January 31, 2022

# XORTX Provides Corporate Update

## Synopsis of 2021 Achievements and Key Activities for 2022

CALGARY, Alberta, Jan. 31, 2022 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("**XORTX**" or the "**Company**") (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANU), a pharmaceutical therapeutics company focused on developing innovative therapies to treat progressive kidney disease, is pleased to take this opportunity to provide an update on the Company's progress in 2021 and provide insights on 2022 goals.

Dr. Allen Davidoff stated, "I would like to thank our Board of Directors, employees, vendors and shareholders for their continued support as we continue to develop XORTX into a high value company. Our significant progress in 2021 laid the foundation for our 2022 goals which should contribute to further de-risking our technology and the creation of significant shareholder value going forward."

In 2021, the Company made significant progress advancing its strategic plan in key areas, including the non-clinical development of its proprietary XRx-008 and XRx-101 and strengthening the corporate leadership team. Most notably, the Company completed two equity financings raising gross proceeds of ~$20 million setting the stage for advancement of XRx-008, a late-stage clinical program focused on demonstrating the potential of our novel therapy for autosomal dominant polycystic kidney disease ("**ADPKD**") and XRx-101, for use in treating patients infected with coronavirus COVID-19 with associated acute kidney injury ("**AKI**"). XRx-008 is the development name given to XORTX's proprietary oral formulation of oxypurinol, and shows increased oral bioavailability compared to oxypurinol alone and XRx-101 is a second oral formulation of oxypurinol, for use in treating patients infected with the coronavirus COVID-19 with associated AKI.

### Clinical and Pre-Clinical Highlights

2021 was a breakout year for XORTX in terms of validation of our technology and value creation for shareholders including a successful partnership with Mount Sinai Hospital and patent grants and filings including the following:

- On January 12, 2021, **Notice of Grant received by the European Patent Office for the patent entitled - "Formulations of Xanthine Oxidase Inhibitors".** This patent covers compositions and methods of using XORTX's proprietary formulations of xanthine oxidase for, renal and other diseases where aberrant purine metabolism has been implicated in disease progression.

- On March 8, 2021, the **filing of a provisional patent related to Coronavirus infection entitled "Compositions and Methods for Enhancing Anti-Viral Therapies"**; based on retrospective clinical data from XORTX scientific partners suggesting that an important therapeutic opportunity lies with addressing aberrant

purine metabolism combined with hyperuricemia in patients most at risk to severe COVID-19 outcomes, including due to kidney disease.

- On September 1, 2021, the **European Patent Grant for "Compositions and Methods for Treatment and Prevention of Hyperuricemia Related Health Consequences"**

- On December 7, 2021, the **publication of the peer reviewed paper entitled "Prevalence and Outcomes Associated with Hyperuricemia in Hospitalized Patients with COVID-19"** in the American Journal of Nephrology resulting from an independent research partnership with Mount Sinai Hospital at Icahn School of Medicine.

- On December 20, 2021, the **filing of a provisional patent entitled - "Compositions and Methods for Diagnosis, Treatment of and Prevention of Kidney Disease"**. This provisional patent filing was based upon findings of two independent investigator led studies that: (1) studied the role of aberrant purine metabolism in ADPKD kidney tissue, the results showing that xanthine oxidase enzyme expression and activity in kidney tissue in ADPKD is increased substantially and significantly, potentially revealing a new mechanism of injury in ADPKD; and (2) explored the health consequences of high uric acid in a mouse model of autosomal dominant polycystic kidney disease that successfully demonstrated that increased levels of serum uric acid can accelerate structural and functional changes in kidneys of ADPKD.

- On December 21, 2021, **completed novel synthesis method development and manufacture of non-GMP active pharmaceutical ingredient** for upcoming bridging pharmacokinetic studies: including scale-up, process validation and initiated ongoing stability as part of the CMC requirements.

