Gamida Cell Reports First Quarter 2019 Financial Results and Provides Company Update

May 7, 2019

– Announces USAN selection of omidubicel as nonproprietary name for company's investigational NAM-expanded hematopoietic stem cells (formerly known as NiCord®) –

– Patient enrollment in Phase 3 study of omidubicelexpected to be completed in second half of 2019; Topline results expected in first half of 2020 –

– Phase 1 clinical study of GDA-201 (formerly known as NAM-NK) continues to progress, with additional data expected in 2H19 –

BOSTON--(BUSINESS WIRE)--May 7, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), a leading cellular and immune therapeutics company, today reported financial results for the quarter ended March 31, 2019. The company also highlighted continued progress in advancing its clinical development candidates: omidubicel[1] (formerly known as NiCord®), an investigational advanced cell therapy in Phase 3 clinical development designed to enhance the life-saving benefits of hematopoietic stem cell (bone marrow) transplant, and GDA-201 (formerly known as NAM-NK), an investigational, natural killer (NK) cell-based cancer immunotherapy in Phase 1 development in patients with non-Hodgkin lymphoma and multiple myeloma.

"Gamida Cell is focused on transforming the treatment landscape for patients with blood cancers and rare, serious hematologic diseases. We are pleased that omidubicel has been selected as the nonproprietary name for NiCord, highlighting our progress toward bringing this important cell therapy to patients in need of a bone marrow transplant," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We have also made several key personnel appointments this year that reflect our strategic focus on commercial preparedness, including hiring our first chief commercial officer and nominating new board members who bring commercial, operational and financial experience to Gamida Cell's board of directors."

Dr. Adams continued, "We are pleased that the multi-center, randomized Phase 3 study of omidubicel is progressing, with patient enrollment expected to be complete by the end of this year and topline data anticipated in the first half of 2020. Positive data from the study would enable the submission of our first biologics license application next year, which would be a significant achievement."

"Earlier this year, we also reported encouraging data from the Phase 1 clinical study of our natural killer cell product candidate, GDA-201, previously known as NAM-NK. The multiple complete responses observed emboldened us to begin scaling up our manufacturing process to enable the evaluation of a cryopreserved formulation of GDA-201 in a multi-center, multi-dose Phase 1/2 clinical study in patients with non-Hodgkin lymphoma next year," Dr. Adams concluded.

Company Highlights

- **Omidubicel selected as nonproprietary name for NiCord:** Today Gamida Cell announced that the United States Adopted Names (USAN) Council selected omidubicel as the nonproprietary name for Gamida Cell's investigational hematopoietic stem cell expanded through the company's proprietary nicotinamide-based, or NAM, technology. The USAN Council aims for global standardization and unification of drug nomenclature to ensure that drug information is communicated accurately and unambiguously. Gamida Cell's lead investigational product has two components: omidubicel (hematopoietic stem cells expanded through the company's proprietary nicotinamide-based, or NAM, technology) and differentiated immune cells, including T cells. Gamida Cell refers to the two components collectively as "omidubicel." Going forward, Gamida Cell will use the name "omidubicel" in publications and public statements, at conferences and other forums, and in medical and commercial-related materials.
- **Reported encouraging data for omidubicel and GDA-201 at TCT Annual Meeting:** In February, data from the omidubicel and GDA-201 clinical programs were reported 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 from the completed Phase 1/2 clinical study of omidubicel demonstrated that recipients who received omidubicel had rapid and robust reconstitution of key immune cells. Successful immune reconstitution is an important factor in the recovery of patients undergoing bone marrow transplant.

  Data were also reported from the ongoing Phase 1/2 study of omidubicel in patients with severe aplastic anemia. In the initial cohort of three patients, all successfully underwent a bone marrow transplant consisting of omidubicel plus a haploidentical stem cell graft. The results enable the initiation of a second cohort of patients to be treated with omidubicel as a stand-alone graft.Patient enrollment in the second cohort is expected to begin in the first half of 2019.

  Additionally, data reported from the ongoing Phase 1 study of GDA-201 in patients with non-Hodgkin lymphoma (NHL) and multiple myeloma (MM) demonstrated that GDA-201 was clinically active, with three complete responses observed in patients with NHL and one complete response in a patient with MM. These data, along with safety data showing that GDA-201 was generally well tolerated, support continued clinical development. Gamida Cell is planning to initiate a multi-center, Phase 1/2 clinical study of GDA-201 in patients with NHL in 2020.

- **Evolved Board of Directors to reflect company's progress toward commercialization:** In March,the company announced the nominations of Shawn Cline Tomasello and Stephen T. Wills to its board of directors. These nominations require approval at the Annual Shareholders Meeting, which will take place in June 2019. Ms. Tomasello has extensive experience in commercializing first-in-class medicines for the treatment of cancer, including Yescarta® (at Kite Pharma, now part of Gilead Sciences) and Imbruvica® (at Pharmacyclics, now part of AbbVie). Mr. Wills has extensive operational, financial and transactional experience over nearly three decades in the life sciences and accounting industries. He has served as chief financial officer of Palatin Technologies, a publicly-traded biotechnology company developing peptide therapeutics, since 1997 and also serves as Palatin's chief operating officer and executive vice president.

  In January, the company appointed Nurit Benjamini to Gamida Cell's board of directors and chair of the board's audit committee. Ms. Benjamini has served as chief financial officer of TabTale Ltd. since 2013. Previously, she held a number of chief financial officer positions, including at Wix.com Ltd., Sigma Designs Israel Ltd. and Compugen Ltd.

