



APPLIED THERAPEUTICS TO PRESENT NEW DATA ON NOVEL  
INVESTIGATIONAL TREATMENT FOR GALACTOSEMIA AT 2018 NORD  
SUMMIT

**New York, October 15, 2018** - Applied Therapeutics Inc., a privately-held biotechnology company focused on developing transformative drugs in areas of high unmet medical need, announced today that it will present data on AT-007, a novel investigational aldose reductase inhibitor, at the 2018 National Organization for Rare Disorders' (NORD) Rare Diseases & Orphan Products Breakthrough Summit, taking place October 15-16, in Washington, D.C. The presentation, entitled "A Novel Investigational Treatment in Preclinical Development for Galactosemia," will be available for viewing throughout the conference.

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, including Gal1P. Additional data on prevention of complications associated with galactitol formation will be presented at the NORD Summit.

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 often fatal in infants if undiagnosed; early identification through newborn screening and prompt initiation of a restricted diet can prevent the acute fatal consequences of the disease. However, despite strict dietary control, patients with galactosemia suffer from serious long-term complications, due to endogenous production of galactosemia within the body. These complications include cognitive and intellectual deficiencies, speech, neurological and motor problems, cataracts, and premature ovarian insufficiency in women. The long-term consequences of galactosemia significantly diminish patients' health and quality of life and represent a significant unmet medical need.

"Galactosemia can be a debilitating disease to many patients who suffer from long term complications and developmental disabilities. Unfortunately, galactosemia is often misunderstood as a disease that can be completely controlled by dietary restriction. This is untrue. While dietary restriction of lactose does prevent fatalities in infancy, and is important in limiting exposure of the body to external intake of galactose, it does not halt the long-term complications that develop over time due to the body's internal production of galactose," said Riccardo Perfetti, MD, PhD, Chief Medical Officer of Applied Therapeutics.

“Like many rare diseases, there are no treatments available for galactosemia. Applied Therapeutics is committed to advancing research in galactosemia, and is working closely with both researchers and patient organizations to bring treatments to this debilitating disease,” said Shoshana Shendelman, PhD, Founder and CEO of Applied Therapeutics.

Presentation details:

*Poster #02, A Novel Investigational Treatment in Preclinical Development for Galactosemia; October 15-16<sup>th</sup>, Washington Marriott Wardman Park Hall C*

**About the NORD Rare Summit**

The NORD Rare Summit, convened annually in Washington, D.C., gathers leaders from FDA, NIH, industry, patient groups, payers and research institutions to address issues of critical importance to the rare disease community. With over 700 attendees, the 2018 Summit will highlight new and innovative efforts to drive progress in rare disease research, drug development, and advocacy.

**About AT-007**

AT-007 is an investigational drug in preclinical development for galactosemia. 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. 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 and intellectual deficiencies, speech, neurological and motor problems, and premature ovarian insufficiency in women. For more information about galactosemia, please visit the Galactosemia Foundation website or the National Organization for Rare Disorders (NORD) website.

CONTACTS:

Applied Therapeutics  
Erin Fleming  
efleming@appliedtherapeutics.com  
212.220.9437