Gamida Cell Reports Fourth Quarter and Full Year 2021 Financial Results and Provides Company Update

March 15, 2022

- Initiated rolling BLA submission for omidubicel in the first quarter of 2022 with full BLA submission on track for first half of 2022 -

- Finished fourth quarter of 2021 with approximately $96 million in cash; sufficient cash to fund the company's operations into mid-2023 -

- Company to host conference call at 8:00 a.m. ET today -

BOSTON--(BUSINESS WIRE)--Mar. 15, 2022-- 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 year and quarter ended December 31, 2021. Net loss for 2021 was $89.8 million, compared to a net loss of $61.6 million in 2020. As of December 31, 2021, Gamida Cell had total cash and cash equivalents of $95.9 million.

During the past quarter and into 2022, Gamida Cell:

- Continued to advance omidubicel, a potentially life-saving cell therapy treatment for patients with blood cancers in need of stem cell transplant. In the first quarter of 2022, Gamida Cell initiated a rolling Biologics License Application (BLA) submission for omidubicel following the receipt of positive Type B meeting correspondence from the U.S. Food and Drug Administration (FDA).
- Progressed activities to address the FDA's clinical hold on the Investigational New Drug (IND) application for GDA-201, which was imposed based on FDA questions about donor eligibility procedures and assay qualification prior to the initiation of the study in patients with follicular and diffuse large B-cell lymphomas.
- Advanced the company's NAM-enabled natural killer (NK) cell pipeline, including targets GDA-301, GDA-501 and GDA-601, which focus on solid-tumor and hematological cancers. These targets utilize CAR, membrane bound- and CRISPR-mediated technologies to increase targeting, potency and persistence against hematologic malignancies and solid tumors.

"Throughout 2021, Gamida Cell made meaningful progress advancing our broad immunotherapy pipeline of potentially curative cell therapies for patients with solid tumor and blood cancers and other serious blood diseases, resulting in the recent initiation of our rolling BLA submission for omidubicel, which we expect to complete in the second quarter of this year. Additionally, we are continuing to work diligently to respond to the FDA regarding our IND for GDA-201, and expect to initiate our Phase 1/2 study in patients with follicular and diffuse large B-cell lymphomas once the clinical hold has been removed," said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "Looking ahead in 2022, we are continuing to progress our genetically modified NK cell immunotherapy programs leveraging CAR- and CRISPR-mediated technologies focused on addressing unmet needs for patients with hematologic malignancies and solid tumors and we will select a product candidate for IND-enabling studies by the end of the year."

Fourth Quarter and Recent Developments

Omidubicel: Advanced Cell Therapy

- **BLA submission:** Following the receipt of positive Type B meeting correspondence from the FDA confirming that analytical comparability has been established between Gamida Cell's wholly-owned commercial manufacturing facility and the product that was manufactured for the Phase 3 study, Gamida Cell initiated a rolling BLA submission for omidubicel in the first quarter of 2022. As part of the rolling BLA, Gamida Cell submitted the nonclinical and clinical modules of the omidubicel BLA and is on-track to complete submission of all remaining modules of the BLA by the first half of 2022. In parallel with the BLA submission, the company is assessing alternatives for the commercialization of omidubicel, including potential U.S. or global partnerships.
- **New data presented at ASH:** At the 63rd American Society of Hematology (ASH) Annual Meeting and Exposition in December 2021, Gamida Cell presented clinical updates and a health economic analysis on hospital resource utilization.
    - Data collected from a subset of patients in the omidubicel Phase 3 trial showed 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, NK cells, and dendritic cells than the control arm. The robust recovery of a broad range of the immune system correlated with and supported clinical data showing fewer severe bacterial, fungal, and viral infections in patients treated with omidubicel.
    - Resource utilization data during the first 100 days after transplant showed that omidubicel-treated patients had 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.
    - An analysis of outcomes of patients with hematologic malignancies treated with omidubicel over a 10-year period showed 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 diligently to address the clinical hold on the IND for a Phase 1/2 study of GDA-201. Gamida Cell expects to initiate a company-sponsored Phase 1/2 clinical study in patients with follicular and diffuse large B-cell lymphomas in 2022.
- **New data presented at ASH:** Gamida Cell presented 2-year survival and correlation data with cytokine IL7 at the ASH Annual Meeting and Exposition in December 2021. This analysis provided 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 with a median duration of response of 16 months.

