Gamida Cell Announces First Patient with Sickle Cell Disease Transplanted in Phase 1/2 Study of CordIn™ as the Sole Graft Source

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CordIn has the potential to be a sole graft source for rare genetic diseases where transplantation is the only clinically established cure

Jerusalem, Israel, July 27, 2016 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, announced today that the first patient with sickle cell disease (SCD) has been transplanted with CordIn™. The transplant took place at UCSF Benioff Children's Hospital Oakland. Mark Walters, MD, Director of the Blood and Marrow Transplantation (BMT) Program is the Principal Investigator.

CordIn is an experimental curative treatment for rare non-malignant diseases where bone marrow transplantation is the only currently available cure. These include hemoglobinopathies such as SCD and thalassemia, bone marrow failure syndromes such as aplastic anemia, genetic metabolic diseases and refractory autoimmune diseases. This unmet medical need represents a multi billion dollar market potential.

"The successful enrollment and transplantation of our first SCD patient with CordIn as a single graft marks an important milestone in our clinical development program. We are eager to demonstrate the potential of CordIn as a transplantation solution to cure SCD and to broaden accessibility to patients with rare genetic diseases in need of bone marrow transplantation," said Gamida Cell CEO Dr. Yael Margolin. "In the first Phase 1/2 study with SCD, the expanded graft was transplanted along with a non-manipulated umbilical cord blood unit in a double graft configuration. In the second phase 1/2 study we updated the protocol from our first Phase 1/2 study so that patients would be transplanted with CordIn as a standalone graft, which is expanded from one full umbilical cord blood unit and enriched with stem cells using the company's proprietary NAM technology."

Approximately 100,000 patients in the U.S suffer from SCD; and around 200,000 patients suffer from thalassemia worldwide. The financial burden of treating these patients over their lifetime is estimated at $8-9M. Bone marrow transplantation is the only clinically established cure for SCD. However, only a few hundred patients have actually received a transplantation in the last ten years, as most patients were not successful in finding a suitable match. Unrelated cord blood could be available for most of the patients eligible for transplantation, but the rate of successful engraftment of un-manipulated cord in these patients is low and therefore cord blood is not considered for SCD patients. Without a transplant, these patients suffer from very high morbidity and low quality of life.

Eight patients with SCD were transplanted in the first Phase 1/2 study performed in a double graft configuration. This study is still ongoing. Preliminary data from the first study will be summarized and published later this year. A Phase 1/2 of CordIn for the treatment of patients with aplastic anemia will commence later this year.

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 of products are in development to treat 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 current shareholders include: Novartis, Elbit Imaging, Clal Biotechnology Industries, Israel Healthcare Venture, Teva Pharmaceutical Industries, Denali Ventures and Auriga Ventures. For more information please visit gamida-cell.com.

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