



Gamida Cell Announces FDA Acceptance of Biologics License Application for Omidubicel with Priority Review

August 1, 2022

- If approved, omidubicel will be the first allogeneic advanced stem cell therapy donor source for patients with blood cancers in need of a stem cell transplant -

- PDUFA target action date is January 30, 2023 -

- Company to host conference call on BLA acceptance and commercial opportunity today at 8:00 a.m. ET -

BOSTON--(BUSINESS WIRE)--Aug. 1, 2022-- [Gamida Cell Ltd.](#) (Nasdaq: GMDA), the leader in the development of NAM-enabled cell therapy candidates for patients with hematologic and solid cancers and other serious diseases, announced today that the U.S. Food and Drug Administration (FDA) has accepted for filing the Company's Biologics License Application (BLA) for omidubicel for the treatment of patients with blood cancers in need of an allogeneic hematopoietic stem cell transplant. Omidubicel is a first-in-class, advanced NAM-enabled stem cell therapy candidate with breakthrough and orphan drug designations.

The FDA granted Priority Review for the BLA and has set a Prescription Drug User Fee Act (PDUFA) target action date of January 30, 2023. The FDA grants Priority Review to product applications that, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. At this time, the FDA has indicated that it is not planning an advisory committee meeting as part of the BLA review.

"The FDA's acceptance of our BLA with Priority Review signifies a critical milestone in our mission to deliver a new stem cell therapy option for patients in need of a donor for an allogeneic stem cell transplant," said Julian Adams, Ph.D., chief executive officer of Gamida Cell. "We are encouraged by the positive and sustained follow-up results from patients participating in the Phase 3 trial of omidubicel, including a positive overall survival trend one-year out from treatment. These results provide promising rationale that, if approved, omidubicel could become a treatment of choice for patients in need of an allo-HSCT transplant. We look forward to working with the FDA throughout the review process to bring omidubicel to patients as quickly as possible."

Upon FDA approval, omidubicel will be manufactured at the Gamida Cell owned manufacturing facility in Israel. This is a newly constructed, state-of-the-art, modular facility which allows for additional capacity to be added to address growing demand. Batches from this facility were used to support the BLA for omidubicel and the facility is currently manufacturing clinical batches.

The omidubicel BLA is supported by the statistically significant results from Gamida Cell's pivotal Phase 3 study, the results of which were published in *Blood*, the official journal of the American Society of Hematology. Results for the study's primary endpoint, the median time to neutrophil engraftment in patients with hematologic malignancies undergoing allogeneic bone marrow transplant with omidubicel compared to standard umbilical cord blood (UCB), demonstrated a median time to neutrophil engraftment of 12 days for patients randomized to omidubicel compared to 22 days for the comparator group ($p < 0.001$). The secondary endpoints of this Phase 3 study were all achieved and were statistically significant. These secondary endpoints were platelet engraftment, the rate of infection, and days alive and out of hospital. Omidubicel was generally well tolerated in the Phase 3 study.

The full *Blood* manuscript is available here: <https://ashpublications.org/blood/article/doi/10.1182/blood.2021011719/476235/Omidubicel-Versus-Standard-Myeloablative-Umbilical>.

In 2019, approximately 8,000 patients who were 12 years old and up with hematologic malignancies underwent an allogeneic stem cell transplant in the United States.¹ Unfortunately, it is estimated that another 1,200 patients were eligible for transplant but could not find a donor source.² If approved, omidubicel has the potential to improve outcomes for patients based on transplant feedback and to potentially increase access for patients to get to transplant. If approved, omidubicel has the potential to treat approximately 2,000 – 2,500 patients each year in the U.S.

Conference Call Information

Gamida Cell will host a conference call today, August 1, 2022, at 8:00 a.m. ET to discuss this update. To access the conference call, please register [here](#) and please be advised to do so at least 10 minutes prior to joining the call. 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. A replay of the webcast will be available approximately two hours after the event, for approximately 30 days.

About Omidubicel

Omidubicel is an advanced cell therapy candidate developed as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplant for patients with blood cancers. Omidubicel demonstrated a statistically significant reduction in time to neutrophil engraftment in comparison to standard umbilical cord blood in an international, multi-center, randomized Phase 3 study (NCT0273029) in patients with hematologic malignancies undergoing allogeneic bone marrow transplant. The Phase 3 study also showed reduced time to platelet engraftment, reduced infections and fewer days of hospitalization. One-year post-transplant data showed sustained clinical benefits with omidubicel as demonstrated by significant reduction in infectious complications as well as reduced non-relapse mortality and no significant increase in relapse rates nor increases in graft-versus-host-disease (GvHD) rates. Omidubicel is the first stem cell transplant donor source to receive Breakthrough Therapy Designation from the FDA and has also received Orphan Drug Designation in the US and EU.

Omidubicel is an investigational stem cell therapy candidate, and its safety and efficacy have not been established by the FDA or any other health authority. For more information about omidubicel, please visit <https://www.gamida-cell.com>.

About NAM Technology

Our NAM-enabling technology is designed to enhance the number and functionality of targeted cells, enabling us to pursue a curative approach that moves beyond what is possible with existing therapies. Leveraging the unique properties of NAM (nicotinamide), we can expand and metabolically modulate multiple cell types — including stem cells and natural killer cells — with appropriate growth factors to maintain the cells' active phenotype and enhance potency. Additionally, our NAM technology improves the metabolic fitness of cells, allowing for continued activity throughout the expansion process.

About Gamida Cell

Gamida Cell is pioneering a diverse immunotherapy pipeline of potentially curative cell therapy candidates 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 therapy candidates 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 omidubicel), regulatory filings submitted to the FDA (including the potential timing of the FDA's review of the BLA for omidubicel), commercialization planning efforts, and the potentially life-saving or curative therapeutic and commercial potential of Gamida Cell's product candidates (including 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 Quarterly Report on Form 10-Q, filed with the Securities and Exchange Commission (SEC) on May 12, 2022, 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.

¹CIBMTR 2019 – allogeneic transplants in patients 12+ years with hematological malignancies.

²Gamida Cell market research

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