MDA Collaborates with AVI BioPharma on First Phase 2 Placebo-Controlled Clinical Trial of Exon-51 Skipping Drug as Potential Therapy for Duchenne Muscular Dystrophy

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TUCSON, Ariz., Aug. 15, 2011 /PRNewswire-USNewswire/ -- More than a decade of targeted Muscular Dystrophy Association-funded research, made possible as a result of generous public support of the MDA Labor Day Telethon and thousands of grass-roots special events, has today culminated in MDA providing financial assistance for the start of the first Phase 2 placebo-controlled, multiple dose efficacy, safety, tolerability and pharmacokinetics clinical trial of an exon-51 skipping drug, eteplirsen, as a potential therapy for Duchenne muscular dystrophy (DMD).

The first three of 12 DMD boys participating in the AVI BioPharma clinical trial at Nationwide Children's Hospital in Columbus, Ohio today received the first of 24 weekly doses of eteplirsen or a placebo by intravenous infusion (i.v.). Four more participants had muscle biopsies vital to measuring the presence of the essential muscle protein dystrophin both before and after treatments. The seven boys traveling in for the study launch are from Calif., Ill., Fla., Wis., Va. and Mo.

"This is an important day for families fighting muscular dystrophy," said R. Rodney Howell, M.D., Chairman of the MDA Board of Directors. "AVI BioPharma already completed a 19-patient clinical trial in the United Kingdom confirming the potential of eteplirsen to be a safe and effective disease-modifying drug for DMD (The Lancet, July 25, 2011). Now, a team led by Dr. Jerry Mendell is receiving funding from MDA to help initiate this randomized, double-blind, placebo-controlled 12-patient trial needed to further test safety, efficacy and optimal dosing."

"Twenty-five years ago, MDA-funded investigators identified the dystrophin gene that, when mutated (or defective) causes Duchenne muscular dystrophy as well as the somewhat milder Becker muscular dystrophy (BMD)," explained Mendell, Curran-Peters Chair of Pediatric Research at Nationwide Children's Hospital, and Professor of Pediatrics and Neurology at
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