

## **Akashi Therapeutics Reports Positive Clinical Data on HT-100 in Patients with Duchenne Muscular Dystrophy**

Interim Clinical Data from Ongoing Phase 1b/2a Clinical Program Highlights Statistically Significant Improvements in Muscle Strength

**Cambridge, Mass. - June 18 , 2015** - Akashi Therapeutics Inc., a clinical stage biopharmaceutical company developing treatments for Duchenne muscular dystrophy (DMD), today announced positive interim clinical data from an ongoing Phase 1b/2a clinical program with HT-100 (delayed-release halofuginone) an orally available, small molecule developed to reduce fibrosis and inflammation and promote healthy muscle fiber regeneration in boys with DMD. In the clinical program, statistically significant differences in muscle strength as compared to a matched external control cohort and a favorable safety profile were observed.

“We are excited by these interim Phase 1b/2a clinical data for HT-100, a powerful anti-inflammatory and anti-fibrotic, which further demonstrate its potential in the treatment of DMD,” said Marc B. Blaustein, CEO of Akashi Therapeutics. “As a group, the boys in this study showed an increase in muscle strength over their baseline and a statistically significant increase relative to a comparable external control cohort, and we look forward to further evaluating and reporting on the progress of HT-100 as a promising treatment option for all boys with DMD as the study continues.”

Highlights of the interim data as of June 12 include:

- The 10 DMD patients participating in the trial for 18 to 22 months and with at least six months of continuous dosing achieved mean total muscle strength 22.3% greater than levels predicted by comparable steroid-treated external control ( $p=0.027$ ) as measured by quantitative muscle testing (QMT) of upper and lower extremity muscle groups.
- The mean increase in total muscle strength compared to baseline (study entry) over 18-22 months was 11.7%.
- These efficacy findings are in the trial’s 2 lowest dose cohorts (mean age[SD]=10.4[2.55]). All study participants are on a stable dose of corticosteroids.
- HT-100 continues to be well-tolerated with no serious adverse events. The safety database in this study reflects a cumulative 10.5 years of dosing, with 6 patients dosed for a total of 12-13 months, and 10 patients dosed continuously for 9-10 months.

### **About HT-100**

HT-100 (delayed-release halofuginone) is an orally available, small molecule drug candidate designed to reduce fibrosis and inflammation and promote healthy muscle fiber regeneration in DMD patients. HT-100 has been granted orphan designation for DMD in both the U.S. and E.U., and fast track designation in the U.S. A phase 1b/2a clinical program is currently underway at five hospitals across the U.S.



HT-100 development is generously supported by patient advocacy organizations. A list of these organizations can be found at <http://akashirx.com/financial-supporters/>.

### **About Duchenne muscular dystrophy (DMD)**

Affecting approximately 1 in 3,600 boys worldwide, DMD is the most common of the muscular dystrophies and the most lethal genetic disorder of childhood. It is caused by a genetic mutation that renders boys unable to make functional dystrophin, a protein critical for normal muscle function. Young men with DMD show progressive signs of physical impairment as early as age three, lose the ability to walk in their teens, and die of cardiac or respiratory failure in their late twenties or early thirties.

### **About Akashi Therapeutics**

Akashi Therapeutics is a clinical stage biopharmaceutical company whose mission is to develop treatments for Duchenne muscular dystrophy. Akashi was founded by leading patient organizations and biotechnology industry veterans and is managed by a seasoned team of drug development experts to impact a central problem in rare diseases: rapid therapy development. Akashi is developing a pipeline of therapies with the goal of transforming Duchenne from a 100% fatal, aggressive muscle-wasting disease to a chronic, manageable condition. For more information, please visit [www.akashirx.com](http://www.akashirx.com).

### **Akashi Therapeutics Contact:**

Marc B. Blaustein, CFA  
info@akashirx.com  
617.431.7250

### **Media Contact:**

Gina Nugent, The Yates Network  
[gina@theyatesnetwork.com](mailto:gina@theyatesnetwork.com)