XORTX THERAPEUTICS INC.

Developing Orphan Drug Therapies To Slow Progressive Kidney Disease

开发孤儿药疗法以减缓进展性肾脏疾病

GCFF– Oct 21st, 2023

GCFF– 2023年10月21日

Nasdaq : XRTX

TMX TORONTO STOCK EXCHANGE TSX VENTURE EXCHANGE :XRTX

BÖRSE FRANKFURT : ANU

Forward Looking Statements / 前瞻性声明

Certain information included in this Presentation constitutes forward-looking information or forward-looking statements under applicable securities legislation ("**forward-looking statements**"). These statements relate to future events or future performance of the Company. Forward-looking statements are statements that are not historical facts and are often, but not always, identified using words or phrases such as "can", "continue", "develop", "expect", "forecast", "future", "may", "milestone", "plan", "potential", "proposed", "will" and other similar expressions. In particular, but without limiting the foregoing, this Presentation contains forward-looking statements pertaining to, among other things: the industry in which Company operates, including the value thereof; strategic plans, including the timing thereof; the Company's operations, including with respect to accelerated approval processes, licencing deals, patent filings and clinical trials, including the timing and results thereof; the effects of end-stage renal disease and onset delay of end-stage renal disease; timing and occurrence of certain milestones, including the success of preclinical studies and clinical trials; and the Company's products, services and assets, including the benefits thereof. In addition, this Presentation may contain forward-looking statements attributed to third party industry sources.

By their nature, forward-looking statements involve known and unknown risks, uncertainties, and other factors that may cause actual results or events to differ materially from those anticipated. Such forward-looking statements are provided for the purpose of providing information about management's current expectations and plans relating to the future. Readers are cautioned that reliance on such statements may not be appropriate for other purposes, such as making investment decisions. These factors and risks include, without limitation: incorrect assessments of the value of acquisitions, licenses and development programs; technical, manufacturing and processing problems; actions by governmental authorities, including increases in taxes; the availability of capital on acceptable terms; fluctuations in foreign exchange, currency, or interest rates and stock market volatility; failure to realize the anticipated benefits from licenses or acquisitions; and potential labor unrest. This list is not exhaustive of the factors that may affect any of the Company's forward-looking statements. Some of the important risks and uncertainties that could affect forward-looking statements are described further under the heading "Key Information - Risk Factors" in its annual report on Form 20-F filed with the Securities and Exchange Commission and under the heading "Risks Related to the Business" in its management's discussion and analysis filed as an Exhibit to its annual report on Form 20-F, which annual report is available on www.sec.gov.

With respect to forward-looking statements in this Presentation, the Company has made assumptions, regarding, among other things: the availability of capital to fund planned expenditures; prevailing regulatory, tax and environmental laws and regulations; the ability to secure necessary personnel, equipment, supplies and services; the Company's ability to manage the Company's growth effectively; the absence of material adverse changes in the Company's industry or the global economy; trends in the Company's industry and markets; the Company's ability to maintain good business relationships with the Company's strategic partners; the Company's ability to comply with current and future regulatory standards; the Company's ability to protect the Company's intellectual property rights; the Company's continued compliance with third-party license terms and the non-infringement of third-party intellectual property rights; the Company's ability to manage and integrate acquisitions; the Company's ability to raise sufficient debt or equity financing to support the Company's continued growth.

Although the Company believes that the expectations reflected in such forward-looking statements are reasonable, such statements are not guarantees of future performance and actual results may differ materially from those in forward-looking statements. Undue reliance should not be placed on forward-looking statements because the Company can give no assurance that such expectations will prove to be correct and such statements are based on the beliefs, estimates and opinions of the Company's management on the date such statements are made. Many factors could cause the Company's actual results, performance or achievements to vary from those described herein. Should one or more of these risks or uncertainties materialize, or should assumptions underlying forward-looking statements prove incorrect, actual results may differ materially from those described in this Presentation as intended, planned, anticipated, believed, estimated or expected.

The forward-looking statements included in this Presentation are expressly qualified in their entirety by this cautionary statement. The Company cautions that the foregoing lists of assumptions, risks and uncertainties are not exhaustive. The forward-looking statements contained in this Presentation are made as of the date hereof and the Company undertakes no obligation to update publicly or revise any forward-looking statements, whether as a result of new information, future events or otherwise, unless required by applicable securities laws.

