

## **AVI BioPharma to Present Safety Data in Duchenne Muscular Dystrophy at 14th Annual International Congress of the World Muscle Society**

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### **AVI-4658 Demonstrates Compelling Safety Profile, Targeted Exon Skipping and New Dystrophin Production in Intramuscular Injection Study; Preliminary Data from Ongoing Systemic Study Also to be Presented During Late Breaking News Session**

#### **For Immediate Release**

BOTHELL, WA — Aug. 25, 2009 — AVI BioPharma, Inc. (Nasdaq: AVII), a developer of RNA-based drugs, today announced that it will present the full data from its completed Phase 1 clinical trial of its splice skipping oligomer (SSO) AVI-4658 in patients with Duchenne Muscular Dystrophy (DMD) at the 14th Annual International Congress of the World Muscle Society on Saturday, September 12, 2009 at 2:30 p.m. local time in Geneva, Switzerland. Preliminary safety data from AVI's currently ongoing systemic Phase 1b/2 clinical trial of AVI-4658 in patients with DMD also will be presented during the conference's Late Breaking News session on Saturday, September 12, 2009 at 4:00 p.m. local time.

Data from the completed single-blind, placebo-controlled and dose escalation Phase 1 trial showed that AVI-4658 was safe when injected intramuscularly and successfully induced the production of dystrophin protein in patients in a dose-responsive manner. AVI also announced today that these data have been published online in the journal *Lancet Neurology* and will be featured in the October print edition. This safe and well-tolerated production of new dystrophin is believed to be the key to restoring muscle function and successfully treating patients with DMD - a condition for which there is no currently approved therapy.

Additionally, preliminary data from the ongoing Phase 1b/2 systemic trial will be presented that demonstrate that AVI-4658 has been well tolerated in patients with DMD in the first two completed cohorts (0.5 mg/kg and 1.0 mg/kg) and two ongoing cohorts (2.0 mg/kg and 4.0 mg/kg). There have been no serious adverse events or safety issues identified and the independent Data Safety Monitoring Board approved each dose escalation. AVI could initiate dosing in the final two dosing cohorts (10.0 mg/kg and 20 mg/kg) in the next few weeks.

"We are extremely excited about these data and look forward to sharing the full findings of our Phase 1 intramuscular injection study and the preliminary data from our ongoing Phase 1b/2 study with our peers and colleagues at the World Muscle Society conference," said Dr. Stephen B. Shrewsbury, Chief Medical Officer and Senior Vice President of Preclinical, Clinical and Regulatory Affairs of AVI. "These findings further support AVI's belief that SSO-induced exon skipping via AVI-4658 offers a very promising therapeutic strategy for treating DMD. These data offer compelling new insights into the long-term safety of our drug candidates, have helped shape our ongoing systemic administration study and, most importantly, could support potential future treatments for patients living with DMD."

The first AVI presentation is entitled "Restoration of dystrophin expression in Duchenne Muscular Dystrophy: a single blind, placebo-controlled dose escalation study using morpholino oligomer AVI-4658" and will be presented by Dr. Virginia Arechavala-Gomez, a researcher associated with Professor Francesco Muntoni, Professor of Pediatric Neurology at the University College London Institute of Child Health and principal investigator of the study and of the MDEX Consortium.

Dr. Shrewsbury, Chief Medical Officer and Senior Vice President of Preclinical, Clinical and Regulatory Affairs of AVI, will present the preliminary data from the ongoing Phase 1b/2 in a late breaking news presentation entitled "Current progress with the systemic administration trial of AVI-4658, a novel Phosphorodiamidate Morpholino Oligomer (PMO) skipping exon 51 in Duchenne muscular dystrophy (DMD)."

The currently ongoing Phase 1b/2 dose-finding clinical trial is evaluating the systemic delivery of AVI-4658. This is an open label, 12-week safety trial, which includes measures of drug efficacy and pharmacokinetics, being conducted in London, UK at the UCL Institute of Child Health / Great Ormond Street Hospital NHS Trust facilities and at the Royal Victoria Infirmary,

Newcastle-Upon-Tyne, UK which is the coordinating center for the European Treat Neuromuscular Diseases (Treat-NMD) initiative. The clinical costs for the trial are provided, in part, by the UK Medical Research Council.

## **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 by age three. Progressive muscle weakness of the legs and pelvis eventually spreads to the arms, neck, and other areas. By age 10, braces may be required for walking, and most patients are confined to a wheelchair by age 12. Eventually, this progresses to complete paralysis and increasing difficulty in breathing, requiring ventilatory support. 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. There is currently no cure for DMD, but for the first time ever, there are promising therapies in or moving into clinical development.

## **About the MDEX Consortium**

The MDEX consortium led by Professor Francesco Muntoni, is a multidisciplinary enterprise to promote translational research into muscular dystrophies, and is formed by the clinical groups of Professor Francesco Muntoni (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), Oxford University (Dr. Matthew Wood) and University of Western Australia (Professor 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).

## **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 therapeutic approaches, AVI's antisense technology has 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).