



Gamida Cell Presents Updated, Expanded Results from Phase 1 Study of Natural Killer Cell Therapy GDA-201 at ASH Annual Meeting and Exposition

December 5, 2020

—GDA-201 was well-tolerated and no dose-limiting toxicities were observed—

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

—Phase 2 clinical trial of GDA-201 in non-Hodgkin lymphoma planned; IND submission anticipated in 2021—

BOSTON--(BUSINESS WIRE)--Dec. 5, 2020-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious hematologic diseases, today announced in an oral presentation the updated and expanded 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 62nd American Society of Hematology (ASH) Annual Meeting & Exposition, which is being held virtually December 5–8.

GDA-201 was well-tolerated and no dose-limiting toxicities were observed in 35 patients (19 with NHL and 16 with MM). The data show that therapy using GDA-201 with monoclonal antibodies demonstrated significant clinical activity in heavily pretreated patients with advanced NHL. Of the 19 patients with NHL, 13 complete responses and one partial response were observed, with an overall response rate of 74 percent and a complete response rate of 68 percent. The maximum tolerated dose was not achieved, as no dose limiting toxicities were observed in patients who received the maximum target dose (2×10^8 cells/kg).

“Data from an expanded group of patients in this Phase 1 clinical study for GDA-201 show that NK cell therapies continue to exhibit impressive therapeutic potential to treat relapsed and refractory patients with lymphomas, while maintaining a favorable safety profile,” said Veronika Bachanova, M.D., Ph.D., Professor of Medicine in the Division of Hematology, Oncology and Transplantation at the University of Minnesota and principal investigator of the study. “Despite recent advancements in therapies for patients with hematologic malignancies, too many patients progress to develop refractory or resistant disease. I look forward to the continued clinical development of this novel investigational therapy.”

NK cell immunotherapies are thought to offer tremendous potential for transforming the care of hematologic malignancies. With GDA-201, Gamida Cell is pioneering a novel approach that harnesses the power of its cell expansion technology to improve antibody-dependent cellular cytotoxicity (ADCC) and tumor targeting of NK cells.

“These additional results again show that GDA-201 has striking signs of efficacy and safety in patients with heavily pre-treated NHL,” said Julian Adams, Ph.D., chief executive officer of Gamida Cell. “With these results in hand, we plan to initiate a Phase 2 clinical study, with the goal of submitting an IND in 2021.”

GDA-201 Phase 1 Clinical Data

The presentation, “Results of a Phase 1 Trial of GDA-201, Nicotinamide-Expanded Allogeneic Natural Killer (NK) Cells in Patients with Refractory Non-Hodgkin Lymphoma (NHL) and Multiple Myeloma,” described results from the Phase 1 clinical study of GDA-201 in heavily pre-treated patients with advanced NHL and MM. Preliminary results from this study [were presented](#) at the 2019 ASH Annual Meeting.

In the study, cell therapy using GDA-201 with monoclonal antibodies was shown to be safe; there were no dose-limiting toxicities, neurotoxic events, confirmed cytokine release syndrome, graft versus host disease or marrow aplasia. Overall survival and progression-free survival at one year in the NHL cohort suggest durable disease control, with a median follow-up of ten months (range 1–28 months). The most common adverse events were decreased neutrophil count, febrile neutropenia, anemia and low platelet counts.

In the NHL cohort, durable complete responses were observed in patients with both follicular and diffuse large B cell lymphoma, with an overall response rate of 74 percent. Future development of GDA-201 may include cryopreservation and the exploration of multiple treatment cycles in a multi-center Phase 2 trial in patients with NHL.

About GDA-201

Gamida Cell applied the capabilities of its NAM-based cell expansion technology to develop GDA-201, an innate natural killer (NK) cell immunotherapy for the treatment of hematologic and solid tumors in combination with standard of care antibody therapies. GDA-201 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. GDA-201 is in Phase 1 development through an investigator-sponsored study 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.

GDA-201 is an investigational therapy, and its safety and efficacy have not been established 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 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 or follow Gamida Cell on [LinkedIn](#) or Twitter at [@GamidaCellTx](#).

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 timing of initiation and progress of and data reported from the clinical trials of Gamida Cell's product candidates, anticipated regulatory filings, commercialization efforts and Gamida Cell's expectations regarding its projected ongoing operating activities, 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 clinical trials and ramifications for the cost thereof; and 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 and other sections of Gamida Cell's Annual Report on Form 20-F, filed with the Securities and Exchange Commission (SEC) on February 26, 2020, its Reports on Form 6-K filed with the SEC on May 18, 2020, August 11, 2020 and November 10, 2020, 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.

¹ [Clinicaltrials.gov](https://clinicaltrials.gov) identifier NCT03019666

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