centre Utrecht receiving unmanipulated cord blood transplantation (n=27) and T cell-replete unrelated bone marrow transplantation (n=20).

Key findings from the analysis include:

- More than 90% of patients achieved successful CD4+ IR at 100 days after transplantation with NiCord.
- T-cell IR with NiCord (median age 41.5 years) was similar to that observed in younger cohorts receiving unmanipulated CB and unrelated bone marrow without ATG (median ages 15.4 and 14.3 years, respectively).
- Immune reconstitution of NK cells ($p<0.001$), B cells ($p=0.026$) and monocytes ($p<0.001$), was faster after transplantation with NiCord when compared to the other groups.

“Immune reconstitution following transplantation is crucial for disease and viral control, but historically cord blood transplantation has had limitations in timely immune reconstitution in patients,” said Dr. Nierkens. “In addition to the rapid neutrophil and platelet engraftment with NiCord, the results from this study showed robust immune reconstitution, even when compared to younger patients who typically achieve more rapid recovery than adults.”

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.

Contact:
Katie Engleman
Pure Communications
katie@purecommunications.com
+1 910.509.3977

Investor Contact:
Daniel Ferry
LifeSci Advisors
daniel@lifesciadvisors.com
+1 617.535.7746