Gamida Cell Reports First Quarter 2021 Financial Results and Provides Company Update

May 11, 2021

- *BLA submission for omidubicel, a potentially life-saving treatment for patients with blood cancers in need of stem cell transplant, expected in fourth quarter of 2021*
- *Pre-commercial and manufacturing activities underway to support potential launch of omidubicel in 2022*
- *Phase 1/2 clinical trial of allogeneic, off-the-shelf GDA-201 in NHL planned with IND submission anticipated in the second half of 2021*
- *Strengthened financial position with sale of $75M exchangeable senior notes in February 2021; sufficient liquidity to fund the company's operations into the second half of 2022*
- *Company to host conference call at 8:00 a.m. ET today*

BOSTON--(BUSINESS WIRE)--May 11, 2021-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious blood diseases, today reported financial results for the quarter ended March 31, 2021. The company also highlighted progress with omidubicel, an advanced cell therapy with positive Phase 3 clinical data, as a potentially life-saving treatment option for patients in need of an allogeneic hematopoietic stem cell (bone marrow) transplant, and GDA-201, a natural killer (NK) cell immunotherapy in Phase 1/2 development for patients with non-Hodgkin lymphoma (NHL).

"In the first quarter of this year, we made significant progress on key initiatives across all functions of our business, starting with omidubicel, a potentially transformative treatment option for patients with hematological malignancies," said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We are working diligently to bring this novel therapy to patients, with submission of a BLA to the FDA anticipated in the fourth quarter of this year. We are progressing well with our manufacturing readiness activities in response to the clear feedback from the FDA regarding registration of our commercial manufacturing facilities and are actively building our launch readiness capabilities, including market access and support services, to ensure a positive patient experience at transplant at the time of potential FDA approval."

"We also continue to expand our clinical pipeline with plans to submit an IND for our GDA-201 natural killer cell therapy, initiate a multi-center Phase 1/2 clinical study in NHL and continue to advance our R&D activities to pursue the development of genetically modified NAM-enabled NK cells in solid tumors. Importantly, we are well positioned to deliver our 2021 corporate goals and objectives toward improving the lives of the patients we serve," Dr. Adams continued.

Omidubicel, a proprietary, investigational advanced cell therapy for allogeneic bone marrow transplant

Omidubicel is the foundational product based on Gamida Cell's proprietary cell expansion technology. During the quarter, Gamida Cell continued to advance omidubicel, the first cell therapy for bone marrow transplant to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA). The company anticipates submitting a Biologics License Application (BLA) to the FDA in the fourth quarter of this year, based on the results of an international, randomized Phase 3 study of omidubicel that was designed to evaluate the safety and efficacy of omidubicel in patients with hematologic malignancies undergoing a bone marrow transplant compared to patients who received a standard umbilical cord blood transplant. The study achieved its primary endpoint, a statistically significant reduction in time to neutrophil engraftment, as well as all key secondary endpoints. A key milestone in a patient's recovery, neutrophil engraftment is a measure of how quickly the stem cells a patient receives in a bone marrow transplant are established and begin to make healthy new cells. In the recently completed Phase 3 study, the median time to neutrophil engraftment was 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p < 0.001$). Additionally, the study met key secondary endpoints related to the speed of platelet engraftment, decrease in infections and reduction in hospitalizations, all significant clinical measures in bone marrow transplant.

In February 2021, the company presented details of the results of the omidubicel Phase 3 study at the Transplantation & Cellular Therapy Meetings of the American Society of Transplantation and Cellular Therapy and Center for International Blood & Marrow Transplant Research. The study's intent-to-treat analysis included 125 patients aged 13–65 years with a median age of 41. Patients were enrolled at more than 30 clinical centers in the United States, Europe, Asia, and Latin America. Racial and ethnic diversity and baseline characteristics which were well-balanced across the two study groups. Diseases included acute lymphoblastic leukemia, acute myelogenous leukemia, chronic myelogenous leukemia, myelodysplastic syndrome or lymphoma.

In addition to the efficacy results described above, safety results were also presented, showing decreased incidence related to grade III/IV acute GvHD (14 percent for omidubicel, 21 percent for the comparator) and comparable results for all grades chronic GvHD at one year (35 percent for omidubicel, 29 percent for the comparator). Transplants with umbilical cord blood, the comparator, have been historically shown to result in low incidence of GvHD in relation to other graft sources and, in this study, omidubicel demonstrated a similar GvHD profile.

