



AVI BioPharma Publishes Preclinical Data in Muscular Dystrophy Research

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Sustained Dystrophin Expression Found in Heart and Other Key Organs

For Immediate Release

CORVALLIS, OR — June 12, 2008 — AVI BioPharma, Inc. (NASDAQ: AVII) today announced the publication of preclinical results of a study designed to demonstrate the ability of AVI's NeuGene® class of drugs to induce sustained expression of dystrophin in the mdx mouse model of Duchenne muscular dystrophy (DMD). Treatment with the AVI compound resulted in production of functional dystrophin in numerous appropriate tissues, including the heart, diaphragm and skeletal muscles; key organs for the treatment of the disease. The findings were published in the peer-reviewed journal, [Molecular Therapy](#).

The paper, titled "[Sustained Dystrophin Expression Induced by Peptide-Conjugated Morpholino Oligomers in the Muscles of mdx Mice](#)," was a collaborative study by scientists from AVI, the University of North Carolina at Chapel Hill and Mahidol University, Thailand.

In this study, a PMO-peptide conjugate, termed PPMO-B, that showed potent activity in skeletal muscles, diaphragm and, importantly, the heart was initially selected from a panel of other conjugates and subsequently applied for treatment of DMD in the mdx mouse model. The first finding of this investigation was that the systemically delivered PPMO-B induced high level of exon skipping in dystrophin mRNA in body-wide muscles, including diaphragm and cardiac muscle of treated mice, without detectable toxicity. The induced dystrophin mRNA persisted for several weeks, indicating that the drug is retained and functional in the target organs for an extended period of time. Furthermore, the mRNA effectively translated substantial amounts of dystrophin protein in all target muscles. Dystrophin protein persisted in the muscles even longer than the RNA. Additional tests indicated improvement in muscle integrity and, specifically, reduction of inflammation of the heart. This is the first report of PPMO-mediated exon skipping and functional dystrophin protein induction in the heart of treated animals.

"For DMD patients, restoration of functional dystrophin in heart and diaphragm is critical because respiratory and cardiovascular complications are the ultimate cause of mortality," said Dr. Ryszard Kole, co-author and AVI's Senior Vice President of Discovery Research. "This is the first study to establish peptide-conjugated NeuGene drugs as potential treatment for DMD."

About Alternative RNA Splicing Technology

In normal genetic function, gene transcription produces a full-length pre-messenger RNA (pre-mRNA) that is then processed to a much shorter and functional messenger RNA (mRNA). The mRNA is the template for creating a protein. During pre-mRNA processing, packets of useful genetic information, called exons, are spliced together to make the functional mRNA template, while unnecessary fragments called introns are snipped out of the full-length RNA. In some diseases, such as DMD, mutations derail this process and prevent production of functional dystrophin protein. AVI's proprietary third-generation NeuGene chemistry can be used to target splicing sites in the pre-mRNA, thus forcing the cell machinery to skip over targeted exons, providing altered mRNA, which in turn produces altered proteins. Skipping a defective exon can restore proper RNA reading frame and restore production of the protein to overcome the devastating clinical consequences of the mutation.

About Duchenne Muscular Dystrophy

DMD is the most common fatal genetic disorder to affect children around the world. It is a devastating and incurable muscle-wasting disease associated with specific inborn errors in the gene that expresses dystrophin, a protein that is an essential component for striated muscle function. When dystrophin is missing or nonfunctional due to a mutation in the genetic code of the dystrophin gene, as it is in DMD, the result is membrane leakage and fiber damage, ultimately leading to degeneration and death of the muscle fiber. Approximately one in 3,500 boys is born with DMD, and an estimated 15,000 to 20,000 children are afflicted in the United States alone.

About AVI BioPharma

AVI BioPharma develops therapeutic products for the treatment of life-threatening diseases using third-generation NeuGene® antisense drugs and alternative RNA splicing technology. AVI's alternative RNA splicing technology is initially being applied to potential treatments for Duchenne muscular dystrophy. AVI's NeuGene compounds are also designed to treat post-operative cardiovascular restenosis. In addition to targeting specific genes in the body, AVI's antiviral program uses NeuGene antisense compounds to combat disease by targeting single-stranded RNA viruses, including Marburg Musoke and Ebola Zaire viruses. More information about AVI is available at www.avibio.com.