APB-102

Name: APB-102
Synonyms: APB 102
Indication: Amyotrophic lateral sclerosis (ALS)
Company: Apic Bio

APB-102, as a one-time treatment for amyotrophic lateral sclerosis (ALS) caused by a mutation in the SOD1 gene. This Apic sponsored program now employs a second-generation vector design with a novel delivery route for the one-time treatment of genetic (SOD1) ALS. This program is based on a compassionate use IND study begun by Robert H. Brown, Jr., DPhil, MD; and Christian Mueller, PhD, in 2017 at the University of Massachusetts Medical School, with collaborators at the Massachusetts General Hospital.

Apic Bio is committed to finding cures for patients with genetic diseases. The company is a spin-off from the University of Massachusetts Medical School (UMMS) and is based upon nearly 30 years of gene therapy research by Apic's scientific founders. Apic is developing best-in-class treatment options for rare, devastating neurological and liver diseases. Its current pipeline focuses on new and effective treatments for Alpha-1 Antitrypsin Deficiency (Alpha-1, or AATD) and genetic Amyotrophic Lateral Sclerosis (ALS.)

THRIVE™

Numerous diseases are associated with inherited or somatic mutations. In many cases, these mutations can lead to both a toxic-gain-of function and loss-of function as in the case for Alpha-1 antitrypsin deficiency. In other cases, mutations in a single allele lead to dominant toxicity without a clear loss-of function, but approaches to reduce their toxicity by non-specifically silencing both alleles can unmask or result in a loss-of function. In both these scenarios, the THRIVE™ platform provides a superior therapeutic approach than simple gene silencing or gene replacement approaches. The THRIVE™ platform both silences a mutant gene product and replaces a normal gene product in a single "dual function" vector.