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

August 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*
- *Commercial readiness activities underway to support potential launch in 2022*
- *Phase 1/2 clinical trial of GDA-201 in patients with follicular and diffuse large B-cell lymphomas expected to start by the end of the year*
- *Four new development programs announced leveraging next-generation, NAM-enabled, genetically-modified NK cells in solid tumor and hematological cancers*
- *Company to host conference call at 8:00 a.m. ET today*

BOSTON--(BUSINESS WIRE)--Aug. 11, 2021-- Gamida Cell Ltd. (Nasdaq: GMDA), an advanced cell therapy company committed to cures for cancer and other serious diseases, today reported financial results for the quarter ended June 30, 2021. Net loss for the second quarter of 2021 was $21.3 million, compared to a net loss of $15.1 million for the same period in 2020. As of June 30, 2021, Gamida Cell had total cash and cash equivalents of $150.2 million.

During the quarter, the company 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. This submission is expected to occur by the end of the year, subject to a pre-BLA meeting with the U.S. Food and Drug Administration (FDA) planned for the fourth quarter. In addition, Gamida prepared to begin a Phase 1/2 trial of GDA-201 in non-Hodgkin lymphoma (NHL), expected to occur by the end of 2021. Also, the company expanded its 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.

“Our progress this quarter represents a major step forward for Gamida Cell and our mission to bring cancer patients potentially curative cell therapies,” said Julian Adams, Ph.D., chief executive officer of Gamida Cell. “We are delivering against key process development, quality and manufacturing milestones in preparation for a BLA submission for omidubicel while also advancing our go-to-market strategy for our planned commercial launch. In parallel, we bolstered our NAM-enabled NK pipeline both by readying to advance GDA-201 into the clinic based on its encouraging clinical data in patients with hematological cancers and by expanding our NK cell pipeline to address solid and liquid tumors.”

Q2 and Recent Developments

Omidubicel: Advanced Cell Therapy

- Continued advancement toward planned BLA submission for omidubicel to the FDA in the fourth quarter of this year. The company’s activities included CMC qualification requirements at both the Gamida–owned facility in Israel and at Lonza, a contract manufacturing organization that will be supplying commercial material upon FDA approval. Advancements were made in analytical methods validation, analytical comparability and clinical manufacturing for Expanded Access Program patients, which are also planned to be used for clinical comparability.
- Advanced launch planning activities by expanding Gamida’s commercial, operational and medical affairs teams. Conducted further market research and health economic and outcomes research (HEOR) to support planned market entry and market access activities. Readied Gamida Cell Assist, supply chain and logistics programs to facilitate positive patient and transplant center experiences at time of launch.
- Announced that results of the international, multi-center, randomized Phase 3 clinical study of omidubicel were published in *Blood*, the official journal of the American Society of Hematology. This pivotal trial compared the safety and efficacy of omidubicel to standard umbilical cord blood transplant in patients with high-risk hematologic malignancies undergoing a bone marrow transplant. The results demonstrate that transplantation with omidubicel leads to faster neutrophil and platelet recovery, and results in fewer bacterial, viral and fungal infections and less time in the hospital, compared to a standard umbilical cord blood graft.

GDA-201: NAM-Enabled NK Immunotherapy

- Prepared for filing of an Investigational New Drug (IND) application with the FDA.
- Finalized clinical study protocol and statistical plan for a planned Phase 1/2 clinical trial of allogeneic, cryopreserved GDA-201 in patients with follicular and diffuse large B-cell lymphoma.
- Conducted study start-up activities, including contract research organization (CRO) and clinical site selections.

NAM-Enabled NK Cell Pipeline Expansion

- Advanced four new development programs that involve modifications intended to direct NK cells against specific tumor markers to improve their cancer killing capabilities against both hematological and solid tumors. Newly designated product candidates include:
    - 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. Designed to improve tumor killing by promoting activation and inhibiting negative feedback signals. Potential applications exist across a range of solid tumors and lymphoma.
    - GDA-401: Undisclosed target genetically engineered to enhance NK cell survival in the solid tumor microenvironment for potential application across a broad range of solid tumors.
    - GDA-501: CAR-engineered NK cells to target HER2+ solid tumors with the potential to enhance homing and activation against cancers with HER2 overexpression, including breast, ovarian, lung, bladder, gastric and others.
    - GDA-601: Knockout of CD38 on NK cells to avoid fratricide by CD38 targeted antibodies in combination treatment of multiple myeloma, combined with a CD38 CAR designed to enhance killing of cancerous cells.
- Advanced additional NAM-enabled research programs targeting immunosuppressive pathways using both CRISPR/Cas9 and CAR, with potential to treat solid tumor and blood cancers.