**Organizational Highlights**

Along with the US listing on Nasdaq and uplisting to the TSX Venture Exchange, 2021 also saw the transformation and bolstering of XORTX's board, management team, clinical team and advisory board with the following appointments:

- **Bill Farley appointed as Director** on May 13, 2021. Bill has over 35 years' experience in business development, sales and leading efforts in drug discovery, development and partnering. Prior to joining the board of directors of XORTX, he held a senior leadership position at Sorrento Therapeutics. Bill began his career at Johnson and Johnson and has also held senior management positions at Pfizer and HitGen Ltd. and Vice President, WuXi Apptec, Inc. creating, building and leading global business development teams, and Vice President, Business Development at ChemDiv where he led numerous efforts to create new therapeutic companies in CNS, oncology and anti-infectives.

- **Jacqueline Le Saux appointed as Director** on June 17, 2021. Jacqueline is a seasoned Canadian health care legal executive who has held senior positions at large and small public and private life science companies. Her legal experience is focused on securities, pharmaceutical regulatory and intellectual property law. As a Vice

President, Legal in both public and private companies Jacqueline led multiple financings, mergers and acquisitions and product licensing transactions.

- **Stephen Haworth appointed Chief Medical Officer** on June 30, 2021. Dr. Haworth has over 25+ years of successful global drug development and senior leadership in both “start-up” and Fortune 500 pharmaceutical firms in the US and Europe. Stephen has a broad clinical and regulatory experience that ranges from infectious disease through nephrology, cardiovascular disease and most recently on programs for treatment and prevention of SARS-CoV infection. He has held key roles in numerous FDA and EMA submissions and has been involved in several licensing and merger transactions. Dr. Haworth holds a medical degree from University College Hospital Medical School, University of London, having graduated with Honors.

- **Amar Keshri appointed Chief Financial Officer** on July 14, 2021. Amar has over 15 years accounting experience in a number of sectors including in the life science and oil and gas industries through public practice audit and finance and accounting consulting roles including with Suncor, PricewaterhouseCoopers LLP and Ernst & Young. He is a Member of the Chartered Professional Accountants of Alberta.

- **Dr. Charles Edelstein, MD, PhD. appointed as Clinical Advisory Board Member** on August 31, 2021. Charles is Professor of Medicine and Nephrologist at the University of Colorado. He is board certified in Nephrology and has a doctoral degree (PhD) in Internal Medicine. His academic research focuses on both therapeutic studies in animal models of polycystic kidney disease (PKD) as well as acute kidney injury (AKI) and biomarkers of AKI. Dr. Edelstein is a world leader in PKD research and PKD care and has received the WSCI Outstanding Investigator Award and is a former president of the Western Section of the American Federation of Clinical Research and is also an International Society of Nephrology member and American Society of Nephrology Advisory Committee Member.

- **XORTX common shares commenced trading on the NASDAQ** exchange under the symbol XRTX on October 13, 2021; and

- **XORTX common shares commenced trading on the TSX Venture Exchange** under the symbol XRTX and its common shares ceased trading on the Canadian Securities Exchange on November 4, 2021.

- **Dr. Raymond Pratt appointed as Director** on December 20, 2021. Ray is an accomplished Physician Executive in clinical medicine, nephrology, drug development, and the pharmaceutical industry. Dr. Pratt is currently Chief Development Officer, Rockwell Medical Inc. He has extensive experience troubleshooting issues concerning regulatory approval of drugs and devices and providing development strategies for global pharmaceutical companies.

**Financial Highlights**

The completion of approximately $20M CAD in financings brought key institutional shareholders to XORTX:

- On February 9, 2021, the Company issued 2,085 units in a private placement offering at a subscription price of $2.94 per unit (adjusted for the 11.74 consolidation effected in September 2021) for gross proceeds of $6,121,572. Key institutional crossover funds represented the primary investors for this oversubscribed offering.

