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XORTX Announces Revision to Polycystic Kidney Disease Clinical Development Plan

● Shorter Path to XRx-008 Marketing Approval ●

CALGARY, Alberta, Oct. 23, 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 ("PKD"), is pleased to announce that the Company's clinical development plan has been reviewed and accelerated. This major revision to the Company's original clinical development plan was acceptable to the US Food and Drug Administration ("FDA") subsequent to XORTX's submission of its pre-Investigational new drug ("IND") information package and the Company's in-person meeting held September 20, 2018 to discuss the comprehensive development plan.

Discussions with the FDA ranged across developmental topics including manufacturing, formulation, non-clinical study plans and clinical development strategy for XRx-008, including:

- Review of XORTX's proposed chemistry, manufacturing and formulation was confirmed by the FDA as acceptable with no material changes;
- The proposed non-clinical development proposal was reviewed and confirmed the importance of characterizing the scale of increased bioavailability of XRx-008 in advance of clinical study initiation;
- The proposed clinical development program for XRx-008, as a therapy for patients with PKD was outlined by the XORTX team and was composed of separate phase 2 and phase 3 clinical trials, followed by marketing application (NDA). Discussion and exploration with the FDA suggested a substantially shorter path to marketing approval for XRx-008 for ADPKD; and
- The outcome of the pre-IND meeting is an accelerated clinical development plan composed of a study to characterize bioavailability XRx-008 in man, then a single, pivotal phase 2/3 clinical trial which would be eligible for special protocol assessment (SPA – see further information below).

Dr. Allen Davidoff, XORTX's CEO stated, "We are very pleased with the positive meeting we had with the FDA that clarified that XORTX can simplify and accelerate its clinical study plan. The Company has now defined four clear steps to develop XRx-008 for autosomal dominant polycystic kidney disease patients ("ADPKD"): (i) manufacture clinical study ready drug for our upcoming clinical trials; (ii) file the IND and characterize the bioavailability and pharmacokinetics of XRx-008 in humans; (iii) complete the orphan drug designation ("ODD")

process for this program (see further information below); and, (iv) complete a pivotal phase 2/3 clinical trial. XORTX will seek a special protocol assessment (SPA) for this pivotal study. This accelerated clinical development plan substantially decreases the time and cost to bring this therapy to patients with PKD."

Special Protocol Assessment ("SPA") is one optional type of agreement submission that is available to sponsors, such as XORTX, for pivotal phase 3 trials. Through the SPA process, the sponsor and the FDA negotiate the design of a clinical trial that will support an efficacy claim for marketing approval. One advantage of the SPA is that, if an agreement is reached, XORTX would then have clarity in writing of the endpoints that must be achieved to support marketing approval.

Orphan Drug programs in the United States are programs for the treatment of rare disease which 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.

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Source: XORTX Therapeutics Inc.