



Gamida Cell Receives Orphan Drug Designation from the FDA for NiCord® as a Treatment for Hematopoietic Stem Cell Transplantation

July 18, 2018

CAMBRIDGE, Mass., July 17, 2018 – [Gamida Cell](#), a leading cellular and immune therapeutics company, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for NiCord as a treatment for hematopoietic stem cell transplantation (HSCT).

Gamida Cell was previously granted orphan drug designation for NiCord by the FDA as a treatment for several hematologic malignancies, and by the European Medicines Agency (EMA) as a treatment for HSCT.

The FDA grants orphan drug designation to investigational drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug status is intended to facilitate drug development and may provide benefits to drug developers, including up to seven years of market exclusivity upon regulatory product approval and exemptions from certain FDA application fees.

About NiCord

NiCord, Gamida Cell'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 clinical-stage biopharmaceutical company leveraging its proprietary technology to develop cell therapies that are designed to cure cancer and rare, serious hematologic diseases. The company is developing a diverse pipeline based on its nicotinamide-, or NAM, -based cell expansion technology, addressing limitations of current cell therapies to provide new treatment alternatives for patients. To learn more about Gamida Cell, including current clinical studies, please visit gamida-cell.com and on [Twitter](#), [LinkedIn](#) and [Facebook](#).

###

Contact:

Melanie Higham
W2O Group
mhigham@w2ogroup.com
+1 617.315.1530

Investor Contact:

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