The data from the study relating to exploratory endpoints also supported the clinical benefit demonstrated by the study's primary and secondary endpoints. The rate of infection was significantly reduced for patients randomized to omidubicel, with the cumulative incidence of first grade II or grade III bacterial or invasive fungal infection for patients randomized to omidubicel of 37 percent, compared to 57 percent for the comparator ($p = 0.027$). Additionally, the study demonstrated a reduction in the incidence of viral infections. Non-relapse mortality was 11 percent for patients randomized to omidubicel and 24 percent for patients randomized to the comparator ($p=0.09$). Overall survival at 15 months following randomization was 73 percent for patients randomized to omidubicel and 62 percent for patients randomized to control ($p=0.16$), median overall survival was not yet reached. Non-relapse mortality and overall survival were exploratory endpoints that were not powered for statistical significance. When considering the patient experience following transplant, faster hematopoietic recovery, fewer bacterial and viral infections and fewer days in hospital are all meaningful results

and represent potentially important advancements in care. Learn more.

Gamida Cell also reported data from the Phase 3 study in March 2021, in an oral session at the Presidential Symposium of the 47th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT 2021). Additionally, the session was featured in a panel discussion, "EBMT Talks: Live with the Best Abstracts."

Additional omidubicel highlights:

- **Progress with commercial manufacturing readiness:** Gamida Cell is making important progress to address the clear feedback received during a Type B meeting with the FDA in December 2020 for commercial manufacturing facilities to be ready for BLA submission. These facilities include the Gamida Cell facility in Israel and a commercial facility for which the company has a contractual relationship with Lonza. Both of these facilities are currently on track to meet the FDA requirements that will be required for BLA submission.
- **Continued launch readiness:** The company continues to progress commercial launch readiness activities for the potential launch of omidubicel in 2022, pending FDA approval. Based on market research insights, there is a clear opportunity to improve outcomes based on clinical needs with current donor sources, increase access for patients who are eligible and not matched for transplant, and increase patient eligibility based on the encouraging clinical profile of omidubicel.
- **Gamida Cell announced the Gamida Cell Assist program.** The transplant process can be challenging and complex for patients, caregivers and the entire transplant care team. Gamida Cell Assist is designed to focus on patient access and support at each step of the process. Once the program is launched, the Gamida Cell Assist case management team will provide a consistent, single point of contact for patients and health care professionals, work with the transplant center to track production of omidubicel for each individual patient, and provide real-time updates on the status of the therapy. The services provided will include coverage and reimbursement support, which may include financial, travel and lodging assistance. Gamida Cell is committed to supporting a positive journey for patients and their transplant teams so they can focus on what matters most, the patient experience and successful clinical outcomes. Learn more.
- **Phase 1/2 study of omidubicel in patients with severe aplastic anemia**: Gamida Cell is actively evaluating omidubicel in an investigator-sponsored Phase 1/2 study in patients with severe aplastic anemia (SAA). Results to date have shown 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.

GDA-201, a proprietary innate NK cell immunotherapy

- **Continued advancement of Phase 1/2 study of GDA-201:** Gamida Cell is preparing for the submission of an investigational new drug (IND) application for cryopreserved, off-the-shelf GDA-201 to enable a multi-center, Phase 1/2 clinical study in patients with NHL in the second half of this year. Gamida Cell is pioneering a potentially curative, novel approach that harnesses the power of its cell expansion technology, which improves antibody-dependent cellular cytotoxicity and tumor targeting of NK cells. Learn more.
- **Advancing NK cell R&D activities:** The company continues to advance R&D activities to support pipeline growth, including the development of genetically modified NK cells.

Corporate Highlights

- **Strengthened financial position:** In February 2021, the company completed a $75 million financing with Highbridge Capital Management, LLC, before deducting offering expenses. This financing will be used to support manufacturing, regulatory and potential commercial development activities for omidubicel and to further the preclinical and clinical development of GDA-201.