- **Appointed Thomas Klima as chief commercial officer:** In January, the company announced the appointment of Thomas Klima as chief commercial officer.In this newly created role, Mr. Klima will be responsible for building the team and executing the strategy to potentially bring omidubicel to patients, including oversight of reimbursement and patient services.Klima brings nearly 20 years of global experience in the pharmaceutical industry with expertise in cellular therapy, hematology, oncology and transplantation. During his career, he has played key roles in building commercial organizations and leading multiple successful product launches.

Anticipated 2019-2020 Milestones
Gamida Cell's anticipated program milestones in 2019-2020 are as follows:

Omidubicel

- Initiate Cohort 2 in the Phase 1/2 study evaluating omidubicel as stand-alone graft in severe aplastic anemia in the first half of 2019
- Complete enrollment in Phase 3 study of omidubicel in patients with hematologic malignancies in the second half of 2019
- Report topline data from the Phase 3 study of omidubicel in patients with hematologic malignancies in the first half of 2020
- Complete BLA submission for omidubicel in hematologic malignancies in the second half of 2020, should Phase 3 data be positive

GDA-201

- Complete patient enrollment in the ongoing Phase 1 study in the second half of 2019
- Present additional data at a medical meeting in the second half of 2019
- Initiate multi-center, Phase 1/2 clinical study in patients with NHL in 2020

First Quarter 2019 Financial Results

- As of March 31, 2019, Gamida Cell had total cash, cash equivalents and available-for-sale securities of $50.3 million, compared to $60.7 million as of December 31, 2018.
- Research and development expenses in the first quarter of 2019 were $7.3 million, compared to $5.1 million in the same period in 2018. The difference was attributable mainly to a $1.2 million increase in clinical activities relate to the advancement of omidubicel and GDA-201, $0.5 million reduction in grants received from the Israeli Innovation Authority (IIA) and an increase of $0.5 million in compensation and other R&D expenses.
- General and administrative expenses were $3.8 million for the first quarter of 2019, compared to $1.7 million in the same period in 2018. The increase was due mainly to a $1.0 million increase in expenses related to hiring and establishing the U.S. headquarters, an increase of $0.5 million in non-cash stock-based compensation expenses, and $0.6 million in professional services, rent and other expenses.
- Finance expenses, net, were $4.4 million for the three months ended March 31, 2019, compared to $0.7 million in income in the same period in 2018. The increase was primarily due to noncash expenses resulting from revaluation of warrants and the revaluation of royalty-bearing grant IIA liability.
- Net loss for the first quarter of 2019 was $15.5 million, compared to a net loss of $7.4 million in the same period in 2018.

2019 Financial Guidance
Gamida Cell continues to expect cash used for ongoing operating activities in 2019 to range from $35-$40 million, reflecting anticipated expenditures to advance the company's clinical programs.

Gamida Cell expects that its cash, cash equivalents, available-for-sale securities and short-term debt will support the company's capital needs through the data readout for the Phase 3 clinical study of omidubicel, which is expected in the first half of 2020. This cash runway guidance is based on the company's current operational plans and excludes any additional funding that may be received or business development activities that may be undertaken.

Conference Call Information

Gamida Cell will host a conference call today, May 7, 2019, at 8:30 a.m. ET to discuss these financial results and company updates. A live webcast of the conference call can be accessed in the “Investors” section of Gamida Cell’s website at www.gamida-cell.com. To participate in the live call, please dial 866-930-5560 (domestic) or 409-216-0605 (international) and refer to conference ID number 2277888. A replay of the webcast will be available for approximately 30 days.

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).[1] 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., 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.

About GDA-201

Gamida Cell applied the capabilities of its NAM-based cell expansion technology to develop GDA-201 (formerly known as NAM-NK), 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.[5]

Omidubicel and GDA-201 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 the patient enrollment in and timing of initiation and progress of and data reported from the clinical trials of Gamida Cell’s product candidates, and Gamida Cell’s expectations regarding its projected operating expenses and cash runway, 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 variability, 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 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.

INTERIM CONSOLIDATEDSTATEMENTS OF FINANCIAL POSITION

U.S. dollars in thousands

	March 31, 2019	December 31, 2018
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 43,749	$ 40,272
Available-for-sale financial assets	6,507	20,417
Prepaid expenses and other current assets	684	1,502
Totalcurrent assets	50,940	62,191
NON-CURRENT ASSETS:		
Property and equipment, net	2,782	2,311
Right-of-use assets	6,668	-

Other assets	657	662
Totalnon-current assets	10,107	2,973
Total assets	$ 61,047	$ 65,164
LIABILITIES AND EQUITY		
CURRENT LIABILITIES:		
Trade payables	$ 1,341	$ 1,985
Employees and payroll accruals	2,580	2,888
Current maturities of lease liabilities	2,156	-
Accrued expenses and other payables	1,739	1,832
Total current liabilities	7,816	6,705
NON-CURRENT LIABILITIES:		
Liabilities presented at fair value	25,031	24,049
Employee benefit liabilities, net	276	183
Lease Liabilities	4,671	-
Liability to Israel Innovation Authority (IIA)	10,108	9,540
Total non-current liabilities	40,086	33,772
SHAREHOLDERS' EQUITY:		
Share capital	68	67
Share premium	197,967	193,953
Capital reserve due to actuarial gains	(160)	(77)
Available-for-sale reserve	(10)	(43)
Accumulated deficit	(184,720)	(169,213)
Total shareholders' equity	13,145	24,687
Total liabilities and shareholders' equity	$ 61,047	$ 65,164

[1] Gamida Cell's lead development candidate consists of omidubicel (expanded hematopoietic stem cells) and differentiated immune cells, including T cells. Gamida Cell refers to the two components collectively as "omidubicel."

[2] 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.

[3] ClinicalTrials.gov identifier NCT02730299.

[4] ClinicalTrials.gov identifier NCT03173937.

[5] ClinicalTrials.gov identifier NCT03019666.

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