

February 28, 2023



# Acasti Pharma Recognizes Rare Disease Day

LAVAL, QC, Feb. 28, 2023 /PRNewswire/ -- Acasti Pharma Inc. ("Acasti" or the "Company") (NASDAQ: ACST and TSX-V: ACST), a late-stage, specialty pharma company advancing three clinical stage drug candidates addressing rare and orphan diseases, recognizes and celebrates Rare Disease Day (February 28), as established by the European Organization for Rare Diseases.



"Rare diseases impact approximately 300 million patients around the world and are often chronic, progressive and frequently life-threatening," commented Jan D'Alvise, CEO of Acasti Pharma. "Acasti's aim is to improve clinical outcomes by applying our proprietary formulation and drug delivery technologies to existing pharmaceutical compounds with a goal to achieve improvements over the current standard of care, or to provide treatment for diseases with no currently approved therapies. Our focus today is on developing therapies for three rare and orphan diseases, aneurysmal subarachnoid hemorrhage (aSAH), ataxia telangiectasia (A-T) and postherpetic neuralgia (PHN), which all combined effect approximately 200,000 people in the United States. We remain committed to effectively treating the debilitating symptoms that result from these underlying rare diseases in these underserved patient populations."

## **Subarachnoid Hemorrhage (SAH)**

Acasti is developing GTX-104, an IV formulation of nimodipine designed to treat SAH. SAH is bleeding over the surface of the brain in the subarachnoid space between the brain and the skull, which contains blood vessels that supply the brain. A primary cause of such bleeding is rupture of an aneurysm (aSAH). The company estimates that approximately 50,000 individuals experience aSAH each year in the US based on third party market research. There are an estimated 150,000 aSAH patients each year in China and approximately 55,000 patients in Europe.

## **Ataxia Telangiectasia (A-T)**

Acasti is developing GTX-102, an oral-mucosal betamethasone spray for the treatment of A-T. A-T is a rare genetic progressive autosomal recessive neurodegenerative disorder that affects children, with the hallmark symptoms of cerebellar ataxia and other motor dysfunction, and dilated blood vessels (telangiectasia) that occur in the sclera of the eyes.

A-T is caused by mutations in the ataxia telangiectasia gene, which is responsible for modulating cellular response to stress, including breaks in the double strands of DNA. It is estimated that A-T affects approximately 4,500 patients in the United States and currently, there are no FDA approved therapies for A-T.

### **Postherpetic Neuralgia (PHN)**

Acasti is developing GTX-101, a topical, bio-adhesive, film-forming bupivacaine spray for PHN. PHN is associated with significant loss of function and reduced quality of life, particularly in the elderly. It has a detrimental effect on all aspects of a patient's quality of life. The nature of PHN pain varies from mild to severe, constant, intermittent, or can be triggered by various stimuli. Approximately half of patients with PHN describe their pain as "horrible" or "excruciating," ranging in duration from a few minutes to constant on a daily or almost daily basis (Katz, 2004). The pain can disrupt sleep, mood, work, and activities of daily living, adversely impacting the quality of life and leading to social withdrawal and depression. It is estimated that PHN affects approximately 120,000 patients per year in the United States, and there are significant unmet medical needs with the current standard of care.

### **About Acasti**

Acasti is a late-stage 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 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 "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. 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.

**Acasti Contact:**

Jan D'Alvise  
Chief Executive Officer  
Tel: 450-686-4555  
Email: [info@acastipharma.com](mailto:info@acastipharma.com)  
[www.acastipharma.com](http://www.acastipharma.com)

**Investor Contact:**

Lytham Partners, LLC  
Robert Blum  
Tel: 602-889-9700  
Email: [acst@lythampartners.com](mailto:acst@lythampartners.com)

 View original content to download multimedia <https://www.prnewswire.com/news-releases/acasti-pharma-recognizes-rare-disease-day-301758324.html>

SOURCE Acasti Pharma Inc.