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XORTX Provides Update on 2022 Achievements and 2023 Planned Milestones

CALGARY, Alberta, Feb. 07, 2023 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("XORTX" or the "Company") (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANU), a late-stage clinical pharmaceutical company focused on developing innovative therapies to treat progressive kidney disease, is pleased to provide a summary of Company's progress in 2022 and milestones planned for 2023.

Dr. Allen Davidoff stated, "2022 marked a year of substantial technological and clinical progress and established the foundation for the Company's 2023 goals. The advancements and accomplishments made could not have happened without the efforts of our Board of Directors, employees, consultants, vendors, and shareholders. During 2023 we believe the goals set for 2023 will advance our technology closer to marketing approval and transform XORTX into a high value company."

In 2022, the Company made substantial progress advancing its strategic plan in key areas, including chemistry, manufacturing, formulation development, non-clinical studies using XORLO™ to attenuate PKD progression in animal models and completion of the first clinical study XR-008 in support of the XR-008 program for autosomal dominant polycystic kidney disease ("ADPKD").

2022 Synopsis

Chemistry and Manufacturing, Clinical and Pre-Clinical Highlights – Confirmed new synthetic pathway for oxypurinol production and produced GMP drug substance; finalized XORLO™ formulation and produced clinical supply of tablets for clinical trials.

- **Regulatory Submissions to US Food and Drug Administration ("FDA") and Health Canada and Clinical Trial conduct** - submitted clinical trial application for the XR-008 Bridging Pharmacokinetics Study of XORLO™ supporting the XR-008 program for ADPKD (the "Study") followed by receipt of Health Canada no objection letter; on April 12th; announced Topline pharmacokinetic results from Part 1 – July 13th; Topline results for Part 2 announced August 22nd; October 26th, the Company announced the expansion of the Study with Part 4 and no objection letter; dosing of Parts 3 and 4 completed December 19th.
- On March 31, 2022, the Company filed an Investigational New Drug ("IND") application for XR-008 for treatment of progressing kidney disease due to ADPKD with receipt of FDA grant received May 5th.

Technology and Patent Advancements

- On March 23, 2022, the Company submitted a Patent Cooperation Treaty patent application seeking international patent protection for the patent entitled “Compositions and Methods for Enhancing Anti-Viral Therapies”.
- On April 7, 2022, the Company announced patent grant of “Formulations of Xanthine Oxidase Inhibitors” by the United States Patent Office.
- On April 20, 2022, the Company received Small and Medium Enterprise status for the European Union.
- On July 19, 2022, Registration protocol and regulatory package submitted to EMA for Scientific Advice from the EMA Committee for Medicinal Products for Human Use.
- On August 4, 2022, clinical protocol submitted for pre-phase 3 meeting with FDA – Type B resulting in the grant of a virtual meeting that was held on September 16.
- November 4, 2022 presentation at American Society of Nephrology (“**ASN**”) under Session Title: Genetic Diseases of the Kidneys, by Dr. Charles Edelstein of the University of Colorado.

Organizational Highlights

In addition to substantial technology advancement, the Company continued to bolster its management team with the following appointments:

- On June 6, 2022, XORTX welcomed Anthony Giovinazzo to the Board of Directors and as Chair of the Board. Anthony Giovinazzo is an internationally recognized expert in intellectual property, drug development and commercialization, including numerous licensing agreements, with more than 25 years’ experience in Central Nervous System diseases. He was the Chief Executive Officer and Director of Cynapsus Therapeutics, a NASDAQ listed specialty pharmaceutical company that developed the first successful sublingual apomorphine thin film strip for Parkinson’s disease Mr. Giovinazzo was a co-inventor of the drug, built the leadership team, set the strategy, raised US \$136 million including an over-subscribed IPO and NASDAQ listing. Ultimately negotiations with several pharmaceutical companies that resulted in a CAD \$841 million, all cash acquisition, by Sunovion Pharmaceuticals (Dainippon Sumitomo Pharmaceuticals).
- On November 16, 2022, the Company appointed Stacy Evans, M.D., MBA as Chief Business Officer initiating a business development outreach program associated with the Company’s XRx-008 program, with the goal of identifying a global partner for Phase 3 development and commercialization. Stacy Evans, M.D., MBA, is an executive consultant with nearly 25 years of commercial development and business development experience, including 12 years at Pfizer where he led multiple transactions for Pfizer, including significant deal experience in the rare disease space. In addition, XORTX appointed Russo Partners as the Company’s Public Relations firm, with David Melamed, Ph.D., as lead contact.

Financial Highlights

- On October 7, 2022, the Company completed a US \$5 million public offering supported

by a key institutional shareholder of XORTX.

In summary, activity during 2022 advances the Company's intellectual property portfolio, characterizes the XORLO™ formulation pharmacokinetics for dose modeling and dose selection in individuals with kidney disease. The XR_x-OXY-101 data gathered provides key understanding for communications with the FDA, EMA and Health Canada to advance the optimal design of XORTX's registration trial for ADPKD. These communications continue to de-risk the XR_x-008 program and drug candidate for treating ADPKD and sets up XORTX for more in-depth and meaningful partnering discussions in the near future.

