



Gamida Cell Presents Data From Phase 1/2 Study of NiCord® for High Risk Hematological Malignancies at ASCO 2016

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Patients treated with NiCord showed improved engraftment, reduced morbidity and less transplant related mortality compared to controls

Results support commencement of Phase 3 Study

Jerusalem, Israel, Monday, June 6, 2016 — Gamida Cell, a leader in cellular and immune therapies for the treatment of cancer and orphan genetic diseases, announced today the presentation of results from the Company's international, multi-center, Phase 1/2 study of NiCord at the 2016 American Society of Clinical Oncology (ASCO) Annual Meeting. NiCord is in development as a novel transplant modality for high risk hematological malignancies (blood cancers). These results demonstrated statistically significant improvements across key measures including time to engraftment of neutrophils and platelets.

"The meaningful data presented today at ASCO further strengthen previously reported results regarding the key benefits of NiCord. We remain enthusiastic by the potential of NiCord as a curative treatment for high-risk hematological malignancies and hope to confirm these results in a larger patient cohort with a randomized controlled, Phase 3 registration study, on track to begin later this year," said Dr. Yael Margolin, president and CEO of Gamida Cell.

The data were highlighted in an oral presentation, entitled *NiCord®: results of phase 1/2 trials set the stage for a definitive phase 3 clinical trial*, by Mitchell Horwitz M.D. of Duke Cancer Institute, and co-chair of the NiCord study, along with Professor **Guillermo Sanz** of Hospital Universitario La Fe in Valencia, Spain. The data comprised the results of 16 patients, ages 12-65, with high-risk hematological malignancies, who were transplanted with NiCord as a standalone graft following myeloablative therapy. The outcomes of these 16 patients were evaluated at one year post-transplantation. In this analysis, the study outcomes were compared to a retrospective cohort, provided by the CIBMTR registry, of 125 similar patients transplanted with cord blood.

Highlights of the Phase 1/2 Study Results of NiCord demonstrate:

- A 101-fold increase in the number of CD34+ (stem and progenitor) cells in NiCord compared to unmanipulated cord blood.
- Faster neutrophil engraftment for NiCord recipients ($p < 0.0001$) at a median of 10 days post-transplantation compared to 21 days in registry controls.
- By 16 days post-transplant (the usual median time to engraftment after standard peripheral blood transplantation), 75% of NiCord recipients had achieved neutrophil engraftment, compared to only 18% in registry controls ($p < 0.0001$).
- Faster platelet engraftment for patients engrafting with NiCord ($p < 0.001$) at a median of 32 days, compared to 46 days in registry controls.
- At 42 days post-transplant, 56% of NiCord recipients achieved platelet engraftment, compared to 27% in registry controls ($p = 0.015$).
- Transplant related mortality at one year was 19% compared to 39% in registry controls ($p = 0.12$).

The findings announced today are consistent with data presented earlier this year at the 42nd annual meeting of the EBMT where patients transplanted with NiCord demonstrated a significantly lower frequency of grade 2-3 bacterial infections and a shorter hospital stay.

"We have been encouraged by the early clinical data on NiCord to date and believe the results reported today have the potential to suggest clinical benefit to an ex vivo expanded umbilical cord blood graft. Several promising, although early, observations have been documented including rapid recovery of neutrophils and platelets. If the data in a larger study confirm these findings, it could translate into reduced morbidity and transplant-related mortality following umbilical cord blood transplantation. There remain key unmet needs in transplant medicine, so the development of alternative therapies is important," said Dr. Horwitz.

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. This platform technology, called NAM technology, was developed by Gamida Cell scientists and is a proprietary asset of the Company.

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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