Gamida Cell Reports Immune Reconstitution Data from Completed Phase 1/2 Clinical Study of NiCord® Presented at ASH 2018 Annual Meeting

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– NiCord Recipients Demonstrated Rapid and Robust Immune Reconstitution following Transplantation –

SAN DIEGO--(BUSINESS WIRE)--Dec. 3, 2018-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, today reported translational data showing that recipients who received NiCord®, an investigational cell therapy in Phase 3 clinical development for allogeneic hematopoietic stem cell (bone marrow) transplant had rapid and robust reconstitution of key immune cells. Successful immune reconstitution is an important factor in the recovery of patients undergoing bone marrow transplant. These translational data from the completed Phase 1/2 study of NiCord were presented in a poster session during the American Society of Hematology (ASH) 2018 Annual Meeting being held December 1-4 in San Diego, CA.[1]

"Immune reconstitution following transplantation is critical for disease and viral control, but historically cord blood transplantation has had limitations in timely immune reconstitution in patients," said Jaap-Jan Boelens, M.D., Ph.D., Chief, Pediatric Stem Cell Transplantation and Cellular Therapies Service, Memorial Sloan Kettering Cancer Center. "We were pleased to see that NiCord treatment resulted in rapid and robust immune reconstitution when compared to younger patients who typically achieve more rapid recovery than adults."

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.[2] 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.

Data Presented at ASH 2018
The poster presentation, "Rapid and robust CD4+ and CD8+ T-, NK-, B- and monocyte cell reconstitution after nicotinamide-expanded cord blood transplantation" (Abstract 2123), described early, in-depth immune reconstitution data from the completed Phase 1/2, multicenter clinical study of NiCord as a stand-alone graft after myeloablative therapy in patients with high-risk hematologic malignancies.[3] A random subgroup of 27 patients from this study had extensive immune monitoring evaluated throughout the first year after transplant. The primary endpoint was the probability of achieving CD4+ immune reconstitution (>50×106/L) within the first 100 days, and the secondary endpoints included the recovery of B cells, CD4+ T cells and natural killer (NK) cells during the first year after transplantation. These data were compared to cohorts of adolescent and young adults with hematologic malignancies receiving unmanipulated cord blood transplantation (n=27) or unrelated bone marrow transplantation (n=20). Analyses were performed at the University Medical CentreUtrecht, Laboratory of Translational Immunology.

Key findings from the analysis include the following:

- 91 percent of patients achieved successful immune reconstitution of CD4+ T cells at 100 days after transplantation with NiCord.
- Immune reconstitution of T cells was similar in the NiCord group (median age: 41.5 years) compared to the younger cohorts receiving unmanipulated cord blood and unrelated bone marrow (median ages 15.4 and 14.3 years, respectively).
- Immune reconstitution of B cells ($p = 0.02$) and NK cells ($p < 0.001$) was significantly faster after transplantation with NiCord compared to the other groups.
- Immune reconstitution after NiCord transplantation was associated with recovery of a broad spectrum of T cell, B cell and NK cell subsets representing a range of effector functions similar to that observed with other graft sources.

"These data, combined with the clinical data from our Phase 1/2 study of NiCord in patients with high-risk blood cancers, suggest that NiCord has the potential to be an important treatment option for patients undergoing bone marrow transplant," stated Ronit Simantov, M.D., chief medical officer at Gamida Cell. "We are working to advance our NiCord clinical development program and expect to complete patient enrollment in our ongoing Phase 3 study 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 patient enrollment in Gamida Cell's ongoing Phase 3 study of NiCord, 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 study. 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]de Koning C., Horwitz M.E., Sanz G., Jagasia M. et al. Rapid and robust CD4+ and CD8+ T-, NK-, B- and monocyte cell reconstitution after nicotinamide-expanded cord blood transplantation. Presented at the American Society of Hematology 2018 Annual Meeting.

[2]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.

[3]ClinicalTrials.gov identifier NCT01816230.

[4]ClinicalTrials.gov identifier NCT02730299.

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