



Gamida Cell Reports Full Year 2020 Financial Results and Provides Company Update

March 9, 2021

- Primary and secondary endpoints were met, key exploratory endpoints supported clinical benefit in Phase 3 study of omidubicel in patients with hematologic malignancies; BLA submission anticipated in fourth quarter of 2021—
- Omidubicel commercial preparation underway, including the creation of Gamida Cell Assist, to support potential launch —
- GDA-201 demonstrated significant clinical activity in Phase 1 study of patients with non-Hodgkin lymphoma, with multiple complete responses observed; Phase 1/2 clinical trial planned with IND submission anticipated in the second half of 2021—
- Strengthened financial position with sale of \$75M ordinary shares in December 2020 and \$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 8:30 a.m. ET today –

BOSTON--(BUSINESS WIRE)--Mar. 9, 2021-- [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 year and quarter ended December 31, 2020. The company also highlighted progress with [omidubicel](#), an advanced cell therapy in Phase 3 clinical development as a potentially life-saving treatment option for patients in need of bone marrow transplant, and [GDA-201](#), a natural killer (NK) cell immunotherapy in Phase 1 development in patients with non-Hodgkin lymphoma (NHL).

"It has been a significant year for Gamida Cell, marked by a number of important achievements that have brought us closer to developing cures for blood cancers and serious hematologic diseases. Omidubicel, an advanced cell therapy that has met all primary, secondary and exploratory endpoints in our Phase 3 study in patients with hematological malignancies, represents a potentially transformative treatment option. The data we presented in 2020, demonstrating the clinical benefit of omidubicel, position us to submit our first BLA for omidubicel in the fourth quarter of 2021," said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We remain focused on both the BLA submission and preparing for potential commercial readiness, with the announcement of Gamida Cell Assist, a program designed to focus on patient access and a positive omidubicel experience for the patient and the transplant team. We are diligently working to bring omidubicel to patients as soon as possible."

"We believe our NAM-based cell expansion technology has potential for NK cell expansion and we are developing GDA-201, an NK-cell immunotherapy for the treatment of hematologic and solid tumors in combination with antibody therapies. This year, we made meaningful progress with GDA-201, which has demonstrated impressive early results in patients with heavily pre-treated NHL in a Phase 1 investigator-sponsored study. Following these results, we plan to submit an IND to the FDA in the second half of 2021 and initiate a Phase 1/2 study," Dr. Adams continued.

Omidubicel, an investigational advanced cell therapy for allogeneic bone marrow transplant

During the year, Gamida Cell made significant progress to advance its Phase 3 product candidate omidubicel, which is the first cell therapy for bone marrow transplant to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) and which has the potential to be the first FDA-approved engineered cell therapy which can be used as a bone marrow transplant graft. The company presented primary, secondary and exploratory endpoints from the company's international, multi-center, randomized Phase 3 study of omidubicel demonstrating its clinical benefit as a treatment option for patients in need of a bone marrow transplant.

[In May](#), Gamida Cell reported that the phase 3 study of omidubicel achieved its primary endpoint, demonstrating a statistically significant reduction in time to neutrophil engraftment, a key milestone in recovery from a bone marrow transplant. It was shown that the median time to neutrophil engraftment was 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p < 0.001$). The Phase 3 study was designed to evaluate the safety and efficacy of omidubicel in patients with hematologic malignancies undergoing a bone marrow transplant compared to a comparator group of patients who received a standard umbilical cord blood transplant.

The Phase 3 study additionally met key secondary endpoints related to platelet engraftment, infections and hospitalization, all significant clinical measures in bone marrow transplant, as reported in [October 2020](#). The prespecified secondary endpoints, analyzed in all randomized patients (intent-to-treat), were the proportion of patients who achieved platelet engraftment by day 42, the proportion of patients with Grade 2 or Grade 3 bacterial or invasive fungal infections in the first 100 days following transplant, and the number of days alive and out of the hospital in the first 100 days following transplant. All three secondary endpoints demonstrated a statistically significant improvement among patients who were randomized to omidubicel compared to the comparator group.

[Recently](#), the results of the company's Phase 3 study of omidubicel were presented at the Transplantation & Cellular Therapy Meetings of the American Society of Transplantation and Cellular Therapy and Center for International Blood & Marrow Transplant Research. The data from the study relating to exploratory endpoints also supported the clinical benefit demonstrated by the study's primary and secondary endpoints. Safety results were also presented, showing no significant difference between the two patient groups related to grade III/IV acute GvHD (14 percent for omidubicel, 21 percent for the comparator) or 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 rate of infection was significantly reduced for patients randomized to omidubicel, with the cumulative incidence of first grade 2 or grade 3 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 one year following transplant was 73 percent for patients randomized to omidubicel and 62 percent for patients randomized to control ($p = 0.16$). 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.

