APPLIED THERAPEUTICS ANNOUNCES SUPPORT FOR RARE DISEASE DAY 2018

INITIATES GALACTOSEMIA RESEARCH COLLABORATION WITH EMORY UNIVERSITY SCHOOL OF MEDICINE

NEW YORK, N.Y. – February 20, 2018 - Applied Therapeutics Inc., a privately-held biotechnology company focused on developing transformative drugs in areas of high unmet medical need, today announced its support of Rare Disease Day 2018. Rare Disease Day is an international campaign organized by the European Organisation for Rare Diseases (EURORDIS), which unites the community in an effort to improve the awareness, diagnosis and treatment of rare diseases.

February 28, 2018 marks the 11th International Rare Disease Day. This year's theme, "Research," emphasizes the importance of rare disease research toward the development of diagnostic tools, treatments and cures, as well as improved health and social care for patients and their families. In celebration of this theme, Applied Therapeutics is pleased to announce a research collaboration with Emory University to develop treatments for galactosemia.

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.

Applied Therapeutics recognizes the urgent need to develop safe and effective treatments to prevent or reverse consequences of galactosemia, and is committed to supporting research that will drive forward a more precise understanding of the disease's complications and potential therapeutic targets. This effort will benefit from the contributions of scientific and clinical researchers across the globe, including Applied Therapeutics' collaborators at Emory University, who are working to determine the exact causes of long-term galactosemia complications.

"Newborn screening has greatly prevented galactosemia fatalities, but the long-term consequences of disease persist and have a profound effect on patient quality of life," said Shoshana Shendelman, Ph.D., Founder, Chairman and CEO of Applied Therapeutics. "We are proud to announce our research collaboration with Emory, and hope that through this research we can, for the first time, bring therapies to galactosemia patients that will prevent disease complications and improve quality of life."

“The long-term complications of galactosemia in children and adults have been recognized for decades, but the mechanism remains unclear,” said Judith Fridovich-Keil, Ph.D., Professor of Human Genetics at Emory University School of Medicine. “We believe that by identifying the specific metabolites and pathways responsible for these complications, we may be able to intervene—either by preventing the metabolites from forming, or compensating elsewhere in the pathway. We are excited about our collaboration with Applied Therapeutics and are hopeful that our work together will provide new therapeutic options for patients with galactosemia.”

For more information about galactosemia, please visit the Galactosemia Foundation website or the National Organization for Rare Disease (NORD) website.

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.

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.

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