



Gamida Cell and the CIBMTR Announce Collaboration to Advance Research for Life-Saving Cellular Therapy

September 11, 2019

– Multi-Year Observational Study Designed to Evaluate Outcomes of Patients with Hematologic Malignancies Who Undergo Allogeneic Bone Marrow Transplantation –

BOSTON & MILWAUKEE--(BUSINESS WIRE)--Sep. 11, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, and the CIBMTR[®] (Center for International Blood and Marrow Transplant Research[®]) today announced the entry into a research agreement to collect and analyze health outcomes data in patients with hematologic malignancies who receive an allogeneic hematopoietic stem cell transplant (HSCT, or bone marrow transplant) from various donor sources. The CIBMTR is an organization that collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy worldwide.

The newly launched observational study by the CIBMTR and Gamida Cell will include both retrospective and prospective data contemporaneous to the international, randomized, Phase 3 study of omidubicel, Gamida Cell's investigational advanced cell therapy designed to enhance the life-saving benefits of bone marrow transplant. Topline data from the ongoing Phase 3 study is anticipated in the first half of 2020, and initial data from the multi-year observational study is anticipated next year. Omidubicel has not yet been approved for marketing in the United States or any other jurisdiction.

"We are pleased to announce this collaboration with Gamida Cell, a company aiming to make bone marrow transplant an option for more patients facing life-threatening blood diseases," said Mary M. Horowitz, M.D., M.S., chief scientific director of the CIBMTR. "This collaboration will leverage the CIBMTR's deep experience collecting and analyzing data on both the short- and long-term outcomes of patients undergoing a bone marrow transplant. We look forward to contributing to efforts to better understand real-world clinical outcomes."

"This agreement marks the beginning of Gamida Cell's health outcomes research initiatives, and we are excited to partner with the CIBMTR, an organization with deep expertise in bone marrow transplantation and cellular therapy," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We are committed to improving outcomes for patients who are in need of a bone marrow transplant and look forward to better understanding the variables that influence health outcomes, as well as elucidating how omidubicel may fit into the treatment landscape."

Randomized clinical trials comparing different donor types suggest that clinical outcomes may vary significantly depending on the donor type. The goal of this real-world, observational study is to better understand the variables that influence the health outcomes of patients receiving a transplant from a source other than a fully matched family donor. As part of the research agreement, the CIBMTR will use its registry, which consists of clinical outcomes data on more than 500,000 stem cell transplants, to analyze long-term safety and efficacy data for patients with hematologic malignancies who underwent a bone marrow transplant with an alternative donor source following myeloablative conditioning. The criteria for inclusion of patients and the endpoints evaluated in the analysis will be consistent with the design of the Phase 3 study of omidubicel.

About Omidubicel

Omidubicel (formerly known as NiCord[®]), the company's lead clinical program, is an advanced cell therapy under development as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplant solution for patients with hematologic malignancies (blood cancers). Omidubicel is the first bone marrow transplant product to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration and has also received Orphan Drug Designation in the U.S. and EU. In a Phase 1/2 clinical study, omidubicel demonstrated rapid and durable time to engraftment and was generally well-tolerated.¹ A Phase 3 study evaluating omidubicel in patients with leukemia and lymphoma is ongoing in the U.S., Europe and Asia.² Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.³ The aplastic anemia investigational new drug application is currently filed with the FDA under the brand name CordIn[®], which is the same investigational development candidate as omidubicel. For more information on clinical trials of omidubicel, please visit www.clinicaltrials.gov.

Omidubicel is an investigational therapy, and its safety and efficacy has not been evaluated by the U.S. Food and Drug Administration or any other health authority.

About Gamida Cell

Gamida Cell is a clinical-stage biopharmaceutical company committed to developing advanced cell therapies with the potential to cure blood cancers and rare, serious hematologic diseases. We are leveraging our proprietary nicotinamide-based, or NAM-based, cell expansion platform to develop product candidates designed to expand the possibility of cell therapy. For additional information, please visit www.gamida-cell.com.

About CIBMTR

The CIBMTR[®] (Center for International Blood and Marrow Transplant Research[®]) is a research collaboration between the National Marrow Donor Program[®] (NMDP)/Be The Match[®] and the Medical College of Wisconsin (MCW). Through a close partnership with [Be The Match BioTherapies[®]](http://www.bethematchbiotherapies.com), a subsidiary of NMDP/Be The Match, the organizations offer end-to-end

solutions for cell and gene therapy developers, including cell sourcing and collection, clinical trial services, supply chain and logistics, manufacturing and commercialization support, and outcomes management. The CIBMTR collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy worldwide to increase survival and enrich quality of life for patients. The CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of transplant centers, and a unique and extensive clinical outcomes database.

Cautionary Note Regarding Forward Looking Statements

This press release contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including with respect to Gamida Cell's collaboration with the CIBMTR and Gamida Cell's Phase 3 study, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the timing and outcome of such collaboration and Phase 3 study. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of Gamida Cell's public filing on Form 20-F, filed with the SEC on February 25, 2019, and other filings that Gamida Cell makes with the SEC from time to time (which are available at <http://www.sec.gov>), the events and circumstances discussed in such forward-looking statements may not occur, and Gamida Cell's actual results could differ materially and adversely from those anticipated or implied thereby. Any forward-looking statements speak only as of the date of this press release and are based on information available to Gamida Cell as of the date of this release.

References

¹Horwitz M.E., Wease S., Blackwell B., Valcarcel D. et al. Phase I/II study of stem-cell transplantation using a single cord blood unit expanded ex vivo with nicotinamide. *J Clin Oncol.* 2019 Feb 10;37(5):367-374.

²ClinicalTrials.gov identifier NCT02730299.

³ClinicalTrials.gov identifier NCT03173937.

⁴ClinicalTrials.gov identifier NCT03019666.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190911005036/en/>

Source: Gamida Cell

Gamida Cell Contacts:

Jaren Irene Madden

jaren@gamida-cell.com

617-892-9084

Krystle Gibbs (*media*)

krystle@tenbridgecommunications.com

508-479-6358

CIBMTR Contact:

Liz Siepmann

Communications Specialist

esiepmann@mcw.edu

(414) 805-0665