First Quarter 2021 Financial Results

- Research and development expenses in the first quarter of 2021 were $11.4 million, compared to $7.9 million for the same period in 2020. The increase was mainly due to omidubicel commercial manufacturing readiness activities and advancing the GDA-201 program, including broadening the company's scientific capabilities and talent.

- Commercial expenses in the first quarter of 2021 were $4.4 million compared to $1.5 million for the first quarter of 2020. The increase was mainly attributed to progress with commercial readiness activities, including the hiring of an experienced commercial leadership team.

- General and administrative expenses were $3.4 million for the first quarter of 2021 compared to $3.0 million for the same period in 2020. The increase was mainly due to the hiring of key management positions to support the growth of the business.

- Finance income, net, was $0.7 million for the first quarter of 2021, compared to finance income, net, of $1.7 million for the first quarter of 2020. The decrease was primarily due to interest expenses following the recent $75M financing with Highbridge Capital Management, and non-cash expense resulting from revaluation of warrants, and Israeli Innovation

Authority royalty-bearing grant liability.

- Net loss for the first quarter of 2021 was $18.0 million, compared to a net loss of $10.6 million for the same period in 2020.
- As of March 31, 2021, Gamida Cell had total cash and cash equivalents of $174.8 million, compared to $127.2 million as of December 31, 2020.

2021 Financial Guidance

Gamida Cell expects cash used for ongoing operating activities in 2021 to range from $110 million to $120 million.

Gamida Cell expects that its current cash and cash equivalents will support the company's ongoing operating activities into the second half of 2022. This cash runway guidance is based on the company's current operational plans and excludes any additional funding and any business development activities that may be undertaken.

Expected 2021-2022 Milestones and Key Events

Gamida Cell expects the following milestones and key events through 2022:

Omidubicel

- BLA submission to the FDA in the fourth quarter of 2021
- Manufacturing and launch readiness activities ongoing for potential FDA approval in 2022

GDA-201

- Submit company-sponsored IND application to the FDA and initiate a Phase 1/2 clinical study in NHL patients in the second half of 2021

Conference Call Information

Gamida Cell will host a conference call today, May 11, 2021, at 8:00 a.m. ET to discuss these financial results and company updates. A live webcast of the conference call can be accessed in the "Investors & Media" 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 5258448. A recording of the webcast will be available approximately two hours after the event, for approximately 30 days.

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 FDA 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 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 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 have not been established by the FDA or any other health authority.

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 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 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 March 9, 2021, as amended, 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

INTERIM CONSOLIDATED STATEMENTS OF FINANCIAL POSITION
U.S. dollars in thousands

	March 31,		December 31,
	2021	2020	2020
	Unaudited		Audited
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	$ 174,798	$40,292	$ 127,170
Prepaid expenses and other current assets	3,352	1,637	2,815
Total current assets	178,150	41,929	129,985
NON-CURRENT ASSETS:			
Property, plant and equipment, net	21,162	8,543	18,238
Right-of-use assets	5,920	5,820	6,474
Other assets	772	637	786
Total non-current assets	28,854	15,000	25,498
Total assets	$ 206,004	$56,929	$ 155,483

	March 31,		December 31,
	2021	2020	2020
	Unaudited		Audited
LIABILITIES AND EQUITY			
CURRENT LIABILITIES:			
Trade payables	$ 7,204	$ 3,098	$ 6,329
Employees and payroll accruals	3,854	2,549	4,705
Current maturities of lease liabilities	2,076	1,535	2,532
Accrued interest	634	-	-
Accrued expenses and other payables	6,099	2,922	7,988
Total current liabilities	19,867	10,104	21,554
NON-CURRENT LIABILITIES:			
Liabilities presented at fair value	9,758	2,773	12,043
Employee benefit liabilities, net	768	773	768
Other long-term liabilities	4,988	4,920	5,378
Liability to Israel Innovation Authority (IIA)	18,080	13,077	17,003
Convertible senior notes, net	68,646	-	-
Total non-current liabilities	102,240	21,543	35,192

