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

November 15, 2021

- *New data being presented at American Society of Hematology (ASH) Annual Meeting demonstrating GDA-201 overall survival rate of 78% at two years with a median duration of response of 16 months and long-term clinical benefit of omidubicel with long-lasting hematopoietic recovery*
- *Finished third quarter of 2021 with $121 million in cash; reassessing expected spending and prior financial guidance due to the revised timing of the omidubicel BLA submission*
- *Company to host conference call at 8:00 a.m. ET today*

BOSTON--(BUSINESS WIRE)--Nov. 15, 2021-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for cancer and other serious diseases, today provided a business update and reported financial results for the quarter ended September 30, 2021. Net loss for the third quarter of 2021 was $19.6 million, compared to a net loss of $14.8 million for the same period in 2020. As of September 30, 2021, Gamida Cell had total cash and cash equivalents of $120.8 million.

During the past quarter, Gamida Cell:

- Continued to execute on plans to submit a Biologic License Application (BLA) for omidubicel, a potentially life-saving treatment for patients with blood cancers in need of stem cell transplant. As previously disclosed, in a recent pre-BLA meeting, the FDA requested a revised analysis of the manufacturing data generated at Gamida Cell's wholly owned commercial manufacturing facility to demonstrate the comparability to the omidubicel that was produced at the clinical manufacturing sites for the Phase 3 study. The FDA did not request additional clinical data to initiate the BLA submission once analytical comparability is demonstrated.
- Progressed activities with objective to address the FDA's Clinical Hold on the Investigational New Drug (IND) application for GDA-201, which was imposed based on questions about donor eligibility procedures and sterility assay qualification prior to the initiation of the study in patients with follicular and diffuse large B-cell lymphomas.
- Expanded the company's NAM-enabled natural killer (NK) cell pipeline targeting solid-tumor and hematological cancers, including genetically modified variants of proprietary NK therapies using both CRISPR/Cas9 and CAR methodologies.

"We are committed to advance our programs and bring our important potential therapies to patients as quickly as possible. We are working diligently to respond to the FDA's information requests for omidubicel and GDA-201, and now expect to submit the BLA for omidubicel to the FDA in the first half of 2022 and we hope to promptly address outstanding issues regarding our IND application relating to GDA-201." said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "Additionally, at our recent NK-focused R&D Day, we provided details on our genetically modified NK cell immunotherapy programs leveraging CAR- and CRISPR-mediated strategies against hematologic malignancies and solid tumors. The company remains focused on our goal of bringing patients with cancer potentially curative cell therapies."

Recent Developments and Planned Presentations at ASH

Omidubicel: Advanced Cell Therapy

- **BLA Submission:** During a recent pre-BLA meeting, the FDA requested that Gamida Cell provide revised analysis of the manufacturing data generated at Gamida Cell's wholly owned commercial manufacturing facility. Upon completing those requirements, the company anticipates submitting the BLA in the first half of 2022.
- **New data to be presented at ASH:** Gamida Cell will have three omidubicel presentations - two presentations of additional data from the phase III randomized trial of omidubicel, and a poster presentation summarizing long term omidubicel data from multiple studies - at the 63rd American Society of Hematology (ASH) Annual Meeting and Exposition (December 11-14, 2021).
    - Oral presentation of "Hematopoietic Stem Cell Transplantation (HSCT) with Omidubicel is Associated with Robust Immune Reconstitution and Lower Rates of Severe Infection Compared to Standard Umbilical Cord Blood Transplantation" on Saturday, December 11, 2021, at 4:30 p.m. ET. Data collected from a subset of 37 patients in the omidubicel Phase III trial shows that, in addition to more rapid short-term hematopoietic recovery, omidubicel-treated patients had more rapid recovery of a wide variety of immune cells including CD4+ T cells, B cells, monocytes, natural killer cells, and dendritic cells. The robust recovery of the broad range of the immune system correlated with and supports clinical data showing fewer severe bacterial, fungal, and viral infections in patients treated with omidubicel.
    - Poster presentation of "Hospitalization and Healthcare Resource Use of Omidubicel vs. Cord Blood Transplantation

