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XORTX Completes Positive Pre-Phase 3 Meeting with the US Food and Drug Administration

CALGARY, Alberta, Sept. 19, 2022 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("XORTX" or the “Company”) (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANU), a clinical development stage pharmaceutical company focused on developing innovative therapies to treat progressive kidney disease, is pleased to announce completion of the previously announced Type B Pre-phase 3 meeting with the US Food and Drug Administration (“FDA”) held on September 16, 2022. In advance of this meeting, XORTX submitted a “Pre-Phase-3 Briefing Package” to the FDA on Thursday, July 28, 2022 and received responses from, and responded to the FDA prior to the meeting.

To date, the Company has successfully completed numerous research and development activities leading to this meeting and is advancing its XRx-008 program for the treatment of autosomal dominant polycystic kidney disease (“ADPKD”). R&D activities during the past year leading to this meeting included manufacturing clinical quality GMP oxypurinol, finalizing formulation of drug product, and a number of non-clinical studies including the characterization of improved oral bioavailability of its proprietary formulation of oxypurinol in animal models. The Company has commenced the OXY-XRX-101 clinical pharmacokinetics study, reporting part 1 and 2 topline results from this study earlier this year. These key milestones and secondary pharmacokinetics modeling of the data from the Company’s XRx-008 program well positioned XORTX for in-depth discussions during the Pre-Phase 3 meeting with the FDA.

Prior to the meeting with the FDA, the Pre-Phase 3 Briefing Package provided an up-to-date summary of the extensive work completed for the XRx-008 program, including updates regarding chemistry, manufacturing, pharmacology, toxicology, clinical results to date, and regulatory and clinical plans. The FDA reviewed this package of data and accompanying questions to the agency and responded with minimal comments on the work accomplished to date and focused their feedback on XORTX clinical planning. As a result of minimal concerns by the FDA, the focus of the meeting was on optimizing trial design. Based on the discussion, XORTX believes it is well-position to complete a successful, single registration phase 3 clinical trial and associated considerations. Discussion during the meeting provided valuable guidance to the Company, and clarity on critical steps needed to achieve a successful clinical trial and marketing approval. A particularly valuable aspect of FDA advice was an emphasis on how the Company might gain market approval with a single phase 3 registration trial to demonstrate efficacy and safety. Following this meeting with the FDA, XORTX’s plans for a single phase 3 registration trial remains the focus.

Dr. Allen Davidoff stated, “We are pleased to have had the opportunity to discuss advancement of the XRx-008 program with the FDA and are grateful for the depth of

consideration and guidance provided by the agency. The discussions with the FDA provided key confirmation of our progress and clarify the key clinical steps needed in advance of XORTX's plans for an eventual filing of a new drug application (NDA). The Company will now refine the phase 3 registration clinical trial design in advance of initiation of a Special Protocol Assessment (SPA) discussion, in parallel with ongoing scientific review with the European Medicines Agency (EMA) and completion of the bridging pharmacokinetic trial."

About Type B meetings

Type B meetings, which are routine meetings occurring at pre-defined points between FDA and a sponsor. Meetings typically occur right after or right before the submission of clinical data or a new drug filing. Type B meetings can be for the following purposes among others

- Pre-investigational new drug application (pre-IND) meetings
- Certain end-of-phase 1 meetings
- End-of-phase 2 and pre-phase 3 meetings
- Pre-new drug application/biologics license application meetings

About Special Protocol Assessment (SPA)

SPA is a process in which sponsors may ask to meet with FDA to reach agreement on the design and size of certain clinical trials, clinical studies, or animal studies to determine if they adequately address scientific and regulatory requirements for a study that could support marketing approval. An SPA agreement indicates concurrence by FDA with the adequacy and acceptability of specific critical elements of overall protocol design (e.g., entry criteria, dose selection, endpoints, and planned analyses) for a study intended to support a future marketing application. These elements are critical to ensuring that the trial conducted under the protocol can be considered an adequate and well-controlled study that can support marketing approval. Feedback on these issues provides the greatest benefit to sponsors in planning late-phase development strategy. However, an SPA agreement does not indicate FDA concurrence on every protocol detail. An SPA does not guarantee that the FDA will accept an NDA or that the results of the study subject to the SPA will be adequate to support approval.

About ADPKD

ADPKD is a rare disease that affects more that 10 million individuals worldwide. ADPKD is typically diagnosed based upon expansion of fluid-filled cysts in the kidneys. Over time, the increasing number and size of cysts can contribute to structural and functional changes to kidneys and is frequently accompanied by chronic pain which is a common problem for patients with ADPKD. Expansion of cysts is thought to compress healthy functioning tissue surrounding the cysts and contribute to further loss of kidney function, fibrosis, impaired nutrient exchange and impaired kidney function, accompanied later by end-stage renal disease. For individuals with progressing ADPKD, treatment recommendations include anti-hypertensive treatment, dietary restrictions, and, for a limited percentage of suitable patients, pharmacotherapy. New, more broadly applicable therapies to effectively slow decline of kidney function in patients with progressive kidney disease including those with ADPKD are needed.

About XORTX Therapeutics Inc.

XORTX is a pharmaceutical company with two clinically advanced products in development – XRx-008 for ADPKD, XRx-101 for acute kidney and other acute organ injury associated with Coronavirus / COVID-19 infection and XRx-225 is a pre-clinical stage program for Type 2 Diabetic Nephropathy (T2DN). XORTX is working to advance its clinical development stage products that target aberrant purine metabolism and xanthine oxidase to decrease or inhibit production of uric acid. At XORTX, we are currently dedicated to developing two medications to improve the quality of life and future health of patients. Additional information on XORTX is available at www.xortx.com.

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The TSX Venture Exchange and Nasdaq have neither approved nor disapproved the contents of this news release. No stock exchange, securities commission or other regulatory authority has approved or disapproved the information contained herein.

Forward Looking Statements

This press release may contain express or implied forward-looking statements pursuant to Canadian and U.S. federal securities laws. These forward-looking statements and their implications are based on the current reasonable expectations of the management of XORTX only, and are subject to a number of factors and uncertainties that could cause actual results to differ materially from those described in such forward looking statements. Forward-looking statements in this press release include, but are not limited to, the FDA's views on Xortx's clinical trial and study pathway for its XRx-008 program. Except as otherwise required by law, XORTX undertakes no obligation to publicly release any revisions or updates to these forward-looking statements to reflect events or circumstances after the date hereof or to reflect the occurrence of unanticipated events. More detailed information about the risks and uncertainties affecting XORTX is contained in the Company's most recently filed Annual Information Form and the Management Discussion and Analysis for its most recent financial reporting period filed on the Company's SEDAR profile (www.sedar.com) and under the heading "Risk Factors" in XORTX's annual report on Form 20-F filed with the Securities and Exchange Commission ("SEC") available on the SEC's website, www.sec.gov.

XORTX THERAPEUTICS INC.

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