SHAREHOLDERS' EQUITY:			
Share capital -			
Ordinary shares of NIS 0.01 par value - Authorized: 100,000,000 shares at March 31, 2021 and 2020 (unaudited) and December 31, 2020; Issued and outstanding: 59,247,838 and 33,696,582 shares at March 31, 2021 and 2020 (unaudited), respectively and 59,000,153 shares at December 31, 2020.	167	92	166
Share premium	378,478	239,897	375,280
Capital reserve	-441	-541	-441
Accumulated deficit	-294,307	-214,166	-276,268
Total shareholders' equity	83,897	25,282	98,737
Total liabilities and shareholders' equity	$ 206,004	$56,929	$ 155,483

INTERIM CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
U.S. dollars in thousands

	Three months ended March 31,		Year ended December 31,
	2021	2020	2020
	Unaudited		Audited
Operating expenses:			
Research and development, net	$ 11,366	$ 7,879	$ 41,385
Commercial activities	4,430	1,468	8,748
General and administrative	3,413	2,994	12,167
Operating loss	19,209	12,341	62,300
Finance expense	1,690	919	10,640
Finance income	-2,413	-2,658	-236
Loss before tax benefit	18,486	10,602	72,704
Tax benefit	-447	-	-
Net loss	18,039	10,602	72,704
Other comprehensive loss:			
Items that will be reclassified subsequently to profit or loss:			
Actuarial net gain of defined benefit plans	-	-	-100
Changes in the fair value of marketable securities	-	4	4
Total comprehensive loss	$ 18,039	10,606	$ 72,608
Net loss per share:			
Basic loss per share	$ 0.31	$ 0.31	$ 1.66
Diluted loss per share	$ 0.34	$ 0.31	$ 1.66
Weighted average share count	60,011,038	33,674,506	43,725,584

INTERIM CONSOLIDATED STATEMENTS OF CASH FLOWS
U.S. dollars in thousands (except share and per share data)

	Three months ended March 31,	Year ended December 31,

	2021	2020	2020
	Unaudited		Audited
Cash flows from operating activities:			
Net loss	$ (18,039)	$ (10,602)	$ (72,704)
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	635	550	2,397
Financial income (expense) net	485	-132	483
Share-based compensation	1,014	899	2,864
Change in employee benefit liabilities, net	-	-	94
Amortization of premium on available-for-sale financial assets	-	4	4
Revaluation of liabilities presented at fair value	-2,285	-2,448	6,822
Revaluation of liability to IIA	1,026	722	4,302
Deferred income taxes	-447	-	-
	428	-405	16,966
Changes in asset and liability items:			
Increase in prepaid expenses, other current assets and other assets	-523	-458	-1,626
Increase in trade payables	875	1,934	5,083
Increase (decrease) in accrued expenses and other payables	-2,724	-3,096	3,454
	-2,372	-1,620	6,911
Cash received during the period for:			
Interest received	-	348	361
Interest paid	-51	-47	-161
Net cash used in operating activities	-20,034	-12,326	-48,627
Cash flows from investing activities:			
Purchase of property, plant and equipment	-2,806	-2,119	-11,804
Proceeds from maturity of marketable securities	-	-	-158
Proceeds from sale of marketable securities	-	13,551	13,551
Net cash provided by (used in) investing activities	$ (2,806)	$ 11,432	$ 1,589

	Three months ended March 31,		Year ended December 31,
	2021	2020	2020
	Unaudited		Audited
Cash flows from financing activities:			
Proceeds from secondary offering, net	-	-	133,316
Receipt of grants from the IIA	52	53	399
Proceeds from issuance of convertible senior notes, net of issuance costs	71,012	-	-
Payment of lease liabilities	-664	-787	-1,985
Exercise of options	502	6	650
Payment of issuance costs related to public offering	-468	-	-
Net cash (used in) provided by financing activities	70,434	-728	132,380

Exchange differences on balances of cash and cash equivalents	34	76	-10
Increase (decrease) in cash and cash equivalents	47,628	-1,546	85,332
Cash and cash equivalents at beginning of period	127,170	41,838	41,838
Cash and cash equivalents at end of period	$ 174,798	$ 40,292	$ 127,170
Supplemental disclosure of non-cash financing activities:			
Significant non-cash transactions:			
Lease liabilities arising from new right-of-use asset	$ -	$ -	$ 3,409
IIA liability for grants to be received	$ 49	$ -	$ 103
Issuance expenses on credit	$ 235	$ -	$ 468
Purchase of property, plant and equipment on credit	$ 216	$ 206	$ 415

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