for Hematological Malignancies in a Global Randomized Phase III Clinical Trial" on Monday, December 13, 2021, 6:00-8:00 p.m. ET. Resource utilization data during the first 100 days after transplant were analyzed for 108 patients in the phase III trial and shows that omidubicel-treated patients has significantly shorter durations of hospitalization, intensive care unit time, consultant visits, procedures, and transfusions than the control arm. These data provide further evidence of the clinical benefit associated with the more rapid hematopoietic recovery in patients treated with omidubicel and the corresponding reduction in healthcare resource utilization.

- Poster presentation, "Allogeneic Stem Cell Transplantation with Omidubicel: Long-Term Follow-up from a Single Center" on Saturday, December 11, 2021, 5:30-7:30 p.m. ET. Analysis of outcomes of 22 patients with hematologic malignancies treated with omidubicel at Duke University over a 10-year period shows long-term sustained bone marrow function and immune recovery, with a 10-year overall survival of 48%. These data provide further support for the long-term clinical benefit of omidubicel with long-lasting hematopoietic recovery.

GDA-201: NAM-Enabled NK Cell Therapy

- **IND for Phase 1/2 Study:** Gamida Cell is working to address the clinical hold on the IND for a Phase 1/2 study of GDA-201. As a result of the clinical hold, the initiation of our planned Phase 1/2 study of GDA-201 will be delayed beyond the end of 2021, as the company previously projected.
- **New data presented at SITC**: Gamida Cell recently presented promising new preclinical data in two posters characterizing the NAM-enabled mechanisms of action that contribute to the metabolic modulation properties and enhanced tumor cytotoxicity activity of GDA-201 at the Society for Immunotherapy of Cancer's 36 th Annual Meeting (SITC 2021) held from November 10-14, 2021.
- **New data to be presented at ASH:** A poster titled "GDA-201, A Novel Metabolically Enhanced Allogeneic Natural Killer (NK) Cell Product Yields High Remission Rates in Patients with Relapsed/Refractory Non-Hodgkin Lymphoma (NHL): 2-year survival and correlation with cytokine IL7" will be presented at the upcoming ASH Annual Meeting and Exposition on Monday, December 13, 2021, 6:00-8:00 p.m. ET. This analysis provides longer follow-up in the investigator-led study of GDA-201 in patients with non-Hodgkin lymphoma and demonstrated an overall survival rate of 78% at two years, median duration of response of 16 months, and a safety profile that was similar to what had been previously reported.

NAM-Enabled NK Cell Pipeline Expansion

- **Advanced NAM-enabled genetically modified NK pipeline:** During Gamida Cell's NK-focused virtual R&D Day, the company presented new data and additional details on its genetically modified NK cell immunotherapy programs, which utilize CAR, membrane bound- and CRISPR-mediated strategies to increase targeting, potency and persistence against hematologic malignancies and solid tumors:
  - GDA-301: Knockout of CISH (cytokine inducible SH2 containing protein) in NK cells using CRISPR/Cas9 in combination with a membrane-bound IL-15/IL-15Ra;
  - GDA-501: anti HER2 CAR-engineered NK cells to target solid tumors expressing HER2, based on a single-chain variable fragment of the widely used humanized monoclonal antibody trastuzumab; and
  - GDA-601: CRISPR Knockout of CD38 on NK cells combined with anti CD38 CAR. CD38 is an established immunotherapeutic target in multiple myeloma, but its expression on NK cells and its further induction during ex vivo NK cell expansion represents a barrier to the development of an anti CD38 CAR-NK cell therapy. Gamida Cell recently announced a research collaboration with the Dana-Farber Cancer Institute to study the in vitro cytotoxicity of GDA-601 in fresh samples from multiple myeloma patients.
- **New data presented at PEGS Europe:** Data from early-stage studies of GDA-501 demonstrated enhanced potency and cytotoxicity against a HER2-expressing tumor cell line. Data presented on GDA-301 showed cytotoxic activity against a chronic myelogenous leukemia cell line (K562) and a multiple myeloma cell line (RPMI). These data were presented at the 13th Annual Protein and Antibody Engineering Summit (PEGS) in Barcelona, Spain November 2-4, 2021.

