

May 5, 2022



XORTX Opens an IND for XRx-008 Program for Autosomal Dominant Polycystic Kidney Disease

• FDA Provides All Clear Notification for IND and Bridging Pharmacokinetics Study •

CALGARY, Alberta, May 05, 2022 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("XORTX" or the "Company") (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANU), a clinical stage pharmaceutical company focused on developing innovative therapies to treat progressive kidney disease, is pleased to announce that the Company has received official notification from the US Food and Drug Association ("FDA") that the Company's recent IND (Investigative New Drug) application has been reviewed and cleared. Accompanying this notification is a "Study May Proceed Letter" regarding the XRx-OXY-101 bridging pharmacokinetics study ("PK Study"). This FDA approval of the IND supports the Company's XRx-008 program for treatment of progressing kidney disease due to autosomal dominant polycystic kidney disease ("ADPKD").

XORTX is focused on advancing XRx-008 through the PK Study as well as registration clinical trials for the treatment of ADPKD. At the present time, very few approved therapeutic options exist to treat progressive kidney disease in individuals due to this disease. There is reason for hope, however, as recent clinical study evidence confirms that uric acid is an independent risk factor for progression of progressive kidney disease and that managing purine metabolism and serum uric acid concentrations can positively affect the kidney health thereby improving the lives of patients with progressive kidney disease.

Dr. Allen Davidoff, CEO of XORTX stated, "The grant of this IND for our XRx-008 program for our Company's lead program for ADPKD, provided an important opportunity for the FDA to review, comment and confirm XORTX's development plans. This grant is an important step in the Company's progress. We are grateful for the FDA review and communications as we optimize the critical path steps needed to proceed with our clinical trial programs and seek marketing approval of XRx-008 for ADPKD patients."

XORTX's XRx-008 therapeutic development program for ADPKD, is advancing a proprietary composition of xanthine oxidase inhibitor, to manage aberrant purine metabolism and chronically high serum uric acid concentration. At present, there are few therapeutic options available to treat progressing kidney disease due to ADPKD. XORTX's IND provided a robust overview of program status with respect to chemistry, manufacturing, pharmacology, toxicology, and clinical work to date and will facilitate formal communications with the FDA regarding development of XRx-008 for the treatment of progressive kidney disease due to ADPKD. Further announcements regarding future meetings with the FDA, other regulatory agencies will be forthcoming.

XORTX is currently conducting our clinical trial - XR-X-OXY-101 - a “bridging pharmacokinetics” study. Part 1 of the PK Study involves dosing under fasted conditions. Part 2 measures the effect of food on pharmacokinetics (PK) and Part 3 is a multiple dose PK evaluation. Safety evaluation is also an important aspect of the XR-X-OXY-101 clinical trial. The PK Study is designed to permit XORTX to characterize the safety and relative bioavailability of the XR-X-008 program formulations. Knowledge gained during the conduct of this trial will provide critical guidance regarding the oral dose for our planned registration trial in ADPKD. Additionally, the PK Study will provide fundamental information for the 505(b)2 filing of the XR-X-008 program.

About ADPKD

ADPKD is a rare disease that affects more than 10 million individuals worldwide.^{1,2} 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.³ 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.¹ For individuals with progressing ADPKD, treatment recommendations include anti-hypertensive treatment, dietary restrictions, and, for a limited percentage of suitable patients, pharmacotherapy.⁴ 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: 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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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.

References:

1. Wiley C., Kamat S., Stelhorn R., Blais J., Analysis of nationwide data to determine the

incidence and diagnosis of autosomal dominant polycystic kidney disease in the USA, *Kidney Disease*, 5(2): 107-117, 2019

2. Bergmann C., Guay-Woodford L.M., Harris P.C., Horie S., Peters D.J., Torres V.E., Polycystic Kidney Disease, *Nat Rev Dis Primers*. 4(1): 50, 2018
3. <https://pkdcure.org/living-with-pkd/chronic-pain-management/>
4. Gimpel C., Bergmann C., Bockenhauer D., et al., International consensus statement of the diagnosis and management of autosomal dominant polycystic kidney disease in children and young people, *Nat Rev Nephrol* 15(11):713-726, 2019

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. 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 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.