April 16, 2019

Dear Duchenne community:

We are excited to share that on April 16th, 2019, we shared safety and tolerability data from our Phase 1 clinical trial evaluating investigational suvodirsen (WVE-210201) in boys with Duchenne muscular dystrophy. The results from this trial support progressing to a Phase 2/3 clinical trial, which we intend to initiate in July 2019, so we wanted to review what comes next.

The Phase 1 clinical trial was a placebo-controlled, single ascending dose study to evaluate safety, tolerability and pharmacokinetics of suvodirsen. Thirty-six boys with Duchenne muscular dystrophy amenable to exon 51-skipping therapy were given a single infusion of suvodirsen or placebo and followed by their clinical teams for 85 days. The results of the study demonstrated that a single infusion of suvodirsen was generally safe and well-tolerated at doses up to and including 5 mg/kg and support initiation of a Phase 2/3 trial to evaluate efficacy and safety.

The Phase 2/3 clinical trial, called DYSTANCE 51, is a global, multi-center, placebo-controlled study designed to evaluate the efficacy and safety of suvodirsen in boys with Duchenne muscular dystrophy amenable to exon 51 skipping. The trial design has been accepted into the FDA's Complex Innovative Trial Design (CID) Pilot Program. Through the CID pilot program, our goal is to reduce the number of patients required for the study, thereby minimizing the number of patients required in the placebo treatment arm and potentially accelerating completion of the trial. For more information on this study please contact us at clinicaltrials@wavelifesci.com or visit ClinicalTrials.gov here (ClinicalTrials.gov Identifier: NCT03907072).

Our deepest thanks go to all the courageous boys who participated in the Phase 1 study and the families that support them. In addition, we are ever grateful to all of those in the Duchenne community, including the families, advocacy partners, regulators, and clinicians who have provided invaluable guidance on this program from the very beginning.

Sincerely,

Michael Panzara, MD, MPH
Chief Medical Officer

Questions and Answers

What's next for the suvodirsen clinical program?
Suvodirsen is currently being evaluated in an ongoing multi-dose open-label extension (OLE) study with boys from the Phase 1 clinical trial. We are also planning to initiate a global Phase 2/3 efficacy and safety trial, called DYSTANCE 51. DYSTANCE 51 is a multicenter, randomized, double-blind, placebo-controlled clinical trial that will enroll ambulatory boys 5 – 12 years of age with Duchenne muscular dystrophy amenable to exon 51 skipping.

What is suvodirsen?
Suvodirsen (formerly known as WVE-210201) is an investigational exon 51 skipping stereopure antisense oligonucleotide. Exon skipping is an approach that may restore the DMD mRNA reading frame in people with amenable mutations, resulting in restoration of dystrophin protein. Suvodirsen was developed using PRISM™, our proprietary discovery and drug development platform. Suvodirsen has been granted orphan drug designation for the treatment of DMD by the U.S. Food and Drug Administration (FDA) and the European Commission, as well as rare pediatric disease designation by the FDA.

What is the status of Wave's other exon skipping research?
We are advancing a lead candidate for exon 53 are we are actively pursuing research programs designed to skip exons 44, 45, 52, 54 and 55.