Third Quarter 2021 Financial Results

- Research and development expenses in the third quarter of 2021 were $12.4 million, compared to $10.5 million for the same period in 2020. The increase was mainly due to omidubicel commercial manufacturing readiness activities, and the advancement of the GDA-201 program, including broadening scientific capabilities and talent.
- Commercial expenses in the third quarter of 2021 were $6.0 million, compared to $1.9 million for the third quarter of 2020. The increase was mainly attributed to progress with omidubicel commercial readiness activities. Going forward, the company anticipates reducing its near-term commercial readiness expenses in line with the revised omidubicel BLA submission timing.
- General and administrative expenses were $4.8 million for the third quarter of 2021, compared to $2.7 million for the same period in 2020. The increase was mainly due to professional services and the hiring of key management positions, to support business growth.
- Finance income, net, was $3.5 million for the third quarter of 2021, compared to $0.3 million for the third quarter of 2020.

The increase was primarily due to non-cash income, resulting from revaluation of warrants offset by convertible note interest expenses.

Net loss for the third quarter of 2021 was $19.6 million, compared to a net loss of $14.8 million for the same period in 2020.

2021 Financial Guidance

Gamida Cell is re-assessing its planned spending and prior financial guidance as a result of the revised timing of the expected omidubicel BLA submission.

Expected Milestones in 2022

Omidubicel

- BLA submission to the FDA in the first half of 2022

GDA-201

- Initiation of a company-sponsored Phase 1/2 clinical study in NHL in 2022

NK cell pipeline expansion

- Establish preclinical proof of concept studies of the NAM-enabled, genetically modified NK therapeutic targets in 2022
- Select pipeline candidate(s) for IND enabling studies by end of 2022

Conference Call Information

Gamida Cell will host a conference call today, November 15, 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 4347485. 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 blood cancers. Omidubicel is the first bone marrow transplant graft to receive Breakthrough Therapy Designation from the U.S. FDA and has also received Orphan Drug Designation in the U.S. and EU. For more information about omidubicel, please visit https://www.gamida-cell.com.

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 nicotinamide (NAM)-enabled 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, the lead candidate in the NAM-enabled NK cell pipeline, has demonstrated promising initial clinical trial results. 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. Furthermore, GDA-201 improves antibody-dependent cellular cytotoxicity (ADCC) and tumor targeting of NK cells. For more information about GDA-201, please visit https://www.gamida-cell.com.

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 pioneering a diverse immunotherapy pipeline of potentially curative cell therapies for patients with solid tumor and blood cancers and other serious blood diseases. We apply a proprietary expansion platform leveraging the properties of NAM to allogeneic cell sources including umbilical cord blood-derived cells and NK cells to create therapies with potential to redefine standards of care. These include omidubicel, an investigational product with potential as a life-saving alternative for patients in need of bone marrow transplant, and a line of modified and unmodified NAM-enabled NK cells targeted at solid tumor and hematological malignancies. For additional information, please visit www.gamida-cell.com or follow Gamida Cell on LinkedIn, Twitter, Facebook or Instagram 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 (including GDA-201), anticipated regulatory filings (including the timing of submission of the BLA for omidubicel to the FDA), commercialization planning efforts, and the potentially life-saving or curative therapeutic and commercial potential of omidubicel, and Gamida Cell's expectations for the expected clinical development milestones set forth herein.. Any statement describing Gamida Cell's goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to a number of risks, uncertainties and assumptions, including those related to the impact that the COVID-19 pandemic could have on our business, and including the scope, progress and expansion of Gamida Cell's clinical trials and ramifications for the cost thereof; clinical, scientific, regulatory and technical developments; and those inherent in the process of developing and commercializing product candidates that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such product candidates. 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. Although Gamida Cell's forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Gamida Cell. As a result, you are cautioned not to rely on these forward-looking statements.

