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

November 13, 2019

– Completion of patient enrollment in Phase 3 clinical study of omidubicel expected by year-end; Topline data anticipated in first half of 2020 –

– Additional results from Phase 1 study of GDA-201 and new data on NAM technology platform to be presented at 61st Annual Meeting of the American Society of Hematology –

BOSTON--(BUSINESS WIRE)--Nov. 13, 2019-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to finding cures for blood cancers and serious blood diseases, today reported financial results for the quarter ended September 30, 2019. The company also highlighted continued progress in advancing its clinical development candidates: omidubicel, an advanced cell therapy in Phase 3 clinical development as a potential life-saving treatment option for patients in need of bone marrow transplant, and GDA-201, an investigational, natural killer (NK) cell-based cancer immunotherapy in Phase 1 development in patients with non-Hodgkin lymphoma and multiple myeloma.

"During the quarter, we made important progress toward our goal of developing next-generation cell therapies with the potential to redefine standards of care for patients with blood cancers and rare, serious hematologic diseases," stated Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We are nearing completion of patient enrollment in our multi-center, randomized Phase 3 study of omidubicel and are on track to report topline data from the study in the first half of 2020. Positive data from the study would enable us to file our first biologics license application next year."

"Our second cell therapy program, GDA-201, is also advancing, and next month we will report additional data from the ongoing Phase 1 study at the Annual Meeting of the American Society of Hematology," Dr. Adams continued. "During the meeting, we will also show new gene expression data that reinforce our understanding of the mechanism of action underlying our NAM technology platform which enabled the generation of both of our development candidates."

Program Highlights

- **Announced presentation of new data at the 61st Annual Meeting of the American Society of Hematology (ASH):** Last week, Gamida Cell announced that additional data from the ongoing Phase 1 clinical study of GDA-201 will be presented during an oral session at the ASH 2019 Annual Meeting, which is being held December 7 – 10 in Orlando, FL. The 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), will take place on Monday, December 9, at 3:15 p.m. ET.

  Additionally, new research 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, will also be shared during the meeting. 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), will take place on Monday, December 9, from 6:00 – 8:00 p.m. ET.

- **Advanced the Phase 3 clinical study of omidubicel:** Patient enrollment continued to progress in the Gamida Cell's Phase 3 study of omidubicel in patients with high-risk hematologic malignancies. The international, randomized, multi-center study is designed to evaluate the safety and efficacy of omidubicel compared to standard umbilical cord blood for allogeneic bone marrow transplant in approximately 120 patients with no available matched donor. The company anticipates completing patient enrollment by the end of this year with topline data anticipated in first half of 2020.

- **Initiated health outcomes research for omidubicel:** In September, Gamida Cell and the CIBMTR® (Center for International Blood and Marrow Transplant Research) announced a research agreement to evaluate outcomes of patients with hematological malignancies who undergo allogeneic hematopoietic stem cell transplant (bone marrow transplant) from various donor sources. The recently launched observational study includes both retrospective and prospective data contemporaneous to the Phase 3 study of omidubicel. The goal of this real-world, observational study is to better understand the variables that influence the health outcomes of patients receiving a transplant from a source other than a fully matched family donor.

- **Continued to focus on activities required to successfully bring omidubicel to patients:** Gamida Cell is continuing to advance key activities required to bring omidubicel to patients in a commercial setting, including building out manufacturing infrastructure, assembling an experienced commercial team with expertise in cell therapy and transplant, establishing hospital services and patient assistance programs, and exploring coverage and reimbursement models to enable access.

- **Progressed enrollment in the Phase 1/2 study of omidubicel in patients with severe aplastic anemia:** Enrollment is

ongoing in a Phase 1/2 clinical study of omidubicel in patients with severe aplastic anemia, a rare, life-threatening bone marrow failure disease. Earlier this year, encouraging data from the first cohort of patients were reported at the 2019 Transplantation & Cellular Therapy (TCT) Meeting that demonstrated that all three patients in the cohort successfully underwent a bone marrow transplant consisting of omidubicel plus a haploidentical stem cell graft. The rapid engraftment, sustained hematopoiesis and accelerated immune recovery observed enabled the initiation of a second cohort, where patients will be treated with omidubicel as a stand-alone graft.

