



Gamida Cell Announces Results from Phase 1 Study of GDA-201 and New Mechanism of Action Data at ASH 2019 Annual Meeting

December 9, 2019

—GDA-201 demonstrated early evidence of clinical activity in patients with non-Hodgkin lymphoma, with multiple complete responses observed —

— Research on mechanism of action for the NAM technology platform provides further scientific rationale for stem cell engraftment and patient outcomes reported in previous Phase 1/2 clinical study of omidubicel —

BOSTON--(BUSINESS WIRE)--Dec. 9, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to finding cures for blood cancers and serious blood diseases, today announced updated results from a Phase 1 clinical study of GDA-201, an investigational, natural killer (NK) cell-based cancer immunotherapy for the treatment of patients with non-Hodgkin lymphoma (NHL) and multiple myeloma (MM), at the 61st Annual Meeting of the American Society of Hematology (ASH), which is being held December 7–10 in Orlando, FL. Data from 22 patients in the ongoing study showed GDA-201 in combination with monoclonal antibodies was generally well tolerated and demonstrated early evidence of clinical activity in heavily pre-treated patients, including five complete responses observed among nine patients with NHL. Gamida Cell plans to initiate a Phase 1/2 multi-dose, multi-center study of GDA-201 in patients with NHL in 2020.

“NK cells are increasingly recognized as a potential breakthrough approach in immunotherapy, and the data reported today provide early evidence that GDA-201 has the potential to be an important new treatment option,” said Veronica Bachanova, M.D., Ph.D., Associate Professor of Medicine in the Division of Hematology, Oncology and Transplantation at the University of Minnesota and principal investigator of the study through the Masonic Cancer Center. “Given the population of heavily pre-treated patients with advanced disease, it’s particularly encouraging to witness multiple complete responses. I look forward to the continued development of this investigational therapy.”

New research was also presented today on the mechanism of action of Gamida Cell’s NAM-based cell expansion platform, which is designed to enhance the number and functionality of allogeneic donor cells. These data provide further scientific rationale for the favorable stem cell engraftment and patient outcomes observed in the [Phase 1/2 clinical study](#) of omidubicel, the company’s advanced cell therapy currently in Phase 3 clinical development as a potential life-saving treatment option for patients in need of an allogeneic bone marrow transplant.

“These mechanism of action data reinforce the transformative potential of our NAM therapeutic platform, which can be used to expand multiple cell types. Specifically for omidubicel, this research suggests that NAM modulates certain gene expression pathways that, collectively, mimic the hypoxic environment of the bone marrow to help preserve stem cell function and long-term engraftment ability,” said Tracey Lodie, Ph.D., chief scientific officer of Gamida Cell. “We expect to build on our findings by characterizing the metabolites produced when we expand stem cells to make omidubicel, and we are also beginning to conduct similar mechanism of action studies with GDA-201.”

GDA-201 Phase 1 Clinical Data Presented at ASH

The oral presentation, “Results of a Phase 1 Trial of GDA-201, Nicotinamide-Expanded Allogeneic Natural Killer Cells (NAM-NK) in Patients with Refractory Non-Hodgkin Lymphoma (NHL) and Multiple Myeloma (MM)” (Abstract #777), described data from the Phase 1 clinical study of GDA-201 in heavily pre-treated patients with advanced NHL and MM. Twenty-two patients were enrolled in the study, including nine patients with NHL and 13 patients with MM. Of these 22 patients, all were evaluable for safety and 21 were evaluable for response (NHL = 9; MM = 12).

In the study, cell therapy using GDA-201 with monoclonal antibodies was generally well tolerated and demonstrated early evidence of clinical activity. Of the nine patients with NHL, five achieved a complete response and one achieved a partial response. Among the patients with MM, one patient achieved a complete response, and five patients achieved stable disease.

GDA-201 was generally well tolerated, with no graft vs. host disease (GvHD), no tumor lysis syndrome, no neurotoxicity and no marrow aplasia observed. No dose limiting toxicities were observed. Hypertension and hematologic events were the most common Grade 3/4 adverse events observed. Most non-hematologic toxicities were attributed to cyclophosphamide/fludarabine, which was used as a pre-conditioning treatment.

NAM Therapeutic Platform Mechanism of Action Data Presented at ASH

The poster presentation, “Nicotinamide (NAM) Modulates Transcriptional Signature of Ex Vivo Cultured UCB CD34+ Cells (Omidubicel) and Preserves Their Stemness and Engraftment Potential” (Abstract #3718), included transcriptome, transcription factor, and pathway analysis to elucidate the pathways leading to the preservation of engraftment after ex vivo expansion of CD34+ hematopoietic stem cells derived from umbilical cord blood (the starting point for omidubicel) compared to CD34+ cells grown in the absence of NAM.

Analyses showed that the presence of NAM reduced the expression of genes involved in the production of reactive oxygen and nitrogen species, suggesting that cell stress was minimized during expansion. In addition, NAM also decreased growth factor pathways responsible for activation and differentiation of hematopoietic stem cells, suggesting NAM expanded cells while keeping them in an undifferentiated state. The presence of NAM also led to a decrease in the expression of genes responsible for matrix-metallo proteinase secretion, simulating the microenvironment of the bone marrow. Additionally, NAM led to an increased expression of telomerase genes, which is believed to enable cells to remain in a more quiescent, stem-like state. These data provide further scientific rationale for the favorable stem cell engraftment and patient outcomes that were observed in the Phase 1/2 clinical study of omidubicel.

About GDA-201

GDA-201 (formerly known as NAM-NK) is being developed as an innate natural killer (NK) cell immunotherapy for the treatment of hematologic and

solid tumors in combination with standard-of-care antibody therapies. NK cells have the ability to kill tumor cells, representing a novel immunotherapeutic approach to cancer treatment. GDA-201 is designed to address 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. GDA-201 is in Phase 1 development in patients with refractory non-Hodgkin lymphoma and multiple myeloma.¹ For more information on the clinical study of GDA-201, please visit www.clinicaltrials.gov.

About Omidubicel

Omidubicel (formerly known as 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). Omidubicel 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, omidubicel demonstrated rapid and durable time to engraftment and was generally well tolerated.² A Phase 3 study evaluating omidubicel in patients with leukemia and lymphoma is ongoing in the U.S., Latin America, Europe and Asia.³ Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.⁴ 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 omidubicel. For more information on clinical trials of omidubicel, please visit www.clinicaltrials.gov.

GDA-201 and omidubicel 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 the NAM Therapeutic Platform

Gamida Cell's proprietary NAM-based cell expansion platform is designed to enhance the number and functionality of donor cells in culture, enabling the creation of potentially transformative therapies that move beyond what is possible with existing approaches. The NAM therapeutic platform leverages the unique properties of nicotinamide to enable the expansion of multiple cell types — including stem cells and natural killer (NK) cells — with appropriate growth factors to maintain the cells' original phenotype and potency. This can enable the administration of a therapeutic dose of cells with the potential to improve patient outcomes.

About Gamida Cell

Gamida Cell is an advanced cell therapy company committed to finding 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.

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 of new clinical trials and the continuation of the Company's clinical development program, 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 clinical trials and other 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 of Gamida Cell's public filing on Form 20-F, filed with the SEC on February 25, 2019, 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 NCT03019666.

²Horwitz 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.

³ClinicalTrials.gov identifier NCT02730299

⁴ClinicalTrials.gov identifier NCT03173937

View source version on businesswire.com: <https://www.businesswire.com/news/home/20191209005662/en/>

Source: Gamida Cell Ltd.

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

Max Stendahl (media)
max@tenbridgecommunications.com
508-277-8117