Management Team / 管理团队

Dr. Allen W. Davidoff, Ph.D., Chief Executive Officer / Allen W. Davidoff 博士、博士、首席执行官

Dr. Davidoff has 20 years of drug development experience and is the founder and CEO of XORTX. Allen has a broad range of clinical and regulatory experience and senior management experience in pharmaceutical R&D. Previously, Allen was co-founder, CSO at Stem Cell Therapeutics Corp. which merged with Trililium and thereafter was acquired by Pfizer. Prior to his role at Stem Cell, Allen was Senior Scientist/Head of Pharmacology at Cardiome Pharma Corp. Allen earned his PhD in Cardiovascular Physiology and Biophysics at University of Calgary. / Davidoff博士拥有20年药物开发经验，是XORTX的创始人兼首席执行官，在制药研发领域拥有丰富的临床和监管经验以及高级管理经验，此前曾担任Stem Cell Therapeutics Corp.的联合创始人兼首席战略官，该公司与Trililium合并，随后被辉瑞收购 。在加入Stem Cell之前，Allen是Cardiome Pharma Corp的高级科学家/药理学主管。他在卡尔加里大学获得了心血管生理学和生物物理学博士学位。

Dr. Stephen Haworth, MD, Chief Medical Officer / Stephen Haworth 博士、医学博士、首席医疗官

Dr. Haworth has >25 years of successful global drug development and senior leadership in both “start-up“ and Fortune 500 pharmaceutical firms in both the US and Europe. Stephen has a broad clinical and regulatory experience that ranges from nephrology, cardiovascular, and infectious disease. He has extensive regulatory experience with FDA and EMA submissions, as well as licensing and M&A transactions. Stephen holds a MD from University College Hospital Medical School, University of London, having graduated with Honors. / Haworth博士拥有超过 25 年的成功全球药物开发经验，并在美国和欧洲的“初创”制药公司和财富 500 强制药公司中担任高级领导职务，拥有广泛的临床和监管经验，涉及肾脏病、心血管和传染病等领域，在 FDA 和 EMA 报批、 以及许可和并购交易方面拥有丰富的监管经验。 Stephen 以优异的成绩毕业于伦敦大学大学学院医院医学院，获得医学博士学位。

Dr. Taryn Boivin, Ph.D., Head Chemistry, Manufacturing and Controls (CMC) / Taryn Boivin博士，博士、化学、制造和控制 (CMC) 主管

Dr. Boivin is a pharmaceutical veteran with >30 years of experience leading all aspects of CMC and related disciplines. Her experience includes multiple worldwide drug submissions, post approval support activities, and commercial supply chain operations. Early in her career, Taryn was among a key group of leaders who established the Glaxo Canada (later GSK) pharmaceutical development organization, and was a pivotal contributor to this world-class research, development, and manufacturing facility. Dr. Boivin has held numerous Senior VP positions in large and small biopharma. / Boivin博士是一位制药资深人士，在领导CMC和相关学科的各个方面拥有超过30年的经验。她的经验包括多次全球药物申请、审批后支持活动和商业供应链运营。在她职业生涯的早期，Taryn是建立葛兰素加拿大公司（后来的葛兰素史克）药物开发组织的关键领导者之一，并且是这个世界级研究、开发和制造工厂的关键贡献者，曾在大型和小型生物制药公司担任过多个高级副总裁职位。

James Fairbairn, CPA, Chief Financial Officer / James Fairbairn，注册会计师、首席财务官

James was the Company’s Chief Financial Officer from November 2018 through July 2021. He is a Chartered Professional Accountant having obtained his CPA designation in 1987, and an Institute-certified Director, he holds a B.A. from Western Ontario. He has more than 30 years of experience with publicly traded companies. / James在2018年11月至2021年7月期间曾担任公司首席财务官，是一名特许专业会计师，于1987年获得注册会计师资格，也是一名经协会认证的董事，拥有西安大略大学文学学士学位，在上市公司拥有30多年的经验。

Dr. Stacy Evans, M.D., MBA Chief Business Officer (Consultant) / Stacy Evans博士、医学博士、工商管理硕士、首席商务官（顾问）

Dr. Evans has >20 years of commercial development and business development experience, including 12 years at Pfizer where he was last responsible for leading transactions across all TAs. Stacy has been consulting at an executive level for small to mid-size private and public biopharma companies for the past 7 years including as part-time Chief Business Officer for Avillion, LLP, a Blackstone-backed late-stage financing and co-development company. Stacy holds a MD from McGill University and an Executive MBA from Columbia University. / Evans博士拥有超过20年的商业开发和业务开发经验，其中包括在辉瑞公司工作了12年，最后负责领导所有TA的交易，在过去7年里一直为中小型私营和上市生物制药公司提供高管层咨询，包括担任Avillion, LLP（一家Blackstone支持的后期融资和联合开发公司）的兼职首席商务官，拥有麦吉尔大学的医学博士学位和哥伦比亚大学的行政工商管理硕士学位。

XORTX THERAPEUTICS INC.