- **Continued to prepare for the next clinical study of GDA-201:** The company is continuing its work to enable a Phase 1/2 multi-dose, multi-center study of GDA-201 in patients with non-Hodgkin lymphoma, which is expected to begin in 2020. The decision to focus the next clinical study on non-Hodgkin lymphoma is based on the encouraging clinical data reported at the 2019 TCT Meeting which demonstrated the GDA-201 was generally well tolerated and clinically active, with multiple complete responses observed.

Third Quarter 2019 Financial Results

- Research and development (R&D) expenses in the third quarter of 2019 were $7.4 million compared to $5.1 million for the same period in 2018. R&D expenses were higher in the third quarter of 2019 compared to the same period in 2018 due to the advancement of omidubicel and GDA-201.

- General and administrative expenses were $4.6 million for the third quarter of 2019, compared to $2.4 million for the same period in 2018. The difference was attributable mainly to a $1.2 million increase in activities related to commercial readiness, as well as $1.0 million increase in professional services and other expenses, including an increase in expenses associated with being a publicly-traded company.

- Finance income, net, was $1.7 million for the third quarter of 2019, compared to finance expenses, net, of $2.2 million for the same period in 2018. The net increase was primarily due to non-cash income resulting from the re-valuation of warrants, offset by non-cash expenses from the re-valuation of the Israeli Innovation Authority royalty-bearing grant liability.

- Net loss for the third quarter of 2019 was $10.1 million, compared to a net loss of $9.8 million for the same period in 2018.

- As of September 30, 2019, Gamida Cell had total cash, cash equivalents and available-for-sale assets of $68.1 million, compared to $60.7 million as of December 31, 2018.

2019 Financial Guidance

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

Gamida Cell expects that its current cash, cash equivalents and available-for-sale securities will support the company's ongoing operating activities into the fourth quarter 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, November 13, 2019, at 4:30 p.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 8653335. A replay of the webcast will be available approximately two hours after the event, 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). 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.[1] A Phase 3 study evaluating omidubicel in patients with leukemia and lymphoma is ongoing in the U.S., Europe and Asia.[2] Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.[3] 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.[4]

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 an advanced cell therapy company committed to finding cures for 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 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 CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

U.S. dollars in thousands

	September 30,		**Dec 31,**
	2019	**2018**	**2018**
	Unaudited		**Audited**
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	$ 39,573	$ 14,109	$ 40,272
Available-for-sale financial assets	28,544	9,570	20,417
Prepaid expenses and other current assets	1,134	1,018	1,502
Total current assets	69,251	24,697	62,191
NON-CURRENT ASSETS:			
Property and equipment, net	4,209	1,743	2,311
Right-of-use assets	5,568	-	-
Other assets	651	354	662
Deferred issuance cost	-	1,718	-

Total non-current assets	10,428	3,815	2,973
Total assets	$ 79,679	$ 28,512	$ 65,164

LIABILITIES AND EQUITY

CURRENT LIABILITIES:			
Trade payables	$ 2,105	$ 3,060	$ 1,985
Employees and payroll accruals	3,096	2,128	2,888
Current maturities of lease liabilities	1,926	-	-
Accrued expenses and other payables	1,979	2,111	1,832
Total current liabilities	9,106	7,299	6,705
NON-CURRENT LIABILITIES:			
Liabilities presented at fair value	5,434	15,400	24,049
Employee benefit liabilities, net	280	194	183
Lease liability	4,342	-	-
Liability to Israel Innovation Authority (IIA)	11,594	10,474	9,540
Total non-current liabilities	21,650	26,068	33,772
SHAREHOLDERS' EQUITY:			
Total shareholders' equity	48,923	(4,855)	24,687
Total liabilities and shareholders' equity	$ 79,679	$ 28,512	$ 65,164

INTERIM CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME

U.S. dollars in thousands (except share and per share data)

