

## **AVI BioPharma Receives Grants Totaling \$500,000 from CureDuchenne and the Foundation to Eradicate Duchenne to Support Continuing Development of Drug Candidates to Treat Duchenne Muscular Dystrophy**

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### **For Immediate Release**

BOTHELL, WA — February 15, 2010 — AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA-based drugs, today announced that CureDuchenne and the Foundation to Eradicate Duchenne (FED), each awarded grants of \$250,000 to AVI BioPharma to support continued research and development of the Company's exon skipping drug candidates for the treatment of Duchenne Muscular Dystrophy (DMD), a genetic muscle wasting disease caused by failure to produce dystrophin. Cure Duchenne and FED are US not-for-profit foundations fully dedicated to supporting the research and development of a cure for DMD.

"AVI shares a commitment with the Foundation to Eradicate Duchenne and CureDuchenne to advance the research and development of disease-modifying drugs, treat DMD, and significantly help patients," said Leslie Hudson, Ph.D., President and Chief Executive Officer of AVI BioPharma. "We are grateful for the generous financial support of both organizations. This funding will help us continue to advance our drug candidates, including our lead drug candidate, AVI-4658, and move them closer to becoming new treatment options for patients."

"Exon skipping holds promise as a treatment for Duchenne muscular dystrophy. CureDuchenne is very happy to support AVI BioPharma as it advances these treatments to boys with DMD as soon as possible," stated Debra Miller, President and Founder, CureDuchenne. "As the parent of a 13-year old boy afflicted with DMD, I shall be very pleased to see AVI's programs progress as quickly as possible."

"The exon-skipping strategies being developed by AVI offer the greatest prospect for meaningful clinical therapies for the majority of boys and young men afflicted with this cruel disorder. We are gratified by the partnership with CureDuchenne, Children's National Medical Center and AVI," commented Joel Wood, President and Founder of the Foundation to Eradicate Duchenne. "Speaking as the parent of a 12-year-old with DMD, I'm tremendously optimistic that we can punch through the remaining hurdles in time for this generation of DMD kids. This is an anxious and exciting time in the history of this disorder."

### **AVI-4658 Study 28 Overview**

AVI is currently conducting a dose-finding clinical trial evaluating the systemic delivery of AVI-4658. Known as Study 28, this ongoing Phase 1b/2 open label clinical trial is assessing the safety, tolerability, pharmacokinetics and exploratory efficacy of AVI-4658 in ambulatory DMD boys between the ages of 5 and 15 years of age who have an error in the gene coding for dystrophin that could be treated by skipping exon 51. Patients are dosed once per week for 12 weeks by intravenous infusion. Nineteen patients were enrolled in total and assigned to one of six dose cohorts: 0.5, 1.0, 2.0, 4.0, 10.0 or 20.0 mg/kg. After completion of dosing, patients are followed for a further 14 weeks. The primary objective of the trial is to assess the safety of AVI-4658 at these doses over the 26-week duration of the trial. The trial is fully enrolled and the final cohort is being dosed.

Data from patients dosed to date demonstrate that AVI-4658 continues to be generally well tolerated. Adverse events reported to date are mostly mild, unrelated to drug treatment and transient. In the patients who completed dosing, two serious adverse events, both deemed unrelated to AVI-4658, were reported in different patients after they completed their 12-week treatment period and during the 14-week follow-up period.

In Study 28, efficacy data from patients in the first four dose cohorts who completed 12 weeks of treatment demonstrates that all patients in the 2 and 4 mg/kg cohorts (3 of 3 in total) showed correctly spliced mRNA for the dystrophin protein. One of these patients, a boy in the 2mg/kg cohort, showed a robust treatment response: a fivefold increase in dystrophin expression (from 0.9% to 5.3% of normal) on a western blot analysis, and an increase from 1% pre-treatment to 21%, in the percentage of dystrophin positive muscle fibers in patient muscle biopsies as measured by immunofluorescence analysis. After completing treatment, no RNA or protein expression signal was detected in patients in the lowest dose cohorts, 0.5 mg/kg or 1.0 mg/kg. Restoration of functional dystrophin expression is considered critical for successful treatment of DMD.

### **Studies Towards US IND**

AVI has completed a series of 12-week preclinical studies of AVI-4658 under Good Laboratory Practice (GLP) conditions required to open an Investigational New Drug (IND) application in the US. The studies tested doses up to 960 mg/kg in both mdx and wild type mice, and up to 320 mg/kg in non-human primates, both doses being the maximum feasible single doses in these animals. In all cases the PMO was well tolerated at doses equivalent to 80 mg/kg and 110 mg/kg in humans respectively (based on standard allometric scaling), suggesting the potential for a wide therapeutic index. These studies were conducted by AVI in cooperation with Eric Hoffman, Ph.D., of the Children's National Medical Center, Washington DC, and supported by a U.S. Defense Department grant.

An additional GLP study of AVI-4225 PMO, to skip exon 23, in the mdx mouse has also been completed, with similar encouraging reports of good tolerability. The histopathology is currently being reviewed but initial reports suggest that the muscles of treated mice show improvement over the 12 weeks of study.

### **About Duchenne Muscular Dystrophy**

Duchenne Muscular Dystrophy (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 DMD 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 development.

### **About CureDuchenne**

CureDuchenne is a nonprofit organization that raises awareness and funds research specifically aimed at taking on Duchenne Muscular Dystrophy (DMD). By working closely with the world's leading DMD scientists CureDuchenne works to determine the most viable research projects that will accelerate the clinical trial process and bring potential life saving drugs to help this generation of young boys living with the deadly disease. Our vision is our name...to Cure Duchenne. Learn more at: [www.cureduchenne.org](http://www.cureduchenne.org).

### **About The Foundation to Eradicate Duchenne**

The Foundation to Eradicate Duchenne is a 501c3 charitable organization established in 2001 to pursue therapeutics for Duchenne Muscular Dystrophy. It is headquartered in Alexandria, VA. Since its inception, the FED has funded millions of dollars in aggressive research and is a principal funder of the Cooperative International Neuromuscular Research Group, an international clinical trials network founded at Children's National Medical Center in Washington, DC.

### **About Children's National Medical Center/Children's Research Institute**

Children's National Medical Center, located in Washington, DC, is a leader in the development of innovative new treatments for childhood illness and injury. Children's has been serving the nation's children for more than 135 years. Children's National is consistently ranked among the best pediatric hospitals by U.S. News & World Report and the Leapfrog Group. For more information, visit [www.ChildrensNational.org](http://www.ChildrensNational.org). Children's Research Institute, the academic arm of Children's National Medical Center, encompasses the translational, clinical, and community research efforts of the institution. Learn more about Children's Research Institute at [www.childrensnational.org/research](http://www.childrensnational.org/research).

### **About AVI BioPharma**

AVI BioPharma is focused on the discovery and development of RNA-based medicines 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, including an ongoing systemic Phase 1b/2 clinical trial of exon skipping with AVI-4658. 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 Junín, influenza, HCV or Dengue viruses. For more information, visit [www.avibio.com](http://www.avibio.com).

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“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.