



## **Gamida Cell Announces Positive Topline Data on Secondary Endpoints from Phase 3 Clinical Study of Omidubichel in Patients with Hematologic Malignancies**

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— Study met secondary endpoints related to platelet engraftment, infections and hospitalizations, key clinical measures in bone marrow transplant —

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

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

BOSTON--(BUSINESS WIRE)--Oct. 6, 2020-- [Gamida Cell Ltd.](#) (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious blood diseases, today announced that the Phase 3 study of omidubichel, an investigational advanced cell therapy in development as a potential life-saving treatment option for patients in need of bone marrow transplant, met all three of its secondary endpoints. Omidubichel 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 engineered bone marrow transplant graft.

The international, multi-center, randomized Phase 3 study was designed to evaluate the safety and efficacy of omidubichel in patients with hematologic malignancies undergoing a bone marrow transplant compared to a comparator group of patients who received a standard umbilical cord blood transplant. In [May](#), Gamida Cell reported that omidubichel achieved its primary endpoint, demonstrating a highly statistically significant reduction in time to neutrophil engraftment, a key milestone in recovery from a bone marrow transplant. The prespecified secondary endpoints of the study, analyzed in all randomized patients (intent-to-treat), were the proportion of patients who achieved platelet engraftment by day 42, the proportion of patients with Grade 2 or Grade 3 bacterial or invasive fungal infections in the first 100 days following transplant, and the number of days alive and out of the hospital in the first 100 days following transplant. All three secondary endpoints demonstrated a statistically significant improvement among patients who received omidubichel compared to the comparator group. The company anticipates reporting the full data set at a medical meeting in the fourth quarter of 2020.

"These data, obtained in a global, randomized, multi-institutional setting could represent an important step forward in the field. In addition to more rapid platelet engraftment, a key step toward recovery, reducing infections and hospitalizations are considered meaningful patient outcomes and have the potential to provide substantial value for patients, their families and the healthcare system," said Mitchell Horwitz, M.D., principal investigator and professor of medicine at the Duke Cancer Institute. "The totality of these data strengthen my belief that omidubichel has the potential to be a graft source for any patient who does not have access to a matched related donor and could help make stem cell transplantation more accessible and more successful for patients with lethal blood cancers."

"These additional data reinforce the potential of omidubichel and move us another step closer toward bringing potentially curative therapies to patients. We look forward to presenting data at a future medical meeting, and we are continuing our work to enable the submission of our biologics license application for omidubichel to the FDA on a rolling basis, both expected in the fourth quarter," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We deeply appreciate the patients who participated in this study, the incredible encouragement from their caregivers and the support we have received from investigators and their teams."

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.<sup>1</sup> 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.<sup>2</sup> Omidubichel 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.

### **About Omidubichel**

Omidubichel 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), omidubichel demonstrated rapid and durable time to engraftment and was generally well tolerated.<sup>3,4</sup> Omidubichel 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<sup>®</sup>, which is the same investigational development candidate as omidubichel. For more information on clinical trials of omidubichel, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

*Omidubichel 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 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](http://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 anticipated timing of the presentation of clinical data and regulatory filing submissions for omidubichel, which statements are subject to a

number of risks, uncertainties and assumptions, including, but not limited to the ongoing global COVID-19 pandemic 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.

<sup>1</sup>U.S. Department of Health and Human Services: Health Resources and Services Administration. Bone Marrow and Cord Blood Donation and Transplantation.

<sup>2</sup>Carreras et al. The EBMT Handbook. Springer 2019.

<sup>3</sup>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.

<sup>4</sup>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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