

## **AVI BioPharma to Present Additional Data From the Phase IIB Study of Eteplirsen for the Treatment of Duchenne Muscular Dystrophy at the 2012 AAN Annual Meeting**

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### **Previously Reported Data Demonstrated Study Met Primary Endpoint**

BOTHELL, WA, Apr 25, 2012 (MARKETWIRE via COMTEX) --AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based therapeutics, announced that data from a Phase IIB study evaluating eteplirsen as a treatment for boys with Duchenne muscular dystrophy (DMD) will be presented today at the American Academy of Neurology 64th Annual Meeting in New Orleans, Louisiana. Principal investigator, Jerry R. Mendell, M.D. of Nationwide Children's Hospital, will describe the data via a brief oral presentation of the abstract titled "A Phase IIB Placebo-Controlled Study of the Exon-Skipping Drug Eteplirsen in Subjects with Duchenne Muscular Dystrophy" during the AAN Emerging Science Session (abstract #004 at 5:54 pm CDT), followed by a more detailed poster presentation (6:30 to 7:00 pm CDT).

The abstract will describe new and previously reported efficacy and safety data from the Phase IIB study examining 24 weeks of treatment with eteplirsen in boys with DMD. Results from the randomized, double-blind, placebo-controlled study confirm that the trial met its primary endpoint, demonstrating a significant increase in dystrophin at 24 weeks compared to placebo. Data showed that 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.

Additional data to be presented today include:

- Individual patient data on the primary endpoint of change in dystrophin-positive fibers from baseline;
- Biochemical findings across the 24-week treatment cohort (30 mg/kg/wk dose) including RT-PCR and western blot images for each individual patient in this cohort;
- An exploratory analysis of performance on the 6-minute walk test comparing eteplirsen-treated patients compared to placebo. Two patients in the 30 mg/kg cohort showed rapidly progressive decline on the 6-minute walk test and were excluded from the analysis. Of the remaining patients, the eteplirsen-treated patients demonstrated a 17.8 meter benefit versus placebo over 24 weeks (-3.2 meter decline from baseline vs. -21.0 decline, respectively). This was an exploratory analysis and not a statistically significant finding.
- A summary of treatment-emergent adverse events comparing eteplirsen-treated patients versus placebo, which demonstrated that eteplirsen was well tolerated through 24 weeks of treatment. No treatment-related adverse events, serious adverse events, and treatment discontinuations related to eteplirsen were observed. In addition, no treatment related changes were detected on any safety laboratory parameters, including several biomarkers for renal function.

Dr. Mendell's presentation will be posted on the AVI BioPharma web site in the "Events & Presentations" section after the session is completed.

### **About Duchenne Muscular Dystrophy**

DMD is one of the most common fatal genetic disorders to affect children around the world. Approximately one in every 3,500 boys worldwide is affected with DMD. A devastating and incurable muscle-wasting disease, DMD is associated with specific inborn errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness eventually spreads to the arms, neck and other areas. Eventually, this progresses to complete paralysis and increasing difficulty in breathing due to respiratory muscle dysfunction requiring ventilatory support as well as cardiac muscle dysfunction leading to heart failure. The condition is terminal, and death usually occurs before the age of 30.

### **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 disease. For more information, please visit [www.avibio.com](http://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.

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 safety and efficacy of eteplirsen and/or AVI's antisense-based technology platform; 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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