	Nine months ended September 30,		Three months ended September 30,		Year ended December 31,
	2019	2018	2019	2018	2018
	Unaudited				Audited
Operating expenses:					
Research and development, net	$ 21,682	$ 17,169	$ 7,363	$ 5,132	$ 22,045
General and administrative	12,195	7,008	4,621	2,438	11,599
Operating loss	33,877	24,177	11,984	7,570	33,644
Finance expenses	2,499	6,560	895	2,356	20,259
Finance income	(16,665)	(434)	(2,613)	(104)	(1,042)
Loss (income) before taxes on income	19,711	30,303	10,266	9,822	52,861
Taxes on income (benefit)	(70)	-	(170)	-	70
Net loss	19,641	30,303	10,096	9,822	52,931
Net loss (income) per share:					
Basic net loss per share	$ 0.70	$ 43.92	$ 0.30	$ 14.23	$ 10.53
Diluted net loss per share	$ 1.24	$ 43.92	$ 0.30	$ 14.23	$ 10.53

INTERIM CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Nine months ended September 30,		Three months ended September 30,		Year ended December 31,
	2019	**2018**	**2019**	**2018**	**2018**
	Unaudited				**Audited**
Cash flows from operating activities:					
Net loss	$ (19,641)	$ (30,303)	$ (10,096)	$ (9,822)	$ (52,931)
Adjustments to reconcile net loss to net cash used in operating activities:					
Adjustments to the profit or loss items:					
Depreciation of property, plant and equipment and right-of-use assets	1,792	146	547	49	269
Financial (income) expenses, net	(768)	(161)	(199)	214	(858)
Cost of share-based compensation	3,731	2,503	1,321	880	3,575
Change in employee benefit liabilities, net	14	(6)	6	(23)	(15)
Amortization of premium on available-for-sale financial assets	150	191	49	200	272
Revaluation of financial derivatives	(15,691)	5,100	(2,220)	1,700	17,600
Revaluation of liability to IIA	1,852	3,167	653	567	2,037
	(8,920)	10,940	157	3,587	22,880
Changes in asset and liability items:					
Decrease (increase) in prepaid expenses and other current assets and other assets	113	(1,266)	(4)	(1,637)	942
Increase (decrease) in trade payables	120	670	(124)	1,902	(405)

Increase (decrease) in accrued expenses and other payables and employee and payroll accrual	680	1,071	518	(8)	2,296
	913	475	390	257	2,833
Cash received during the period for:					
Interest received	1,132	570	302	179	792
Interest paid	(92)	-	(41)	-	-
	1,040	570	261	179	792
Net cash used in operating activities	(26,608)	(18,318)	(9,288)	(5,799)	(26,426)
Cash flows from investing activities:					
Purchase of property and equipment	(2,139)	(949)	(1,261)	(246)	(1,645)
Purchase of available-for-sale financial assets	(32,021)	-	(32,021)	-	(10,905)
Proceed from sale of available-for-sale financial assets	-	4,984	-	-	4,949
Proceed from maturity of available-for-sale financial assets	23,789	-	8,049	-	-
Proceeds from bank deposits	-	5,000	-	-	5,000
Investment in restricted bank deposits	-	(150)	-	(150)	(150)
Net cash provided by (used in) investing activities	(10,371)	8,885	(25,233)	(396)	(2,751)

INTERIM CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Nine months ended September 30,		**Three months ended September 30,**		**Year ended December 31,**

	2019	2018	2019	2018	2018
	Unaudited				
Cash flows from financing activities:					
Proceeds from secondary offering, net	37,235	-	37,343	-	-
Receipt of grants from the IIA	202	2,953	35	1,300	612
Proceeds from issuance of shares, initial public offering (payment of issuance expenses), net	(238)	-	-	-	47,479
Payment of lease liabilities	(1,144)	-	(380)	-	-
Exercise of options	120	-	3	-	2
Increase in deferred issuance cost	-	(736)	-	-	
Net cash provided by financing activities	36,175	2,217	37,001	1,300	48,093
Exchange differences on balances of cash and cash equivalents	105	-	15	-	31
Increase (decrease) in cash and cash equivalents	(699)	(7,216)	2,495	(4,895)	18,947
Cash and cash equivalents at beginning of period	40,272	21,325	37,078	19,004	21,325
Cash and cash equivalents at end of period	$ 39,573	$ 14,109	$ 39,573	$ 14,109	$ 40,272

[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]ClinicalTrials.gov identifier NCT02730299.

[3]ClinicalTrials.gov identifier NCT03173937.

[4]ClinicalTrials.gov identifier NCT03019666.

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