



## **Gamida Cell Presents New Data from Ongoing Phase 1 Study of NAM-NK and Initial Data from Phase 1/2 Study of NiCord® in Severe Aplastic Anemia at 2019 TCT Annual Meeting**

February 21, 2019

- *NAM-NK therapy clinically active and generally well tolerated, with multiple complete responses observed* –
- *Data in severe aplastic anemia support potential of NiCord to treat non-malignant bone marrow failure disorders* –
- *Gamida Cell to host conference call today at 8:00 a.m. ET* –

HOUSTON--(BUSINESS WIRE)--Feb. 21, 2019-- Gamida Cell Ltd. (Nasdaq:GMDA), a leading cellular and immune therapeutics company, today announced that new data from its NAM-NK and NiCord® programs was presented at the 2019 Transplantation & Cellular Therapy (TCT) Meetings of American Society for Blood and Marrow Transplantation and Center for International Blood and Marrow Transplant Research taking place in Houston, Texas. Data reported from the first 14 patients in the ongoing Phase 1 study of NAM-NK, an investigational, cell-based cancer immunotherapy, in patients with non-Hodgkin lymphoma (NHL) and multiple myeloma (MM) demonstrated that NAM-NK was highly active, with three complete responses observed in patients with NHL and one complete response in a patient with MM. These data, along with additional safety data showing that NAM-NK was generally well tolerated, support continued clinical development. Gamida Cell is planning to initiate a multi-center, Phase 1/2 clinical study of NAM-NK in 2020. NAM-NK cells are natural killer cells that have been expanded using Gamida Cell's proprietary nicotinamide-based, or NAM, technology.

"I am encouraged by the emerging clinical profile of NAM-NK, and it is particularly exciting to witness complete responses in this heavily-pretreated patient population. Following treatment, two of the patients who were in complete remission received a bone marrow transplant, which has curative potential," stated M Health Hematologist/Oncologist Veronika Bachanova, M.D., Ph.D., Associate Professor of Medicine, Section Head for Malignant Hematology in the Division of Hematology, Oncology and Transplantation, University of Minnesota Medical School and Masonic Cancer Center member. "I look forward to continuing to evaluate the potential of NAM-NK as the study progresses."

Additionally, data were reported from the ongoing Phase 1/2 study of NiCord, an investigational advanced cell therapy designed to enhance and expand the life-saving benefits of hematopoietic stem cell (bone marrow) transplant, in patients with severe aplastic anemia. In the initial cohort of three patients, all three successfully underwent a stem cell transplant consisting of NiCord plus a haploidentical stem cell graft. The rapid engraftment, sustained hematopoiesis and accelerated immune recovery observed in these patients enable the initiation of a second cohort of patients to be treated with NiCord as a stand-alone graft.

"Data from our NiCord and NAM-NK programs continue to demonstrate the transformative potential of our proprietary nicotinamide-, or NAM-based, cell expansion technology. We are pleased to be at the forefront of exploring NK-therapy, which we believe has potential to advance treatment paradigms for patients just as CAR T therapy provided ground-breaking treatment options for patients," stated Julian Adams, Ph.D., chief executive officer at Gamida Cell. "We are also encouraged by the initial NiCord data in severe aplastic anemia, which supports further exploring a reduced intensity regimen and highlights the potential of NiCord as a bone marrow transplant solution not only for patients with hematologic malignancies but also for patients with severe bone marrow failure disorders. We look forward to continued progress with both programs throughout 2019."

### **NAM-NK Data in Patients with NHL and MM**

The safety and activity of NAM-NK is currently being evaluated in a Phase 1 dose-escalation study. Patients received rituximab (NHL patients) or elotuzumab (MM patients) prior to and after NAM-NK infusion. The presentation, "First-in-Human Phase I Study of Nicotinamide-Expanded Related Donor Natural Killer Cells for the Treatment of Relapsed/Refractory Non-Hodgkin Lymphoma and Multiple Myeloma" (Poster #242), included six patients with NHL and eight patients with MM. All 14 patients were evaluable for safety, and 12 of 14 patients were evaluable for activity (all six NHL patients and six of eight MM patients). The majority of patients were heavily pre-treated and had advanced disease.

