XORTX THERAPEUTICS INC.

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REDEFINING HOW KIDNEY DISEASE IS TREATED

XORTX is a biopharmaceutical company focused on developing late stage drug based therapies for progressive kidney disease (PKD) markets. Strong clinical evidence suggests a high probability of translating phase 3 registration trial into marketing approval targeting Autosomal Dominant Polycystic Kidney Disease (ADPKD) and in an upcoming Phase 2b clinical trial in Diabetic Nephropathy (DN).

Our Technology: Is focused on inhibiting xanthine oxidase (XOI) as a method of controlling intracellular and serum uric levels - independent risk factors for kidney disease progression. XORTX's most advanced program is a therapy for treatment of ADPKD – with plans to initiate a Phase 3 registration clinical trial using a proprietary formulation of Oxypurinol. The Company's DN program aims to advance into a Phase 2b clinical trial in the near term with testing of a potent, new class of xanthine oxidase inhibitor. Recent successful completion of a phase 2a trial in DN suggest a potent dual action mechanism of action that decreases proteinuria. Currently no drug development programs for either ADPKD or DN progression target XOI, making both programs - first-in-class. Recently, XORTX initiated – XRx-101 to treat and protect against the acute kidney injury currently noted in coronavirus COVID-19 infections.

XORTX's strategy is to develop a proprietary formulation of Oxypurinol as a candidate for polycystic kidney disease, an orphan disease where only one drug is currently approved. To accelerate this program and minimize program risk a proprietary novel formulation was developed and a PCT patent application was submitted in 2014. FDA has re-confirmed the view that Oxypurinol is safe, well tolerated and effective. This program is preparing for a phase 3 registration trial. Multiple industry specialty pharmaceutical companies have expressed interest in the ADPKD program.

The Market: In the United States, Europe and Japan, PKD is considered an orphan disease with approximately 150,000 patients each. Type 2 diabetes is growing and projected to double in the next decades from ~10,000,000 patients to ~20,000,000 in the US. Together DN and PKD cause nearly 50% of all kidney disease leading to end stage renal failure and the need for life saving dialysis.

Value Proposition and Competitive Advantage: XORTX's strategy is to rapidly develop our proprietary Oxypurinol formulation (XRx-008) with improved bioavailability for the treatment of ADPKD – an orphan indication - and builds upon strong, existing, clinical trial successes. Orphan drug programs receive seven year market exclusivity in the US, and 10 years in Europe combined with strong pricing, tax credits and margin protection.

Total and Target Market Opportunity for Uric Acid Related Disease: Recent discussions with the FDA confirmed the view that Oxypurinol is well characterized as safe, well tolerated and effective as a XOI and for managing uric acid levels. Current industry interest in the ADPKD program suggests this program has the potential for developmental partnering during the pivotal trial or shortly thereafter.

Type of Incidence – North America (Past 5 Years)	Individuals Affected	% of US Population	US Market ($USD)
Hyperuricemia	76 Million	22%	500 M
Diabetic Nephropathy – Type I [2]	0.1 Million	0.03%	2 B
Diabetic nephropathy – Type II [3]	10 Million	3%	6 B
Diabetes	86 Million	34%	55 B
ADPKD [1]	~120,000	0.004%	2 B
IgA nephropathy	3,000-6,000	Rare	2 B
[1,2,3] **Early target markets for XORTX**	**Total Market opportunity:** **Target Market opportunity:**		**$258 B** **$10 B**

- Developing first in class therapies to treat progressive kidney disease accelerated by improper uric acid metabolism
- XORTX technology improves management of purine metabolism, and can be used to treat major and rare a progressive kidney diseases.
- XORTX technology treats rare diseases such as ADPKD and large market Type 2 DN
- Patent portfolio - University of Florida, Washington and XORTX
- US PKD Foundation recognition and collaboration for the development of treatments that will redefine how physicians treat ADPKD
- Big 10 Pharma interested in T2DN program.
- Orphan disease focus provides a fast track through FDA, seven years exclusivity in US and 10 years in Europe combined with lucrative product pricing, resulting in a strong financial model.
- Significant current Specialty Pharma interest in licensing ADPKD program.
- **Funding**
  - ~$10M Research: pre-company formation
  - $3.7 M equity investment to date
- **Shares Outstanding**
  - Current – 81.2 million
  - FD – 101.7 million

Intellectual Property & Competitive Advantage

Our multi-tiered patent portfolio is composed of granted patents and patent applications that claim the "use of uric acid lowering agents" for the treatment of hypertension, insulin resistance and diabetic nephropathy (DN) (granted). XORTX's patent applications include a PCT filing claiming new formulations for xanthine oxidase inhibitors and includes claims for the treatment of chronic kidney disease (CKD), cardiovascular disease, metabolic syndrome and diabetes. A recent PCT prophetic patent filing claims the use of xanthine oxidase inhbitors as a means of treating acute kidney injury due to coronavirus COVID-19 infection.