Additional omidubicel highlights:

- **Presented new Phase 1 data from study of omidubicel in patients with severe aplastic anemia (SAA) at ASH:** In [a poster presentation at ASH](#), Gamida Cell presented data demonstrating that patients with severe aplastic anemia treated with omidubicel achieved sustained early engraftment and robust immune reconstitution following reduced intensity conditioning. The data suggest 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. The study remains open for accrual of patients with SAA.
- **Advanced commercial launch readiness:** Gamida Cell recently announced plans for the Gamida Cell Assist program. The transplant process can be challenging and complex for the patient, caregivers and the entire transplant care team. Gamida Cell Assist has been designed to focus on patient access and support of every individual and their caregivers 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, and 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.
- **Expanded collaboration with Be The Match BioTherapies®:** In [October](#), Gamida Cell and Be The Match Therapies® expanded their existing strategic collaboration for omidubicel. In building upon the existing collaboration, Gamida Cell will work through Be The Match BioTherapies® for the supply of cord blood units, which serve as the starting material for omidubicel. The expanded agreement is designed to provide a smooth process throughout the omidubicel therapy supply chain.

GDA-201, an innate NK cell immunotherapy

- **Presented updated Phase 1 data at the 62nd ASH Annual Meeting:** In [December](#), Gamida Cell announced updated data from the ongoing Phase 1 study of GDA-201 in combination with monoclonal antibodies in patients with NHL and multiple myeloma at the ASH Annual Meeting. GDA-201 in combination with rituximab demonstrated significant clinical activity in relapsed and refractory NHL patients, with 13 complete responses and one partial response observed in the first 19 NHL patients, for an overall response rate of 74 percent. Overall survival and progression-free survival at one year in the NHL cohort suggest durable disease control, with a median follow-up of ten months (range 1–28 months), in heavily pretreated patients. Additionally, there were no dose-limiting toxicities, neurotoxic events, confirmed cytokine release syndrome, GvHD or marrow aplasia.
- **Continued advancing Phase 1 study of GDA-201:** Gamida Cell continues to advance activities to enable 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 2021. Gamida Cell is pioneering a novel approach that harnesses the power of its cell expansion technology, which uniquely improves antibody-dependent cellular cytotoxicity and tumor targeting of NK cells.

Corporate Highlights

- **Strengthened financial position:** In [December](#) 2020, the company executed an underwritten public offering raising approximately \$75 million before deducting underwriting discounts, commissions and offering expenses. Also, in [February](#) 2021, the company announced a \$75 million financing with Highbridge Capital Management, LLC before deducting offering expenses. These capital infusions 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.

Full Year 2020 Financial Results

- Research and development (R&D) expenses in 2020 were \$41.4 million, compared to \$31.5 million in 2019. The increase was mainly due to advancing the GDA-201 clinical program and clinical activities relating to concluding our Phase 3 clinical trial, as well as additional headcount within the R&D organization.
- Commercial expenses in 2020 were \$8.7 million, compared to \$4.7 million in 2019. The increase was attributed to an increase in omidubicel commercial readiness activities as well as additional headcount within the Commercial organization.
- General and administrative expenses were \$12.2 million in 2020, compared to \$12.1 million in 2019. The increase was mainly due to a \$1.3 million increase in professional services expenses, including Legal and Insurance, offset by decrease

of \$1.2 million in Travel and non-cash compensation expenses.

- Finance expenses, net, was \$10.4 million for 2020, compared to finance income, net, of \$13.8 million for 2019. The decrease was primarily due to non-cash expenses resulting from revaluation of warrants owned by certain of the company's shareholders and the revaluation of the Israeli Innovation Authority royalty-bearing grant liability.
- Net loss for 2020 was \$72.7 million, compared to a net loss of \$34.4 million in 2019.
- As of December 31, 2020, Gamida Cell had total cash and cash equivalents of \$127.2 million, compared to \$55.4 million as of December 31, 2019. In addition, on February 16, 2020, Gamida Cell announced the sale of \$75 million exchangeable senior notes due in 2026 to Highbridge Capital Management, LLC.

2021 Financial Guidance

Gamida Cell expects cash used for ongoing operating activities in 2021 to range from \$100 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 Milestones

Gamida Cell plans to achieve the following milestones during 2021:

Omidubicel

- BLA submission to the FDA in the fourth quarter of 2021
- Commercial readiness activities underway for potential launch at approval

GDA-201

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

Conference Call Information

Gamida Cell will host a conference call today, March 9, 2021, at 8:30 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 1996281. 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 Phase 1 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 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.

