Long-Term Data from Omidubicel Phase 3 Trial Demonstrates Overall Survival and Sustainable Durable Outcomes for Patients with Blood Cancers at the Society of Hematologic Oncology Meeting

September 29, 2022

At three years, new data showed overall survival and disease-free survival of 63% and 56% respectively

At ten years, follow-up data demonstrated sustained long-term bone marrow recovery

Omidubicel patients reported clinically meaningful health-related quality of life scores compared to umbilical cord blood

BOSTON--(BUSINESS WIRE)--Sep. 29, 2022-- Gamida Cell Ltd. (Nasdaq: GMDA), the leader in the development of NAM-enabled cell therapies for patients with hematologic and solid cancers and other serious diseases, today announced the presentation of new long term follow-up data and health-related quality of life scores of patients treated with omidubicel at the Tenth Annual Meeting of the Society of Hematologic Oncology (SOHO), being held in Houston, Texas.

"These data reinforce our commitment to advance transformational cell therapy research and underscore the potential of our NAM technology platform. Our lead stem cell therapy candidate, omidubicel, addresses the unmet need for patients with hematologic malignancies, demonstrated by the robust and growing body of encouraging clinical evidence, including the long-term follow up data and quality of life improvement," said Ronit Simantov, M.D., Chief Medical Officer of Gamida Cell. "As we approach the PDUFA date of January 30, 2023, and upon potential FDA approval, we are prepared to execute our plan that ensures access to those patients who can benefit from omidubicel as quickly as possible."

The long-term, durable clinical benefit of omidubicel was observed at three years across a patient population that typically has a poor prognosis. A study titled, "Multicenter Long-Term Follow Up of Allogeneic Hematopoietic Stem Cell Transplantation with Omidubicel: A Pooled Analysis of Five Prospective Clinical Trials," highlighted long-term follow-up of 105 patients transplanted with omidubicel between 2006-2020 (median follow-up of 22 months). The data demonstrated an overall survival and disease-free survival of 63% (95% CI, 53%-73%) and 56% (95% CI, 47%-67%) at three years, respectively, as well as durable long-term hematopoiesis and immune competence. Learn More

Overall well-being health-related quality of life scores for patients treated with omidubicel demonstrated clinical benefit compared to standard of care. A study titled, "Health-Related Quality of Life Following Allogeneic Hematopoietic Stem Cell Transplantation with Omidubicel Versus Standard Umbilical Cord Blood" featured an analysis of 108 patients that completed validated health-related quality of life (HRQL) surveys on screening and days 42, 100, 180, and 365 post-transplant. Measures of physical and functional well-being and other HRQL scores were more favorable with omidubicel. These data suggest clinically meaningful and sustained improvements in physical, functional, and overall well-being compared to umbilical cord blood transplantation. Learn More

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 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 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.

[1]CIBMTR 2019 – allogeneic transplants in patients 12+ years with hematological malignancies.
[2]Gamida Cell market research

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