May 2020

The Food and Drug Administration (FDA) granted orphan drug designation for Shift Pharmaceutical's lead drug compound to treat all forms of spinal muscular atrophy (SMA). Orphan Drug Status provides incentives for companies to research drug developments to treat rare diseases such as SMA.

Spinal Muscular Atrophy (SMA) is the most common inherited motor neuron disease and occurs in 1 out of every 11,000 live births with a carrier frequency of 1 in 40 worldwide. Most children born with SMA fail to reach their second birthday. Treating SMA should be a global priority due to the relatively high frequency for a "rare" disease combined with the often catastrophic impact on families.

Shift Pharmaceuticals' $E1^{v1.11}$ aims to treat all types of SMA by using the patient's own genome to increase the production of active SMN protein throughout the neuromuscular system. $E1^{v1.11}$ is an Antisense oligo (ASO) that shows great efficacy, dose-response, safety, and target engagement. Shift's work has been published by Chief Scientific Officer Christian Lorson and his team at the University of Missouri-Columbia.

About Shift Pharmaceuticals

Shift Pharmaceuticals (a privately held company) is developing Antisense Oligonucleotides as drug candidates for a variety of genetic disorders. Shift was co-founded by serial entrepreneur Dr. Steve O'Connor and world-leading Spinal Muscular Atrophy (SMA) research expert Dr. Chris Lorson. The company has licensed the core intellectual property from The University of Missouri, where the initial discovery and development was performed by Dr. Lorson.

Shift Pharmaceuticals
Dr. Steve O'Connor, CEO
(626) 893-2514
steve.oconnor@shiftpharmaceuticals.com