



## Gamida Cell Presents Analysis of Observational Data Demonstrating the Impact of Donor Age in Hematopoietic Stem Cell Transplant Outcomes at the Virtual Cord Blood Connect Meeting

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— Study shows younger donor age is associated with improved outcomes following bone marrow transplant —

BOSTON--(BUSINESS WIRE)--Sep. 10, 2020-- [Gamida Cell Ltd.](#) (Nasdaq: GMDA), an advanced cell therapy company committed to cures for blood cancers and serious blood diseases, today announced data from an observational study demonstrating that younger donor age is associated with more rapid time to neutrophil engraftment and increased overall survival in patients who received hematopoietic stem cell transplant (HSCT, or bone marrow transplant) for the treatment of hematological malignancies. These data are being presented at the Cord Blood Connect Meeting, which is taking place virtually today and on September 17.

The study is the result of a [research agreement](#) between Gamida Cell and the [CIBMTR](#)<sup>®</sup> (Center for International Blood and Marrow Transplant Research<sup>®</sup>) designed to collect and analyze health outcomes data in patients with hematologic malignancies who receive a hematopoietic stem cell transplant or cellular therapy infusion, including bone marrow transplant graft from various donor sources. The study evaluated clinical outcomes for 660 patients in the CIBMTR registry who underwent a bone marrow transplant with a matched unrelated, mismatched unrelated or haploidentical graft source contemporaneous to the Phase 3 study of omidubicel, Gamida Cell's investigational advanced cell therapy in development as a treatment option for patients in need of a bone marrow transplant. Key clinical outcomes, including time to neutrophil engraftment and overall survival, were improved for patients with donors under the age of 30.

"As new graft options evolve for bone marrow transplant, and we as a field learn more about the long-term patient outcomes of these graft options, the selection algorithms that are used to match patients with donors must also evolve to take into account the most current clinical data," said Ronit Simantov, M.D., chief medical officer at Gamida Cell. "These data indicate that donor age is a factor in clinical outcomes and that donor age should be considered when matching patients with a graft source."

In [May](#), Gamida Cell reported that its Phase 3 study of omidubicel achieved its primary endpoint, demonstrating a highly statistically significant reduction ( $p < 0.001$ ) in time to neutrophil engraftment, a key milestone in recovery from a bone marrow transplant. Omidubicel is the first bone marrow transplant product to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA). Gamida Cell expects to begin submitting the biologics license application for omidubicel to the FDA on a rolling basis in the fourth quarter of 2020.

"This analysis reinforces the importance of considering donor age when selecting a bone marrow graft source for patients, as donors aged 30 or younger resulted in improved overall survival," said Julian Adams, Ph.D., chief executive officer at Gamida Cell. "As we look toward our anticipated regulatory submission of omidubicel to the FDA and potential approval, given that omidubicel is derived from cord blood, we believe these findings could potentially have future implications for considering omidubicel for any patient who does not have an available related or unrelated donor of suitable age."

### More Details About the Study

This observational study utilizes data from the CIBMTR<sup>®</sup> registry to analyze long-term safety and efficacy data for patients with hematologic malignancies who underwent a bone marrow transplant with a matched unrelated, mismatched unrelated or haploidentical graft source. The criteria for inclusion of patients and the outcomes evaluated in the analyses were consistent with those in the Phase 3 study of omidubicel. The median donor age was 30 (range 2-74) years.

Statistical analyses, performed by Gamida Cell, compared important clinical outcomes for patients with donors  $\leq 30$  years old ( $n=334$ ) to patients with donors  $> 30$  years of age ( $n=326$ ). Patient demographics were well-balanced across the two donor age groups. The study demonstrated that neutrophil recovery was more rapid in patients with donors  $\leq 30$  years old ( $p = 0.013$ ). Additionally, patients with donors  $\leq 30$  years of age showed improved overall survival at one year ( $p = 0.016$ ).

### 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 and NCT02730299), omidubicel demonstrated rapid and durable time to engraftment and was generally well tolerated.<sup>1,2</sup> 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<sup>®</sup>, which is the same investigational development candidate as omidubicel. For more information on clinical trials of omidubicel, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

*Omidubicel is an investigational therapy, and its 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 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](http://www.gamida-cell.com) or follow Gamida Cell on [LinkedIn](#) or Twitter at [@GamidaCellTx](#).

## About the CIBMTR

The [CIBMTR](#)<sup>®</sup> (Center for International Blood and Marrow Transplant Research<sup>®</sup>) is a research collaboration between the National Marrow Donor Program<sup>®</sup> (NMDP)/Be The Match<sup>®</sup> and the Medical College of Wisconsin (MCW). The CIBMTR collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy worldwide to increase survival and enrich quality of life for patients. The CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a network of more than 300 transplant centers, and one of the largest databases worldwide for clinical outcomes of cellular therapy, and a biorepository with tissue samples.

For more information on the CIBMTR, please visit [www.cibmtr.org](http://www.cibmtr.org) or follow the CIBMTR on [Facebook](#), [LinkedIn](#), or Twitter at [@CIBMTR](#).

## About the National Marrow Donor Program/Be The Match

The National Marrow Donor Program<sup>®</sup> (NMDP)/Be The Match<sup>®</sup> is the global leader in providing a cure to patients with life-threatening blood and marrow cancers like leukemia and lymphoma, as well as other diseases. The NMDP/Be The Match manages the world's largest registry of potential blood stem cell donors and cord blood units, connects patients to their donor match for a life-saving marrow or umbilical cord blood transplant and educates health care professionals and patients. In 2016, the NMDP/Be The Match established [Be The Match BioTherapies](#)<sup>®</sup> to accelerate patient access to life-saving therapies, by providing proven services and support to companies developing and delivering cell and gene therapies.

Learn more at [BeTheMatchClinical.org](http://BeTheMatchClinical.org).

## About the Medical College of Wisconsin

With a history dating back to 1893, The Medical College of Wisconsin (MCW) is dedicated to leadership and excellence in education, patient care, research and community engagement. More than 1,200 students are enrolled in MCW's medical school and graduate school programs in Milwaukee, Green Bay, and Central Wisconsin in 2016. MCW's School of Pharmacy opened in 2017. A major national research center, MCW is the largest research institution in the Milwaukee metro area and second largest in Wisconsin. In FY2016, faculty received more than \$184 million in external support for research, teaching, training and related purposes. This total includes highly competitive research and training awards from the National Institutes of Health (NIH). Annually, MCW faculty direct or collaborate on more than 3,100 research studies, including clinical trials. Additionally, more than 1,500 physicians provide care in virtually every specialty of medicine for more than 525,000 patients annually.

## 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 the anticipated timing of regulatory filing submissions, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to the ongoing global COVID-19 pandemic 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 February 26, 2020, 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.

## References

<sup>1</sup>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.

<sup>2</sup>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.

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## Investors:

Jaren Irene Madden  
[jaren@gamida-cell.com](mailto:jaren@gamida-cell.com)  
1-617-286-6264

## Media Inquiries:

Matthew Corcoran  
[mcorcoran@tenbridgecommunications.com](mailto:mcorcoran@tenbridgecommunications.com)  
1-617-866-7350

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