Gamida Cell Announces First Patient Transplanted in Phase 3 Registration Study of NiCord for Blood Cancers

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Jerusalem, Israel, February 28, 2017 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, announced today that the first patient has been transplanted in the Company's Phase 3 study of NiCord®, in development as a cure for patients with blood cancer who do not have a fully matched donor required for bone marrow transplantation, a potentially $3 billion market. NiCord has an FDA Breakthrough Therapy Designation as well as FDA and EMA orphan drug designations.

"NiCord can provide a transplantation solution for patients in need. With clinical outcomes that potentially compare to a matched donor, NiCord is positioned to become the graft of choice for patients who do not have a high probability of rapidly finding a match," said Gamida Cell President and CEO Dr. Yael Margolin. "This registration study is the last stage in the clinical development of NiCord towards regulatory approval and market introduction, with the goal of saving lives, curing disease and reducing treatment costs."

The Phase 3 clinical trial is an international, randomized, controlled, open-label, multicenter, registration study. The trial will evaluate the safety and efficacy of transplanting NiCord® as compared to transplanting un-manipulated cord blood in patients with hematological malignancies (blood cancers) following a preparative conditioning regimen. The trial is expected to enroll 120 patients, ages 16-60. The primary end point is the time to neutrophil engraftment.

"We are very excited to start the Phase 3 study where we hope to reproduce the encouraging results demonstrated in the Pilot and Phase 1/2 data in a broader population," said Guillermo Sanz, M.D., Principal Investigator and Co-Study Chair, Hospital Universitario La Fe.

"There is a clinical need for a transplant option for the many patients who do not have a fully matched donor. Data from the Phase 1/2 study strongly support the further study of NiCord in this important Phase 3 trial," said Mitchell Horwitz, M.D., Principal Investigator and Co-Study Chair, Duke Cancer Institute.

Data from the Pilot and Phase 1/2 studies has demonstrated better clinical outcomes for patients and improved pharmaco-economic parameters, compared to transplantation of un-manipulated umbilical cord blood. These data were presented at EBMT 2016 and ASCO 2016.
Confirmation of data to date by Phase 3 study results will establish NiCord as a transformative therapy in the clinical practice of bone marrow transplantation.

More information on enrolling transplantation centers and study inclusion and exclusion criteria can be found 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, Teva Pharmaceutical Industries, Denali Ventures and Auriga Ventures. For more information please visit gamida-cell.com

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