Gamida Cell Announces Publication of Phase 1/2 Clinical Data of NiCord® in the Journal of Clinical Oncology

December 4, 2018

BOSTON--(BUSINESS WIRE)--Dec. 4, 2018-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, today announced the publication of data from the previously reported, multi-center Phase 1/2 clinical study evaluating the safety and efficacy of NiCord® as a stand-alone, hematopoietic stem cell (bone marrow) transplant in the *Journal of Clinical Oncology*[1,2]. NiCord is an investigational product candidate in Phase 3 development as a universal bone marrow transplant solution for patients with high-risk hematologic malignancies, or blood cancers.

Results from the Phase 1/2 study showed that patients transplanted with NiCord had rapid and durable engraftment of neutrophils and platelets, as well as prompt immune reconstitution. The median time to neutrophil recovery was shortened by nearly 50 percent for patients who received NiCord compared to a retrospective cohort of patients who received standard umbilical cord blood. NiCord also demonstrated an acceptable safety profile for patients undergoing bone marrow transplant.

“In this study, patients who received NiCord had a clinically meaningful reduction in their time to neutrophil and platelet recovery compared to a retrospective cohort of patients who received a standard umbilical cord blood transplant. The neutrophil recovery observed with NiCord also resulted in fewer days spent in the hospital compared to the comparator cohort,” said Mitchell Horwitz, M.D., principal investigator and professor of medicine at the Duke Cancer Institute. “These data suggest a potential step toward making stem cell transplantation safer and more accessible to patients with lethal blood cancers.”

Despite the curative potential of bone marrow transplants, it is estimated that more than 40 percent of eligible patients do not receive one for various reasons, including finding a matched donor.[3] While umbilical cord blood provides a source of stem cells for patients who do not have a matched related donor, it provides a smaller number of stem cells, which can delay engraftment and put patients at a greater risk for prolonged hospitalizations and life-threatening infections. NiCord is designed to address these limitations by offering a therapeutic dose of expanded cells while preserving the functional characteristics of stem cells.

NiCord Phase 1/2 Study Design and Results
The publication, “Phase I/II study of stem cell transplantation using a single cord blood unit expanded ex vivo with nicotinamide,” described results from the completed multicenter, Phase 1/2 clinical trial of NiCord 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 NiCord 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).

Key findings included the following:

- Patients transplanted with NiCord had rapid and durable engraftment of neutrophils and platelets, as well as prompt immune reconstitution. The age-adjusted cumulative incidence of neutrophil engraftment at 42 days following transplantation was 94 percent for NiCord recipients compared to 85 percent for the CIBMTR cohort.
- Among patients who engrafted, the median time to neutrophil recovery was 11.5 days (95% CI: 9-14 days) for NiCord recipients compared to 21 days (95% 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% CI:32-42 days) and 46 days (95% CI:42-50 days) for the NiCord and CIBMTR cohorts, respectively ($p<0.001$).
- NiCord demonstrated an acceptable safety profile, with hypertensionreported as the most common adverse event attributable to NiCord 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. NiCord recipients spent a median of 73 days alive and out of hospital during the first 100 days following UCB transplantation.

“These data demonstrate the potential of NiCord to give patients with high-risk blood cancers an opportunity for a cure, particularly patients who would otherwise not be able to receive a bone marrow transplant using a matched donor source,” stated Ronit Simantov, M.D., chief medical officer at Gamida Cell. “We are actively enrolling patients in our Phase 3 study, which is designed to confirm the potential of NiCord to be an effective transplantation solution. We look forward to completing patient enrollment expected in the second half of 2019.”

About NiCord
NiCord, the company’s lead clinical program, is under development as a universal bone marrow transplant solution for patients with high-risk hematologic malignancies. NiCord has been granted breakthrough status by the U.S. Food and Drug Administration, making it the first bone marrow transplant alternative to receive this designation. It has also received U.S. and EU orphan drug designation. A Phase 3 study evaluating NiCord in patients with leukemia and lymphoma is ongoing in the United States, Europe and Asia.[4] For more information on NiCord clinical trials, please visit www.clinicaltrials.gov.

About Gamida Cell
Gamida Cell is a clinical stage biopharmaceutical company leveraging its proprietary technology to develop cell therapies that are designed to cure

cancer and rare, serious hematologic diseases. The company is leveraging its nicotinamide-, or NAM-, based cell expansion technology to develop a pipeline of products designed to address the limitations of cell therapies.

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 timing of completion of enrollment of the ongoing Phase 3 clinical study of NiCord and the results of clinical development of NiCord, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the timing and success of clinical trials and potential complications thereof and the scope, progress and expansion of developing and commercializing NiCord. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of our Registration Statement on Form F-1 filed with the SEC on September 28, 2018, 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.

References

[1]ClinicalTrials.gov identifier NCT01816230.

[2] 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

[3]U.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. Last accessed November 27, 2018.

[4]ClinicalTrials.gov identifier NCT02730299.

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