Among the six NHL patients, three patients achieved a complete response, one patient achieved a partial response, and two patients experienced progressive disease. Two of the patients who achieved a complete response subsequently received a bone marrow transplant. Among the six MM patients evaluable for activity, one patient achieved a complete response, two patients experienced stable disease, and three patients experienced progressive disease. Activity was observed at all three dose levels evaluated.

NAM-NK was generally well tolerated, with no graft vs. host disease (GvHD), no tumor lysis syndrome and no neurotoxicity syndrome observed. Grade 3 (n = 3) and Grade 4 (n = 1) hematologic adverse events were observed. Non-hematologic adverse events were mostly Grade 1 and Grade 2. There was one case of Grade 3 cytokine release syndrome and one death due to sepsis.

### **NiCord Data in Patients with Severe Aplastic Anemia**

The safety and activity of NiCord in patients with severe aplastic anemia is being evaluated in an ongoing Phase 1/2 study. The presentation, "Ex Vivo Nicotinamide-Expanded (NAM-Expanded) Unrelated Cord Blood Transplantation (UCB) for Refractory Severe Aplastic Anemia Results in Rapid Engraftment and Expedites Immune Recovery" (Poster #295), included data from three severe aplastic anemia patients with severe neutropenia who failed immunosuppressive therapy.

All three patients enrolled in the first cohort were successfully treated with reduced intensity conditioning regimens and underwent a bone marrow transplant consisting of NiCord plus a haploidentical stem cell graft. Engraftment occurred rapidly, with a median neutrophil recovery of 6 days (range: 6-7 days), which was sustained at day 100, and was superior to that observed in a retrospective cohort of 16 patients who received a single

unexpanded umbilical cord blood transplant and haploidentical cells using the same conditioning regimen (P = 0.006). At median follow-up of 11 months (range 4-18 months), all three patients who received NiCord were alive and GvHD-free.

### Conference Call Information

Gamida Cell will host a conference call and webcast today, Thursday, February 21, 2019, at 8:00 a.m. ET to review the data from its NAM-NK and NiCord programs that are being presented at the 2019 TCT Annual Meeting. A live webcast of the conference call can be accessed in the Investors section of Gamida Cell's website at <https://investors.gamida-cell.com>. To participate in the conference call, please dial 1-866-930-5560 (domestic) or 1-409-216-0605 (international) five minutes prior to start time. The conference ID number is 9462948. An archived version of the webcast will be available on Gamida Cell's website for 30 days.

### About NAM-NK

Gamida Cell applied the capabilities of its NAM-based cell expansion technology to highly functional NK cells to develop NAM-NK, an innate immunotherapy for the treatment of hematologic and solid tumors in combination with standard of care antibody therapies. NAM-NK addresses key limitations of NK cells by increasing the cytotoxicity and in vivo retention and proliferation in the bone marrow and lymphoid organs of NK cells expanded in culture. NAM-NK is in Phase 1 development through an investigator-sponsored study in patients with refractory non-Hodgkin lymphoma and multiple myeloma.<sup>1</sup>

### 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.<sup>2</sup> A Phase 3 study evaluating NiCord in patients with leukemia and lymphoma is ongoing in the U.S., Europe and Asia.<sup>3</sup> NiCord is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.<sup>4</sup> 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 NiCord. For more information on clinical trials of NiCord, please visit [www.clinicaltrials.gov](http://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](http://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 initiation and timing of a Phase 1/2 study of NAM-NK and expansion of the Phase 1/2 study of NiCord for the treatment of severe aplastic anemia, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the scope and progress of Gamida Cell's studies. 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.

<sup>1</sup> ClinicalTrials.gov identifier NCT03019666.

<sup>2</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>3</sup> ClinicalTrials.gov identifier NCT02730299.

<sup>4</sup> ClinicalTrials.gov identifier NCT03173937.

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