

[173] EX VIVO EXPANDED CELLS TO TREAT HEMATOLOGICAL MALIGNANCIES

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- **Investment Rationale**

Gamida Cell is a late-stage biotechnology company developing potentially breakthrough advanced cellular therapies and anticipates multiple milestones in the next 12-18 months, including a Phase 3 data readout for NiCord and the initiation of a multi-center Phase 1/2 clinical study of its natural killer (NK) program.
- **Business Strategy**

Gamida Cell's is focused on delivering curative cell therapies to patients and has worldwide rights to its pipeline. Pending positive Phase 3 data for NiCord, the company expects to submit regulatory applications in 2020 and anticipates U.S. launch-readiness in 2021. Additionally, Gamida Cell is planning for a multi-center clinical study of NAM-NK in 2020.
- **Core Technology**

Gamida Cell's proprietary nicotinamide-based (NAM-based) cell expansion technology is designed to expand cells while maintaining therapeutic functionality. Nicotinamide has been shown to preserve gene expression and modulate cellular metabolism, while reducing cellular stress during expansion. The company's optimized manufacturing process results in robust, replicable production, enabling the next generation of cell therapies with the potential to offer improved clinical outcomes.
- **Product Profile/Pipeline**

Gamida Cell is advancing two programs based on its NAM cell expansion technology: NiCord, an investigational advanced cell therapy in Phase 3 clinical development designed to enhance the life-saving benefits of hematopoietic stem cell (bone marrow) transplant, and NAM-NK, an investigational, cell-based cancer immunotherapy in Phase 1 development in patients with non-Hodgkin lymphoma and multiple myeloma.
- **What's Next?**

The company anticipates the following milestones in 2019-2020

Nicord® - Hematologic malignancies

 - 2H19: Complete enrollment in Phase 3 study
 - 1H20: Report topline Phase 3 data
 - 2H20: Complete BLA filing

Nicord® - Severe aplastic anemia

 - 1H19: Present preliminary data from Cohort 1
 - 1H19: Initiate Cohort 2 evaluating NiCord as stand-alone graft

NAM-NK Phase 1 study

 - 1H19: Present additional data
 - 2H19: Complete patient enrollment
 - 2H19: Present additional data at a medical meeting
 - 2020: Initiate multi-center, Phase 1/2 clinical study