Press release

SQY THERAPEUTICS RECEIVES FDA ORPHAN DRUG DESIGNATION FOR SQY51 FOR THE TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

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SQY Therapeutics, announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to SQY51 for the treatment of Duchenne Muscular Dystrophy (DMD).

SQY51 is a palmitoyl-conjugated tc-DNA antisens oligonucleotide designed to restore semi-functional dystrophin by skipping exon 51 during mRNA splicing of the *DMD* gene in a subpopulation of boys affected by Duchenne Muscular Dystrophy.

"This important designation is a milestone in the development of SQY51 and highlights the need for potential new treatment options for patients with Duchenne Muscular Dystrophy," said Luis Garcia, Ph.D., Special Scientific Advisor of SQY Therapeutics, “The designation will facilitate the development of our molecule which we believe has the potential to serve as a much-needed therapeutic option for patients affected by DMD.”

The FDA's Orphan Drug Designation program supports the development of drugs that address rare diseases which affect fewer than 200,000 people in the United States. Incentives that come with the designation include eligibility for federal grants, tax credits for qualified clinical trials, prescription drug user fee exemptions, and a seven-year marketing exclusivity period upon FDA approval.

About SQY Therapeutics

SQY Therapeutics is a French clinical-stage biotech company founded by parents of DMD boys and researchers, with the aim of implementing clinical R&D programs for genetic diseases, primarily Duchenne Muscular Dystrophy. SQY Therapeutics is developing a unique antisens technological platform focused on tricyclo-DNA, with a view to promoting new therapeutic solutions for severely debilitating diseases for which the current treatment offer remains unsatisfactory.

About DMD

Duchenne Muscular Dystrophy is a rare genetic muscle-wasting disease predominantly affecting boys, with symptoms usually appearing between three and five years of age. Duchenne is a progressive, irreversible, and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated number of 3000 cases in France. It is estimated that 100 to 150 newborn boys in France suffer from Duchenne Muscular Dystrophy every year.

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