



Gamida Cell Announces Data for Omidubicel Presented at the International Society for Cellular and Gene Therapy 2019 Annual Meeting

May 31, 2019

BOSTON--(BUSINESS WIRE)--May 31, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, today announced that two presentations on omidubicel¹ (formerly known as NiCord[®]), an investigational advanced cell therapy in Phase 3 clinical development designed to enhance the life-saving benefits of hematopoietic stem cell (bone marrow) transplant, took place at the International Society for Cell and Gene Therapy (ISCT) 2019 Annual Meeting being held in Melbourne, Australia. The presentations included a summary of clinical and translational data from the completed Phase 1/2 clinical study of omidubicel in patients with high-risk hematologic malignancies, or blood cancers. Results from the Phase 1/2 study showed that patients transplanted with omidubicel had rapid and durable engraftment of neutrophils and platelets, as well as prompt immune reconstitution. An international, randomized Phase 3 study of omidubicel in patients with hematologic malignancies is currently ongoing.²

"In the Phase 1/2 clinical study, patients who received omidubicel had a clinically meaningful reduction in their time to neutrophil and platelet recovery compared to a real-world cohort of patients who received a standard umbilical cord blood transplant. The neutrophil recovery observed with omidubicel also resulted in fewer days spent in the hospital compared to the comparator cohort," said Joanne Kurtzberg, M.D., Director of the Marcus Center for Cellular Cures and the Carolinas Cord Blood Bank at Duke University Medical Center. "These data suggest an important potential step toward making stem cell transplantation safer and more accessible to patients with lethal blood cancers, and I am pleased to be participating in the Phase 3 study currently enrolling patients."

Despite the curative potential of bone marrow transplants, it is estimated that more than 40 percent of eligible patients in the U.S. do not receive one for various reasons, including finding a matched donor.³ Even for patients who do receive a transplant, treatment is not always effective and can lead to serious complications that can dramatically affect quality of life.⁴ Omidubicel is intended to address the current limitations of bone marrow transplant by providing a therapeutic dose of cells while preserving the cells' functional therapeutic characteristics.

"At Gamida Cell, our aspiration is to bring the first FDA-approved cell therapy for bone marrow transplantation to patients," stated Ronit Simantov, M.D., chief medical officer at Gamida Cell. "These data demonstrate the potential of omidubicel to give patients with high-risk blood cancers, particularly those who would not otherwise receive a bone marrow transplant from a matched donor, an opportunity for a cure."

Data Presented at ISCT 2019 Annual Meeting

Phase 1/2 Clinical Data

The presentation, "NiCord, an Expanded Cord Blood Product, Accelerates Engraftment After Myeloablative Conditioning," described results from the completed multicenter, Phase 1/2 clinical trial of omidubicel in 36 patients with high-risk hematologic malignancies and no readily available matched sibling or matched unrelated adult donor. The key primary endpoint was the cumulative incidence of neutrophil engraftment at 42 days. Additionally, the omidubicel patient cohort was compared to a retrospective cohort of patients who received standard cord blood transplant using data from the Center for International Blood and Marrow Transplant Research (CIBMTR).

Data from the study demonstrated that patients transplanted with omidubicel had rapid and durable engraftment of neutrophils and platelets. The age-adjusted cumulative incidence of neutrophil engraftment at 42 days following transplantation was 94 percent for omidubicel recipients compared to 85 percent for the CIBMTR cohort. Among patients who engrafted, the median time to neutrophil recovery was 11.5 days (95 percent confidence interval (CI): 9-14 days) for omidubicel recipients compared to 21 days (95 percent CI: 20-23 days) for the CIBMTR cohort ($p < 0.001$). For patients achieving platelet recovery, the median time to platelet recovery was 34 days (95 percent CI: 32-42 days) and 46 days (95 percent CI: 42-50 days) for the omidubicel and CIBMTR cohorts, respectively ($p < 0.001$). Omidubicel demonstrated an acceptable safety profile, with hypertension reported as the most common adverse event attributable to omidubicel infusion, and moderate to severe chronic graft vs. host disease reported in 9.8 percent of patients at one year following transplantation. Primary hospital discharge occurred at a median of 20 days following transplantation. Omidubicel recipients spent a median of 73 days alive and out of hospital during the first 100 days following transplantation.

Phase 1/2 Translational Data

The presentation, "Rapid and Robust CD4+ and CD8+ T-, NK-, B- and Monocyte Reconstitution after Nicotinamide-Expanded Cord Blood Transplantation," described in-depth immune reconstitution data from the completed Phase 1/2 clinical study of omidubicel. Immune reconstitution for 27 patients receiving omidubicel was compared to retrospective cohorts of adolescent and young adults with hematologic malignancies receiving unmanipulated cord blood transplantation (unCBT, $n = 27$) or unrelated bone marrow transplantation (BMT, $n = 20$). The primary endpoint was the probability of achieving CD4+ immune reconstitution ($> 50 \times 10^6/L$) within the first 100 days. Secondary endpoints included the recovery of B cells, CD4+ T cells and natural killer (NK) cells during the first year after transplantation. Analyses were performed at the University Medical Centre Utrecht, Laboratory of Translational Immunology.

The analysis showed that 91 percent of patients receiving omidubicel achieved successful immune reconstitution of CD4+ T cells at 100 days after transplantation. Reconstitution of T cells in the omidubicel group (median age 41.5 years) was similar to the unCBT and BMT cohorts (median age 15.4 and 14.3 years, respectively), despite the younger age of the cohorts, who would be expected to reconstitute faster. In addition, reconstitution of a number of cell types, including B cells ($p = 0.026$) and NK cells ($p < 0.001$), was significantly faster after transplantation with omidubicel compared to the cohorts, and suggests that omidubicel reconstitutes diverse functions of the immune system.

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).¹ 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.⁵ 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.⁶ 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 technology to develop product candidates designed to address the limitations of cell therapies. 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 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 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 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.

¹ Gamida Cell's lead development candidate consists of omidubicel (expanded hematopoietic stem cells) and differentiated immune cells, including T cells. Gamida Cell refers to the two components collectively as "omidubicel."

²ClinicalTrials.gov identifier NCT02730299.

³U.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.

⁴ Carreras et al. The EBMT Handbook. Springer 2019.

⁵ 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.

⁶ ClinicalTrials.gov identifier NCT03173937.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190531005088/en/>

Source: Gamida Cell Ltd.

Jaren Irene Madden
jaren@gamida-cell.com
617-892-9084

Media Inquiries

Krystle Gibbs
krystle@tenbridgecommunications.com
508-479-6358