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Errant Gene Therapeutics, LLC

Errant Gene Therapeutics, LLC (“EGT”) Pushing Clinical Trial of Potentially Curative Treatment for Beta-Thalassemia and Eventually Sickle Cell Disease

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TAMPA, Fla.--(BUSINESS WIRE)--The technology, developed by scientists at Memorial Sloan Kettering Cancer Center ("MSKCC"), headed by Dr. Michel Sadelain and Errant Gene Therapeutics, is a one-time treatment that inserts an encoded gene into a patient's own bone marrow stem cells restoring the production of normal hemoglobin. This technology is known as Thalagen.

In June of 2020, an abstract was released to the European Hematology Association (EHA) noting the results of patients treated in a clinical trial at MSK with the EGT vector. The abstract is based on patients treated with the 2009 EGT-produced vector in the MSK Clinical Trial. The EHA abstract, submitted by Simona Raso, reports that 2 out of 3 Thalassemia patients treated with EGT’s vector have sustained dramatic reduction in blood transfusions after 8 and 5 years, respectively. These 3 patients are the only Thalassemic patients treated with Lentiglobin in the US for whom there is an 8-year follow-up. The abstract is publicly available on the European Hematology Association’s website at the following address:

https://library.ehaweb.org/eha/2020/eha25th/293982/simona.raso.gene.therapy.with.the.lentiviral.vector.tns9.3.55.html?f=menu%3D14%2Abrowseby%3D8%2Asortby%3D2%2Amedia%3D3%2Aspeaker%3D731017.

The reductions in transfusions for the patients reported in the EHA abstract means a marked reduction in risk and damage created by the chronic transfusions, transferred diseases and iron build-up. One of the patients with significant transfusion deduction used the EGT-produced vector with a mild chemotherapeutic prep-regimen. The abstract does not report any clonal dominance. EGT is the only company with experience in development of a non-myeloablative potential treatment for Thalassemia patients.

EGT produced the world’s first commercial batch of gene therapy vector in 2009. The EGT vector uses the wild-type beta globin gene, the most natural form of the gene.

EGT Founder, Patrick Girondi noted, “After some delay, we are happy to be moving forward once again, and the EHA abstract is incredible news for patients. Today, with modern production, enhancers, improved filtration and other prep regimen drugs, we believe that the vector EGT will produce in 2021, honed by 12 years of advancement in the field and using modern transduction enhancers will cure Thalassemia patients and that EGT will make quick headway towards curing Sickle Cell patients using the same therapy.”

EGT, a gene therapy pioneer’s goal is to make medicines which are safe and accessible to patients. EGT believes the EGT vector to be more natural and therefore safer. Additionally, the cost of the treatment is drastically lower than that of competing products. EGT will work with regulatory agencies to continue the trial, formerly sponsored by Memorial Sloan Kettering Cancer Center NCT01639690.

Ronald Capano of Cooley’s Anemia International says, “This clinical trial means so much to so many and represents the work and dedication of our organization and that of the family and friends of all Thalassemia and Sickle Cell anemia patients, particularly those with compromised organs.”

About Errant Gene Therapeutics, LLC

Errant Gene Therapeutics (also known as EGT) is a privately held biopharmaceutical company established in 2003 headquartered in Tampa, Florida. In addition to its ongoing support of gene therapy for beta-thalassemia and Sickle Cell anemia, EGT is a pioneer in the emerging field of epigenetics, and its patented portfolio of small molecule histone deacetylase inhibitors, which change the way cells express their genetic material. EGT’s lead compound, CG-1521, targets inflammatory breast cancer and hormone refractory prostate cancer. CG-1521 results have been published in scientific venues.

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