gamida cell

COMMITTED TO CURES

COMPANY HIGHLIGHTS

Clinical-stage company with potential for first product launch in 2022

Management team with deep experience in cell therapy, clinical development and commercialization

Worldwide rights to our innovative pipeline built on our proprietary NAM-enabled cell therapy platform

MANAGEMENT TEAM

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Chief Medical Officer

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Chief Regulatory and Quality Officer

ABOUT GAMIDA CELL

We are a clinical-stage advanced cell therapy company committed to finding cures for patients with cancer and other serious diseases. We are harnessing our novel, proprietary nicotinamide (NAM) cell-expansion platform to create therapies with the potential to redefine standards of care in areas of serious medical need and significantly improve patient outcomes. We are advancing omidubicel, which has successfully completed a Phase 3 study in preparation for biologics license application submission to the U.S. Food and Drug Administration as a potential life-saving treatment option for patients in need of bone marrow transplant. Our pipeline also includes several programs using natural killer (NK) cells to treat hematologic malignancies and solid tumor therapies, including GDA-201, an innate NK cell immunotherapy, which has demonstrated positive initial clinical trial results. An allogeneic, off-the-shelf cryopreserved formulation of GDA-201 will begin evaluation in a Phase 1/2 clinical trial in non-Hodgkin lymphoma (NHL) by the end of 2021. Additionally, the company recently announced a significant expansion of its NAM-enabled NK cell pipeline, including genetically modified variants of proprietary NK therapies using both CRISPR/Cas9 and CAR methodologies, which will enable Gamida Cell to target multiple solid-tumor and hematological cancers.

TECHNOLOGY PLATFORM

Our NAM cell-expansion platform is designed to enhance the number and functionality of donor cells, enabling us to create potentially transformative therapies that move beyond what is possible with existing approaches. Leveraging the unique properties of NAM, we are able to expand and metabolically modulate multiple cell types — including stem cells and natural killer cells — with appropriate growth factors to maintain the cells' active phenotype and enhance potency. This potentially allows us to administer a therapeutic dose of cells that may improve patient outcomes.

Omidubicel and GDA-201 are investigational therapies, and their safety and efficacy has not been established by the U.S. Food and Drug Administration or any other health authority. For more information on clinical trials, please visit www.gamida-cell.com.

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PROGRAMS

Our team is working to develop potentially curative advanced cell therapies for cancer and other serious diseases, including high-risk leukemias, lymphomas, solid tumors and severe aplastic anemia.

Omidubicel

Omidubicel is an advanced cell therapy under development as a potential life-saving allogeneic hematopoietic stem cell (bone marrow) transplant solution for patients with blood cancers. Omidubicel is the first bone marrow transplant graft to receive Breakthrough Therapy Designation from the U.S. FDA and has also received Orphan Drug Designation in the U.S. and EU.

An international, multi-center, randomized Phase 3 study published in *Blood* evaluated the safety and efficacy of omidubicel compared to standard umbilical cord blood transplant.[1]

- The study achieved its primary endpoint ($p<0.001$). In the intent-to-treat analysis, median time to neutrophil engraftment was 12 days for patients receiving omidubicel (95% CI: 10-15 days) compared to 22 days for the comparator group (95% CI: 19-25 days).[2]
- Omidubicel was generally well tolerated. Among patients who were transplanted per protocol, rates of acute and chronic graft-versus host disease were similar and cumulative incidence of infections was significantly smaller in omidubicel compared to controls for both viral infections and bacterial or invasive fungal infections.[1]
- The study also met all three of its secondary endpoints, improving platelet engraftment, and reductions in infections and hospitalizations, which are key measures for success for bone marrow transplant.[3,4,5]

We intend to submit a biologics license application to the FDA in the fourth quarter of 2021. Omidubicel is also being evaluated in a Phase 1/2 clinical study in patients with severe aplastic anemia, a rare and life-threatening blood disorder.[6]

NK Programs

The NAM process enables NK cells to undergo expansion while increasing their function, resulting in greater potency. Our lead candidate in this area, GDA-201, is an innate NK cell immunotherapy for the treatment of hematologic malignancies. Combined with standard of care antibody therapies, GDA-201 has shown potentially greater antibody-dependent cellular toxicity, or ADCC.