Corporate

- Hired Vladimir Melnikov as Senior Vice President, Global Operations and Manufacturing. Vladimir has over 25 years of experience in the biopharmaceutical industry. He previously served as general manager at Omrix Biopharmaceuticals and biologic technical operations lead at Ethicon Biosurgery, both part of a Johnson & Johnson Company. In those roles he supervised three Israeli biotech manufacturing sites and technology transfer to external partners. Vladimir will have responsibility for the company's Israeli manufacturing site and manufacturing partnership with Lonza.
- Hired Josh Patterson as General Counsel, effective August 30, 2021. Josh has over 20 years of experience as in-house legal counsel for biopharmaceutical companies. Josh will be joining Gamida Cell from Akcea Therapeutics, a wholly owned subsidiary of Ionis Pharmaceuticals, where he is currently General Counsel. Josh will be responsible for building, leading and managing the legal function for Gamida Cell.

Second Quarter 2021 Financial Results

- Research and development expenses in the second quarter of 2021 were $13.5 million, compared to $9.3 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 second quarter of 2021 were $5.2 million, compared to $1.0 million for the second quarter of 2020. The increase was mainly attributed to progress with omidubicel commercial readiness activities.
- General and administrative expenses were $3.8 million for the second quarter of 2021, compared to $2.5 million for the same period in 2020. The increase was mainly due to the hiring of key management positions to support business growth.
- Finance income, net, was $1.2 million for the second quarter of 2021, compared to $2.2 million for the second quarter of 2020. The increase was primarily due to non-cash income, resulting from revaluation of warrants offset by interest expenses that resulted from the $75 million convertible note financing in February 2021.

Net loss for the second quarter of 2021 was $21.3 million, compared to a net loss of $15.1 million for the same period in 2020.

2021 Financial Guidance

Gamida Cell reiterates its prior financial guidance and expects cash used for ongoing operating activities in 2021 to range from $110 million to $120 million. The company believes that its current cash and cash equivalents will support the 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 Developments and Milestones

Gamida Cell plans to achieve the following key milestones during the second half of 2021:

Omidubicel

- Pre-BLA meeting with FDA in the fourth quarter of 2021
- BLA submission to the FDA in the fourth quarter of 2021
- Commercial readiness activities ongoing for potential launch following approval

GDA-201

- IND submission to FDA in third quarter 2021
- Initiation of a company-sponsored Phase 1/2 clinical study in NHL before year-end 2021

NK cell pipeline expansion

- Advance pipeline of NAM-enabled, genetically-modified NK cells in solid tumor and blood cancers

Conference Call Information

Gamida Cell will host a conference call today, August 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 potentially life-saving[1] 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.[2,3] 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.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)-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, the lead candidate in the NAM-enabled NK cell pipeline, has demonstrated promising initial clinical trial results, as reported at the 2020 American Society of Hematology (ASH) Annual Meeting & Exposition. 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 has been in development through an investigator-sponsored study in patients with refractory NHL and multiple myeloma. 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 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, Instagram, or YouTube 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 (including the submission of the BLA for omidubicel to the FDA), commercialization planning efforts, the potentially life-saving or curative therapeutic and commercial potential of omidubicel, and Gamida Cell's expectations regarding its projected cash to be used for operating activities and cash runway. 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.

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[1] Gragert et al. HLA Match Likelihoods for Hematopoietic Stem-Cell Grafts in the U.S. Registry. N Engl J Med 2014;371:339-48. Bejanyan et al. Myeloablative Conditioning for Allogeneic Transplantation Results in Superior Disease-Free Survival for Acute Myelogenous Leukemia and Myelodysplastic Syndromes with Low/Intermediate but not High Disease Risk Index: A Center for International Blood and Marrow Transplant Research Study. Biol Blood Marrow Transplant 00 (2020) 1-9.

[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] Horwitz M.E., et al. Blood. 2021 Jun 22;blood.2021011719. doi: 10.1182/blood.2021011719. Online ahead of print.

[4] Bachanova et al. ASH 2020 abstract

INTERIM CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

U.S. dollars in thousands

	June 30,		December 31,
	2021	2020	2020
	Unaudited		
ASSETS			
CURRENT ASSETS:			
Cash and cash equivalents	$ 100,490	$ 88,638	$ 127,170
Marketable securities	49,702	-	-
Prepaid expenses and other current assets	3,730	2,241	2,815
Total current assets	153,922	90,879	129,985
NON-CURRENT ASSETS:			
Property, plant and equipment, net	25,607	14,204	18,238
Right-of-use assets	5,404	7,490	6,474
Other assets	1,787	642	786
Total non-current assets	32,798	22,336	25,498
Total assets	$ 186,720	$ 113,215	$ 155,483
LIABILITIES AND EQUITY			
CURRENT LIABILITIES:			
Trade payables	$ 5,435	$ 2,738	$ 6,329
Employees and payroll accruals	4,796	3,187	4,705
Current maturities of lease liabilities	1,937	2,145	2,532
Accrued interest	1,618	-	-
Accrued expenses and other payables	8,839	5,509	7,988
Total current liabilities	22,625	13,579	21,554
NON-CURRENT LIABILITIES:			
Liabilities presented at fair value	6,233	4,551	12,043
Employee benefit liabilities, net	768	773	768
Other long-term liabilities	4,839	5,946	5,378
Liability to Israel Innovation Authority	19,146	13,816	17,003
Convertible senior notes, net	69,025	-	-
Total non-current liabilities	100,011	25,086	35,192
SHAREHOLDERS' EQUITY:			
Share capital	167	137	166
Share premium	379,981	304,175	375,280
Capital reserve	(441)	(541)	(441)
Reserve from financial assets measured at FVOCI	(25)	-	-
Accumulated deficit	(315,598)	(229,221)	(276,268)
Total shareholders' equity	64,084	74,550	98,737
Total liabilities and shareholders' equity	$ 186,720	$ 113,215	$ 155,483