Board of Directors / 董事会

Anthony Giovinazzo – Chairman / Anthony Giovinazzo – 董事会主席

43 years of total work experience, is an internationally recognized expert in intellectual property, drug development and commercialization, including numerous licensing agreements, with more than 25 years' experience in Central Nervous System diseases. Co-inventor, Chief Executive Officer and Director of Cynapsus Therapeutics, led a NASDAQ listed specialty pharmaceutical company that developed the first successful sublingual apomorphine thin film strip for Parkinson's disease and led a highly successful exit of Cynapsus by way of a 120% premium to market of an $841 MM all cash M&A deal. Mr. Giovinazzo is currently Executive Chairman of Kalgene, and director of Titan Medical Inc. / 拥有43年工作经验，是国际公认的知识产权、药物开发和商业化专家，包括多项许可协议，在中枢神经系统疾病领域拥有超过25年的经验。他是Cynapsus Therapeutics的联合发明人、首席执行官兼董事，领导一家纳斯达克上市的专业制药公司，该公司成功开发了首款治疗帕金森病的阿扑吗啡舌下膜剂，领导了 Cynapsus 的成功退出，以 120% 的市场溢价完成了 8.41 亿美元的全现金并购交易，现任Kalgene执行主席和Titan Medical Inc.董事。

Dr. Allen W. Davidoff Ph.D. – President and CEO / Allen W. Davidoff博士、博士 – 总裁兼首席执行官

Over 20 years drug development experience) is the founder and CEO of XORTX. Allen has a broad range of clinical and regulatory experience and senior management experience in pharmaceutical R&D including four investigational new drug ("IND") applications or supplemental IND's, two phase I studies (four of which were multi-country), eight phase II studies, and one NDA. Prior to co-founding XORTX, Allen was the Chief Scientific Officer, VP Product Development and co-founder of Stem Cell Therapeutics Corp. (seven years) Trillium TRIL:NASDAQ acquired by Pfizer and Senior Scientist and Head of Pharmacology at Cardiome Pharma Corp. / 拥有超过20年的药物开发经验，是XORTX的创始人兼首席执行官。Allen在药品研发方面拥有广泛的临床和监管经验以及高级管理经验，包括四项新药临床研究("IND")申请或补充IND、两项I期研究（其中四项是多国研究）、八项II期研究，以及一份保密协议。在共同创立XORTX之前，Allen曾担任辉瑞收购的Trillium （TRIL:NASDAQ）（前称Stem Cell Therapeutics Corp.）的首席科学官、产品开发副总裁和联合创始人长达七年、以及Cardiome Pharma Corp.的高级科学家和药理学主管。

Dr. Raymond Pratt / Raymond Pratt博士

Raymond Pratt is an accomplished Physician Executive in clinical medicine, Nephrology, drug development, and the pharmaceutical industry. He has extensive experience troubleshooting issues concerning regulatory approval of drugs and devices and providing development strategies for global pharmaceutical companies. / RaymondPratt是临床医学、肾脏病学、药物开发和制药行业中的一位卓有成就的医生。在解决有关药物和器械监管审批的问题以及为全球制药公司提供开发策略方面拥有丰富的经验。

Paul Van Damme

Held senior positions with a number of Canadian and US public companies. His experience focused on the biotech industry in Toronto when he joined GlycoDesign, a private biotech company. While at Allelix Pharmaceuticals Inc., he participated in the sale of that company to NPS Pharmaceuticals, Inc. / 曾在多家加拿大和美国上市公司担任高级职务。当他加入 GlycoDesign（一家私营生物科技公司）时，他的经验主要集中在多伦多的生物科技行业。 在 Allelix Pharmaceuticals Inc. 任职期间，他参与了该公司出售给 NPS Pharmaceuticals, Inc.。