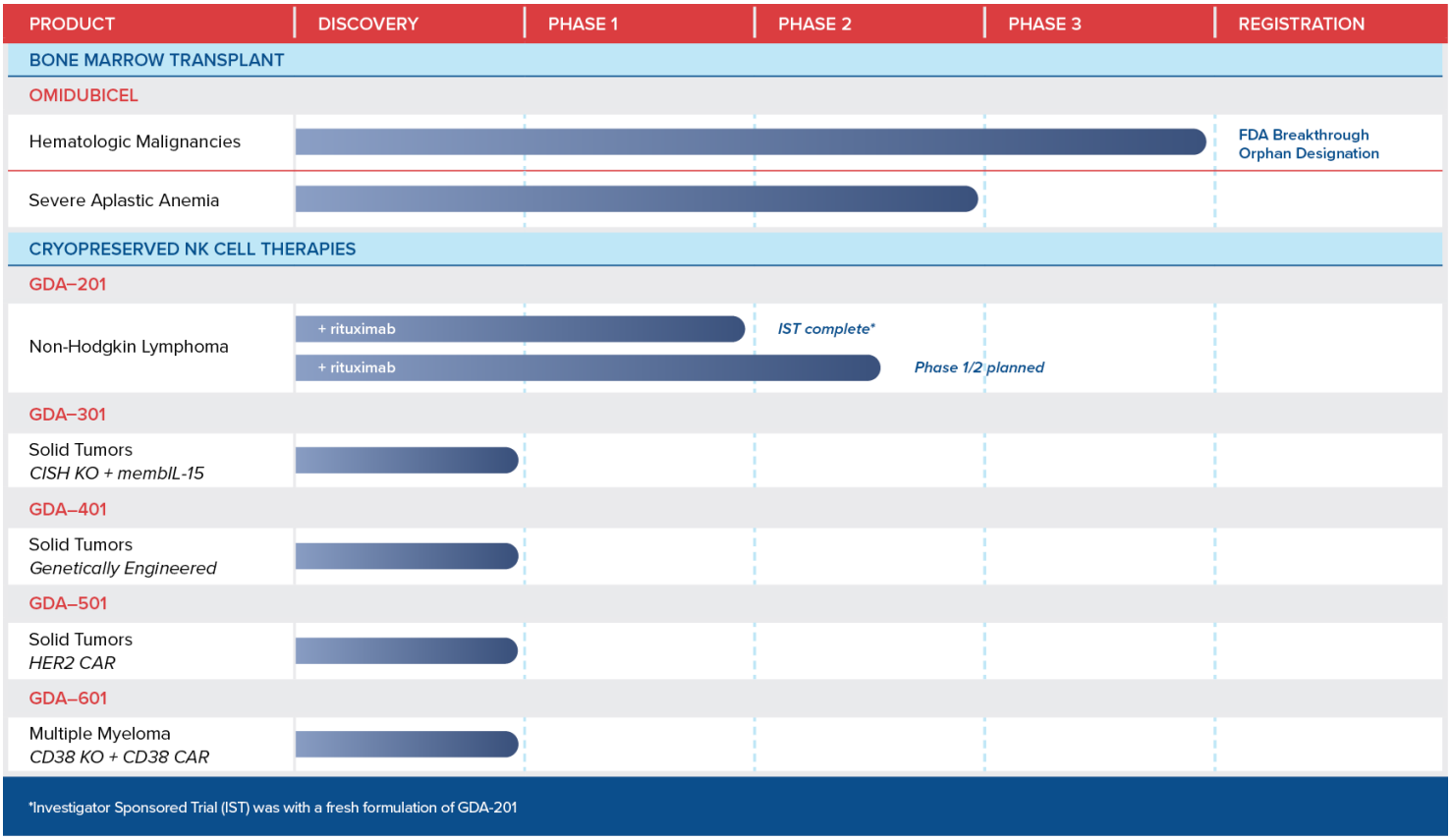
GDA-201 is currently in Phase 1/2 development in patients with refractory NHL and multiple myeloma (MM). We have reported encouraging Phase 1 study data, demonstrating that GDA-201 generally was well tolerated in 35 patients.[7,8] Of the 19 patients with NHL, 13 complete responses and one partial response were observed (CRR = 68%, ORR = 74%). No dose-limiting toxicities were observed.

A Phase 1/2 clinical trial of a new cryopreserved form of GDA-201 in NHL is planned, with IND submission anticipated in Q3 2021 to enable study initiation by end of 2021.

Multiple engineered NK cell programs are currently in development:

- GDA-301 is a knockout of CISH (cytokine inducible SH2 containing protein) in NK cells using CRISPR/Cas9, in combination with a membrane-bound IL-15/IL-15Ra, and is designed to improve tumor killing by promoting activation and inhibiting negative feedback signals.
- GDA-401 is an undisclosed target genetically engineered to enhance NK cell survival in the solid tumor microenvironment for potential application across a broad range of tumors.
- GDA-501 is CAR-engineered to target HER2+ solid tumors with the potential to enhance homing and activation against cancers with HER2 over-expression, including breast, ovarian, lung, bladder, gastric and others.
- GDA-601 combines a CRISPR/Cas9 knockout of CD38 to avoid fratricide in combination treatment with CD38 targeted antibodies and a CD38 CAR designed to enhance killing in MM.
- Undisclosed targets: Additional programs targeting immunosuppressive pathways using both CRISPR/Cas9 and CAR, with potential to treat solid tumor and blood cancers.

OUR PIPELINE



*Investigator Sponsored Trial (IST) was with a fresh formulation of GDA-201

1. Horwitz, ME et al. Omidubicel Versus Standard Myeloablative Umbilical Cord Blood Transplantation: Results of a Phase III Randomized Study. Blood 2021; blood.2021011719.
2. Gamida Cell Announces Positive Topline Data from Phase 3 Clinical Study of Omidubicel in Patients with High-Risk Hematologic Malignancies, May 12, 2020.
3. Gamida Cell Announces Positive Topline Data on Secondary Endpoints from Phase 3 Clinical Study of Omidubicel in Patients with Hematologic Malignancies
4. Gamida Cell Presents Efficacy and Safety Results of Phase 3 Study of Omidubicel in Patients with Hematologic Malignancies. March 15, 2021.
5. **ClinicalTrials.gov** identifier NCT03173937
6. **ClinicalTrials.gov** identifier NCT03019666
7. Bachanova et al. ASH 2020 abstract
8. Bachanova et al. ASH 2020 abstract