AVI BioPharma Initiates Dosing in Phase 2 Study of Eteplirsen in Duchenne Muscular Dystrophy Patients

August 15, 2011 4:06 PM ET

BOTHELL, WA, Aug 15, 2011 (MARKETWIRE via COMTEX) --

AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based therapeutics, today announced that it has initiated dosing in a Phase 2 study of eteplirsen, the Company's lead exon-skipping therapeutic candidate for the treatment of Duchenne muscular dystrophy (DMD).

The placebo-controlled study of twelve patients, which will be conducted at Nationwide Children's Hospital in Columbus, Ohio, is designed to evaluate the efficacy and safety of eteplirsen in DMD patients over 24 weeks of dosing. Patients enrolled in the study will receive once weekly intravenous infusions of either 50mg/kg of eteplirsen, 30mg/kg of eteplirsen or placebo, and will be evaluated on a number of safety and efficacy endpoints. The efficacy endpoints will include biochemical markers in muscle biopsies, such as the production of the dystrophin protein and markers of immune-inflammatory response, as well as clinical outcomes to measure muscle strength, function and degree of ambulation.

"In this Phase 2 study we will evaluate eteplirsen at higher doses and over a longer duration of treatment, which will help us understand the potential disease-modifying effects and safety of eteplirsen as chronically-administered therapy," said Chris Garabedian, AVI's CEO and president. "We expect data from this study around the end of the second quarter of 2012, which will guide our design for a pivotal study."

Jerry R. Mendell, M.D., of Nationwide Children's Hospital and principal investigator of the study added, "Despite many years of awareness and investment in therapeutic development, we only have supportive treatments available for DMD patients today. We have seen tremendous promise for eteplirsen to potentially modify the progression of DMD in patients and we look forward to further understanding its potential through this longer study."

The travel costs for the patients participating in the Phase 2 clinical study are supported in part by grants from Parent Project Muscular Dystrophy and Muscular Dystrophy Association.

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. The results of an AVI sponsored UK based trial were recently published in The Lancet. The abstract and full paper can be accessed at http://www.thelancet.com/journals/lancet/article/PIIS0140-6736%2811%2960756-3/abstract.

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 a 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 AVI's product candidates and their efficacy, potency and utility in the treatment of rare and infectious diseases and the availability of study results from the Phase 2 study of eteplirsen around the end of the second quarter of 2012.

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 any of AVI's drug candidates and/or AVI's antisense-based technology platform; and any of AVI's drug candidates 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 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.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995: The statements that are not historical facts contained in this release are forward-looking statements that involve risks and uncertainties, including, but not limited to, the results of research and development efforts, the results of preclinical and clinical testing, the effect of regulation by the FDA and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, and other risks detailed in the company's Securities and Exchange Commission filings.

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