September 10, 2018

XORTX Announces Submission of Orphan Drug Designation Application

- **Phase II Clinical Study in ADPKD Planned**

CALGARY, Alberta, Sept. 10, 2018 (GLOBE NEWSWIRE) -- XORTX Therapeutics Inc. ("**XORTX**" or the "**Company**") (CSE:XRX; OTCQB:XRTXF), a biopharmaceutical company focused on developing innovative therapies to treat progressive kidney disease, is pleased to announce that the Company along with its collaborative partner, Cato Clinical Research, has submitted documents to the US Food and Drug Administration (FDA) to receive Orphan Drug Designation (ODD) status for XORTX's XRx-008 (a proprietary formulation of Oxypurinol) program for the treatment of autosomal dominant polycystic kidney disease (ADPKD).

Dr. Allen Davidoff, XORTX's CEO commented, "Orphan Drug Designation status is an important milestone for the XRx-008 program for ADPKD. ODD recognition by the FDA provides a variety of tax incentives, enhanced patent protection and marketing rights as well as, clinical research subsidies. The benefit to this XORTX program and patients with ADPKD is that XRx-008 can be advanced more quickly. That in turn permits XORTX to efficiently establish the merit of this program and create future value for shareholders. Our hope is that the planned phase II clinical trial for this program will demonstrate the ability of this therapy to slow or stop the deleterious effects of uncontrolled uric acid levels."

XORTX is focused on advancing XRx-008 through phase II clinical trials for the treatment of ADPKD. Strong scientific and clinical evidence suggests that in this patient population, uric acid concentration can deleteriously affect progression of kidney disease. Managing purine and uric acid levels in patients shows promise as a therapy to slow the rate at which filtering capacity in kidneys decreases. Currently there are few FDA approved therapeutic options to treat progressive kidney disease (PKD) in patients with polycystic kidney disease.

Dr. Richard Johnson, a respected Thought Leader and Scientific and Clinical Advisory Board member for XORTX stated, "ADPKD is one of the most important causes of kidney disease in the world. Serum uric acid is commonly elevated in this disease and there is increasing evidence that it may contribute to the progression of kidney disease. I am delighted to see XORTX take a major step in announcing its desire to perform a clinical trial on the effect of uric acid-lowering on ADPKD. New treatments are badly needed and the potential for this treatment to have a significant beneficial effect is high."

Orphan Drug programs in the United States – programs for the treatment of rare disease – were passed into law in 1983 to facilitate development of orphan drugs – drugs for rare diseases such as ADPKD, Huntington's disease, ALS and muscular dystrophy. These rare diseases typically have fewer than 200,000 patients living in the US and due to small patient numbers would not be considered economically feasible without government programs to

support their economic viability. ODD does not indicate that the therapeutic is either safe and effective or legal to manufacture and market in the United States. That process is handled through other offices in the FDA, however an ODD designation would qualify XORTX for a number of benefits from the US federal government, such as reduced taxes and grants to fund future clinical trial work – a potentially substantial non-dilutive funding benefit to shareholders. Similar programs for rare diseases exist in European Union, Japan and other countries. Orphan drugs generally follow the same regulatory development path as any other pharmaceutical product, in which testing focuses on pharmacokinetics and pharmacodynamics, dosing, stability, safety and efficacy, however, some statistical burdens are lessened in an effort to maintain development momentum. As a result of world wide support for the development of therapeutic solutions to disease, orphan programs are some of the most successful, time and cost effective programs to develop.

For more information on ODD program fundamentals, see:
www.optum.com/resources/library/world-of-orphan-drugs.html

For more information on the robust market for Orphan Drug programs globally, see:
http://info.evaluategroup.com/rs/607-YGS-364/images/EPOD17.pdf

About XORTX Therapeutics Inc.

XORTX Therapeutics Inc. is a BioPharmaceutical company focused on developing innovative therapies to treat progressive kidney disease. XORTX has lead programs to develop treatments for progressive kidney disease due to diabetes, diabetic nephropathy and polycystic kidney disease. Secondary programs focus on developing therapies for health consequences that accompany pre-diabetes, diabetes and cardiovascular disease. Additional information on XORTX Therapeutics is available at www.xortx.com.

For further information, please contact:

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

or Erik Matthews, Corporate Communications & Investor Relations
erik@xortx.com or +1 747 203 5240

The CSE has 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.

This news release includes forward looking statements that are subject to assumptions, risks and uncertainties. Statements in this news release which are not purely historical are forward looking statements, including without limitation any statements concerning the Company's intentions, plans, estimates, beliefs or expectations regarding the future. Although the Company believes that any such intentions, plans, estimates, beliefs and expectations in this news release are reasonable, there can be no assurance that any such intentions, plans, beliefs and expectations will prove to be accurate. The Company cautions readers that all forward looking statements, including without limitation those relating to the Company's future operations and business prospects, are based on assumptions none of which can be assured, and are subject to certain risks and uncertainties that could cause

actual events or results to differ materially from those indicated in the forward looking statements. Readers are advised to rely on their own evaluation of such risks and uncertainties and should not place undue reliance on forward looking statements. Any forward looking statements are made as of the date of this news release, and the Company assumes no obligation to update the forward looking statements, or to update the reasons why actual events or results could or do differ from those projected in the forward looking statements. The Company assumes no obligations to update any forward looking statements, whether as a result of new information, future events or otherwise.

Source: XORTX Therapeutics Inc.