Gamida Cell Announces Publication of Positive Clinical Outcomes from NiCord-Transplanted Patients in the Journal of Biology of Blood and Marrow Transplantation

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NiCord-transplanted patients demonstrated improvements in time to engraftment, bacterial and non-viral grade 2-3 infection rate, and duration of hospitalization

Jerusalem, Israel, April 20, 2017 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, announced today the publication of positive clinical outcomes from an investigator-initiated study of NiCord®-transplanted patients in *Biology of Blood and Marrow Transplantation (BBMT),* the official publication of the American Society for Blood and Marrow Transplantation.

The article, titled "Transplantation of Ex Vivo Expanded Umbilical Cord Blood (NiCord) Decreases Early Infection and Hospitalization", highlights data underscoring key benefits associated with NiCord over standard umbilical cord blood (UCB) transplantation, including shorter time to engraftment, fewer infections and shorter hospital stays.

"Our results indicate that rapid hematopoietic recovery from Gamida Cell's NiCord transplantation approach is associated with clinical benefit. Reducing the burden of infections is important, as these are a major cause of early morbidity and death of patients following UCB transplantation," said Mitchell Horwitz, M.D., Professor of Medicine and Director, Adult Blood and Marrow Transplant Program at Duke University Medical Center and principal investigator of the study.

"NiCord continues to demonstrate significant advantages over standard UCB transplantation in clinical efficacy and in pharmaco-economic parameters enhancing our understanding of the market potential of the product," said Yael Margolin, Ph.D., President and Chief Executive Officer of Gamida Cell. "Our Phase 3 registration trial of NiCord is ongoing in which we hope to reproduce these important results in a larger patient population."

The study compared 18 consecutive NiCord-transplanted patients and 86 consecutive standard umbilical cord blood (UCB) transplanted patients with all patients receiving TBI-based myeloablative conditioning. The data highlights from the study that were published in BBMT include:

- **Statistically significant improvement in time to engraftment**: Median time to neutrophil engraftment in NiCord patients was 12.5 days (range 10-18) compared to 27 days (range 23-28) in standard UCB patients (p<.001).
- **Decrease in bacterial and non-viral grade 2-3 infections**: Frequency with NiCord patients versus standard UCB patients was 22% vs. 54%, respectively (p=.015). In addition, a univariate and multivariate analysis revealed significantly reduced risk ratio for all bacterial (0.35, p=.001) and grade 2-3 non-viral infection (0.16, p<.001) in NiCord patients versus standard UCB patients.
- **Meaningful reduction in duration of hospitalization:** NiCord patients had 21.51 (95% CI 8.25, 34.78) more days alive and out of the hospital in the first 100 days compared to standard UCB patients (p=.002) after adjustment for age, KPS and acute grade 3-4 GVHD.

NiCord is currently being studied in an international, multi-center, Phase 3 registration study as a graft for bone marrow transplantation for patients with blood cancer who do not have a rapidly available fully matched donor. The Company announced in February 2017 that the first patient in the study had been transplanted. NiCord has an FDA Breakthrough Therapy Designation as well as FDA and EMA orphan drug designations, the most recent granted in March 2017.

For more information on enrolling transplantation centers and study inclusion and exclusion criteria please click here.

About NiCord

NiCord is a stand-alone graft derived from a single umbilical cord blood unit which has been expanded in culture and enriched with stem and progenitor cells using Gamida Cell's proprietary NAM technology. NiCord leverages the advantage of umbilical cord blood which does not need full tissue matching to the patient, and can therefore be available to practically all patients in need. It also aims to address the major barrier of umbilical cord blood transplantation – delayed hematopoietic recovery – by demonstrating an advantage with a primary endpoint that is clinically meaningful.

About Gamida Cell

Gamida Cell is a world leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases. The company's pipeline of products are in development to treat a wide range of conditions including cancer, genetic hematological diseases such as sickle cell disease and thalassemia, bone marrow failure syndromes such as aplastic anemia, genetic metabolic diseases and refractory autoimmune diseases. Gamida Cell's current shareholders include Novartis, Elbit Imaging, Clal Biotechnology Industries, Israel Healthcare Venture, Denali Ventures and Auriga Ventures. For more information please visit gamida-cell.com.

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