Gamida Cell Announces Publication in Blood, the Journal of the American Society of Hematology, of the First Pivotal Trial to Evaluate a Cell Therapy (Omidubicel) for Patients with Blood Cancer who Require an Allogeneic Stem Cell Transplant

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- Omidubicel is a first-in-class, NAM-enabled, advanced cell therapy being evaluated as a potential life-saving treatment for patients with blood cancers in need of an allogeneic hematopoietic stem cell (bone marrow) transplant
- The Phase 3 clinical trial achieved both primary and secondary endpoints
- Gamida Cell remains on track to submit a Biologics License Application for omidubicel in the fourth quarter of this year

BOSTON--(BUSINESS WIRE)--Jun. 23, 2021-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious hematologic diseases, today announced that the results of a Phase 3 clinical study of omidubicel have been published in *Blood*, the official journal of the American Society of Hematology. Omidubicel is an advanced cell therapy under development as a potential life-saving allogeneic hematopoietic stem cell transplant solution for patients with hematologic malignancies.

The results demonstrate that transplantation with omidubicel leads to faster neutrophil and platelet recovery compared to a standard umbilical cord blood graft, and results in fewer early bacterial and viral infections and less time in the hospital.

“We are pleased that the data from this well-conducted international Phase 3 trial have been published in *Blood*, the highly respected, peer-reviewed journal of the American Society of Hematology,” said Ronit Simantov, M.D., chief medical officer of Gamida Cell. “The robust results of this clinical trial have demonstrated that omidubicel could provide an important new option for patients with hematologic malignancies in need of a bone marrow transplant.”

Data from this study were previously presented at the Transplantation & Cellular Therapy Meetings of the American Society of Transplantation and Cellular Therapy and Center for International Blood & Marrow Transplant Research, and most recently during the Presidential Symposium at the 47th Annual Meeting of the European Society for Blood and Marrow Transplantation. The pivotal study was an international, multi-center, randomized Phase 3 trial designed to compare the safety and efficacy of omidubicel to standard umbilical cord blood transplant in patients with high-risk hematologic malignancies undergoing a bone marrow transplant.

“Previous studies have shown that engraftment with omidubicel is durable, with some patients in the Phase 1/2 study now a decade past their transplant. The Phase 3 data reinforce omidubicel’s potential to be a new standard of care for patients who are in need of stem cell transplantation but do not have access to an appropriate matched donor,” said Mitchell Horwitz, M.D., lead author of the paper and a professor of medicine at the Duke Cancer Institute.

The full *Blood* manuscript is available here: https://ashpublications.org/blood/article/doi/10.1182/blood.2021011719/476235/Omidubicel-Versus-Standard-Myeloablative-Umbilical.

Details of Phase 3 Efficacy and Safety Results Shared in *Blood*

The intent-to-treat analysis included 125 patients aged 13–65 years with a median age of 41. Forty-four percent of the patients treated on study were non-Caucasian, a population known to be underrepresented in adult bone marrow donor registries. Patient demographics and baseline characteristics were well-balanced across the two study groups. Patients with acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, myelodysplastic syndrome or lymphoma 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 omidubicel 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 omidubicel compared to 22 days for the comparator group ($p<0.001$).

All three secondary endpoints, details of which **were first reported** in December 2020, demonstrated a statistically significant improvement among patients who were randomized to omidubicel compared to patients randomized to standard cord blood graft. Platelet engraftment was significantly accelerated with omidubicel, with 55 percent of patients randomized to omidubicel achieving platelet engraftment at day 42, compared to 35 percent for the comparator ($p = 0.028$). Hospitalization in the first 100 days after transplant was also reduced in patients randomized to omidubicel, with a median number of days alive and out of hospital for patients randomized to omidubicel of 61 days, compared to 48 days for the comparator ($p=0.005$). The rate of infection was significantly reduced for patients randomized to omidubicel, with the cumulative incidence of first grade 2 or grade 3 bacterial or invasive fungal infection for patients randomized to omidubicel of 37 percent, compared to 57 percent for the comparator ($p=0.027$). Additional data reported in the manuscript included a comparison of infection density, or the number of infections during the first year following transplantation, which showed that the risk for grade 2 and grade 3 infections was significantly lower among recipients of omidubicel compared to control (risk ratio 0.5, $p<0.001$).

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 in incidence of grade 3/4 acute GvHD (14 percent for omidubicel, 21 percent for the comparator) or all grades chronic GvHD at one year (35 percent for omidubicel, 29 percent for the comparator). Non-relapse mortality

was shown to be 11 percent for patients randomized to omidubicel and 24 percent for patients randomized to the comparator (p=0.09).

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

About Omidubicel

Omidubicel is an advanced cell therapy under development as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplants for patients with hematologic malignancies (blood cancers), for which it has been granted Breakthrough Status by the FDA. 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 the potential for omidubicel to become a new standard of care and the anticipated submission of a BLA for omidubicel, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to Gamida Cell's ability to prepare regulatory filings and the review process therefor; complications in Gamida Cell's plans to manufacture its products for commercial distribution; 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 March 9, 2021, as amended on March 22, 2021, 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.

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