INTERIM CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

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

	Six months ended June 30,		Three months ended June 30,		Year ended December 31,

	2021	2020	2021	2020	2020
	Unaudited		Unaudited		
Operating expenses:					
Research and development, net	$ 24,817	$ 17,198	$ 13,451	$ 9,319	$ 41,385
Commercial activities	9,660	2,497	5,230	1,029	8,748
General and administrative	7,230	5,490	3,817	2,496	12,167
Operating loss	41,707	25,185	22,498	12,844	62,300
Finance expense	4,150	1,366	2,594	2,320	10,640
Finance income	(6,080)	(894)	(3,801)	(109)	(236)
Loss before tax benefit	39,777	25,657	21,291	15,055	72,704
Tax benefit	(447)	-	-	-	-
Net loss	39,330	25,657	21,291	15,055	72,704
Net loss per share:					
Basic loss per share	$ 0.66	$ 0.69	$ 0.36	$ 0.37	$ 1.66
Diluted loss per share	$ 0.76	$ 0.69	$ 0.42	$ 0.37	$ 1.66
Weighted average share count	59,725,076	37,141,582	59,336,633	49,589,719	43,725,584

INTERIM CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Six months ended June 30,		Three months ended June 30,		Year ended December 31,
	2021	2020	2021	2020	2020
	Unaudited		Unaudited		
Cash flows from operating activities:					
Net loss	$(39,330)	$(25,657)	$ (21,291)	$ (15,055)	$ (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	1,277	1,106	642	556	2,397
Financial (income) expense, net	1,007	(260)	522	(128)	483
Share-based compensation	2,463	1,221	1,449	322	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	(5,810)	(670)	(3,525)	1,778	6,822
Revaluation of liability to IIA	1,858	1,315	832	593	4,302
Deferred income taxes	(447)	-		-	-
	348	2,716	(80)	3,121	16,966
Changes in asset and liability items:					
Decrease (increase) in prepaid expenses, other current assets, and other assets	68	(1,065)	591	(607)	(1,626)
Increase (decrease) in trade payables	(893)	1,574	(1,768)	(360)	5,083
Increase (decrease) in accrued expenses and other payables	(201)	(624)	2,523	2,472	3,454
	(1,026)	(115)	1,346	1,505	6,911
Cash received during the period for:					

Interest received	268	357	268	9	361
Interest paid	(85)	(80)	(34)	(33)	(161)
Net cash used in operating activities	(39,825)	(22,779)	(19,791)	(10,453)	(48,627)
Cash flows from investing activities:					
Purchase of property, plant and equipment	(5,390)	(7,109)	(2,584)	(4,990)	(11,804)
Investment in long term deposit	(1,000)	-	(1,000)	-	-
Purchase of marketable securities	(68,151)	-	(68,151)	-	-
Proceeds from maturity of marketable securities	17,824	-	17,824	-	(158)
Proceeds from sale of marketable securities	-	13,551	-	-	13,551
Net cash provided by (used in) investing activities	$(56,717)	$ 6,442	$ (53,911)	$ (4,990)	$ 1,589

INTERIM CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Six months ended June 30		Three months ended June 30		Year ended December 31,
	2021	2020	2021	2020	2020
	Unaudited				
Cash flows from financing activities:					
Proceeds from secondary offering, net	-	-	-	-	133,316
Receipt of grants from the IIA	52	200	-	147	399
Proceeds from secondary offering, net	-	63,860	-	63,860	-
Proceeds from issuance of convertible senior notes, net of issuance costs	70,777	-	(235)		-
Payment of lease liabilities	(1,129)	(1,122)	(465)	(335)	(1,985)
Exercise of options	556	147	54	141	650
Payment of issuance costs related to public offering	(468)	-	-	-	-
Net cash (used in) provided by financing activities	69,788	63,085	(646)	63,813	132,380
Exchange differences on balances of cash and cash equivalents	74	52	40	(24)	(10)
Increase (decrease) in cash and cash equivalents	(26,680)	46,800	(74,308)	48,346	85,332
Cash and cash equivalents at beginning of period	127,170	41,838	174,798	40,292	41,838
Cash and cash equivalents at end of period	$100,490	$88,638	$ 100,490	$ 88,638	$ 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	$ 656	$ -	$ 607	$ -	$ 103
Issuance expenses on credit	$ -	$ -	$ -	$ -	$ 468
Purchase of property, plant and equipment on credit	$ 1,563	$ 960	$ 1,563	$ 960	$ 415
Borrowing costs capitalization	$ 574	$ -	$ 574	$ -	$ -

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