Gamida Cell Receives FDA Breakthrough Therapy Designation For NiCord®

October 12, 2016

NiCord is a novel graft modality for bone marrow transplantation in patients with hematological malignancies including leukemia and lymphoma

Jerusalem, Israel, October 11, 2016 —Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to the Company's lead product candidate, NiCord®, in development as a novel graft modality for bone marrow transplantation in patients with high risk hematological malignancies (blood cancers) such as leukemia and lymphoma. The international, multi-center Phase 3 registration study of NiCord is planned to begin before the end of the year.

Breakthrough therapy designation may be granted to a drug that is intended to treat a serious or life-threatening condition, and where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on at least one clinical significant endpoint over available therapies. A breakthrough therapy designation entitles the company to more intensive FDA guidance on an efficient and accelerated drug development program, and eligibility for other actions to expedite the FDA review, such as a rolling submission and priority review.

"We are very pleased the FDA has recognized the potential of NiCord to address the unmet clinical need in bone marrow transplantation," said Dr. Yael Margolin, president and CEO of Gamida Cell. "The breakthrough therapy designation creates the foundation for a joint and concerted effort between the FDA and Gamida Cell to bring this important therapy faster to patients. We look forward to continuing our close cooperation with the FDA and other regulatory agencies to a positive conclusion as we prepare for commercialization."

Data from the Pilot, and Phase 1/2 studies of NiCord to date, have demonstrated clinically meaningful improvement in time to neutrophil engraftment over cord blood transplantation. Additionally, NiCord study data have shown fewer infections, reduced length of hospitalization, quicker platelet engraftment and improved non-relapse mortality when compared to unmanipulated cord blood transplantation. Click here to review the results presented at ASCO 2016 and here for the data presented at EBMT 2016.

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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