INTERIM CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

U.S. dollars in thousands

	September 30,		December 31,
	2021	2020	2020
	Unaudited		Audited
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	$ 80,613	$ 73,311	$ 127,170
Marketable securities	40,223	-	-
Prepaid expenses and other current assets	2,785	1,734	2,815
Total current assets	123,621	75,045	129,985
NON-CURRENT ASSETS:			
Property, plant and equipment, net	30,023	15,838	18,238
Right-of-use assets	4,918	7,023	6,474
Other assets	6,599	802	786
Total non-current assets	41,540	23,663	25,498
Total assets	$ 165,161	$ 98,708	$ 155,483

INTERIM CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

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

	September 30,		December 31,
	2021	2020	2020
	Unaudited		Audited
LIABILITIES AND EQUITY			
CURRENT LIABILITIES:			
Trade payables	$ 7,833	$ 2,704	$ 6,329
Employees and payroll accruals	5,870	3,872	4,705
Current maturities of lease liabilities	1,622	2,345	2,532
Accrued interest	525	-	-
Accrued expenses and other payables	7,810	5,005	7,988
Total current liabilities	23,660	13,926	21,554
NON-CURRENT LIABILITIES:			
Liabilities presented at fair value	-	3,252	12,043
Employee benefit liabilities, net	768	773	768
Other long-term liabilities	4,621	5,460	5,378
Liability to Israel Innovation Authority	20,858	14,729	17,003
Convertible senior notes, net	69,298	-	-
Total non-current liabilities	95,545	24,214	35,192
SHAREHOLDERS' EQUITY:			

Share capital -			
Ordinary shares of NIS 0.01 par value - Authorized: 100,000,000 shares at September 30, 2021 and 2020 (unaudited) and December 31, 2020; Issued and outstanding: 59,298,846 and 49,556,663 shares at September 30, 2021 and 2020 (unaudited), respectively and 59,000,153 shares at December 31, 2020.	167	138	166
Share premium	381,504	304,944	375,280
Capital reserve	(441)	(541)	(441)
Reserve from financial assets measured at FVOCI	(42)	-	-
Accumulated deficit	(335, 232)	(243,973)	(276,268)
Total shareholders' equity	45,956	60,568	98,737
Total liabilities and shareholders' equity	$ 165,161	$ 98,708	$ 155,483

INTERIM CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

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,
	2021	2020	2021	2020	2020
	Unaudited		Unaudited		Audited
Operating expenses:					
Research and development, net	$ 37,213	$ 27,652	$ 12,396	$ 10,454	$ 41,385
Commercial activities	15,633	4,413	5,973	1,916	8,748
General and administrative	12,004	8,180	4,774	2,690	12,167
Operating loss	64,850	40,245	23,143	15,060	62,300
Finance expense	6,330	2,367	2,218	1,001	10,640
Finance income	(11,769)	(2,203)	(5,727)	(1,309)	(236)
Loss before tax benefit	59,411	40,409	19,634	14,752	72,704
Tax benefit	(447)	-	-	-	-
Net loss	58,964	40,409	19,634	14,752	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	42	4	17	-	4
Total comprehensive loss	$ 59,006	$ 40,413	$ 19,651	$ 14,752	$ 72,608
Net loss per share:					
Basic loss per share	$ 1.00	$ 0.98	$ 0.33	$ 0.30	$ 1.66
Diluted loss per share	$ 1.18	$ 0.98	$ 0.33	$ 0.30	$ 1.66
Weighted average share count	59,219,757	41,281,970	59,281,243	49,472,749	43,725,584

