



Gamida Cell Announces Immune Reconstitution Data from Completed Phase 1/2 Clinical Study of NiCord® Presented at 2019 TCT Annual Meeting

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– Data support ongoing Phase 3 study of NiCord in patients with hematologic malignancies –

HOUSTON--(BUSINESS WIRE)--Feb. 23, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, today announced that translational data from the completed Phase 1/2 clinical study of NiCord® were reported in an oral presentation at the 2019 Transplantation & Cellular Therapy (TCT) Meetings of American Society for Blood and Marrow Transplantation (ASBMT) and Center for International Blood and Marrow Transplant Research (CIBMTR) in Houston, Texas. The data demonstrated that treatment with NiCord, an investigational advanced cell therapy designed to enhance and expand the life-saving benefits of bone marrow transplant for patients with hematologic malignancies, resulted in rapid and robust immune reconstitution. NiCord is currently being evaluated in an international, randomized Phase 3 study in patients with hematologic malignancies.¹

“Reconstitution of a patient’s bone marrow and immune system is a crucial factor in recovery following allogeneic hematopoietic stem cell transplant,” said Jaap-Jan Boelens, M.D., Ph.D., Chief, Pediatric Stem Cell Transplantation and Cellular Therapies Service, Memorial Sloan Kettering Cancer Center. “We were particularly encouraged by the finding that reconstitution of CD4+ T cells with NiCord treatment was at least as fast as transplant with unmanipulated cord blood and unrelated bone marrow in adolescents and young adults, 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 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.³ NiCord 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.

Data Presented at TCT Annual Meeting

The oral presentation, “Rapid and Robust CD4+ and CD8+ T-, NK-, B-Cell, Dendritic Cell, and Monocyte Reconstitution after Nicotinamide-Expanded Cord Blood Transplantation” (Abstract 69), described in-depth immune reconstitution data from the completed Phase 1/2, multi-center clinical study of NiCord as a stand-alone graft after myeloablative therapy in patients with high-risk hematologic malignancies.⁴ Immune reconstitution for 27 patients receiving NiCord 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 (>50×10⁶/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 NiCord achieved successful immune reconstitution of CD4+ T cells at 100 days after transplantation. Reconstitution of T cells in the NiCord 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 = 0.02) and NK cells (p < 0.001), was significantly faster after transplantation with NiCord compared to the cohorts, and suggests that NiCord reconstitutes diverse functions of the immune system. These findings may be explained by the higher stem cell dose and proliferative capacity of NiCord.

“Our goal is to bring a potentially transformative new treatment option to patients in need of bone marrow transplant, and these data further reinforce our belief in the clinical potential of NiCord,” stated Ronit Simantov, M.D., chief medical officer at Gamida Cell. “We are continuing to enroll patients in our ongoing Phase 3 study and expect to complete patient enrollment in the second half of 2019, followed by an anticipated topline data readout in the first half of 2020.”

During the TCT Annual Meeting, new data were also presented from Gamida Cell’s NAM-NK clinical program, as well as initial data from a Phase 1/2 study of NiCord in patients with severe aplastic anemia. More information on those presentations can be found [here](#).

About NiCord

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). NiCord 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, NiCord demonstrated rapid and durable time to engraftment and was generally well-tolerated.⁵ A Phase 3 study evaluating NiCord in patients with leukemia and lymphoma is ongoing in the U.S., Europe and Asia.¹ NiCord 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 NiCord. For more information on clinical trials of NiCord, please visit www.clinicaltrials.gov.

NAM-NK and NiCord are investigational therapies, and their 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 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 translational data from the completed Phase 1/2 study of NiCord for the treatment of hematologic malignancies and the ongoing Phase 3 study of NiCord in patients with leukemia and lymphoma, 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 studies and clinical, scientific and technical developments. 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.

¹ClinicalTrials.gov identifier NCT02730299.

²U.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.

³ Carreras et al. The EBMT Handbook. Springer 2019.

⁴ ClinicalTrials.gov identifier NCT01816230. Last accessed February 20, 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.

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Source: Gamida Cell Ltd.

Jaren Irene Madden
jaren@gamida-cell.com
617-286-6264

Media Inquiries

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