



Gamida Cell Presents Efficacy and Safety Results of Phase 3 Study of Omidubichel in Patients with Hematologic Malignancies at the 2021 TCT Meetings of ASTCT and CIBMTR

February 9, 2021

— *Omidubichel represents a potentially transformative cell therapy treatment option for patients in need of a bone marrow transplant* —

— *Newly presented data support clinical benefit demonstrated by primary and secondary endpoints* —

— *Company anticipates BLA submission in second half of 2021* —

BOSTON--(BUSINESS WIRE)--Feb. 9, 2021-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious hematologic diseases, today announced the results of a Phase 3 clinical study of omidubichel presented in an oral session at the Transplantation & Cellular Therapy Meetings of the American Society of Transplantation and Cellular Therapy (ASTCT) and Center for International Blood & Marrow Transplant Research (CIBMTR), or the TCT Meetings. Omidubichel is an advanced cell therapy under development as a potential life-saving allogeneic hematopoietic stem cell transplant solution for patients with hematologic malignancies.

This clinical data set was from the international, multi-center, randomized Phase 3 study of omidubichel that was designed to evaluate the safety and efficacy of omidubichel 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. This is the first presentation of these data in a peer-reviewed conference. [The full presentation is available on the Gamida Cell website.](#)

"The results of this global Phase 3 study of omidubichel in patients with hematologic malignancies show that omidubichel resulted in faster hematopoietic recovery, fewer bacterial and viral infections and fewer days in hospital, all of which are meaningful results and represent potentially important advancements in care when considering the patient experience following transplant," said Mitchell Horwitz, M.D., principal investigator and professor of medicine at the Duke Cancer Institute. "The comparator, a transplant with umbilical cord blood, has been historically shown to result in low incidence of graft versus host disease (GvHD) in relation to other graft sources, and in this study, omidubichel demonstrated a GvHD profile similar to the comparator. Moreover, previous studies have shown that engraftment with omidubichel is durable, with some patients in the Phase 1/2 study receiving their transplant more than 10 years ago. The data presented at this meeting indicate that omidubichel has the potential to be considered a new standard of care for patients who are in need of stem cell transplantation but do not have access to a matched donor."

Details of Phase 3 Efficacy and Safety Results Shared at the TCT Meetings

Patient demographics including racial and ethnic diversity and baseline characteristics were well-balanced across the two study groups. The study's intent-to-treat analysis included 125 patients aged 13–65 years with a median age of 41. Diseases included acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, myelodysplastic syndrome or lymphoma. Patients were enrolled at more than 30 clinical centers in the United States, Europe, Asia, and Latin America.

Gamida Cell previously reported in May 2020 that the study [achieved its primary endpoint](#), showing that omidubichel demonstrated a statistically significant reduction in time to neutrophil engraftment, a measure of how quickly the stem cells a patient receives in a transplant are established and begin to make healthy new cells, and a key milestone in a patient's recovery from a bone marrow transplant. The median time to neutrophil engraftment was 12 days for patients randomized to omidubichel compared to 22 days for the comparator group ($p < 0.001$).

All three secondary endpoints demonstrated a statistically significant improvement among patients who were randomized to omidubichel in relation to patients randomized to the comparator group (intent-to-treat). Platelet engraftment was significantly accelerated with omidubichel, with 55 percent of patients randomized to omidubichel achieving platelet engraftment at day 42, compared to 35 percent for the comparator ($p = 0.028$). The rate of infection was significantly reduced for patients randomized to omidubichel, with the cumulative incidence of first grade 2 or grade 3 bacterial or invasive fungal infection for patients randomized to omidubichel of 37 percent, compared to 57 percent for the comparator ($p = 0.027$). Hospitalization in the first 100 days after transplant was also reduced in patients randomized to omidubichel, with a median number of days alive and out of hospital for patients randomized to omidubichel of 60.5 days, compared to 48.0 days for the comparator ($p = 0.005$). The details of these [data were first reported](#) in December 2020.

Previously unpublished data from the study relating to exploratory endpoints also support the clinical benefit demonstrated by the study's primary and secondary endpoints. There was no statistically significant difference between the two patient groups related to grade 3/4 acute GvHD (14 percent for omidubichel, 21 percent for the comparator) or all grades chronic GvHD at one year (35 percent for omidubichel, 29 percent for the comparator). Non-relapse mortality was shown to be 11 percent for patients randomized to omidubichel and 24 percent for patients randomized to the comparator ($p = 0.09$).

These clinical data results will form the basis of a Biologics License Application (BLA) that Gamida Cell expects to submit to the U.S. Food and Drug Administration (FDA) in the second half of 2021.

"We believe that omidubichel has the potential to transform the field of hematopoietic bone marrow transplant by expanding access to this potentially curative cell therapy treatment for thousands of patients who are in need of a transplant but lack access to a matched related donor," said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "Sharing the results of the Phase 3 study of omidubichel with the transplant community is a major moment for Gamida Cell, and we are forever grateful to the patients who participated in this study, their caregivers, and the work of the investigators and their teams."

About Omidubicel

Omidubicel 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). In both Phase 1/2 and Phase 3 clinical studies (NCT01816230, NCT02730299), omidubicel demonstrated rapid and durable time to engraftment and was generally well tolerated.^{1,2} Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia (NCT03173937). 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 established by the FDA or any other health authority.

About Gamida Cell

Gamida Cell is an advanced cell therapy company committed to cures for patients with 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 or follow Gamida Cell on [LinkedIn](#) or Twitter at [@GamidaCellTX](#).

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 timing of anticipated regulatory submissions, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the progress and expansion of Gamida Cell's manufacturing capabilities and other commercialization efforts 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, its Report on Form 6-K filed with the SEC on August 12, 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 Horwitz 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.

² Gamida Cell press release, "Gamida Cell Announces Positive Topline Data from Phase 3 Clinical Study of Omidubicel in Patients with High-Risk Hematologic Malignancies," issued May 12, 2020. Last accessed August 31, 2020.

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