NAM-Enabled NK Cell Pipeline Expansion

- **Advanced NAM-enabled genetically modified NK pipeline:** Gamida Cell continues to progress its NAM-enabled genetically modified NK pipeline, which utilizes CAR, membrane bound- and CRISPR-mediated technologies to increase targeting, potency and persistence against hematologic malignancies and solid tumors. The company plans to execute preclinical proof of concept studies for these genetically modified NK therapeutic targets and to select a product candidate for IND enabling studies by the end of 2022. These therapeutic targets 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;
    - 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 is advancing this program with a collaboration with the Dana-Farber Cancer Institute to study the in vitro cytotoxicity of GDA-601 in fresh samples from multiple myeloma patients.
    - GDA-401: A development candidate with the target still undisclosed.

Full Year 2021 Financial Results

- Research and development expenses were $50.2 million in 2021, compared to $38.9 million in 2020. The increase was primarily due to a $5.4 million increase in omidubicel commercial manufacturing readiness activities and advancing the NK programs, as well as an increase of $5.9 million in broadening the company scientific capabilities and talent.
- Commercial expenses in 2021 were $20.0 million, compared to $8.9 million in 2020. The increase was attributable mainly to a $6.5 million increase in commercial readiness expenses and a $4.6 million increase in headcount within the commercial organization. Going forward, the company anticipates reducing its near-term commercial readiness expenses, as it is assessing alternatives for the commercialization of omidubicel, including potential U.S. or global partnerships.
- General and administrative expenses were $17.0 million in 2021, compared to $13.2 million in 2020. The increase was mainly due to a $2.6 million increase in professional services expenses and a $1.2 million increase in headcount and related expenses.
- Finance expenses, net, were $2.6 million for 2021, compared to $0.6 million for 2020. The increase was primarily due to a $4.4 million interest expenses from convertible notes, offset by a $2.1 million capitalization and other non-cash expenses, and a $0.3 million increase in interest income from cash management.
- Net loss for 2021 was $89.8 million, compared to a net loss of $61.6 million in 2020.

2022 Financial Guidance

Gamida Cell expects cash used for ongoing operating activities in 2022 to range from $60 million to $70 million.

Gamida Cell expects that its current cash and cash equivalents will support the company's ongoing operating activities into mid 2023. This cash runaway 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 Milestones in 2022

Omidubicel

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

GDA-201

- Initiation of a company-sponsored Phase 1/2 clinical study in follicular and diffuse large B-cell lymphomas

NK cell pipeline expansion

- Establish preclinical proof of concept studies of the NAM-enabled, genetically modified NK therapeutic targets
- Select pipeline candidate for IND-enabling studies

Conference Call Information

Gamida Cell will host a conference call today, March 15, 2022, 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 1696742. 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 for patients with blood cancers. Omidubicel is the first stem cell transplant donor source to receive Breakthrough Therapy Designation from the U.S. FDA and has also received Orphan Drug Designation in the U.S. and EU. Gamida Cell has completed an international, multi-center, randomized Phase 3 study (NCT0273029) evaluating the safety and efficacy of omidubicel in patients with hematologic malignancies undergoing allogeneic bone marrow transplant compared to a comparator group of patients who received a standard umbilical cord blood transplant. That study achieved its primary endpoint, demonstrating a highly statistically significant reduction in time to neutrophil engraftment, a key milestone in a patient's recovery from a stem cell transplant. The Phase 3 study also achieved its secondary endpoints of reduced time to platelet engraftment, reduced infections and fewer days of hospitalization. Gamida Cell initiated a rolling BLA submission for omidubicel in the first quarter of 2022 with full BLA submission on track for first half of 2022. 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. Gamida Cell expects to initiate a company-sponsored Phase 1/2 clinical study of GDA-201 in patients with follicular and diffuse large B-cell lymphomas in 2022. 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 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.

