Exon 51: WVE-210201

WVE-210201 Phase 1 clinical trial initiated November 2017

– Design: Multicenter, double-blind, placebo-controlled, single ascending dose study with I.V. administration

– Primary endpoint: Safety and tolerability

– Inclusion criteria: ages 5 to 18, amenable to exon 51 skipping

• Ambulatory and non-ambulatory boys eligible, including those previously treated with eteplirsen (following appropriate washout

period)

– Readout expected Q4 2018

– Open-label extension (OLE) with muscle biopsy and ≥2-years of follow-up

• WVE-210201 planned efficacy study

– Efficacy readout anticipated H2 2019

– Design: Double-blind, placebo-controlled, multi-dose study assessing dystrophin expression and clinical outcomes

– Measurement of dystrophin via standardized Western Blot

– Interim analysis of dystrophin expression in muscle biopsies

• Exploring intravenous and subcutaneous formulations for WVE-210201