

## **AVI BioPharma and Action Duchenne Team Up to Support Advancement of PMO-based Therapeutics for Treatment of Duchenne Muscular Dystrophy**

July 28, 2009 6:15 PM ET

### **Action Duchenne provides \$1.2m to support continuing development of drugs to treat Duchenne Muscular Dystrophy**

#### **For Immediate Release**

CORVALLIS, OR., and London, UK — July 28, 2009 — AVI BioPharma, Inc. (Nasdaq: AVII), a developer of RNA-based drugs, and Action Duchenne, a leading UK charity dedicated to increasing awareness, engendering action and raising funds to find a cure for Duchenne Muscular Dystrophy (DMD), today announced a collaboration to support the acceleration of research and development for AVI's exon skipping candidate drugs for the treatment of DMD.

"AVI has a new class of candidate drugs which are an important contribution to Action Duchenne's search for a treatment for DMD. We have teamed up with AVI to help accelerate these clinical programs as part of our commitment to cure this devastating disease," said Nick Catlin, CEO of Action Duchenne. "Our primary goal is to help provide treatment options for the many children and adults afflicted with DMD. We believe we can provide significant value to AVI's ground breaking efforts."

"AVI and Action Duchenne share a common goal to advance new therapeutics and find treatment options for DMD patients," said Leslie Hudson, Ph.D., President and Chief Executive Officer of AVI BioPharma. "We believe this collaboration has high potential and we are also very pleased to receive the financial support from Action Duchenne which will help accelerate our research and development efforts for new exon skipping therapeutics."

The agreement has a one-year term, with an option to extend for additional years, and will provide approximately \$1.2 million in support to AVI over the initial term for advancement of research, regulatory efforts and clinical trial recruitment.

AVI is currently conducting a dose-finding clinical trial 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 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.

AVI-4658 is designed to skip exon 51 of the dystrophin gene, allowing for restoration of the reading frame in the mRNA sequence. By skipping this exon, a truncated, yet potentially functional form of the dystrophin protein is produced, which could ameliorate the disease process and possibly prolong and improve the quality of life of these patients. Results from a Phase 1 proof-of-concept trial showed that injection of the drug into the muscles of a series of DMD boys successfully induced dystrophin production in a dose-responsive manner. Further, the drug was well tolerated, with no significant drug-related adverse events detected. The clinical trial was conducted in collaboration with the MDEX Consortium in London UK. AVI is also developing AVI-5038, a new candidate drug based on second-generation PPMO chemistry and designed to skip exon 50. The preclinical work for AVI-5038 is funded in part by Charlie's Fund. The Company is currently working to advance this new drug candidate into clinical trials under an investigational new drug application (IND) in the United States and an investigational medicinal product dossier (IMPD) in Europe.

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

DMD is the most common fatal genetic disorder 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 six. 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. 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 in decades, there are promising therapies in or moving into development.

## **About Action Duchenne**

Action Duchenne (formally Parent Project UK) was set up by Duchenne families in 2001 to promote new research for a cure for Duchenne. The charity has a strong record in funding research and has to date funded 9 major projects costing over £1m and has been a leading partner in the £1.6m DoH MDEX project. These projects have enabled much needed early work to be completed on exon skipping and other therapeutic approaches. Action Duchenne holds an international conference every year to bring together researchers and families to exchange new research developments and provide a vital meeting venue for scientists.

In 2005 Action Duchenne launched the Duchenne Registry, the first National Duchenne database that holds gene information of people living with Duchenne and can be used to speed up the recruitment of patients for clinical trials. In 2006 Action Duchenne launched a comprehensive learning and behaviour toolkit for use by parents and education professionals. For more information please visit: [www.actionduchenne.org](http://www.actionduchenne.org)

## **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).