



## Gamida Cell Reports Fourth Quarter and Full Year 2018 Financial Results and Provides Company Update

February 25, 2019

– Successfully Completed IPO Raising \$53.2 Million in Gross Proceeds –

– Patient Enrollment in Phase 3 Study of NiCord® on Track for Completion in Second Half of 2019; Topline Results Expected in First Half of 2020 –

– Recently Presented NAM-NK and NiCord Data at 2019 TCT Annual Meeting Demonstrate Clinical Progress and Potential of Proprietary NAM Expansion Technology –

BOSTON--(BUSINESS WIRE)--Feb. 25, 2019-- [Gamida Cell Ltd.](#) (Nasdaq:GMDA), a leading cellular and immune therapeutics company, today reported financial results for the fourth quarter and full year ended December 31, 2018, and provided a business update, which highlights the company's progress advancing its clinical development candidates: NiCord<sup>®</sup>, an investigational advanced cell therapy in Phase 3 clinical development designed to enhance and expand the life-saving benefits of hematopoietic stem cell (bone marrow) transplant, and NAM-NK, an investigational, cell-based cancer immunotherapy in Phase 1 development in patients with non-Hodgkin lymphoma and multiple myeloma.

Gamida Cell continues to anticipate completing patient enrollment in the Phase 3 study of NiCord by the end of this year with topline data anticipated in first half of 2020. The international, randomized, multi-center study is designed to evaluate the safety and efficacy of NiCord compared to standard umbilical cord blood for allogeneic bone marrow transplant in approximately 120 patients with no available matched donor. Additionally, the company continues to make progress in its NAM-NK program, and expects to initiate a multi-center Phase 1/2 study of NAM-NK in patients with blood cancer in 2020.

"We are off to a strong start this year and are making meaningful progress across every dimension of our company. The data presented last week at the TCT Annual Meeting demonstrates the potential for NiCord to offer a new cell therapy option for patients in need of a bone marrow transplant," stated Julian Adams, Ph.D., chief executive officer at Gamida Cell. "Additionally, our collaboration with Be The Match BioTherapies and the appointment of Tom Klima as chief commercial officer underscore our commitment to advancing our clinical development plans and beginning commercial readiness activities for the potential approval and launch of NiCord."

Dr. Adams continued, "We are committed to leveraging the transformative potential of our proprietary nicotinamide-, or NAM-based, cell expansion technology, to deliver a multi-product pipeline. Last week, we presented encouraging data from our ongoing Phase 1 study of NAM-NK in patients with non-Hodgkin lymphoma and multiple myeloma, and the emerging clinical profile suggests it has the potential to address some of the limitations of currently available cell therapies. In addition, we have demonstrated the ability to cryopreserve our NK cells in a laboratory setting, and we are working to scale up our manufacturing process to enable the ability to evaluate multiple doses and schedules of NAM-NK therapy in a multi-center, Phase 1/2 clinical study next year."

### Recent Company Highlights:

- **Reported additional immune reconstitution data for NiCord supporting clinical potential for bone marrow transplant:** In February, translational data from the completed Phase 1/2 study of NiCord were reported at the 2019 Transplantation & Cellular Therapy (TCT) Meetings of American Society for Blood and Marrow Transplantation and Center for International Blood and Marrow Transplant Research demonstrating that recipients who received NiCord had rapid and robust reconstitution of key immune cells. Successful immune reconstitution is an important factor in the recovery of patients undergoing bone marrow transplant.
- **Reported initial data from Phase 1/2 study of NiCord in severe aplastic anemia:** At the 2019 TCT Annual meeting, data were reported from the ongoing Phase 1/2 study of NiCord in patients with severe aplastic anemia. In the initial cohort of three patients, all successfully underwent a bone marrow transplant consisting of NiCord plus a haploidentical stem cell graft. The rapid cord engraftment, sustained hematopoiesis and accelerated immune recovery in treatment refractory observed in these patients enable the initiation of a second cohort of patients to be treated with NiCord as a stand-alone graft. Patient enrollment in the second cohort is expected to begin in the first half of 2019.
- **Announced encouraging data from Phase 1 study of NAM-NK in non-Hodgkin Lymphoma and multiple myeloma:** During the 2019 TCT Annual Meeting, data reported from the ongoing Phase 1 study of NAM-NK in patients with non-Hodgkin lymphoma (NHL) and multiple myeloma (MM) demonstrated that NAM-NK was clinically active, with three complete responses observed in patients with NHL and one complete response in a patient with MM. These data, along with safety data showing that NAM-NK was generally well tolerated, support continued clinical development, and Gamida Cell is planning to initiate a multi-center, Phase 1/2 clinical study of NAM-NK in patients with blood cancers in 2020.
- **Entered into agreement with Editas Medicine to evaluate potential to gene edit NAM-NK cells:** In February, Gamida Cell announced an agreement with Editas Medicine, Inc. to evaluate the potential use of Editas Medicine's CRISPR technology to edit NAM-NK cells. Through this agreement, the companies aim to rapidly discover optimized NAM-NK cells that could be used to improve the treatment of blood cancers and solid tumors.

