Gamida Cell Receives Additional EMA Orphan Drug Designation for NiCord® in Haematopoietic Stem Cell Transplantation (HSCT)

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Phase 3 study of NiCord commenced last month in blood cancer patients

Jerusalem, Israel, March 23, 2017 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, announced today that orphan drug designation has been granted by the European Medicines Agency's (EMA's) Committee for Orphan Medicinal Products (COMP) regarding NiCord® as a treatment for haematopoietic stem cell transplantation, also commonly known as bone marrow transplantation (BMT).

"We are very encouraged that the EMA has widened NiCord's orphan drug designation to include BMT, and believe it is an important recognition of the unmet needs in these indications," said Gamida Cell president and CEO Dr. Yael Margolin. "This broadened regulatory designation adds a key layer of market exclusivity for NiCord as we move forward with our Phase 3 study and prepare for commercialization."

The EMA grants an orphan drug designation to promote the development of products that demonstrate promise of significant benefit for the treatment of rare diseases. Products receiving orphan drug designation are eligible to receive various regulatory and economic benefits, including 10 years of market exclusivity in the EU.

Gamida Cell was previously granted orphan drug designation for NiCord by the EMA for acute myeloid leukemia (AML), and by the U.S. Food and Drug Administration (FDA) for the treatment of several hematologic malignancies including AML, acute lymphoblastic leukemia (ALL), Hodgkin's lymphoma, myelodysplastic syndromes (MDS) and chronic myelogenous leukemia (CML). Gamida Cell also received FDA Breakthrough Therapy Designation for NiCord in blood cancers.

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 last month that the first patient in the study had been transplanted.

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, Teva Pharmaceutical Industries, Denali Ventures and Auriga Ventures. For more information please visit gamida-cell.com.

Press Contact:
Marjie Hadad
MH Communications
+972 54 536 5220
marjierhadad@gmail.com

Investor Contact:
Beth DelGiacco
Stern Investor Relations, Inc.
+ 1 212 362 1200
beth@sternir.com