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,
	2021	2020	2021	2020	2020
	Unaudited		Unaudited		Audited
Cash flows from operating activities:					
Net loss	$ (58,964)	$ (40,409)	$ (19,634)	$ (14,752)	$ (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	1,898	1,716	621	610	2,397
Financial (income) expense, net	1,613	(169)	606	91	483
Share-based compensation	3,976	1,969	1,513	748	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 derivatives	(11,257)	(1,969)	(5,447)	(1,299)	6,822
Revaluation of liability to IIA	3,170	2,227	1,312	912	4,302
Deferred income taxes	(447)	-		-	-
	(1,047)	3,778	(1,395)	1,062	16,966
Changes in asset and liability items:					
Decrease (increase) in prepaid expenses, other current assets, and other assets	1,005	(718)	937	347	(1,626)
Increase (decrease) in trade payables	1,504	1,535	2,397	(39)	5,083
Increase (decrease) in accrued expenses and other payables	(894)	516	(693)	1,141	3,454
	1,615	1,333	2,641	1,449	6,911
Cash received during the period for:					
Interest received	1,122	359	854	2	361
Interest paid	(128)	(120)	(43)	(40)	(161)
	994	239	811	(38)	200
Net cash used in operating activities	(57,402)	(35,059)	(17,577)	(12,279)	(48,627)
Cash flows from investing activities:					
Purchase of property, plant and equipment	(9,577)	(9,792)	(4,187)	(2,683)	(11,804)
Investment in long-term deposit	(5,803)	-	(4,803)	-	-
Purchase of marketable securities	(97,808)	-	(29,657)	-	-
Investment in restricted bank deposits	-	-	-	-	(158)
Proceeds from maturity of marketable securities	56,717	-	38,893	-	-
Proceeds from sale of marketable securities	-	13,551	-	-	13,551
Net cash provided by (used in) investing activities	$ (56,471)	$ 3,759	$ 246	$ (2,683)	$ 1,589

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,
	2021	2020	2021	2020	2020
	Unaudited		Unaudited		Audited
Cash flows from financing activities:					
Proceeds from secondary offering, net	-	-	-	-	133,316
Receipt of grants from the IIA	311	200	259	-	399
Proceeds from secondary offering, net	-	63,860	-	-	-
Proceeds from issuance of convertible senior notes, net of issuance costs	70,777	-	-	-	-
Payment of lease liabilities	(1,782)	(1,539)	(653)	(417)	(1,985)
Payment of interest of Convertible senior notes	(2,191)	-	(2,191)	-	-
Exercise of options	566	169	10	21	650
Payment of issuance costs related to public offering	(468)	-	-	-	-
Net cash provided by (used in) financing activities	67,213	62,690	(2,575)	(396)	132,380
Exchange differences on balances of cash and cash equivalents	103	83	29	31	(10)
Increase (decrease) in cash and cash equivalents	(46,557)	31,473	(19,877)	(15,327)	85,332
Cash and cash equivalents at beginning of period	127,170	41,838	100,490	88,638	41,838
Cash and cash equivalents at end of period	$ 80,613	$ 73,311	$ 80,613	$ 73,311	$ 127,170
Supplemental disclosure of non-cash financing activities:					
Significant non-cash transactions:					
Lease liabilities arising from new right-of-use asset	$ -	$ 3,376	$ -	$ -	$ 3,409
IIA liability for grants to be received	$ 590	$ -	$ 590	$ -	$ 103
Issuance expenses on credit	$ -	$ -	$ -	$ -	$ 468
Purchase of property, plant and equipment on credit	$ 1,561	$ -	$ 1,561	$ -	$ 415
Borrowing costs capitalization	$ 1,287	$ -	$ 713	$ -	$ -

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For investors:
Courtney Turiano
Stern Investor Relations, Inc.
Courtney.Turiano@sternir.com
1-212-362-1200

For media:
Rhiannon Jeselonis
Ten Bridge Communications

rhiannon@tenbridgecommunications.com
1-978-417-1946

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