



## **Nohla Therapeutics Receives FDA Orphan Drug Designation for Dilanubicel for Hematopoietic Stem Cell Transplant Patients**

**Seattle -- (GlobeNewswire – July 16, 2018)** - Nohla Therapeutics, a leading developer of universal, off-the-shelf cell therapies for patients with hematologic malignancies and other critical diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Nohla's lead product candidate, dilanubicel (NLA101), Orphan Drug Designation for reduction of morbidity and mortality associated with hematopoietic stem cell transplant (HSCT). Dilanubicel received Orphan Drug Designation from the European Commission in January 2018.

Under the Orphan Drug Act of 1983, the FDA provides incentives for companies developing treatments that are expected to provide significant therapeutic advantage over existing treatments, and that target rare medical conditions affecting fewer than 200,000 U.S. patients per year. Incentives include seven-year market exclusivity, tax credits on U.S. clinical trials, fast-tracking of regulatory proceedings, exemption from certain fees, such as waiver of filing fees under the Prescription Drug User Fee Act (PDUFA), and orphan drug grants.

"Dilanubicel has shown encouraging initial activity as a novel cell therapy in patients with hematologic malignancies receiving a cord blood transplant," said Katie Fanning, President and CEO of Nohla Therapeutics. "We believe the addition of dilanubicel has the potential to make a meaningful difference for these patients and we look forward to having the top-line results from the fully enrolled randomized Phase 2b trial later this year."

The dilanubicel FDA Orphan Drug Designation was supported by efficacy and safety data from a Phase 2 single arm study in patients with hematologic malignancies who underwent a myeloablative cord blood transplant. The results of this study demonstrated that infusion of dilanubicel was safe and led to faster neutrophil and platelet recovery with excellent long-term survival when compared to a separate control group. In addition, dilanubicel-treated patients experienced no severe acute Graft-versus-Host Disease (GVHD) and no transplant-related mortality.

### **About Dilanubicel**

The company's lead product candidate, dilanubicel, is currently under evaluation in two ongoing Phase 2 trials. A Phase 2b randomized trial (NCT01690520), has completed enrollment of 160 patients with hematologic malignancies undergoing a myeloablative allogeneic cord blood transplant with or without dilanubicel. In addition, the global Phase 2 LAUNCH trial (NCT03301597) is currently enrolling AML patients with chemotherapy-induced myelosuppression.

Dilanubicel is a universal donor, off-the-shelf, ex vivo expanded hematopoietic stem and progenitor cell investigational product intended to provide rapid, transient hematopoiesis with long-term immunologic benefits. Unlike autologous or patient-specific allogeneic cell therapies, dilanubicel does not require Human Leukocyte Antigen (HLA) tissue matching. Intentionally developed to provide short-term, temporary bone marrow function until a patient's immune system recovers, dilanubicel may also induce long-term immunologic benefits with the potential for improved survival. Dilanubicel is manufactured ahead of time, cryopreserved, and intended for immediate off-the-shelf use.

**About Nohla Therapeutics**

Nohla Therapeutics is a leading developer of off-the shelf cell therapies for patients with cancer and other critical diseases. Nohla's proprietary notch ligand technology platform serves as the foundation for its ongoing clinical, preclinical and discovery programs. The company's lead product candidate, dilanubicel, is currently under evaluation in two ongoing Phase 2 trials. Nohla is also pursuing multiple preclinical and discovery programs in the areas of immune tolerance and other diseases. More information is available at [www.nohlatherapeutics.com](http://www.nohlatherapeutics.com) or on Twitter @nohlatx.

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