Gamida Cell Announces Positive Topline Data from Phase 3 Clinical Study of Omidubicel in Patients with High-Risk Hematologic Malignancies

May 12, 2020

— Study met primary endpoint of time to neutrophil engraftment —

— Omidubicel represents potential transformative treatment option for patients in need of a bone marrow transplant —

— Company anticipates initiating BLA submission in fourth quarter of 2020 —

— Company to host conference call today at 8:30 a.m. ET —

BOSTON--(BUSINESS WIRE)--May 12, 2020-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to finding cures for blood cancers and serious blood diseases, today announced positive topline results from its Phase 3 clinical study evaluating the safety and efficacy of omidubicel, an investigational advanced cell therapy in development as a potential life-saving treatment option for patients in need of bone marrow transplant. The median time to neutrophil engraftment was 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p<0.001$). Neutrophil engraftment is a measure of how quickly the stem cells a patient receives in a transplant are established and begin to make healthy new cells, and rapid neutrophil engraftment has been associated with fewer infections and shorter hospitalizations.[1]

Despite the curative potential of bone marrow transplant, it is estimated that more than 40 percent of eligible patients in the United States do not receive a transplant for various reasons, including the lack of a matched donor.[2] Even for patients who do receive a transplant, treatment is not always effective and can lead to serious complications that can dramatically affect their quality of life.[3] Omidubicel 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.

"I'm very encouraged by the data from this rigorous, Phase 3 study that was conducted at more than 50 centers around the world, as there is a significant need for new bone marrow transplant graft modalities," said Mitchell Horwitz, M.D., principal investigator and professor of medicine at the Duke Cancer Institute. "These results have the potential to substantially move the field forward and represent an important step toward making stem cell transplantation more accessible and more successful for patients with lethal blood cancers. Shortening the time to engraftment is clinically meaningful, as it can reduce a patient's time in the hospital and decrease likelihood of infection."

"Omidubicel is the first bone marrow transplant product to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration and has the potential to be the first FDA-approved bone marrow transplant graft. We are very pleased with the results of the Phase 3 data reported today, which move us one step closer toward bringing potentially curative therapies to patients. We expect to begin to submit our biologics license application for omidubicel to the FDA on a rolling basis in the fourth quarter of this year," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We deeply appreciate the participation of patients in this trial and the support we have received from investigators and their teams."

Topline Phase 3 Data

The international, multi-center, randomized Phase 3 study (NCT02730299) was designed to evaluate the safety and efficacy of omidubicel in patients with high-risk hematologic malignancies undergoing a bone marrow transplant compared to a comparator group of patients who received a standard umbilical cord blood transplant. The primary endpoint was time to neutrophil engraftment. The intent-to-treat analysis included 125 patients aged 12–65 years with acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, myelodysplastic syndrome or lymphoma and was conducted at more than 50 clinical centers in the United States, Latin America, Europe and Asia. The demographics and baseline characteristics were well-balanced across the two study groups.

The study achieved its primary endpoint ($p<0.001$). In the intent-to-treat analysis, the median time to neutrophil engraftment was significantly shorter for patients who received omidubicel (12 days; 95% CI: 10-15 days) compared to the comparator group (22 days; 95% CI: 19-25 days). Omidubicel was generally well tolerated. Among patients who were transplanted per protocol, 96 percent of patients who received omidubicel achieved successful neutrophil engraftment, compared to 88 percent of patients in the comparator group.

"We are pleased with the outcome of this global, well-designed study in patients with life-threatening blood cancers who were in need of a bone marrow transplant and did not have an available matched donor," said Ronit Simantov, M.D., chief medical officer of Gamida Cell. "Importantly, these data confirmed the results from our earlier Phase 1/2 clinical study and demonstrated that patients who received omidubicel had more rapid recovery of neutrophils, which are key infection-fighting white blood cells."

The data reported today are consistent with results from a multi-center, Phase 1/2 study in 36 patients with advanced hematologic malignancies, which showed that patients treated with omidubicel demonstrated more rapid neutrophil engraftment compared to a concurrent cohort of 146 patients treated with standard umbilical cord blood as reported by the Center for International Blood and Bone Marrow Transplant Research.[4] In the Phase 1/2 study, the median time to engraftment was 11.5 days (95% CI: 9-14 days) for omidubicel recipients compared to 21 days (95% CI: 20-23 days) for the CIBMTR cohort ($p<0.001$).

Gamida Cell expects to report full efficacy and safety results at a medical conference later this year.

Conference Call Information

Gamida Cell will host a conference call today, May 12, 2020, at 8:30 a.m. ET to discuss the Phase 3 study results. A live webcast of the conference

call can be accessed in the “Investors” section of Gamida Cell’s website at www.gamida-cell.com. To participate in the live call, please dial 1-866-930-5560 (domestic toll-free), 1-409-216-0605 (international) and refer to conference ID number 5454076. A recording of the webcast will be available approximately two hours after the event, for approximately 30 days.

About Omidubicel

Omidubicel, 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 both Phase 1/2 and Phase 3 clinical studies, omidubicel demonstrated rapid and durable time to engraftment and was generally well tolerated. Omidubicel 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 omidubicel. For more information on clinical trials of omidubicel, please visit www.clinicaltrials.gov.

Omidubicel 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 https://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 enrolment in and timing of initiation and progress of and data reported from the clinical trials of Gamida Cell’s product candidates, anticipated regulatory filings, and potential approval of product candidates by the U.S. Food and Drug Administration, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the ongoing global COVID-19 pandemic; the scope, progress and expansion of Gamida Cell’s clinical trials and variability, and ramifications for the cost thereof; 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 and other sections of Gamida Cell’s Annual Report on Form 20-F, filed with the Securities and Exchange Commission (SEC) on February 26, 2020, 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.

[1]Anand S., Thomas S., Hyslop T., Adcock J. et al. 2017. Transplantation of Ex Vivo Expanded Umbilical Cord Blood (NiCord) Decreases Early Infection and Hospitalization. *BBMT* 2017; 23:1151-7.

[2]U.S. Department of Health and Human Services: Health Resources and Services Administration. Bone Marrow and Cord Blood Donation and Transplantation.

[3]Carreras et al. The EBMT Handbook. Springer 2019.

[4]Horwitz M.E., Wease S., Blackwell B., Valcarcel D. et al. 2018. Phase I/II study of stem-cell transplantation using a single cord blood unit expanded ex vivo with nicotinamide. *Journal of Clinical Oncology*. DOI: 10.1200/JCO.18.00053

[5]www.clinicaltrials.gov, NCT03173937.

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