Gamida Cell Provides Pipeline Update, Including Detailed Results of Pivotal Phase 3 Clinical Study of Omidubicel, and Prepares to Start BLA Submission by End of 2020

December 9, 2020

—Omidubicel results in improved clinical outcomes as measured by reduction in time to neutrophil engraftment, time in hospital and infections following bone marrow transplant—

— Company announces omidubicel commercial readiness plan, including the creation of Gamida Cell Assist, to support a positive patient and transplant center experience in preparation for potential launch of omidubicel as early as Q4 2021, subject to ongoing FDA discussions—

—Provides update on Phase 1 study of investigational natural killer cell therapy GDA-201 and that Phase 2 study of omidubicel in patients with severe aplastic anemia has demonstrated sustained early engraftment, as reported at ASH Annual Meeting—

BOSTON--(BUSINESS WIRE)--Dec. 9, 2020-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious hematologic diseases, today will be providing an update on the Phase 3 clinical study of omidubicel, commercial readiness plan and pipeline at its virtual Pipeline Deep Dive event.

“Our goal with omidubicel is to revolutionize the field of bone marrow transplantation and bring a potentially curative cell therapy option to thousands of patients who are in need of a bone marrow transplant, but lack a suitable stem cell donor. These results bring us one step closer towards that goal,” said Julian Adams, Ph.D., chief executive officer of Gamida Cell. “What’s more, transplantation with omidubicel has been shown to result in more rapid neutrophil engraftment, a decrease in the amount of time patients spend in hospital, and a reduction in infections. These are very meaningful outcomes for patients and may also lessen the financial costs of certain aspects of the transplant.”

Gamida Cell previously reported top-line data for omidubicel. In October, the company reported that the omidubicel phase 3 study achieved its secondary endpoints, analyzed in all randomized patients (intent-to-treat). In May, Gamida Cell reported that the study achieved its primary endpoint, demonstrating a highly statistically significant reduction in time to neutrophil engraftment, a key milestone in a patient’s recovery from a bone marrow transplant.

These pivotal data form the basis of a Biologics License Application (BLA) that Gamida Cell expects to initiate on a rolling basis before the end of this year. Gamida Cell is preparing to be launch ready in anticipation of potential FDA approval as early as the fourth quarter of 2021, subject to ongoing FDA discussions on manufacturing, quality and other matters.

The live event will be available here. More information about the Phase 3 study of omidubicel and the other updates included in this release can be found in the Pipeline Deep Dive presentation on the Gamida Cell website immediately following the event.

Details of Phase 3 Endpoints

As previously reported, Gamida Cell achieved positive topline results from its Phase 3 clinical study evaluating the safety and efficacy of omidubicel. The median time to neutrophil engraftment was 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p<0.001$). Neutrophil engraftment is a measure of how quickly the stem cells a patient receives in a transplant are established and begin to make healthy new cells, and rapid neutrophil engraftment has been associated with fewer infections and shorter hospitalizations.

Today, Gamida Cell announced the details of achieving all three of the prespecified secondary endpoints of the study, analyzed in all randomized patients (intent-to-treat). These secondary endpoints were the proportion of patients who achieved platelet engraftment by day 42, the proportion of patients with grade 2 or grade 3 bacterial or invasive fungal infections in the first 100 days following transplant, and the number of days alive and out of the hospital in the first 100 days following transplant. All three secondary endpoints demonstrated statistical significance in an intent-to-treat analysis.

- **Platelet engraftment was significantly accelerated** with omidubicel, with 55 percent of patients randomized to omidubicel achieving platelet engraftment at day 42, compared to 28 percent for the comparator ($p = 0.028$).
- **Infection rates were significantly reduced** for patients randomized to omidubicel. The cumulative incidence of first grade 2 or grade 3 bacterial or invasive fungal infection for patients randomized to omidubicel was 37 percent, compared to 57 percent for the comparator ($p = 0.027$).
- **Total days in hospital were reduced** in patients randomized to omidubicel. The median number of days alive and out of hospital for patients randomized to omidubicel was 60.5 days, compared to 48.0 days for the comparator ($p = 0.005$).

Additionally, Gamida Cell reported that the exploratory endpoints in the study demonstrated a reduction in the cumulative incidence of viral infections.

The international, multi-center, randomized Phase 3 study for omidubicel was designed to evaluate the safety and efficacy of omidubicel in patients with hematologic malignancies undergoing allogeneic bone marrow transplant compared to a comparator group of patients who received a standard umbilical cord blood transplant.

The company anticipates reporting the full data set in a peer-reviewed setting in the first half of 2021.

Commercial Readiness

The company discussed the market potential for omidubicel and launch plans. These included quantifying the market opportunity and keys aspects for a successful launch.

As it prepares for the potential commercial launch of omidubicel, the company also announced plans for the Gamida Cell Assist program, which has been designed to focus on patient access and support of every individual and their caregiver at each step of the transplant process. Once the program is launched, the Gamida Cell Assist case management team would provide a consistent, single point of contact for patients and health care professionals. This team would work with the transplant center to track each individual patient's omidubicel therapy and provide real-time updates on the status of the therapy. Gamida Cell Assist is also designed to provide additional services, including coverage and reimbursement support, and patient and caregiver support, which may include financial, travel, and lodging assistance.

"At Gamida Cell we are inspired to cure, with the goal of pioneering new standards of care for patients with blood cancers and serious blood diseases," said Michele Korfin, chief operating and chief commercial officer of Gamida Cell. "The transplant process can be challenging and complex for the patient, caregivers and the entire transplant care team. As we prepare for commercialization, we have developed Gamida Cell Assist to serve as a comprehensive support program to focus on assuring a positive patient experience with omidubicel. We are committed to supporting patients and their caregivers during every step of their journey and enabling what matters most, a successful clinical outcome that makes a meaningful difference for patients."

Update on Natural Killer Cell Therapy GDA-201

In an oral presentation at the recent American Society of Hematology (ASH) 62nd Annual Meeting, it was shown that GDA-201 was well tolerated and no dose limiting toxicities were observed in the Phase 1 clinical study. GDA-201 demonstrated significant clinical activity in patients with non-Hodgkin lymphoma, with 13 complete responses and one partial response observed in 19 patients, for a response rate of 74 percent. Full details of the presentation can be found in the press release.

Phase 2 Study of Omidubicel in Patients with Severe Aplastic Anemia

In a poster presentation at ASH, it was shown that patients with severe aplastic anemia treated with omidubicel achieved sustained early engraftment. These data, which were presented on December 5 by Mohamed Samour, M.D., Hematology Branch, National Heart, Lung, and Blood Institute, Bethesda, MD, are the first evidence that omidubicel can result in rapid engraftment and can achieve sustained hematopoiesis in patients who are at high risk for graft failure with conventional umbilical cord blood transplant.

About Omidubicel

Omidubicel 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). In both Phase 1/2 and Phase 3 clinical studies (NCT01816230, NCT02730299), omidubicel demonstrated rapid and durable time to engraftment and was generally well tolerated.[1,2] Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia (NCT03173937). 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.

Omidubicel 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 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.[3] 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 has 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, launch readiness and FDA approval, 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.

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[1] 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.

[2] Gamida Cell press release, "Gamida Cell Announces Positive Topline Data from Phase 3 Clinical Study of Omidubicel in Patients with High-Risk Hematologic Malignancies," issued May 12, 2020. Last accessed August 31, 2020.

[3] Clinicaltrials.gov identifier NCT03019666

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