Gamida Cell Announces First Patient Transplanted in Phase 1/2 Study of CordIn for Severe Aplastic Anemia and Hypoplastic MDS

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This investigator initiated study is being conducted in collaboration with
the U.S. National Heart, Lung and Blood Institute (NHLBI)

Jerusalem, Israel, August 21, 2017 — The first subject has been transplanted in an investigator-initiated study of Cordin™ for patients with severe aplastic anemia (AA) or hypoplastic myelodysplastic syndrome (MDS) who have no available matched donor. CordIn™ is produced by Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases.

The study is being conducted by Dr. Richard W. Childs, Clinical Director of the National Heart, Lung, and Blood Institute's (NHLBI) Division of Intramural Research in the National Institutes of Health (NIH) and Assistant United States Surgeon General.

"Severe aplastic anemia and myelodysplastic syndrome are life-threatening bone marrow disorders with few optimal treatment options. Many patients with these diseases fail conventional therapy. Amongst those with severe AA who respond to conventional treatment, up to 30% will suffer relapse or evolve to myelodysplastic syndrome or leukemia which is often fatal," said Dr. Childs. "Promising preclinical and clinical data have shown the efficacy of Gamida Cell's ex-vivo hematopoietic stem cell technology. Based upon exciting prior data, we are now conducting a clinical trial at the NHLBI testing whether umbilical cord blood transplantation using CordIn can be used to improve the results of conventional cord blood transplantation for patients with these life-threatening conditions who lack an available matched donor," said Dr. Childs.

"The study, announced today, represents a significant milestone for Gamida Cell, as we continue our important mission of expanding access to curative transplantation in patients for which engraftment has been historically difficult," said Gamida Cell President and CEO Dr. Yael Margolin. "We highly value our collaboration with the NIH and with Dr. Childs, who is one of the world experts in the field, and we look forward to evaluating the potential of CordIn in patients with life-threatening hematologic diseases."

The study is titled Unrelated Umbilical Cord Blood Transplantation for Severe Aplastic Anemia and Hypo-plastic MDS Using CordIn™, Umbilical Cord Blood-Derived Ex Vivo Expanded Stem and Progenitor Cells to Expedite Engraftment and Improve Transplant Outcome. The primary endpoint of the Phase 1/2 trial is prompt and durable cord engraftment in patients transplanted with CordIn. Additional information can be accessed here on the Clinicaltrials.gov website.

About CordIn
CordIn is in development for patients with rare genetic diseases where bone marrow transplantation is clinically established as a potential cure but no fully matched donors are available. CordIn is also currently being evaluated in a Phase 1/2 study in patients with sickle cell disease, a rare genetic disease of red blood cells.

About Gamida Cell
Gamida Cell is a world leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases. The company's pipeline includes products in development for treatment of a wide range of conditions including cancer, genetic hematological diseases such as sickle cell disease and thalassemia, bone marrow failure syndromes such as aplastic anemia, genetic metabolic diseases and refractory autoimmune diseases. Gamida Cell's shareholders include Novartis, Clal Biotechnology Industries, Elbit Imaging, Israel Healthcare Ventures, Shavit Capital Fund, VMS Investment Group, Denali Ventures, Auriga Ventures and Israel Biotech Fund. For more information please visit gamida-cell.com.

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