



Gamida Cell Announces Completion of Patient Enrollment in Ongoing Phase 3 Clinical Study of Omidubice

January 2, 2020

– Topline data expected in first half of 2020 –

BOSTON--(BUSINESS WIRE)--Jan. 2, 2020-- [Gamida Cell Ltd.](#) (Nasdaq: GMDA), an advanced cell therapy company committed to finding cures for blood cancers and serious blood diseases, announced that in December the company completed patient enrollment in its Phase 3 study of the company's lead clinical program, omidubice, an investigational advanced cell therapy being evaluated as a potential life-saving treatment option for patients with high-risk hematologic malignancies who are in need of a bone marrow transplant. Topline data from the study are expected in the first half of 2020.

"Completing patient recruitment for the Phase 3 study of omidubice is a very significant milestone for our company," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "Positive data would enable us to file our first biologics license application in the second half of 2020 and would represent an important step toward becoming a fully integrated company that can bring breakthrough medicines to patients."

It is estimated that more than 40 percent of eligible patients in the U.S. do not receive a bone marrow transplant for various reasons, including inability to find a matched donor, despite its curative potential.¹ Even for patients who do receive a transplant, the procedure is not always effective and can lead to serious complications that dramatically affect quality of life.² Omidubice is intended to address the current limitations of bone marrow transplant by providing a therapeutic dose of stem cells while preserving the cells' functional therapeutic characteristics.

"For many patients with high-risk hematologic malignancies who are in remission, their only hope of remaining cancer-free is to undergo a bone marrow transplant. While the scientific community has made strides in improving bone marrow transplant, there is still a significant need to make this potentially curative treatment option available to more patients," said Ronit Simantov, M.D., chief medical officer of Gamida Cell. "We truly appreciate the participation of patients and the support we have received from investigators who believe this clinical trial is critical for moving the field forward."

The international, multi-center, randomized Phase 3 study ([NCT02730299](#)) is designed to evaluate the safety and efficacy of omidubice compared to standard umbilical cord blood in patients with high-risk hematologic malignancies who need a bone marrow transplant and do not have an available matched donor. The primary endpoint is time to neutrophil engraftment. The study includes approximately 120 patients aged 12-65 with acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, myelodysplastic syndrome or lymphoma. The study is taking place at over 50 clinical centers in the U.S., Latin America, Europe and Asia.

About Omidubice

Omidubice (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). Omidubice 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, omidubice demonstrated rapid and durable time to engraftment and was generally well tolerated.³ A Phase 3 study evaluating omidubice in patients with leukemia and lymphoma is ongoing in the U.S., Latin America, Europe and Asia.⁴ Omidubice is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.⁵ 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 omidubice. For more information on clinical trials of omidubice, please visit www.clinicaltrials.gov.

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

About Gamida Cell

Gamida Cell is an advanced cell therapy company committed to finding cures for blood cancers and serious blood diseases. We harness our cell expansion platform to create therapies with the potential to redefine standards of care in areas of serious medical need. For additional information, please visit www.gamida-cell.com.

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 the patient enrollment in and timing of initiation and progress of and data reported from the clinical trials of Gamida Cell's product candidates, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope, progress and expansion of Gamida Cell's clinical trials and clinical, scientific, regulatory and technical developments. 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

¹U.S. Department of Health and Human Services: Health Resources and Services Administration. Bone Marrow and Cord Blood Donation and Transplantation. https://bloodcell.transplant.hrsa.gov/about/general_faqs/index.html. Last accessed December 16, 2019.

²Carreras et al. The EBMT Handbook. Springer 2019.

³Horwitz 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.

⁴ClinicalTrials.gov identifier NCT02730299.

⁵ClinicalTrials.gov identifier NCT03173937.

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