

AVI BioPharma Announces Eteplirsen Meets Primary Endpoint, Demonstrating a Significant Increase in Dystrophin at 24 Weeks Compared to Placebo in Phase IIb Trial for the Treatment of Duchenne Muscular Dystrophy

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BOTHELL, WA, Apr 02, 2012 (MARKETWIRE via COMTEX) --AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based therapeutics, today announced that treatment with eteplirsen met the primary efficacy endpoint in a randomized, double-blind, placebo-controlled Phase IIb study in boys with Duchenne muscular dystrophy (DMD). Eteplirsen administered once weekly at 30mg/kg over 24 weeks resulted in a statistically significant ($p \leq 0.002$) increase in novel dystrophin (22.5% dystrophin-positive fibers as a percentage of normal) compared to no increase in the placebo group.

"This study represents a major advance in the field of DMD research as the results indicate that eteplirsen is producing consistent levels of dystrophin, which is the essential protein that these patients need," said Jerry Mendell, M.D., Director of the Centers for Gene Therapy and Muscular Dystrophy at Nationwide Children's Hospital and principal investigator of the Phase IIb study. Dr. Mendell added, "We anticipate that these levels of dystrophin could lead to significant clinical benefit if maintained over a longer course of treatment."

In the study, a shorter duration of eteplirsen treatment, 12 weeks, did not show a significant increase in novel dystrophin (0.79% dystrophin-positive fibers as a percentage of normal; p-value NS), despite administration of the drug at a higher dose (50mg/kg once weekly). This finding suggests that a longer duration of dosing is required before meaningful levels of dystrophin are produced. There were no significant improvements in clinical outcomes in the treated groups compared to placebo. Performance on the 6-minute walk test and other outcome measures were generally stable across most of the patients, including the placebo patients, suggesting that a longer period of observation will be required to demonstrate clinical effects of eteplirsen versus a placebo control.

Eteplirsen was well tolerated at both dose levels through 24 weeks of treatment. There were no treatment-related adverse events, no serious adverse events, and no treatment discontinuations related to eteplirsen. Furthermore, no treatment related changes were detected on any safety laboratory parameters, including several biomarkers for renal function.

"We are very encouraged by the results of this first placebo-controlled study investigating exon-skipping technology in DMD," said Chris Garabedian, President and CEO of AVI BioPharma. "Eteplirsen represents the first drug candidate for DMD to demonstrate the production of novel dystrophin in a robust and consistent manner and these study results support advancing eteplirsen into a pivotal study."

Conference Call AVI BioPharma, Inc. will hold a conference call to discuss these results today at 8:00 a.m. EDT (5:00 a.m. PDT). The conference call may be accessed by dialing 800.561.2718 for domestic callers and 617.614.3525 for international callers. The passcode for the call is 99858553. Please specify to the operator that you would like to join the "AVI BioPharma Phase IIb Top-Line Data Results Call." The conference call will be webcast live under the events section of AVI's website at www.avibio.com and will be archived there following the call for 90 days. Please connect to AVI's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. An audio replay will be available through April 9, 2012 by calling 888.286.8010 or 617.801.6888 and entering access code 16040637.

About Study 201 (Eteplirsen Phase IIb Study) Study 4658-US-201 was conducted at Nationwide Children's Hospital in Columbus, Ohio. Twelve boys meeting the inclusion criteria being between 7 and 13 years of age with appropriate deletions of the dystrophin gene that confirm eligibility for treatment with an exon-51 skipping drug received double-blind IV infusions of placebo (n=4), 30 mg/kg of eteplirsen (n=4), or 50 mg/kg of eteplirsen once weekly for 24 weeks (n=4). Muscle biopsies for evaluation of dystrophin were obtained at baseline for all subjects and after 12 weeks for patients in the 50 mg/kg cohort and after 24 weeks for patients in the 30 mg/kg cohort. Two placebo patients were randomized to the 30 mg/kg cohort and two placebo patients were randomized to the 50 mg/kg cohort. This study design allowed AVI to investigate the relationship of dose and duration of eteplirsen treatment on the production of dystrophin over the course of the 24 week study.

About Eteplirsen Eteplirsen is AVI's lead drug candidate that is systemically delivered for the treatment of a substantial subgroup of patients with DMD. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression.

Eteplirsen uses AVI's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from mRNA. Promoting the synthesis of a truncated dystrophin protein is intended to improve, stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

AVI is also developing other PMO-based exon-skipping drug candidates intended to treat additional patients with DMD.

About AVI BioPharma AVI BioPharma is focused on the discovery and development of novel RNA-based therapeutics for rare and infectious diseases, as well as other select disease targets. Applying pioneering technologies developed and optimized by AVI, the Company is able to target a broad range of diseases and disorders through distinct RNA-based mechanisms of action. Unlike other RNA-based approaches, AVI's technologies can be used to directly target both messenger RNA (mRNA) and precursor messenger RNA (pre-mRNA) to either down-regulate (inhibit) or up-regulate (promote) the expression of targeted genes or proteins. By leveraging its highly differentiated RNA-based technology platform, AVI has built a pipeline of potentially transformative therapeutic agents, including eteplirsen, which is in clinical development for the treatment of Duchenne muscular dystrophy, and multiple drug candidates that are in clinical development for the treatment of infectious diseases. For more information, visit www.avibio.com.

Forward-Looking Statements and Information In order to provide AVI's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the development of eteplirsen and its efficacy, potency and utility in the treatment of DMD and the potential for the creation of novel dystrophin to lead to significant clinical benefit over a longer course of treatment.

These forward-looking statements involve risks and uncertainties, many of which are beyond AVI's control. Known risk factors include, among others: clinical trials may not demonstrate the safety and efficacy of eteplirsen and/or AVI's antisense-based technology platform; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; and any of AVI's drug candidates, including eteplirsen, may fail in development, may not receive required regulatory approvals, or be delayed to a point where they do not become commercially viable.

Any of the foregoing risks could materially and adversely affect AVI's business, results of operations and the trading price of AVI's common stock. For a detailed description of risks and uncertainties AVI faces, you are encouraged to review the official corporate documents filed with the Securities and Exchange Commission. AVI does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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