Goals for 2023

In 2023, XORTX will be focused on advancing XORLO™ as part of the XR_x-008 for ADPKD into a Phase 3 registration clinical trial, the submission of an Orphan Drug Designation ("ODD"), initiation of special protocol assessment discussions with the FDA and initiation of commercialization activities for XORLO™ as well as advancing research in other kidney disease applications. To achieve these objectives, XORTX's action plan includes:

1. **Initiate XR_x-OXY-301 Registration supporting "Accelerated Approval" of XORLO™ for individuals with ADPKD.** This study is a Phase 3, Multi-Center, Double-Blind, Placebo Controlled, Randomized Withdrawal Design Study to Evaluate the Efficacy and Safety of a Novel Oxypurinol Formulation in Patients with Progressing Stage 2-4 ADPKD and Coexistent Hyperuricemia. This study will provide data to support a future "Accelerated Approval" new drug application ("NDA") submissions to the FDA and EMA. This study is planned to start in the second half of 2023 and will enroll individuals with stage 2, 3 or 4 ADPKD accompanied by chronically high uric acid. The objective of this study is to evaluate the ability of XORLO™ to slow the expansion of total kidney volume over a 12-month treatment period.

<https://www.fda.gov/drugs/development-resources/table-surrogate-endpoints-were-basis-drug-approval-or-licensure#:~:text=FDA%E2%80%99s%20surrogate%20endpoint%20table%20provides%20>

2. **Complete Orphan Drug Designation.** XORTX's ODD application was filed in January 2023, with anticipated feedback from the FDA ODD office within 90 days and ODD status during the first half 2023. Acceptance of the XR_x-008 program further de-risks this program regarding the use of XORLO™ as a treatment for ADPKD.
3. **Prepare and Communicate with the FDA and EMA regarding the XR_x-OXY-302 Registration trial in ADPKD.** XR_x-OXY-302 is a Phase 3, Multi-Center, Double-Blind, Placebo Controlled, Randomized Withdrawal Design Study to Evaluate the Efficacy and Safety of a Novel Oxypurinol Formulation in Patients with Progressing Stage 2-4 ADPKD and Coexistent Hyperuricemia. with progressing stage 2, 3, or 4 kidney disease. The objective of this study is to evaluate the safety and effectiveness of XORLO™ for the XR_x-008 program over a 24-month treatment period. The aim of the study is to characterize the ability of XO1 to decrease the rate of decline of glomerular filtration rate. An estimated 300 patients will be enrolled. This study is planned to start in the second half of 2024, subject to Special Protocol Assessment

negotiations with the FDA.

4. **Ongoing CMC Work.** In parallel with the XR_X-OXY-301 and XR_X-OXY-302 studies, XORTX will be focus scale-up, validation and stability testing of clinical drug product supplies of XORLO™ under the Company's granted IND, as well as future clinical and commercial supplies. All development will be performed according to current GMP methodology. This work will be ongoing throughout 2022 and 2023.
5. **Activities Related to Potential Commercial Launch.** In preparation for a possible "Accelerated Approval" NDA filing in 2025 in the U.S. for XORLO™ for XR_X-008, XORTX will conduct commercialization studies, including nephrologist, patient, payer interviews, to support in-depth analysis of pricing and/or reimbursement, as well as product brand name selection and filings, and plans for launch. This work will be ongoing from 2023 to 2025.
6. **Activities Related to European Registration.** XORTX will continue to work with and seek out guidance from the EMA to facilitate the path to approval of XORLO™ in the European Union, including required clinical studies and reimbursement conditions. This work will be ongoing from 2023 through 2026, and will include future drug orphan status.

To achieve the above goals, XORTX will continue to pursue non-dilutive and dilutive funding and expand discussions to partner with a major pharma / biotech companies with a global reach. XORTX will also increase financial and healthcare conference participation to further strengthen and expand our investor base.

About XORTX Therapeutics Inc.

XORTX is a pharmaceutical company with two clinically advanced products in development: 1) our lead, XR_X-008 program for ADPKD; and 2) our secondary program in XR_X-101 for acute kidney and other acute organ injury associated with Coronavirus / COVID-19 infection. In addition, XR_X-225 is a pre-clinical stage program for Type 2 Diabetic Nephropathy. 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 dedicated to developing 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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This press release contains express or implied forward-looking statements pursuant to U.S. Federal securities laws. These forward-looking statements and their implications are based on the current 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 the forward-looking statements. Except as otherwise required by law, XORTX undertakes no obligation to publicly release any revisions 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 under the heading “Risk Factors” in XORTX’s Registration Statement on Form F-1 filed with the SEC, which is available on the SEC’s website, www.sec.gov (including any documents forming a part thereof or incorporated by reference therein), as well as in our reports, public disclosure documents and other filings with the securities commissions and other regulatory bodies in Canada, which are available on www.sedar.com.



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