Development:

In process: IND Submission for ADPKD, Orphan Designation + possible grants; for Type 2 DN phase 2b protocol approval
2020 : Phase 3 registration trial in ADPKD (under SPA)
Ongoing discussions with several pharmaceutical may provide licensing opportunities in the near future.

Milestones Achieved:

- Aggregated and composed 7 patents: 6 methods of use, 1 composition application, other composition patents under preparation.
- Advancing XRx-008 formulation studies to "clinical trial ready" and developed new molecule entities for diabetic nephropathy.
- Positive Phase 2a clinical trial in T2DN suggests high probability for translational success and future marketing approval.
- Recent coronavirus COVID-19 program with XRx-101 advancing toward clinical trial (phase 3)
- Built a team of experienced drug development professionals, manufacturers, clinical trial and regulatory research organizations.
- Since incorporation, XORTX has raised $6.2 M (plus previously ~ $ 10 M spent developing the compound).

MANAGEMENT

A virtual company strategy with experienced, key leaders ready to maximize value creation.

Allen Davidoff, PhD – *Founder, Chief Executive Officer, President and Director* with 14+ years product development and clinical development experience. History of successful fundraising for several publicly traded PharmaCo.s including $6.2 M dollars at XORTX.

James Fairbairn, CA, ICD.D – Experienced public company *CFO* with extensive corporate governance and reporting experience with emerging companies.

Alan Moore, PhD – Founding Board Member and Director with 30+ years pharmaceutical and drug development experience, most with P&G. History of successful fund raising >$20 M.

Brian Mangal, MSc - *Director of Business Development* with 15 years' experience in pharma/biotechnology, including Cardiome Pharma Corp.

Board of Directors: In addition to Allen Davidoff,

Bruce Cousins - Accomplished biopharma industry senior finance and operations executive with experience spanning small, early stage growth to large, international companies.

Bruce Rowlands - *Chairman* **–** Extensive experience in the biotechnology and investment banking industries.

Paul Van Damme, B. Comm., CPA, MBA – *Audit Chair* **-** Biotech industry focused experience with senior positions with a number of Canadian and US public companies.

Allan Williams - Independent businessman with 30+ years capital market and public company experience.

OPPORTUNITY SUMMARY

Strong Management Team – History of success and experience in early drug development to market approval, tailored to developing XRx-008 from concept through New Drug Application (NDA). Historically, developed Oxypurinol through NDA for "allopurinol intolerant gout", an orphan indication, showing effective uric acid lowering and superior safety. Developed Vernakalant – an antiarrhythmic drug now approved and marketed in EU markets.

KOLs – Strong Scientific and Clinical Advisory board members Dr. Petter Bjornstad and Richard Johnson of the Colorado School of Medicine, Dr. Federico Maese, Texas-based Cardiologist, and Dr. Henk ter Keurs, Cardiologist, LIBIN, Cardiovascular Institute of Alberta.

Intellectual Property Portfolio – XORTX claims enable exclusive use of "all uric acid lowering agents" for treatment of Hypertension, Insulin Resistance, Metabolic Syndrome, Diabetes and Diabetic Nephropathy. XORTX has three granted patents and four applications.

Lower Development Costs and Faster Time to Market – Orphan Drug Programs in ADPKD has potential for marketing approval and US product launch possible in 2021.

Multiple Phase 2 Clinical Trials Validate IP Concept – Clinically meaningful and statistically significant effects on Early-Stage Hypertension and Progressive Renal Injury, ADPKD, DN and CKD already completed, increase the probability of pivotal trial success.

Recent Orphan Program Acquisitions – Demonstrable market interest and validation of the return on investment opportunity for orphan indications, with deal values ranging from $300M to $2B. Several license partners identified .

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