

April 21, 2023



# XORTX Announces Receipt of FDA Orphan Drug Designation to Treat Autosomal Dominant Polycystic Kidney Disease

CALGARY, Alberta, April 21, 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 announce the grant of Orphan Drug Designation for oxypurinol – "orphan-drug designation request of oxypurinol is granted for treatment of autosomal dominant polycystic kidney disease".

The orphan drug designation process ("ODD") begins with an application to the US Food and Drug Administration ("FDA") Orphan Drug Designation office, and is accompanied by a comprehensive package that includes the basic science, analysis of addressable number of individuals with autosomal dominant polycystic kidney disease ("ADPKD") and description of the patients projected to be helped by a therapy. The FDA ODD office review of the application package provided by XORTX determined that the evidence that aberrant purine metabolism and high uric acid levels suggest a mechanism of injury in ADPKD and importantly, that the XORLO™ therapy may slow progression of this form of injury.

While this is not approval of XORLO™ for use in patients at the present time, it is a key milestone regarding the new and existing discoveries made by XORTX and regarding a novel approach to slowing progression of kidney disease in ADPKD.

Dr. Allen Davidoff stated, "Orphan drug designation represents a major milestone for the Company in pursuit of marketing approval for XORLO™, our proprietary formulation of oxypurinol, and the XRx-008 program for ADPKD. This designation was a significant and critical milestone for all of the staff and management at the Company. Further our potential partners indicated that it was a critical requirement underpinning their ongoing consideration of the XORLO™ program. We look forward to our upcoming meeting with the FDA on May 1, 2023 to discuss our planned phase 3 clinical program for XORLO™."

Drugs intended to treat orphan diseases (rare diseases that affect less than 200,000 people in the US) are eligible to apply for ODD, which provides multiple benefits to the sponsor during development and after approval. XORTX intends to pursue these benefits as part of the drug development for XRx-008 for treatment of ADPKD.

## Benefits of Orphan Drug Designation

Under the Orphan Drug Act, drug companies can apply for ODD, and if granted, the drug will have a status which gives companies exclusive marketing and development rights along with other benefits to recover the costs of researching and developing the drug. A tax credit

of 25% of the qualified clinical drug testing costs awarded upon drug approval is also possible. Regulatory streamlining and special assistance to companies that develop drugs for rare patient populations is another benefit of ODD. In addition to exclusive rights and cost benefits, the FDA will provide protocol assistance, potential decreased wait-time for drug approval, discounts on registration fees, and eligibility for market exclusivity after approval.

Key benefits of ODD:

- Seven years exclusivity post-approval
- Tax credits of 25% off the clinical drug testing cost awarded upon approval
- Waiver of FDA User Fees

XORTX recently presented positive results in animal models of PKD in both mouse and rat, showing the ability of XORTX's proprietary formulation of oxypurinol, XORLO™, to attenuate expansion of kidneys, due to the cyst promoting effects of aberrant purine metabolism and hyperuricemia - at the American Society of Nephrology meeting during the November 2022 meeting.

### **About the XRx-008 Program**

Oxypurinol is a xanthine oxidase inhibitor (“XOI”) with important pharmacologic characteristics ideal for administration to individuals with ADPKD. Key pharmacologic attributes include:

1/ The ability to act in the circulation, kidney and cardiovascular tissue and inhibit the production of uric acid and so attenuate the mechanism of injury and accelerating effect of xanthine oxidase on progressing diseases.

2/ XORLO™ provides substantially increased absorption of oxypurinol. This approach provides an effective, well tolerated drug with an extensive clinical safety experience suggesting the Company's XRx-008 program has the capacity to provide superior XOI to slow the accelerating decline in kidney function during ADPKD progression.

### **About ADPKD**

ADPKD is a rare disease that affects more than 10 million individuals worldwide.<sup>1,2</sup> 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.<sup>3</sup> 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.<sup>1</sup> For individuals with progressing ADPKD, treatment recommendations include anti-hypertensive treatment, dietary restrictions, and, for a limited percentage of suitable patients, pharmacotherapy.<sup>4</sup> New, more broadly applicable therapies to effectively slow decline of kidney function in ADPKD are needed.

### **About XORTX Therapeutics Inc.**

XORTX is a pharmaceutical company with two clinically advanced products in development:

1) our lead, XRx-008 program for ADPKD; and 2) our secondary program in XRx-101 for acute kidney and other acute organ injury associated with Coronavirus / COVID-19 infection. In addition, XRx-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](http://www.xortx.com).

For more information, please contact:

Allen Davidoff, CEO

[adavidoff@xortx.com](mailto:adavidoff@xortx.com) or +1 403 455 7727

Nick Rigopoulos, Director of Communications

[nick@alpineequityadv.com](mailto:nick@alpineequityadv.com) or +1 617 901 0785

Media Inquiries, David Melamed, Ph.D.

[david.melamed@russopartnersllc.com](mailto:david.melamed@russopartnersllc.com) or +1 212 845 4225

## 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., Bermann C., Bockenbauer 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

*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](http://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](http://www.sedar.com).



Source: XORTX Therapeutics Inc.