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XORTX Highlights Achievements of 2023 and Preparation for Registration Clinical Trial

CALGARY, Alberta, March 19, 2024 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("XORTX" or the "Company") (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANUA WKN: A3UNZ), 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 achievements in 2023 and objectives planned for 2024.

Dr. Allen Davidoff, CEO of XORTX, stated, "2023 marked a year of substantial clinical, technological and regulatory progress, establishing the foundation for the Company's 2024 goals. Key milestones include: 1/ the grant of U.S. Orphan Drug Designation for the XRx-008 program for Autosomal Dominant Polycystic Kidney Disease ("ADPKD") that is being developed under the US FDA 505(b)2 rules further de-risking this program; 2/ ongoing discussions with the US FDA have aligned our endpoints and other Phase 3 clinical trial elements to make XORLO™ the Company's proprietary oxypurinol formulation, eligible for accelerated approval. These key advances on the XRx-008 program during 2023 were made possible by the exceptional efforts of our Board of Directors, employees, consultants, and vendors. We believe the goals set for 2024 will advance our lead program XRx-008 for ADPKD ever closer to conducting a registration clinical trial leading to marketing approval and value creation for XORTX and its shareholders."

In 2023, the Company made steady progress advancing its strategic plan in key areas, including chemistry, formulation, manufacturing, and non-clinical studies using XORLO™ to attenuate polycystic kidney disease ("PKD") progression in animal models, and topline results from the XRx-OXY-101 bridging clinical study of XORLO™. Each of these milestones permit the next step in the Company's clinical development plan, being a "registration" clinical trial – XRx-OXY-201 in pursuit of accelerated approval and support of the Company's lead program XRx-008 program for ADPKD.

Synopsis of 2023 Achievements

Chemistry and Manufacturing, Clinical and Pre-Clinical Highlights – Produced drug substance for oxypurinol production and produced GMP drug substance; confirmed XORLO™ formulation, produced enhanced bioavailability and produced clinical supply of tablets for clinical trials.

Regulatory Submissions to US Food and Drug Administration ("FDA") and European Medicines Agency ("EMA") Supporting Clinical Trial Conduct

- April 21, 2023 – FDA granted Orphan Drug Designation for XRx-008 program for

ADPKD, following review of February 1st submission of a comprehensive scientific review package within an Orphan Drug Designation application for XRx-008 for treatment of progressing kidney disease due to ADPKD with the US FDA.

- May 5, 2023 – FDA confirmed the eligibility of XORLO™ for Accelerated Approval following review of March 14th submission of type D meeting request with US FDA to discuss the clinical development plan (XRx-OXY-201 clinical trial design).
- August 29, 2023 – XORTX submitted an Orphan Drug Designation application to the EMA for the treatment of ADPKD. Following discussion and guidance from EMA, XORTX will expand data package and resubmit to gain EMA Orphan Drug Designation.

Technology and Patent Advancements

- January 3, 2023 – XORTX submitted a new Provisional Patent Application seeking broadened and lengthened future patent protection, in a patent entitled “Compositions and Methods for Diagnosis, Treatment and Prevention of Kidney Disease.
- November 2, 2023 – XORTX sponsored study results were presented at American Society of Nephrology (“ASN”) under Session Title: “Genetic Diseases of the Kidneys”, by Dr. Charles Edelstein of the University of Colorado. Results of these studies suggest that management of xanthine oxidase activity in PKD may be more important than previously appreciated and further that previously unrecognized factors related to diet, genetic factors or prescribed drugs that increase uric acid levels could potentially aggravate the progression of PKD.

Organizational Highlights

- In addition to substantial technological advancement, the Company continued to bolster the XORTX team with the following appointments:
 - June 26, 2023 - James Fairbairn was appointed Chief Financial Officer of XORTX Therapeutics. Mr. Fairbairn has more than 20 years of experience with publicly-traded companies. He is a Chartered Professional Accountant, and an Institute-certified Director.
 - December 31, 2023 – Patrick Treanor was appointed as a member of the XORTX Board of Directors. Patrick Treanor is a seasoned pharmaceutical industry executive with over 25 years experience. He is the current Chief Operating Officer of Pathalys Pharma, Inc., a private company specializing in advanced therapeutics for late-stage chronic kidney disease management. Mr. Treanor earned a BS in Management from Bryant University and an MBA from Rensselaer Polytechnic Institute.
- On November 29, 2023, XORTX returned to NASDAQ compliance following a reverse split to increase share price to greater than \$1.00.

XRx-008 Program Highlights – Independent Commercial Assessment

In support of ongoing pharmaceutical partnership discussions, XORTX initiated an independent commercial assessment of the XRx-008 program for ADPKD with Bluestar BioAdvisors. This evaluation included interviews with 30 Nephrologists and 10 “Payers” with Large national Plans that cover greater than 290 million lives. Outcome of this assessment suggests that the XRx-008 program for ADPKD worldwide peak net sales per year that may

exceed \$1B, with a total product life estimated to surpass 7 to 10 years.

2024 Corporate Objectives

- March 4, 2024 – XORTX completed an oversubscribed financing of \$2.7 million.
- The Company will provide guidance, in the near future, regarding 2024 Corporate Objectives including announcements regarding clinical and regulatory submissions in support of the XR_x-OXY-201 clinical “registration” trial designed to demonstrate the benefit of XORLO™ in slowing the progression of declining filtering capacity in ADPKD.

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. For more information, please contact:

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Neither the TSX Venture Exchange nor Nasdaq has approved or 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 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.sedarplus.ca.



Source: XORTX Therapeutics Inc.