MyoGene Granted Orphan Drug Designation and Rare Pediatric Disease Designation for Duchenne Muscular Dystrophy Program

These designations make MyoDys45-55, a gene editing therapy designed to treat up to 50% of all Duchenne patients, eligible for incentives

SAN DIEGO, CA, UNITED STATES, November 19, 2024 /EINPresswire.com/ -- MyoGene Bio, a biotech dedicated to developing cutting edge therapies for muscle diseases, announced today that the U.S. Food and Drug Administration (FDA) has recently granted Orphan Drug Designation (ODD) and Rare Pediatric Disease (RPD) designation to MyoDys45-55, MyoGene's gene editing therapy for Duchenne muscular dystrophy.

> Gene editing offers a new approach for treating Duchenne. MyoDys45-55 targets the patient's own DNA to permanently correct the mutation and restore gene function."
>
> *Courtney Young, CEO of MyoGene Bio*

Duchenne is a progressive and fatal muscle-wasting disease which affects children and young adults, primarily males. No medical cures exist, and with currently available treatments, the prognosis remains poor.

MyoDys45-55 is an experimental therapy designed to correct the gene mutations that cause half of all Duchenne cases and to slow or stop progression of the disease.

"Gene editing offers a new approach for treating Duchenne. MyoDys45-55 targets the patient's own DNA to permanently correct the mutation and restore gene function," said Courtney Young, PhD, CEO and co-founder of MyoGene Bio. "We are pleased that FDA granted both Rare Pediatric Disease and Orphan Drug Designation to MyoDys45-55. These designations provide important benefits that will help us bring MyoDys45-55 to patients as quickly as possible."

Orphan Drug Designation provides tax credits for clinical trials, exemption from user fees, and seven years of market exclusivity. Rare Pediatric Disease designation allows sponsors to request a Priority Review Voucher (PRV) to be granted upon marketing approval. PRVs cut the marketing application review time in half and can be redeemed for a future program or sold to another sponsor.

About MyoDys45-55

MyoDys45-55 is a gene editing therapy that targets a mutational hotspot within the DMD gene. MyoDys45-55 deletes exons 45-55 to restore the DMD reading frame, and in turn, the expression of an internally-deleted dystrophin protein that is more than 85% identical to wildtype dystrophin. MyoDys45-55 is applicable to all mutation types within DMD exons 45-55, for approximately 50% of all Duchenne patients. MyoDys45-55 is currently in preclinical development.

About MyoGene Bio

MyoGene Bio is a privately held biotech company dedicated to developing next-generation genetic therapies for Duchenne and other muscle diseases. MyoGene Bio has received funding from CIRM, NIH (NINDS and NIAMS), DOD CDMRP, MDA, PPMD, DUK and CureDuchenne Ventures. MyoGene Bio is currently raising additional funding and looking for investors. Learn more here: https://www.myogenebio.com/.

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