December 16, 2019

Dear Duchenne community:

We have a disappointing update to share on the clinical program for suvodirsen, our investigational exon 51 skipping stereopure antisense oligonucleotide. After reviewing interim dystrophin results from the Phase 1 open-label extension (OLE) study, we've made the difficult decision to discontinue the suvodirsen program. In addition, we are suspending further development of our candidate for individuals with Duchenne who have mutations amenable to exon 53 skipping.

We initiated our research in Duchenne with the goal of restoring meaningful levels of dystrophin and we are profoundly saddened to have fallen short of achieving this objective with suvodirsen. Over the past few years, as we've worked to develop suvodirsen, many of us at Wave have been privileged to meet hundreds of families living with Duchenne, and to work with the advocacy organizations that support you. The strength, courage, and perseverance of the Duchenne community has humbled and inspired us every single day. While today's announcement is deeply disappointing, we are hopeful and confident that brighter days are ahead for individuals and families living with Duchenne.

The results from the clinical trial show that there was no change in dystrophin expression with either the 3.5 mg/kg or 5 mg/kg doses of suvodirsen. These results were unexpected given the preclinical results seen with suvodirsen, but clearly demonstrated that treatment with suvodirsen did not result in increased dystrophin restoration in boys. No safety concerns were observed. We will share the findings from this study in hopes that the community can benefit from its contributions to this program.

As a result of this decision, we are working with clinical trial sites to immediately discontinue the OLE study and the Phase 2/3 trial, DYSTANCE 51. No further doses of suvodirsen will be administered, and no further biopsy procedures will be performed. We are also working with investigators to provide what they need to support families participating in these studies. After the final data from the study have been analyzed, we are committed to enabling investigators to share data for each patient with their family.

Our sincere and heartfelt thanks to the individuals and families that have participated in clinical trials for suvodirsen. If you or a family member are participating in the study, we encourage you to contact your clinician for further information. We would also like to thank all of those in the Duchenne community – including the families, advocacy partners, regulators, and clinicians – who have provided invaluable guidance on this program.

Sincerely,

Paul Bolno
President and Chief Executive Officer

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