

[187] ENABLING THE POSSIBILITY OF BONE MARROW TRANSPLANTATION FOR PRACTICALLY ALL PATIENTS IN NEED.

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Investment Rational

Gamida Cell is a cell and immune therapy visionary. Its broad platform technology expands functional cells in culture to create unprecedented, life-saving cures for multi-billion dollar markets including blood cancers and rare genetic diseases. The company has developed a cost effective and robust manufacturing process under GMP for each product and has in-house manufacturing capabilities.

Core Technology

Our NAM technology has successfully overcome the various challenges of expanding functional umbilical cord blood cells in culture, making bone marrow transplantation available for practically all patients in need. It is already being applied to several cell types to develop unprecedented, life-saving treatments for blood cancers and sickle cell disease and thalassemia.

Product Profile/Pipeline

NiCord[®] for blood cancers provides the first evidence that an ex vivo expanded graft can provide a robust and long lasting hematopoietic and immune recovery. It has an FDA breakthrough therapy designation and FDA and EMEA orphan drug designations. A phase 3 registration study is ongoing. Top line data will be available in 2019.

CordIn is a curative treatment for rare genetic diseases, including blood diseases such as sickle cell disease (SCD) and thalassemia. Clinical studies are ongoing.

NAM-NK has shown in pre-clinical studies to improve the killing efficacy and to overcome some of the limitations of culture expanded NK cells. A Phase 1 study will begin in H1/2017 for patients with refractory B-cell lymphoma.

What's Next?

Gamida Cell is preparing for commercialization of NiCord, market launch NiCord is planned for 2020. In parallel, the company will continue to develop CordIn as a curative treatment for rare genetic diseases, in SCD and additional indications. A clinical study with our NK cell platform will begin in 2017 as an immune therapy for cancer.