

## **AVI BioPharma Announces Successful Clinical Trial of AVI-4658 for Treatment of Duchenne Muscular Dystrophy by Exon Skipping**

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### **Company to Host Conference Call Today at 9:00 a.m. ET**

### **For Immediate Release**

PORTLAND, OR — January 21, 2009 — AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based drugs, today announced results from a Phase 1 trial of its drug candidate AVI-4658 for the treatment of Duchenne muscular dystrophy (DMD) by exon skipping. Biopsy data showed that injection of the drug into the muscles of a series of DMD patients successfully induced dystrophin production in each patient.

The proof of principle, single dose escalation study tested the effect of an intramuscular (IM) injection of AVI-4658 in boys with DMD. Each patient received an injection of 0.09 or 0.9 mg of AVI-4658 into the exterior digitorum brevis muscle of one foot and an injection of saline as placebo into the corresponding muscle of the opposite foot to provide an internal treatment comparison. Three to four weeks later, each injected muscle was biopsied and examined for evidence of dystrophin production. Results demonstrated that injection of AVI-4658 elicited dystrophin production in a dose dependent manner in all treated patients. Further, the drug was well tolerated, with no significant detectable drug-related adverse events.

The clinical study was performed in the UK by members of the MDEX Consortium led by Professor Francesco Muntoni. Professor Muntoni commented, “As a clinician and scientist, I am very pleased by these findings and the prospects they offer for the potential treatment of this serious, life threatening condition. Biopsies from muscles injected with the higher dose of test drug showed an unequivocal, widespread and robust response in terms of number of dystrophin positive muscle fibers. We will publish these exciting data in a peer-reviewed journal in due course.”

Patients with DMD have a very low capacity to make dystrophin. In general, and in this study, DMD patients have less than 5% dystrophin positive muscle fibers prior to treatment.

“These are promising data which support the continued development of AVI-4658 as a potential exon skipping treatment for DMD and are highly competitive with data disclosed by other companies working in this field,” said Leslie Hudson, Ph.D., President and Chief Executive Officer of AVI BioPharma. “A multi-dose, dose escalation trial to examine the efficacy of the drug candidate following systemic administration (IV) – also in collaboration with the MDEX Consortium — was opened in December 2008 and our collaborators have begun the work up of the first cohort of DMD patients prior to dosing.”

DMD is an incurable muscle-wasting disease associated with errors in the gene that makes dystrophin, a protein that plays a key structural role in muscle fiber function. The drug candidate AVI-4658 is designed to skip exon 51 of the dystrophin gene, allowing for restoration of the reading frame in the mRNA sequence. Restoration of dystrophin production achieved by skipping this exon may improve or significantly slow the disease process, thus prolonging and improving the quality of life for the affected patient population. It is important to note that different mutations in the dystrophin gene require different oligonucleotide drugs. In principle, approximately 80% of all DMD patients could be treated with exon-skipping drugs. AVI-4658, and the four related exon-skipping drugs under development in AVI BioPharma, could be used to treat more than half of this population — if they prove to be effective — with a potential market value of approximately \$2.0 billion. AVI BioPharma was granted orphan drug designation for AVI-4658 by the U.S. Food and Drug Administration in November 2007 and by the European Medicines Agency (EMA) in December 2008.

The IM injection trial was funded by the Department of Health (UK) and conducted by the members of the MDEX Consortium, led by Professor Muntoni at Imperial College Healthcare NHS Trust facilities. Imperial College London is the sponsor for the trial, with AVI BioPharma serving as its clinical development collaborator.

### **Conference Call Details**

AVI BioPharma, Inc. will hold a conference call today, Wednesday, January 21, 2009, at 9:00 a.m. Eastern time (6:00 a.m.

Pacific) to provide an update on the Company's Duchenne muscular dystrophy program.

Individuals interested in listening to the live conference call may do so by dialing 866-303-7746 toll free within the United States and Canada, or 416-641-6139 for international callers.

A replay of the call will be available by dialing 800-408-3053 toll free within the U.S. and Canada or 416-695-5800. The passcode for the replay is 3281420. In addition, a recording of the call will be available within approximately 24 hours at [www.avibio.com/events.php](http://www.avibio.com/events.php).

## **About Duchenne Muscular Dystrophy (DMD)**

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 afflicted with Duchenne muscular dystrophy with 20,000 new cases reported each year. It is a devastating and incurable muscle-wasting disease associated with specific inborn errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Symptoms usually appear in male children before age 6. Progressive muscle weakness of the legs and pelvis eventually spreads to the arms, neck, and respiratory muscles. By age 12, patients are confined to a wheelchair. Eventually, the condition progresses to complete paralysis with increasing difficulty in breathing. The condition is terminal and death usually occurs before the age of 30. The outpatient cost of care for a non-ambulatory DMD boy is among the highest of any disease and has been estimated at between \$400-500,000 per annum. There is currently no cure for DMD, but for the first time in decades, there are promising therapies in or moving into clinical development.

## **About AVI BioPharma**

AVI BioPharma is focused on the discovery and development of RNA-based drugs utilizing proprietary derivatives of its antisense chemistry (morpholino-modified phosphorodiamidate oligomers or PMOs) that can be applied to a wide range of diseases and genetic disorders through several distinct mechanisms of action. Unlike other RNA-based therapeutic approaches, AVI's antisense technology have been used to directly target both messenger RNA (mRNA) and its precursor (pre-mRNA), allowing for both up- and down-regulation of targeted genes and proteins. AVI's RNA-based drug programs are being evaluated for the treatment of Duchenne muscular dystrophy as well as for the treatment of cardiovascular restenosis through our partner Global Therapeutics, a Cook Group Company. AVI's antiviral programs have demonstrated promising outcomes in Ebola Zaire and Marburg Musoke virus infections and may prove applicable to other viral targets such as HCV or Dengue viruses. For more information, visit [www.avibio.com](http://www.avibio.com).

## **About the MDEX Consortium**

The MDEX consortium led by Professor Muntoni, is a multidisciplinary enterprise to promote translational research into muscular dystrophies, and is formed by the clinical groups of Professor Francesco Muntoni (Imperial College London and UCL Institute of Child Health) and Professor Kate Bushby and Professor Volker Straub (Newcastle University), and scientists from Imperial College London (Professor Dominic Wells), UCL Institute of Child Health (Dr Jennifer Morgan), Royal Holloway University of London (Professor George Dickson and Dr Ian Graham), Oxford University (Dr Matthew Wood) and University of Western Australia (Prof Steve Wilton). In addition, the charities Muscular Dystrophy Campaign (MDC), Action Duchenne and Duchenne Family Support Group also participate in the Consortium. For more information visit [www.mdex.org.uk](http://www.mdex.org.uk).