Gamida Cell Presents Positive Data From Phase 1/2 Study of NiCord® as a Curative Treatment for Sickle Cell Disease at ASH 2016

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SCD patients treated with NiCord showed rapid engraftment and were cured of disease symptoms

Jerusalem, Israel, Tuesday, December 6, 2016 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, today announced results from its Phase 1/2 study of NiCord for Sickle Cell Disease (SCD)**.** The data were presented during a poster session at the 58th American Society of Hematology (ASH) Annual Meeting & Exposition, December 3-5, 2016 in San Diego, CA.

The results presented at ASH demonstrated the potential of NiCord as a novel transplant approach for patients with SCD. The study provided a clinical proof of concept that NiCord, which was transplanted with an un-manipulated unit of umbilical cord blood (UCB), may enable very rapid engraftment in SCD patients transplanted after myeloablative conditioning.

The principal investigators of the study are Joanne Kurtzberg MD, Professor, Department of Pediatrics; Director, Pediatric Blood and Marrow Transplant Program and Chief Scientific and Medical Officer, Robertson Clinical and Translational Cell Therapy Program, andJoel Brochstein, MD, Chief of Division of Pediatrics – Pediatric Hematology-Oncology, North Shore University Hospital and Associate Professor, Hofstra Northwell School of Medicine.

Allogeneic hematopoietic stem cell transplant (HSCT) from a matched related bone marrow donor is the only established curative therapy for SCD to date; however, most patients in need of HSCT do not have matched donors. Banked, unrelated donor umbilical cord blood (UCB) can provide a sufficiently matched donor to practically all patients in need. However, umbilical cord blood transplants (UCBT) are associated with a highgraft failure risk in patients with hemoglobinopathies attributed to inadequate cell dose in the cord blood graft**.**

“The preliminary results from the Phase 1/2 study indicate that NiCordhas the potential to increase access to transplantation for SCD patients. This is very important given the difficulty for most SCD patients with finding a matched related bone marrow donor as well as the limitations associated with the transplantation of un-manipulated UCB,” said Dr. Kurtzberg.

In a poster titled *Successful Engraftment and Cure of Sickle Cell Disease after Co-transplantation of NiCord® (Ex Vivo Expanded UCB Progenitor Cells with Nicotinamide) and a Second Un-manipulated Cord Blood Unit after Myeloablative Chemotherapy in Children with Severe Sickle Cell Disease*, Dr. Suhag Parikh,from Duke Medical Center, presented data from nine patients, ages 3-17, who were transplanted with NiCord and an un-manipulated unit of cord blood. All nine patients engrafted. At a median follow-up of three years post-transplant, seven out of the nine patients are free of disease. Two of the nine patients have since passed away due to transplant complications.

Highlights from the Phase 1/2 study include:

- NiCord appears to overcome the engraftment barrier, with rapid engraftment achieved consistently at a median of seven days.
- None of the survivors had active GvHD at the last follow-up.
- All surviving patients are free from SCD symptoms, including transfusion independence and a normal hemoglobin profile.

“We are very encouraged with the data, reported for the first time today, indicating that NiCord enables more patients to receive a potentially curative treatment for SCD. This is consistent with the encouraging data we are seeing in patients with blood cancers, where NiCord has shown rapid and sustained engraftment, even as a sole graft source. We intend to further advance this treatment approach for SCD as well as for other rare genetic diseases, such as thalassemia, bone marrow failure syndromes and genetic metabolic diseases,” said Gamida Cell CEO Dr. Yael Margolin.

About NiCord

NiCord is a new graft modality for hematopoietic stem cell transplantation, derived from a single cord blood unit and expanded ex vivo, utilizing a small molecule as an epigenetic approach to increase the number of short and long term engrafting cells and improve their functionality. The platform technology, called NAM technology, was developed by Gamida Cell scientists and is a proprietary asset of the Company. Note: NiCord is further being developed as a standalone graft for SCD and other rare genetic diseases, and has subsequently been branded and listed on the Gamida Cell website as “CordIn™”. NiCord was recently granted an FDA Breakthrough Therapy Designation as a curative treatment for patients with hematological malignancies (blood cancers like leukemia) and is at the last stage of clinical development before commercialization for this indication.

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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