CONSOLIDATED BALANCE SHEETS

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

	December 31,

	2021	2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 55,892	$127,170
Marketable securities	40,034	-
Prepaid expenses and other current assets	2,688	3,087
Total current assets	98,614	130,257
NON-CURRENT ASSETS:		
Restricted deposits	3,961	-
Property, plant and equipment, net	35,180	18,238
Operating lease right-of-use assets	7,236	6,841
Severance pay fund	2,148	2,191
Other long-term assets	1,647	786
Total non-current assets	50,172	28,056
Total assets	$148,786	$158,313

CONSOLIDATED BALANCE SHEETS

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

	December 31,	
	2021	2020
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Trade payables	$ 8,272	$ 6,331
Employees and payroll accruals	4,957	4,705
Operating lease liabilities	2,699	2,475
Accrued interest of convertible senior notes	1,640	-
Accrued expenses and current liabilities	7,865	7,988
	25,433	21,499
NON-CURRENT LIABILITIES:		
Convertible senior notes, net	71,417	-
Accrued severance pay	2,396	2,426
Long-term operating lease liabilities	5,603	5,517
Total non-current liabilities	79,416	7,943
CONTINGENT LIABILITIES AND COMMITMENTS		
SHAREHOLDERS' EQUITY:		
Ordinary shares of NIS 0.01 par value - Authorized: 150,000,000 shares at December 31, 2021 and 100,000,000 shares at December 31, 2020; Issued and outstanding: 59,970,389 and 59,000,153 shares at December 31, 2021 and 2020, respectively	169	166
Additional paid-in capital	381,225	376,369
Accumulated deficit	(337,457)	(247,664)
Total shareholders' equity	43,937	128,871
Total liabilities and shareholders' equity	$ 148,786	$ 158,313

CONSOLIDATED STATEMENTS OF OPERATIONS

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

	Year ended December 31,	
	2021	2020
Research and development expenses, net	$ 50,177	$ 38,873
Commercial expenses	20,013	8,894
General and administrative expenses	16,977	13,158
Total operating loss	87,167	60,925
Financial expenses, net	2,626	648
Loss	89,793	61,573
Net loss per share attributable to ordinary shareholders, basic and diluted	$ 1.52	$ 1.41
Weighted average number of shares used in computing net loss per share attributable to ordinary shareholders, basic and diluted	59,246,803	43,725,584

CONSOLIDATED STATEMENTS OF CASH FLOWS

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

	Year ended December 31,	
	2021	2020
Cash flows from operating activities:		
Loss	$ (89,793)	$ (61,573)
Adjustments to reconcile loss to net cash used in operating activities:		
Depreciation of property, plant and equipment	431	357
Financing expense, net	359	166
Share-based compensation	4,233	3,368
Amortization of debt discount and issuance costs	638	-
Operating lease right-of-use assets	2,109	1,891
Operating lease liabilities	(2,193)	(1,318)
Accrued severance pay, net	12	-
Decrease (increase) in prepaid expenses and other assets	1,008	(1,630)
Increase in trade payables	1,941	5,066
Increase (decrease) in accrued expenses and current liabilities	(505)	3,454
Net cash used in operating activities	(81,760)	(50,219)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(15,054)	(11,804)
Purchase of marketable securities	(102,179)	-
Proceeds from maturity of marketable securities	61,534	13,551
Investment in restricted deposits	(5,222)	(158)
Net cash provided by (used in) investing activities	(60,921)	1,589
Cash flows from financing activities:		
Proceeds from issuance of ordinary shares, net	-	133,312
Proceeds from exercise of options	626	650
Proceeds from issuance of convertible senior notes, net	70,777	-

Net cash provided by financing activities	71,403	133,962
Increase (decrease) in cash and cash equivalents	(71,278)	85,332
Cash and cash equivalents at beginning of year	127,170	41,838
Cash and cash equivalents at end of year	$ 55,892	$127,170

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