



Errant Gene Therapeutics Becomes San Rocco Therapeutics

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TAMPA, Fla.--(BUSINESS WIRE)--Errant Gene Therapeutics was founded in 1993, after founder Pat Gironi's son was diagnosed with Thalassemia, a cousin disease to Sickle Cell Anemia.

In 2007, with the help of researchers from Memorial Sloan Kettering, Cornell and National Institute of Health, EGT became the first entity to pass the FDA Recombinant DNA Committee for gene therapy in Sickle Cell Disease and Beta Thalassemia. EGT was the first company to get Orphan Drug Designation for Thalassemia in the US and Europe and first to produce a commercial batch (8-10 patients) of gene therapy for Sickle Cell Disease and Thalassemia. EGT is the company with the longest track record of treating US patients and the only company with experience in both harsh and soft chemotherapeutic prep-regimens.

Errant Gene will become San Rocco Therapeutics (SRT). San Rocco is the patron saint of hopeless disease. EGT opened *Centro Medico San Rocco* in Altamura, Italy, in 1995 and was free of charge for dozens of patients suffering from hemoglobinopathies.

The SRT vector uses the natural wild type beta globin gene. Other companies, such as Bluebird Bio, used a mutant gene. To date, four patients have been treated with the EGT vector with no incidence of Clonal Dominance and no incidence of AML leukemia.

Dr. Lucio Luzzatto, former Chair of the ASGCT's Ethics Committee recently commented on the barrier to access for most patients and was quoted in *Lancet* (Vol 8, April 2021), "Nearly all recently introduced targeted drugs are unavailable or unaffordable." Gironi adds, "Most Orphan Disease research is paid for by the taxpayer through public grants and not for profits. It's time to 'weed the greed.' We cannot have taxpayer-sponsored therapies turn into products which patients cannot afford because of gross executive compensation."

San Rocco aims for a one-time price of \$700,000. The price will be lowered as more patients are treated. This is more than \$1,000,000 less than Bluebird's price of \$1,800,000 and in line with EMEA and FDA wishes of Orphan Disease products which are affordable to patients.

EGT CEO, Pat Gironi says, "Born and raised a true US capitalist, the first thing I do as a leader is to sacrifice myself. A dollar I use is a dollar less for research. I have an allergic reaction to the sheets if my hotel room costs more than \$100. I'm not paying for that room. The patients are."

From the beginning of EGT in 2003, Pat Gironi, the CEO, and Sam Salman the president (2009-2020), took no compensation. "It was and is a labor of love." Gironi says, "We work for our patients."

Gironi continues, "I smirk when I read of colleagues, such as Bluebird's CEO taking a 50% cut to a \$6,000,000 compensation. It's absurd. Should we feel sorry for them? Would the authors of these articles like us to pass a hat?"

The average US family income is under \$60,000. \$6,000,000 is 100 times that, 100 families income for a year, or one family working 100 years.

Gironi says, "We're waiting for FDA guidelines on lentiviral vectors. After many years, there's just us and CRISPR. I believe that our product is safe, as we use the natural wild type beta globin gene. We have one of the world's greatest scientists, Michel Sadelain, at one of the world's premier research institutes, Memorial Sloan Kettering, behind us. I think that we're going to help a lot of folks."

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