AVI BioPharma and Collaborators Demonstrate In Vivo Effectiveness of PPMO-based Splice Switching Oligomers (SSOs) in Genetic Disease Target

January 12, 2009 7:02 PM ET

For Immediate Release

PORTLAND, OR — January 12, 2009 — AVI BioPharma, Inc. (NASDAQ: AVII), a developer of RNA–based drugs, announced today new pre–clinical results published in Proceedings of the National Academy of Sciences (Saovaros Svasti et al (January 12, 2009) Proc. Natl Acad. Sci. USA, 10.1073/PNAS 0812436106) demonstrating the effectiveness of a systemically delivered PPMO–based splice switching oligomer or SSO (a morpholino oligomer conjugated to an arginine–rich peptide) in vivo in a mouse model of an inherited blood disorder. The results show that PPMO–based SSOs may be effective in vivo not only in muscle cells for DMD treatment, but also for another genetic disease and in target cells more challenging than muscle fibers.

The paper's co–authors include Drs. Ryszard Kole and Hong Moulton from AVI as well as Nobel laureate Dr. Oliver Smithies of the University of North Carolina. The research was conducted by scientists at the Departments of Pharmacology, Pathology and Lineberger Comprehensive Cancer Center, University of North Carolina and the Thalassemia Research Center, Mahidol University, Bangkok, Thailand in collaboration with AVI scientists.

"The significance of these findings includes confirmation that our PPMO–based SSOs are effective in additional tissues and cells, establishing the potential that this technology may be selectively applied to commercially viable genetic disease targets in the future," said Ryszard Kole, Ph.D., SVP of Discovery Research at AVI BioPharma. "The results from this research were particularly impressive since immature red blood cells are challenging targets and other oligonucleotides that were tested were ineffective."

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.