- **Formed strategic collaboration with Be The Match BioTherapies:** In January 2019, both organizations announced a collaboration to expand the use of bone marrow transplant to treat hematologic malignancies and serious blood disorders. Under the terms of the collaboration agreement, the organizations will explore opportunities to work together across Gamida Cell's ongoing clinical development program for NiCord, including the ongoing Phase 3 clinical study. Be The Match BioTherapies has an extensive history of involvement in the delivery of cord blood units for transplant and broad access to cord blood banks globally.
- **Appointed Thomas Klima as Chief Commercial Officer:** In January, the company announced the appointment of Thomas Klima as Chief Commercial Officer. Mr. Klima brings nearly 20 years of global experience in the pharmaceutical industry with expertise in cellular therapy, hematology, oncology and transplantation. During his career, he has played key roles in building commercial organizations and leading multiple successful product launches.
- **Appointed Nurit Benjamini to Board of Directors:** In January, the company appointed Nurit Benjamini to Gamida Cell's board of directors and chair of the board's audit committee. Ms. Benjamini has served as chief financial officer of TabTale Ltd. since 2013. Previously, she held a number of chief financial officer positions, including at Wix.com Ltd., Sigma Designs Israel Ltd. and Compugen Ltd.

#### **Anticipated 2019-2020 Milestones**

Gamida Cell's anticipated program milestones in 2019-2020 are as follows:

##### *Nicord®*

- Initiate Cohort 2 in the Phase 1/2 study evaluating NiCord as stand-alone graft in severe aplastic anemia in the first half of 2019
- Complete enrollment in Phase 3 study of NiCord in patients with hematologic malignancies in the second half of 2019
- Report topline data from the Phase 3 study of NiCord in patients with hematologic malignancies in the first half of 2020
- Complete BLA filing for NiCord in hematologic malignancies in the second half of 2020, should Phase 3 data be positive

##### *NAM-NK*

- Complete patient enrollment in the ongoing Phase 1 study in the second half of 2019
- Present additional data at a medical meeting in the second half of 2019
- Initiate multi-center, Phase 1/2 clinical study in 2020

#### **Fourth Quarter and Full Year 2018 Financial Results:**

- At December 31, 2018, Gamida Cell had total cash, cash equivalents and available-for-sale securities of \$60.7 million, compared to \$41.1 million at December 31, 2017.
- Research and Development expenses in 2018 were \$22.0 million, compared to \$15.0 million in 2017. The increase was attributable mainly to a \$5.4 million increase in clinical activities relating to the advancement of Gamida Cell's clinical programs as well as an increase of \$1.6 million in other R&D expenses.
- General and administrative expenses were \$11.6 million in 2018, compared to \$4.5 million in 2017. The increase was attributable mainly to a \$2.9 million increase in expenses related to expanding the management team and establishing the U.S. headquarters, an increase of \$2.0 million in non-cash stock-based compensation expenses, and \$2.2 million in professional services expenses incurred during the IPO process, rent and other expenses.
- Finance expenses, net, were \$19.2 million in 2018, compared to \$0.5 million in income, in 2017. The increase was primarily due to non-cash expenses resulting from revaluation of warrants and the revaluation of royalty-bearing grant IIA liability.
- Net loss for 2018 was \$52.9 million, compared to a net loss of \$19.1 million for 2017.

#### **2019 Financial Guidance**

Gamida Cell expects cash used for ongoing operating activities in 2019 to range from \$35-\$40 million, reflecting anticipated expenditures to advance the company's clinical programs.

Gamida Cell expects that its cash, cash equivalents and available-for-sale securities will support the company's capital needs through the data readout for the Phase 3 clinical study of NiCord, which is expected in the first half of 2020. This cash runway guidance is based on the company's current operational plans and excludes any additional funding that may be received or business development activities that may be undertaken.

