September 13, 2022

Acasti Pharma Announces Initiation of Pharmacokinetic Study for GTX-102, the Company's Drug Candidate for the Treatment of Ataxia Telangiectasia

LAVAL, Québec, Sept. 13, 2022 (GLOBE NEWSWIRE) -- Acasti Pharma Inc. ("Acasti" or the "Company") (Nasdaq: ACST and TSX-V: ACST), today announces the initiation of its planned pharmacokinetic (PK) bridging study to evaluate the comparative bioavailability, pharmacokinetics, and safety of its oral betamethasone spray, GTX-102, compared to an intramuscular injection of betamethasone and to an oral solution of betamethasone, in 48 healthy subjects. The First Subject, First Dose was administered on September 13th. This PK study is the next step in the proposed 505(b)(2) regulatory pathway for GTX-102 and is expected to be completed with top line results reported before year end.

GTX-102 is a novel, proprietary, concentrated oral-mucosal metered spray of betamethasone intended to improve the neurological symptoms of Ataxia Telangiectasia (A-T) in a pediatric population for which there are currently no FDA-approved therapies. GTX-102 can be sprayed conveniently over the tongue of the A-T patients, who often have difficulties swallowing.

Jan D'Alvise, Chief Executive Officer of Acasti, stated, "The initiation of this PK study is yet another important milestone in the advancement of our GTX-102 program designed to provide a novel therapy for treating the chronic symptoms of Ataxia Telangiectasia in children with this rare genetic disorder. GTX-102 is now the third program to advance into the clinic this year as we continue to leverage our novel drug delivery technologies that have the potential to improve the performance of currently marketed drugs by achieving faster onset of action, enhanced efficacy, reduced side effects, and more convenient drug delivery. We look forward to the completion of this PK study later this year, and assuming positive results, we expect to move rapidly into Phase 3 in the second half of 2023. Currently there are no drugs approved for A-T, and we are very motivated to potentially bring this exciting novel treatment to children who suffer from A-T."

This PK study is a Phase 1, randomized, open-label, crossover study in healthy male and female subjects to evaluate the comparative bioavailability, pharmacokinetics, and safety of GTX-102 administered as an oral spray compared to an intramuscular injection of betamethasone, which is the reference product for U.S. filing purposes, and to an oral solution of betamethasone, which is available on the market in Europe and will be the comparator product for bridging purposes under the 505(b)(2) guidelines. The primary objective of the study is to evaluate and characterize the PK profile of GTX-102 as an oral spray.

A total of 48 healthy adult male and female subjects will be enrolled in this single center, 5-

treatment, 2-period cross-over study. 4 groups of subjects will receive 2 treatments each and will be randomized to receive 3 different doses of GTX-102 (Low Dose, Medium Dose and High Dose), and blood levels and safety measures will be compared to the betamethasone oral solution and to the betamethasone IM injectable.

Assuming the PK bridging study meets its primary endpoint, the final development step is to conduct a Phase 3 safety and efficacy trial in A-T patients. The Company plans to request a Type B meeting with the FDA following the completion of the PK study to confirm the Phase 3 study design, and the Phase 3 study is expected to be initiated in the second half of 2023. If both studies meet their primary endpoints, an NDA filing for GTX-102 under Section 505(b)(2) would follow.

Senior Management Changes

The Company also announces that Prashant Kohli has been named Chief Commercial Officer. Mr. Kohli previously served as Acasti's VP of Commercial Operations and held the same title at Grace Therapeutics, prior to its acquisition by Acasti in August 2021.

About Ataxia Telangiectasia (A-T)

A-T is a progressive, genetic, neurodegenerative disorder that primarily effects young children, causing severe disability, impairment of the immune system and an increasing susceptibility to infections and cancer. The hallmark symptoms of A-T are cerebellar ataxia and other motor dysfunction, and dilated blood vessels (telangiectasia) that occur in the sclera of the eyes. Children begin to experience balance and coordination problems when they begin to walk (toddler age), and ultimately become wheelchair bound in their second decade of life. In pre-adolescence (age 5-8 years), patients experience oculomotor apraxia, dysarthria, and dysphagia. They often develop compromised immune systems and are at increased risk of developing respiratory tract infections and cancer (typically lymphomas and leukemia). Patients typically die by age 25 years from complications of lung disease or cancer.

A-T is diagnosed through a combination of clinical assessment (especially neurologic and oculomotor deficits), laboratory analysis, and genetic testing. There is no known treatment to slow disease progression, and treatments that are used are strictly aimed at symptoms (e.g., physical, occupational or speech therapy for neurologic issues), or conditions secondary to the disease (e.g., antibiotics for lung infections, chemotherapy for cancer, etc.).

A market research study commissioned by Acasti found that A-T affects approximately 4,300 patients per year in the United States and has a potential total addressable market of $150 million, based on the number of treatable patients.

About Acasti

Acasti is a specialty pharma company with drug delivery technologies and drug candidates addressing rare and orphan diseases. Acasti's novel drug delivery technologies have the potential to improve the performance of currently marketed drugs by achieving faster onset of action, enhanced efficacy, reduced side effects, and more convenient drug delivery—all which could help to increase treatment compliance and improve patient outcomes.

Acasti's three lead clinical assets have each been granted Orphan Drug Designation by the FDA, which provide the assets with seven years of marketing exclusivity post-launch in the United States, and have additional intellectual property protection with over 40 granted and pending patents. Acasti's lead clinical assets target underserved orphan diseases: (i) GTX-104, an intravenous infusion targeting Subarachnoid Hemorrhage (SAH), a rare and life threatening medical emergency in which bleeding occurs over the surface of the brain in the subarachnoid space between the brain and skull; (ii) GTX-102, an oral mucosal spray targeting Ataxia-telangiectasia (A-T), a progressive, neurodegenerative genetic disease that primarily affects children, causing severe disability, and for which no treatment currently exists; and (iii) GTX-101, a topical spray targeting Postherpetic Neuralgia (PHN), a persistent and often debilitating neuropathic pain caused by nerve damage from the varicella zoster virus (shingles), which may persist for months and even years.

For more information, please visit: https://www.acastipharma.com/en.

Forward-Looking Statements

Statements in this press release that are not statements of historical or current fact constitute "forward-looking information" within the meaning of Canadian securities laws and "forward-looking statements" within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, as amended, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended (collectively, "forward looking statements"). Such forward looking statements involve known and unknown risks, uncertainties, and other unknown factors that could cause the actual results of Acasti to be materially different from historical results or from any future results expressed or implied by such forward-looking statements. In addition to statements which explicitly describe such risks and uncertainties, readers are urged to consider statements containing the terms "assuming" "believes," "belief," "expects," "intends," "anticipates," "potential," "should," "may," "will," "plans," "continue", "targeted" or other similar expressions to be uncertain and forward looking. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this press release. The forward-looking statements in this press release are based upon Acasti's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, including, without limitation: (i) the success and timing of regulatory submissions of the PK bridging study for GTX-102 and Acasti's other pre-clinical and clinical trials; (ii) regulatory requirements or developments; (iii) changes to clinical trial designs and regulatory pathways; (iv) legislative, regulatory, political and economic developments, and (v) the effects of COVID-19 on clinical programs and business operations. The foregoing list of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors detailed in documents that have been and may be filed by Acasti from time to time with the Securities and Exchange Commission. All forward looking statements contained in this press release speak only as of the date on which they were made. Acasti undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by applicable securities laws. Neither NASDAQ, the TSXV nor its Regulation Services Provider (as that term is defined in the policies of the TSXV) accepts responsibility for the adequacy or accuracy of this release.

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