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XORTX Announces Grant of US Patent

Patent supporting Autosomal Dominant Polycystic Kidney Disease Granted – XRx-008 Program

CALGARY, Alberta, April 07, 2022 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("XORTX" or the "Company") (NASDAQ: XRTX | TSXV: XRTX | Frankfurt: ANU), a pharmaceutical company focused on developing innovative therapies to treat progressive kidney disease, is pleased to announce receipt of notification that the patent "Formulations of Xanthine Oxidase Inhibitors" will be granted by the United States Patent Office (USPTO). The patent covers compositions for, and methods of using XORTX's proprietary formulations of xanthine oxidase inhibitors for renal and other diseases where aberrant purine metabolism has been implicated in disease progression.

XORTX's XRx-008, for ADPKD program for autosomal dominant polycystic kidney disease ("ADPKD"), centres on a proprietary combination of uric acid lowering agents and other excipients. At present, there are few therapeutic options available to treat progressing kidney disease due to ADPKD or diabetic nephropathy ("DN"). The 20-year protection afforded by this patent will cover XORTX's first-in-class ADPKD therapy to address unmet medical needs in the US. Market size estimates for ADPKD for the United States, Europe, and globally are estimated 150,000, 160,000, and 3,000,000¹, respectively. ADPKD is a rare disease with orphan disease programs in the EU, the US and Japan protecting market exclusivity for 10, 7 and 10-year periods, respectively. Claims granted with this notice are specific to the composition of novel formulations of oxypurinol. Additional future divisional applications, from the original parent patent, will focus on expanding coverage of new proprietary formulation/compositions and uses of those novel formulations.

Dr. Allen Davidoff, CEO of XORTX stated, "This newly granted patent covers compositions of formulations key to XORTX's platform technology and this issuance strengthens our intellectual property portfolio in the United States. Importantly, this US patent grant protects our novel, proprietary formulation program – XRx-008 for ADPKD, chronic kidney and other diseases where aberrant purine metabolism and chronic hyperuricemia may accelerate disease progression. Grant of this patent provides the protection to expand our clinical trials, commercialization and partnering opportunities in the United States. Including this newly allowed patent, XORTX now has four granted patents in the US and/or EU covering compositions and uses of uric acid lowering agents to treat and prevent kidney disease, insulin resistance and diabetic nephropathy."

About Polycystic Kidney Disease and XRx-008

Polycystic kidney disease (PKD) is considered a rare disease with two main types - ADPKD and autosomal recessive PDK ("ARPKD"), with prevalence of 1:800 and 1:20,000, respectively. PKD is a disorder that originates due to genetic changes and results in

numerous fluid filled cysts that can form in the kidneys. This genetic disorder tends to worsen with progressing age and is characterized by increasing cyst numbers and size that changes the shape of kidneys making them much larger. Progression of this disease reduces kidney function and may lead to kidney failure and the need for transplant or dialysis. Statistically, greater than 50% of individuals with ADPKD reach end stage kidney failure by the age of 60 years. Typically, diagnosis of ADPKD occurs between the ages of 30 and 50, when signs and symptoms begin to appear. Progression of PKD is frequently accompanied by high blood pressure, hyperuricemia, gout, kidney stones, proteinuria, abdominal pain, hematuria and declining kidney filtering ability. Like many progressing kidney diseases, the declining rate of filtering capacity accelerates with time leading to end stage kidney failure and the need for kidney transplant or dialysis.

Recently, non-clinical and clinical evidence has accumulated showing that high serum uric acid concentration may mediate disease progression in ADPKD including:

1. Individuals with ADPKD have high reported incidences of hyperuricemia (>60%) and clinical gout (24%) and conditions that are associated with uric acid crystal formation in the kidneys such as low serum and urine pH^{1,2,3}.
2. A high prevalence of kidney stones of approximately equal uric acid composition or oxalate composition⁴.
3. High serum uric acid is an independent risk factor for cyst genesis, cyst growth and declining filtering capacity of kidneys¹.
4. Xanthine oxidase inhibition in ADPKD patients may reverse progression of glomerular filtration rate decline⁵.

At the present time, few therapeutic options to treat, stabilize or slow this progressing kidney disease are available. At the present time, only a single drug is approved to date - Tolvaptan. Although helpful for slowing cyst growth, the need to develop therapeutic options that slow progressive decline of filtering capacity remains, as this is critical to improving quality of life and kidney health for individuals facing this disease. XRx-008 represents a first-in-class opportunity to help individuals with decreasing renal filtering capacity and slow or prevent end stage renal disease and the need for dialysis.

References:

1. Helal, I., et al., *Serum uric acid, kidney volume and progression in autosomal-dominant polycystic kidney disease*. Nephrol Dial Transplant, 2013. **28**(2): p. 380-5.
2. Torres, J.A., et al., *Crystal deposition triggers tubule dilation that accelerates cystogenesis in polycystic kidney disease*. J Clin Invest, 2019. **129**(10): p. 4506-4522.
3. Errasti, P., et al., *Autosomal-dominant polycystic kidney disease: high prevalence of graft loss for death-related malignancies and cardiovascular risk factors*. Transplant Proc, 2003. **35**(5): p. 1717-9.
4. Idrizi, A., et al., *Prevalence of nephrolithiasis in polycystic kidney disease* Cent. Eur. J. Med, 2011. **6**: p. 497.
5. Han, M., et al., *Hyperuricemia and deterioration of renal function in autosomal dominant polycystic kidney disease*. BMC Nephrol, 2014. **15**: p. 63.

About XORTX Therapeutics Inc.

XORTX Therapeutics Inc. is a pharmaceutical therapeutics company with two clinically advanced products in development – XRx-008 for Autosomal Dominant Polycystic Kidney Disease (ADPKD), XRx-101 for Coronavirus / COVID-19 infection and XRx-221 is a clinical stage program for Type 2 Diabetic Nephropathy (T2DN). The Company has strong intellectual property rights and established proof of concept through independent clinical studies. XORTX is working to advance its clinical development stage products that target uric acid lowering as a method of treating progressive kidney disease. At XORTX, we are dedicated to developing medications to improve the quality of life and future of patients with kidney disease. Additional information on XORTX is available at www.xortx.com.

For further information, please contact:

Allen Davidoff, CEO
adavidoff@xortx.com or +1 403 455
7727

Nick Rigopulos, Director of Communications
nick@alpineequityadv.com or +1 617 901
0785

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.

¹ Source: The National Center for Biotechnology Information, a branch of the US National Institutes of Health and the PKD International Association.



Source: XORTX Therapeutics Inc.