CONSOLIDATED STATEMENTS OF FINANCIAL POSITION

U.S. dollars in thousands

	December 31,	
	2020	2019
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	\$ 127,170	\$ 41,838
Marketable securities	-	13,559
Prepaid expenses and other current assets	2,815	1,306
Total current assets	<u>129,985</u>	<u>56,703</u>
NON-CURRENT ASSETS:		
Property, plant and equipment, net	18,238	6,298
Right-of-use assets	6,474	5,133
Other assets	786	641
Total non-current assets	<u>25,498</u>	<u>12,072</u>
Total assets	<u>\$ 155,483</u>	<u>\$ 68,775</u>
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Trade payables	\$ 6,329	\$ 1,164
Employees and payroll accruals	4,705	3,443
Current maturities of lease liabilities	2,532	1,870
Accrued expenses and other payables	7,988	4,918
	<u>21,554</u>	<u>11,395</u>
NON-CURRENT LIABILITIES:		
Liabilities presented at fair value	12,043	5,221
Employee benefit liabilities, net	768	773
Lease liability	5,378	4,101
Liability to Israel Innovation Authority (IIA)	17,003	12,302
	<u>35,192</u>	<u>22,397</u>
CONTINGENT LIABILITIES AND COMMITMENTS		
SHAREHOLDERS' EQUITY:		
Share capital	166	92
Share premium	375,280	238,992
Capital reserve due to actuarial loss	(441)	(541)
Reserve from financial assets measured at FVOCI	-	4
Accumulated deficit	(276,268)	(203,564)
Total shareholders' equity	<u>98,737</u>	<u>34,983</u>
Total liabilities and shareholders' equity	<u>\$ 155,483</u>	<u>\$ 68,775</u>

CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

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

	Year ended December 31,		
	2020	2019	2018
Operating expenses:			
Research and development expenses, net	\$41,385	\$ 31,462	\$22,045
Commercial activities	8,748	4,692	-
General and administrative expenses	12,167	12,091	11,599
Operating loss	<u>62,300</u>	<u>48,245</u>	<u>33,644</u>
Financial expenses	10,640	3,325	20,259
Financial income	<u>(236)</u>	<u>(17,149)</u>	<u>(1,042)</u>
Loss before taxes on income	72,704	34,421	52,861
Taxes on income	<u>-</u>	<u>(70)</u>	<u>70</u>
Net loss	<u>72,704</u>	<u>34,351</u>	<u>52,931</u>

Net loss per share:

Basic net loss per share	<u>\$ 1.66</u>	<u>\$ 1.17</u>	<u>\$ 10.53</u>
Diluted net loss per share	<u>\$ 1.66</u>	<u>\$ 1.69</u>	<u>\$ 10.53</u>

CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Year ended December 31,		
	2020	2019	2018
<u>Cash flows from operating activities:</u>			
Net loss	\$(72,704)	\$(34,351)	\$(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	2,397	2,143	269
Financial loss (income), net	483	(775)	(858)
Share-based compensation	2,864	4,868	3,575
Change in employee benefit liabilities, net	94	126	(15)
Amortization of premium on marketable securities	4	184	272
Revaluation of financial derivatives	6,822	(15,904)	17,600
Revaluation of liability to IIA	4,302	2,531	2,037
	<u>16,966</u>	<u>(6,827)</u>	<u>22,880</u>
Changes in asset and liability items:			
Increase (decrease) in other receivables, prepaid expenses and other assets	(1,626)	(150)	942
Increase (decrease) in trade payables	5,083	(821)	(405)
Increase in accrued expenses and other payables	3,454	2,807	2,296
	<u>6,911</u>	<u>1,836</u>	<u>2,833</u>
Cash received during the year for:			

Interest received	361	1,546	792
Interest paid	<u>(161)</u>	<u>(134)</u>	<u>-</u>
Net cash used in operating activities	<u>(48,627)</u>	<u>(37,930)</u>	<u>(26,426)</u>
<u>Cash flows from investing activities:</u>			
Purchase of property, plant and equipment	(11,804)	(3,055)	(1,645)
Purchase of marketable securities	-	(32,021)	(10,905)
Proceeds from bank deposits	-	-	5,000
Investment in restricted bank deposits	(158)	-	(150)
Proceeds from maturity of marketable securities	13,551	38,742	-
Proceeds from sale of marketable securities	<u>-</u>	<u>-</u>	<u>4,949</u>
Net cash provided by (used in) investing activities	<u>1,589</u>	<u>3,666</u>	<u>(2,751)</u>

CONSOLIDATED STATEMENTS OF CASH FLOWS

U.S. dollars in thousands

	Year ended December 31,		
	2020	2019	2018
<u>Cash flows from financing activities:</u>			
Proceeds from secondary offerings, net	133,316	37,140	-
Receipt of grants from the IIA	399	224	612
Proceeds from issuance of shares, initial public offering (payment of issuance expenses), net	-	(238)	47,479
Payment of lease liabilities	(1,985)	(1,529)	-
Proceeds from exercise of options	<u>650</u>	<u>132</u>	<u>2</u>
Net cash provided by financing activities	<u>132,380</u>	<u>35,729</u>	<u>48,093</u>
Exchange differences on balances of cash and cash equivalents	<u>(10)</u>	<u>101</u>	<u>31</u>
Increase in cash and cash equivalents	85,332	1,566	18,947
Cash and cash equivalents at beginning of year	<u>41,838</u>	<u>40,272</u>	<u>21,325</u>
Cash and cash equivalents at end of year	<u>\$ 127,170</u>	<u>\$ 41,838</u>	<u>\$ 40,272</u>

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](https://clinicaltrials.gov/ct2/show/study/NCT03019666) identifier NCT03019666

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