



## **Applied Therapeutics to Present New Data on Prevention and Treatment of Galactosemia Complications at the Galactosemia Foundation Conference**

New York, July 11, 2018 - Applied Therapeutics Inc. announced today that it will present data on AT-007, a novel investigational aldose reductase inhibitor, at the Galactosemia Foundation conference, July 12-14, 2018, in Denver, Colorado. The data presentation, entitled "A Novel Investigational Treatment in Preclinical Development for Galactosemia," will be presented on Saturday, July 14 at 3:30pm MT.

The data being presented is the product of a research collaboration with Emory University School of Medicine to explore effectiveness of novel, potent, aldose reductase inhibitors on metabolites associated with galactosemia complications, including galactitol. Oral treatment of neonatal rats once daily with AT-007 effectively prevented complications associated with galactosemia in a model of Classic Galactosemia. AT-007 significantly reduced or normalized galactitol levels in plasma, brain and liver, without increasing levels of galactose or other galactose metabolites, such as Gal1p. Additional data on prevention of complications associated with galactitol formation will be presented.

Galactosemia is a rare genetic disease that affects the body's ability to metabolize galactose, a sugar produced at low levels in all human cells that is also found at high levels in milk and dairy products. Currently, no treatments exist for galactosemia, which is fatal in infants if undiagnosed; early identification through newborn screening and prompt initiation of a restricted diet can prevent the most severe consequences of the disease. However, despite strict dietary control, patients with galactosemia often suffer from serious long-term consequences of disease. These complications, which frequently include cataracts, cognitive, speech, and behavioral disabilities, neurological and motor problems, and premature ovarian insufficiency in women, significantly diminish patients' health and quality of life.

"There are no treatments available to prevent long-term complications associated with galactosemia, which have a profound effect on patients and families," said Shoshana Shendelman, Ph.D., Founder, Chairman and CEO of Applied Therapeutics. "We are hopeful that our research will bring our investigational therapy one step closer to patients, and we are excited to share our data with the galactosemia community."

### **About AT-007**

AT-007 is an investigational aldose reductase inhibitor in preclinical development through Applied Therapeutics. The compound is a novel, potent, CNS penetrant inhibitor of aldose reductase.

**About Applied Therapeutics Inc.**

Applied Therapeutics is a privately held biotechnology company, focused on developing transformative drugs in areas of high unmet medical need – fatal or debilitating diseases for which no therapies are approved. The company applies cutting-edge technology to validated drug targets that have failed to produce meaningful therapies in the past. Applied Therapeutics believes that through innovative science, millions of lives can be saved. For more information, visit [www.appliedtherapeutics.com](http://www.appliedtherapeutics.com).

**About Galactosemia**

Galactosemia is a rare genetic metabolic disease that affects the body's ability to convert galactose to glucose. The incidence of galactosemia is estimated to be between 1/30,000 to 1/60,000 in the US, and varies worldwide based on ethnicity. Newborn screening now identifies almost all infants with classic galactosemia born in the US and many other countries, enabling early dietary intervention to restrict galactose exposure—generally by switching the baby from milk to a soy-based formula. Dietary intervention prevents potentially lethal acute symptoms of the disease, but fails to prevent the many long-term complications that can develop later in childhood and persist through adulthood. The most common complications currently recognized include cataracts, cognitive, behavioral, and speech problems, motor difficulties, and primary or premature ovarian insufficiency in women. For more information about galactosemia, please visit the Galactosemia Foundation website or the National Organization for Rare Disease (NORD) website.

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