Omisirge® (omidubicel-onlv) – New orphan drug approval

- On April 17, 2023, the FDA announced the approval of Gamida Cell's Omisirge (omidubicel-onlv), for use in adults and pediatric patients 12 years and older with hematologic malignancies who are planned for umbilical cord blood transplantation following myeloablative conditioning to reduce the time to neutrophil recovery and the incidence of infection.

- Stem cell transplantation is a common treatment for blood cancers. One source of healthy stem cells is umbilical cord blood. Generally, before receiving this kind of transplant, the patient will undergo a course of treatments to remove their own stem cells and prepare the body for the new stem cells. This process may include undergoing therapies such as radiation or chemotherapy, both of which may weaken an individual's immune system. As a result, a frequent and serious risk of this treatment is the occurrence of severe and sometimes deadly infections.

- Omisirge, administered as a single intravenous dose, is composed of human allogeneic stem cells from umbilical cord blood that are processed and cultured with nicotinamide (a form of vitamin B3). Each dose is patient-specific, containing healthy stem cells from an allogeneic pre-screened donor.

- The efficacy of Omisirge was established in an open-label, randomized study of Omisirge transplantation or umbilical cord blood transplantation following myeloablative conditioning in 125 patients with hematologic malignancies. The efficacy was established based on time to neutrophil recovery following transplantation and the incidence of grade 2/3 bacterial or grade 3 fungal infections through day 100 following transplantation.

    - The median time to neutrophil recovery was 12 days with Omisirge vs. 22 days with umbilical cord blood (absolute difference 10 days, 95% CI: 6, 14).
    - The incidence of infections through 100 days following transplantation was 39% with Omisirge vs. 60% with umbilical cord blood (absolute difference 22, 95% CI: 4, 39).

- Omisirge carries a boxed warning for infusion reactions, graft vs. host disease (GvHD), engraftment syndrome, and graft failure.

- Additional warnings and precautions for Omisirge include hypersensitivity reactions, malignancies of donor origin, transmission of serious infections, and transmission of rare genetic diseases.

- The most common adverse reactions (> 20%) with Omisirge use were infections, GvHD, and infusion reaction.

- Refer to the Omisirge drug label for complete dosing and administration recommendations.

- Omisirge is now available for transplant centers to order for appropriate patients and onboarding of transplant centers is underway.