

## **Results From Phase I Clinical Trials Demonstrate Positive Safety Data of AVI BioPharma's RNA-Based Therapeutics for Treatment of Ebola and Marburg Viruses**

February 9, 2012 8:33 AM ET

### **Data and Safety Monitoring Board Recommends Proceeding With Multiple Ascending Dose Studies**

BOTHELL, WA, Feb 09, 2012 (MARKETWIRE via COMTEX) --AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based therapeutics, today announced positive safety results from all six dose cohorts in the single ascending dose studies of AVI-6002 and AVI-6003, AVI's lead drug candidates being evaluated for the treatment of Ebola virus and Marburg virus, respectively. AVI is conducting this work under a Department of Defense contract managed by the Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office.

Data from 60 subjects in 12 cohorts from the AVI-6002 and AVI-6003 studies combined were evaluated by an independent Data and Safety Monitoring Board (DSMB), which issued recommendations for both studies to progress as planned to multiple ascending dose studies after no safety concerns were identified. The Phase I single ascending dose trials were designed to characterize the safety, tolerability and pharmacokinetics of each therapeutic candidate in healthy adult volunteers.

"The favorable safety profiles of these two candidates show promise for drugs that utilize our PMOplus(R) chemistry," said Chris Garabedian, president and CEO of AVI BioPharma. "These data underscore how our chemistry may be safe independent of the sequence that is targeted, which we believe will support applications to other disease targets."

The Phase I multiple ascending dose studies are planned to characterize the safety, tolerability and pharmacokinetics of multiple doses of AVI-6002 and AVI-6003 in healthy adult volunteers. The randomized, double-blind placebo controlled studies will be overseen by a DSMB, who will review safety and clinical laboratory data after each dose cohort prior to enrolling the next highest dose cohort.

AVI-6002 and AVI-6003 are AVI's lead therapeutic candidates for the Ebola and Marburg viruses, respectively. Both candidates employ AVI's patented PMOplus(R) technology that selectively introduces positive charges to its phosphorodiamidate morpholino oligomer (PMO) backbone to improve interaction between the drug and its target.

#### **About Ebola and Marburg Viruses**

Ebola hemorrhagic fever is a severe and often fatal disease in humans. The disease was first recognized in 1976 and is one of two members of a family of RNA viruses called Filoviridae. The disease is generally understood to be endemic to parts of Africa. Onset of illness from Ebola virus is abrupt with symptoms that include fever, headache, muscle ache, vomiting and stomach pain. Internal and external bleeding may also be observed in some patients. There are currently no treatments for Ebola virus infection beyond supportive care.

Marburg hemorrhagic fever is another severe and potentially fatal disease in humans first recognized in 1967. It is also caused by an RNA virus of the filovirus family and is understood to be endemic to Africa. Onset of the disease is often sudden, and the symptoms include fever, chills, nausea, vomiting, chest pain and diarrhea. Increasingly severe symptoms may also include massive hemorrhaging and multiple organ dysfunctions. There are currently no treatments for Marburg virus infection beyond supportive care.

#### **About AVI's PMOplus(R) Chemistry**

PMOplus(R) chemistry is an advanced generation of AVI's phosphorodiamidate morpholino oligomer, or PMO, technology pioneered by AVI. The PMO platform is designed to provide a stable chemistry backbone with superior drug-like characteristics for AVI's advanced RNA-based therapeutics. PMOplus(R) chemistry includes specific molecular charges positionally inserted into the PMO's inherent charge-neutral backbone. The PMOplus(R) modifications are intended to specifically enhance drug performance characteristics on two key parameters: targeted cell penetration and the maintenance of antiviral performance in the presence of viral mutation.

#### **About JPM-TMT**

The Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office supports the overall mission of the U.S. Department of Defense (DOD) by protecting the Warfighter and the nation from emerging, genetically engineered or unknown biothreats. Chartered within the Joint Program Executive Office for Chemical and Biological Defense (JPEO-CBD), JPM-TMT partners with the DOD, other government agencies, academia, and industry for the advanced development of adaptable platform technologies that can be rapidly tailored and deployed to mitigate the effects of the unknown threat, whether it be naturally occurring or man-made. Program investments target the most difficult challenges of medical capability development and fill gaps not currently addressed by the biodefense community. For more information, visit [www.jpmtmt.mil](http://www.jpmtmt.mil).

#### 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 AVI's product candidates, their efficacy, potency and utility in the treatment of rare and infectious diseases, their potential to treat a broad number of human diseases and AVI's plans to initiate Phase I multiple ascending dose studies of AVI-6002 and AVI-6003.

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; development of AVI-6002 or AVI-6003 may not result in funding from JPM-TMT in the anticipated amounts or on a timely basis, if at all; 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 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.

#### AVI Media and Investor Contact:

Erin Cox  
425.354.5140  
[Email Contact](#)

#### AVI Media Contact:

David Schull  
212.845.4271  
[Email Contact](#)

SOURCE: AVI BioPharma, Inc.