



Nohla Therapeutics Granted Orphan Drug Designation in the European Union for NLA101 in the Treatment of Hematopoietic Stem Cell Transplantation

Seattle, Washington -- (GlobeNewswire – January 23, 2018) - Nohla Therapeutics Inc. (Nohla), a clinical stage biopharmaceutical company focused on the development of universal donor, off-the-shelf cell therapies to treat cancer and other critical diseases, today announced the European Commission (EC) has granted Orphan Drug Designation to NLA101 as an orphan medicinal product for the treatment in hematopoietic stem cell transplantation.

NLA101 is a hematopoietic stem and progenitor stem cell therapy currently being evaluated in a Phase 2b multi-center, open-label randomized study (NCT01690520) of myeloablative cord blood transplantation with or without infusion of NLA101 in patients with hematologic malignancies. The primary endpoint of the Phase 2b study is time to engraftment with an absolute neutrophil count (ANC) ≥ 500 . Secondary endpoints for this study include survival, non-relapse mortality, incidence of severity of graft versus host disease (GVHD) and other healthcare economic outcomes such as days in the hospital and rates of severe infections. Over 90% of this 160-patient trial has been enrolled.

“NLA101 has shown encouraging results when used in combination with a cord blood transplant,” commented Katie Fanning, President and CEO of Nohla. “We believe NLA101 has the potential to make a meaningful difference for patients with hematologic malignancies who are candidates for an allogeneic transplant and we look forward to reporting results from the Phase 2b randomized trial later this year.”

Orphan drug designation from the EC provides regulatory and financial incentives for companies to develop and market therapies that treat life-threatening or very serious conditions that affect no more than 5 in 10,000 people in the European Union (EU), and where there is an unmet medical need. In addition to a 10-year period of marketing exclusivity in the EU upon product approval, orphan drug designation provides fee waivers, protocol assistance, and marketing authorization under the centralized procedure in all EU countries.

About Allogeneic Stem Cell Transplant

Allogeneic hematopoietic stem cell transplantation continues to be the only potentially curative treatment option for patients with high-risk hematologic diseases. Despite the proven benefits of allogeneic stem cell transplants, physicians still face barriers to use. These barriers include complications associated with infections from delayed engraftment and/or acute and chronic GVHD. There are an estimated 13,000 patients with hematologic malignancies in Europe who are candidates for an allogeneic transplant. This number includes those patients who are eligible, but unable to find a suitable match within the current donor registries.

About NLA101

NLA101 is a universal, off-the-shelf stem and progenitor cell therapy that provides a short term bridge for hematopoietic recovery while also providing long term immunologic and clinical benefits. Clinical results to date demonstrate that NLA101 has the potential to provide a patient-ready, short-term bridge to immune repair and essential blood cell production, while improving patient survival. Over 125 infusions of NLA101 have been administered across four clinical trials since 2009 with no drug-related safety issues observed to date. In a 15-patient pilot study in the setting of myeloablative cord blood



transplant, subjects receiving NLA101 experienced a significantly reduced median time to platelet and neutrophil recovery and 86% disease-free survival at five years post-transplant compared to 67% in the concurrent control group. In addition, no treatment related mortality (TRM) or grade 3-4 acute GVHD were observed in recipients of NLA101, compared to the control group in which 29% of patients experienced severe grade 3-4 acute GVHD.

In addition to the ongoing Phase 2b study in patients receiving a cord blood transplant, NLA101 is also being evaluated in a Phase 2 global trial, called LAUNCH (NCT03301597), which is intended to enroll 220 adult patients with AML who are at risk for myelosuppression following high dose chemotherapy. The primary objectives of this trial are to (a) evaluate the effect of NLA101 on the rate of Grade ≥ 3 bacterial and fungal infections associated with chemotherapy-induced neutropenia and (b) obtain evidence about the lowest effective cell dose of NLA101 in reduction of infection when given as a single dose infusion after each cycle of chemotherapy. Site initiation is underway and patient enrollment is expected to begin in Q1 2018. More information can be found at clinicaltrials.gov.

About Nohla

Nohla Therapeutics is a clinical stage cell therapy company that's redefining clinical outcomes for patients with critical diseases by providing a short-term hematopoietic bridge to immune repair and healthy blood production with long-term immunologic benefits. Nohla's proven platform generates universal, off-the shelf therapies that enable improved clinical outcomes across a number of disease indications, with an initial focus on high-risk hematological malignancies. Nohla's lead candidate, NLA101, is an expanded progenitor cell therapy that provides the functional support of bone marrow in response to each patient's unique blood-production needs, without any requirement for HLA matching. The product has demonstrated the potential for robust efficacy in multiple clinical trials, while overcoming the broad safety and logistical risks of patient-customized cell therapies. More information is available at www.nohlatherapeutics.com or Twitter @nohlatx.

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