#### **About NiCord**

NiCord, the company's lead clinical program, is under development as a universal bone marrow transplant solution for patients with high-risk hematologic malignancies. NiCord has been granted Breakthrough Therapy designation by the U.S. Food and Drug Administration, making it the first bone marrow transplant alternative to receive this designation. It has also received U.S. and EU orphan drug designation. A Phase 3 clinical study evaluating NiCord in patients with leukemia and lymphoma is ongoing in the United States, Europe and Asia.<sup>1</sup> NiCord is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia.<sup>2</sup> 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 NiCord. For more information on clinical trials of NiCord, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

#### **About NAM-NK**

Gamida Cell applied the capabilities of its NAM-based cell expansion technology to highly functional NK cells to develop NAM-NK, an innate immunotherapy for the treatment of hematologic and solid tumors in combination with standard of care antibody therapies. NAM-NK 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. NAM-NK is in Phase 1 development through an investigator-sponsored study in patients with refractory non-Hodgkin lymphoma and multiple myeloma.<sup>3</sup>

*NAM-NK and NiCord are investigational therapies, and their safety and efficacy have not been evaluated by the U.S. Food and Drug Administration or any other health authority.*

#### **About Gamida Cell**

Gamida Cell is a clinical-stage biopharmaceutical company committed to developing advanced cell therapies with the potential to cure blood cancers and rare, serious hematologic diseases. We are leveraging our proprietary nicotinamide-based, or NAM-based, cell expansion technology to develop product candidates designed to address the limitations of cell therapies. For additional information, please visit [www.gamida-cell.com](http://www.gamida-cell.com).

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 the patient enrollment in and timing of initiation and progress of and data reported from the clinical trials of Gamida Cell's product candidates, and Gamida Cell's expectations regarding its projected operating expenses 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 variability, 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 of Gamida Cell's public filing on Form 20-F, filed with the SEC on February 25, 2019, 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**

	<b>December 31,</b>	
	<b>2018</b>	<b>2017</b>
<b>ASSETS</b>		
<b>CURRENT ASSETS:</b>		
Cash and cash equivalents	\$ 40,272	\$ 21,325
Available-for-sale financial assets	20,417	14,758
Short term deposits	-	5,000
Prepaid expenses and other current assets	1,502	2,539
<b>Total current assets</b>	<b>62,191</b>	<b>43,622</b>
<b>NON-CURRENT ASSETS:</b>		
Property and equipment, net	2,311	940
Other assets	662	360
<b>Total non-current assets</b>	<b>2,973</b>	<b>1,300</b>
<b>Total assets</b>	<b>\$ 65,164</b>	<b>\$ 44,922</b>

### **LIABILITIES AND SHAREHOLDERS' EQUITY**

#### **CURRENT LIABILITIES:**

Trade payables	\$ 1,985	\$ 2,390
Employees and payroll accruals	2,888	1,517
Accrued expenses and other payables	1,832	669
<b>Total current liabilities</b>	<b>6,705</b>	<b>4,576</b>

#### **NON-CURRENT LIABILITIES:**

Warrants presented at fair value	24,049	10,300
Employee benefit liabilities, net	183	200
Liability to Israel Innovation Authority (IIA)	9,540	6,890
<b>Total non-current liabilities</b>	<b>33,772</b>	<b>17,390</b>

### **CONTINGENT LIABILITIES AND COMMITMENTS**

#### **SHAREHOLDERS' EQUITY:**

Ordinary shares	67	2
Preferred shares	-	38
Share premium	193,953	139,311

Capital reserve due to actuarial loss	(77)	(79)
Available for sale reserve	(43)	(34)
Accumulated deficit	(169,213)	(116,282)
<b>Total shareholders' equity</b>	<b>24,687</b>	<b>22,956</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 65,164</b>	<b>\$ 44,922</b>

## CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

U.S. dollars in thousands

	Year ended	
	December 31, 2018	2017
<b>Operating expenses:</b>		
Research and development expenses, net	\$ 22,045	\$ 15,018
General and administrative expenses	11,599	4,472
<b>Operating loss</b>	<b>33,644</b>	<b>19,490</b>
Financial expenses	20,259	718
Financial income	(1,042)	(1,197)
<b>Loss before taxes on income</b>	<b>52,861</b>	<b>19,011</b>
Taxes on income	70	-
<b>Net Loss</b>	<b>52,931</b>	<b>19,011</b>
Other comprehensive loss:	7	69
<b>Total comprehensive loss</b>	<b>\$ 52,938</b>	<b>\$ 19,080</b>
<b>Loss per share - basic and diluted (in U.S. dollars)</b>	<b>\$ 10.53</b>	<b>\$ 27.56</b>

<sup>1</sup>[ClinicalTrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT02730299) identifier NCT02730299.

<sup>2</sup>[ClinicalTrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT03173937) identifier NCT03173937.

<sup>3</sup>[ClinicalTrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT03019666) identifier NCT03019666.

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