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Acasti to Proceed with Phase 3 Clinical Safety Study for GTX-104 Following FDA Feedback, and Upon Approval of the Full Study Protocol to be Submitted to the IND

Company expects the first patient to be enrolled during the second half of 2023

LAVAL, QC, April 4, 2023 /PRNewswire/ -- Acasti Pharma Inc. ("Acasti" or the "Company") (Nasdaq: ACST), a late-stage, specialty pharma company advancing drug candidates for rare and orphan diseases, today announced that it received a Type C written meeting response and clarifying feedback from the United States Food and Drug Administration (FDA) on Acasti's proposed Phase 3 Safety Study for GTX-104. The FDA provided additional comments on the Company's development plan that, pending submission of the final clinical protocol and FDA approval of same, will allow Acasti to proceed with the initiation of a Phase 3 safety clinical trial in aneurysmal Subarachnoid Hemorrhage (aSAH) patients.

The FDA concurred with the suitability of the 505(b)(2) regulatory pathway with the selected Reference Listed Drug (RLD) Nimotop oral capsules (NDA 018869), and that Acasti's GTX-104-002 PK study may have met the criteria for a scientific bridge.

Based on FDA's proposed Phase 3 Study Design, the company will target enrollment of aSAH patients (across all grades of severity) in a 1:1 randomized trial with oral nimodipine, to be conducted in an estimated 25-30 sites in the U.S.A. The FDA confirmed the use of the Hunt and Hess scale to stratify patients based on severity. The primary endpoint is safety, and it will be measured as the percentage of significant adverse events of hypotension related to study drugs in both arms.

Prashant Kohli, CEO of Acasti Pharma, commented, "We expect to move quickly to submit the final clinical protocol and all required study documentation to the FDA. Once these documents are submitted and following any final feedback and approval from the FDA, the Phase 3 safety study can be initiated. If the Phase 3 study meets the primary endpoint, an NDA filing for GTX-104 under Section 505(b)(2) is expected to follow."

An application filed under Section 505(b)(2) is one that contains full reports of investigations

of safety and effectiveness but where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference.

About aneurysmal Subarachnoid Hemorrhage (aSAH)

aSAH 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. The result is a relatively uncommon type of stroke that accounts for about one-in-twenty (5%) of all strokes and has an incidence of six per 100,000 person years (Becske, 2018). In contrast to more common types of strokes in elderly individuals, aSAH often occurs at a relatively young age, with half the affected patients being younger than 60 years (Becske, 2018). Particularly devastating for patients younger than 45, approximately 10% to 15% of aSAH patients die before reaching the hospital (Rinkel, 2016), and those who survive the initial hours post hemorrhage are admitted or transferred to neurointensive care centers to manage the high risk of complications, including rebleeding, vasospasm and delayed cerebral ischemia (DCI). Systemic manifestations affecting cardiovascular, pulmonary, and renal function are common, and often complicate the management of DCI.

Approximately 70% of aSAH patients experience death or dependence, and half die within one month after the hemorrhage. Of those who survive the initial month, half remain permanently dependent on someone else to maintain daily living (Becske, 2018).

About GTX-104

GTX-104 is a clinical stage, novel formulation of nimodipine being developed for IV infusion in aSAH patients. It incorporates surfactant micelles as the drug carrier to solubilize nimodipine. This nimodipine injectable formulation is comprised of a nimodipine base, an effective amount of a hydrophilic surfactant, and a pharmaceutically acceptable carrier for injection. GTX-104 is an aqueous solution substantially free of organic solvents, such that the nimodipine is contained in a concentrated injection solution, suspension, emulsion or complex as a micelle, a colloidal particle or an inclusion complex, and the formulation is stable and clear. The addressable market in the United States for GTX-104 is estimated to be about $300 million based on market research conducted by Fletcher Spaght.

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 aneurysmal Subarachnoid Hemorrhage (aSAH), 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.acasti.com.

Forward-Looking Statements

Statements in this press release that are not statements of historical or current fact constitute "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, and "forward-looking information" within the meaning of Canadian securities laws (collectively, "forward-looking statements"). Such forward looking statements involve known and unknown risks, uncertainties, and other 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," "estimates", "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 planned Phase 3 safety study for GTX-104; (ii) regulatory requirements or developments and the outcome and timing of the proposed IND application for GTX-104; (iii) changes to clinical trial designs and regulatory pathways; (iv) legislative, regulatory, political and economic developments; and (v) actual costs associated with Acasti's clinical trials as compared to management's current expectations. 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 are filed by Acasti from time to time with the Securities and Exchange Commission and Canadian securities regulators. 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. The NASDAQ does not accept responsibility for the adequacy or accuracy of this release.

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