CSR News

EGT Welcomes FDA Clinical Phase I Trial Approval

An important milestone towards a curative therapy for beta-thalassemia major

Submitted by: **Errant Gene Therapeutics, LLC**
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CHICAGO, Aug. 14 /CSRwire/ - Errant Gene Therapeutics, LLC (“EGT”), a pioneering boutique drug development firm specializing in Rare Diseases, applauds the FDA's approval of Phase I clinical trials for beta-thalassemia patients with autologous CD34+ cells transduced with TNS9.3.55. TNS9.3.55 is a cGMP vector technology previously known as Thalagen(c). Under EGT’s agreement with Sloan Kettering Institute ("SKI"), the trial is sponsored by Memorial Sloan Kettering Cancer Center ("MSKCC") in New York City (ClinicalTrials.gov identifier NCT01639690) and represents an important milestone towards the prospect for a cure for a chronic condition, which would otherwise subject the patient to early death without incurring life-long regular blood transfusions and chelation therapy.

EGT is particularly proud and remains committed to its long-standing collaboration with TNS9.3.55's inventor, Dr. Michel Sadelain of MSKCC, and its decade’s long commitment to finding a cure for beta-Thalassemia (also called Cooley’s Anemia).

The FDA's approval for the Phase I Clinical Trial furthers EGT's work and outreach. From Italy, a major center for beta-thalassemia patients, EGT's founder & CEO, Pat Girondi stated that “the palpable hope shared by patients of all ages in all parts of the world, and by researchers in key centers in the US, EU and Asia, reinforces our dedication to ensuring that this journey only ends with a cure". From New York City, Sam Salman, EGT’s president added that, "the promise of delivering a cure derived from a patient's own bone marrow stem cells, provides the basis of a new standard of care for chronic hemoglobinapothies, many of which are life-threatening. EGT is proud to be advancing the current state-of-the-art therapies targeting Rare Diseases."

About Errant Gene Therapeutics, LLC

Errant Gene Therapeutics (“EGT”) is a privately held biopharmaceutical company established in 2003. In addition to its ongoing support of gene therapy for the cure of beta-thalassemia and Sickle Cell Anemia, EGT is a pioneer in the emerging field of epigenetics focused on the treatment of deadly hormone refractory cancers using its patented portfolio of small molecule

histone deacetylases inhibitors (HDACi). EGT's lead HDACi, CG-1521, targets Inflammatory Breast Cancer and Hormone Refractory Prostate Cancer.

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