- On October 15, 2021, the Company closed its **initial public offering in the US with US$12 million contemporaneous with the NASDAQ listing.** The financing was led by A.G.P. / Alliance Global Partners and attracted participation from key biopharma institutional investors. The underwritten public offering of 2,906,000 units, with each unit consisting of one common share, no par value, and one warrant to purchase one common share at a public offering price of US$4.13 per Unit.

**Goals for 2022**

In 2022, XORTX is focused on advancing XRx-008 into a clinical trial, the submission of an Orphan Drug Designation, initiation of special protocol assessment discussions with the US Food and Drug Administration ("**FDA**") and continue formulation development for other kidney disease applications. To achieve these objectives, XORTX's action plan includes:

1. **Initiate XRX-OXY-101 Bridging Study.** This study is a three-part, single-dose; fed or fasted; then, multi-dose crossover comparative bioavailability and pharmacokinetic study in healthy volunteers. It is designed to permit XORTX to characterize the safety and relative bioavailability of the XRx-008 formulation. Knowledge gained during the conduct of this trial will provide guidance regarding the oral dose of XRx-008 for our planned registration trial in ADPKD. Additionally, this study will provide data to support future New Drug Application ("**NDA**") submissions to the FDA and the European Medicines Agency ("**EMA**"). This study is planned to start in the second quarter of 2022.

2. **Initiate XRX-OXY-102 Bridging Study.** This study is a multi-dose crossover comparative bioavailability and pharmacokinetic study in healthy volunteers. It is designed to permit XORTX to characterize the safety and relative bioavailability of the XRx-101 formulation options. Knowledge gained during the conduct of this trial will provide guidance regarding the oral dose of XRx-101 for future clinical and commercial planning. Additionally, this study will provide data to support future NDA submissions to the FDA and EMA. This study is planned to start in the first half of 2022.

3. **Complete Orphan Drug Designation.** Current research being conducted will be used to file for orphan drug designation in 2022.

4. **Commence XRX-OXY-301 Registration trial in ADPKD.** XRX-OXY-301 is a multi-site, multi-national, placebo controlled, study in ADPKD patients with progressing stage 2 or 3 kidney disease. The objective of this study is to evaluate the safety and effectiveness of XRx-008 over a 24-month period and study the ability of xanthine oxidase inhibition 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 2022, subject to SPA negotiations with the FDA.

5. **Ongoing CMC Work.** In parallel to the XRX-OXY-101 and XRX-OXY-102 studies,

XORTX will be focused on performing the necessary scale-up, process validation and stability as part of the CMC requirements for the filing of the Investigative New Drug ("**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.

6. **Preparation of 505(b)(2) IND.** In parallel with initiation of XRX-OXY-101 a 505(b)2 based IND is expected to be submitted in the first half of 2022 for the XRx-008 program.

7. **Activities Related to Potential Commercial Launch.** In preparation for a possible NDA filing in 2025 in the U.S. for XRx-008, XORTX is planning to conduct additional commercialization studies, including nephrologist, patient, payer, pricing and/or reimbursement studies, as well as product brand name selection and filings, and plans for launch. This work will be ongoing from 2022 to 2025.

8. **Activities Related to European Registration.** XORTX intends to establish guidance from the European Union for path to approval in the European Union, including required clinical studies and reimbursement conditions. This work will be ongoing from 2022 to 2025.

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 – XRx-008 for Autosomal Dominant Polycystic Kidney Disease (ADPKD), XRx-101 for acute kidney and other acute organ injury associated with Coronavirus / COVID-19 infection as well as XRx-225 which 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 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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**Forward Looking Statements**

This press release contains 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 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 in the Company's Management's Discussion and Analysis for the interim period ended September 30, 2021 filed on the Company's SEDAR profile (www.sedar.com) and under the heading "Risk Factors" in XORTX's Registration Statement on Form F-1 filed with the Securities and Exchange Commission ("**SEC**") available on the SEC's website, www.sec.gov.

Source: XORTX Therapeutics Inc.