William Farley

Over 35 years experience in the Business Development, Sales and leading efforts in drug discovery, development and partnering. Prior to joining the board of directors of XORTX, Mr. Farley held a senior leadership position at Sorrento Therapeutics. Bill, began his career at Johnson and Johnson and has held sr. mgmt. positions at Pfizer and HitGen Ltd., V. P., WuXi Apptec, Inc., V.P., Business Development at ChemDiv. / 在业务开发、销售以及药物发现、开发和合作方面拥有超过35年的领导经验。在加入XORTX董事会之前，Farley先生在Sorrento Therapeutics担任高级领导职务。他的职业生涯始于强生公司，并曾在辉瑞和成都先导制药有限公司担任高级管理职务、在药明康德担任副总裁、在ChemDiv担任业务开发副总裁。

Ian Klassen

Brings almost 30 years of business management, public relations and government affairs experience. He previously served as Chief of Staff to the Canadian Speaker of the House of Commons. Ian is the recipient of the Commemorative Medal for the 125th Anniversary of the Confederation of Canada in recognition of his significant contribution to his community and country. / 拥有近30年的企业管理、公共关系和政府事务经验。此前曾担任加拿大国会下议院议员办公室主任。荣获加拿大联邦125周年纪念奖章，以表彰他对社区和国家的重大贡献。

Investment Highlights / 投资亮点

- **Developing drug-based therapies for serious progressive kidney diseases** with a high unmet medical need, including Autosomal Dominant Polycystic Kidney Disease (ADPKD), Diabetes Type-2 Nephropathy (T2DN) and Acute Kidney Injury (AKI). / **开发**医疗需求未得到满足的**严重进行性肾脏疾病的药物疗法**，包括常染色体显性多囊肾病（ADPKD）、2 型糖尿病肾病 (T2DN) 和急性肾脏受伤（AKI）。

- **Three patent families derived from our proprietary pipeline-in-a-product technology** with broad therapeutic claims. Multiple third-party studies with well-understood Oxypurinol have characterized the mechanism of action (MoA) in over 700 patients treated, including multiple Ph2 studies. Patent protection until 2034. / **三个专利系列源自我们专有的产品管线技术**，具有广泛的治疗功效。 多项针对 700 多名治疗患者的众所周知的奥昔嘌醇第三方研究已描述了其作用机制 (MoA)，其中包括多项二期研究。 专利保护期至 2034 年。

- **Clear focus on XRx-008 program for ADPKD with a $1.0-1.8 Billion USD per year revenue potential for XRTX in the US alone:** strong unmet medical need; only one drug approved subject to a black box warning with market exclusivity expiring in 2025; Orphan Drug Designated granted by FDA; path to accelerated approval confirmed; potential for early revenue in 2026/7 to replace Tolvaptan by Otsuka / **明确专注于ADPKD 的 XRx-008 项目，仅在美国，XRTX 每年就有 10亿-18 亿美元的收入潜力**：强烈的未满足医疗需求； 只有一种药物获批但有黑框警告，市场独占权将于 2025 年到期； FDA授予孤儿药认定； 加速审批路径得到确认； 有可能在2026/7 年提前获得收入，取代大冢制药的托伐普坦。

- **Short-term catalyst for the share price:** signing of a global licensing deal in 2023/2024 may provide non-dilutive funding before start of a Ph3 pivotal registration clinical trial with approximately 200 patients / **股价的短期催化剂**：2023/2024 年签署的全球许可协议可能会在约 200 名患者的 3 期关键注册临床试验之前提供非稀释性资金

- **Strong cash position** of $6 Million USD vs. market cap of $12 Million USD - runway for 16 months / **现金状况强劲**，达$600 万美元，而市值为 $1200 万美元， 可持续运营16 个月

- **Senior team led by CEO Allen W. Davidoff, Ph.D was responsible for Oxypurinol development** in prior ventures and knows how to build shareholder value (Cynapsus Therapeutics was acquired for $624 Million USD and Trillium Therapeutics was bought for $2.2 Billion USD by Pfizer). / **由首席执行官Allen W. Davidoff 博士领导的高管团队在之前的企业中负责奥昔嘌醇的开发**并且深谙如何为股东创造价值（Cynapsus Therapeutics 被辉瑞以6.24 亿美元收购 ， Trillium Therapeutics 被辉瑞以22 亿美元收购 ）。

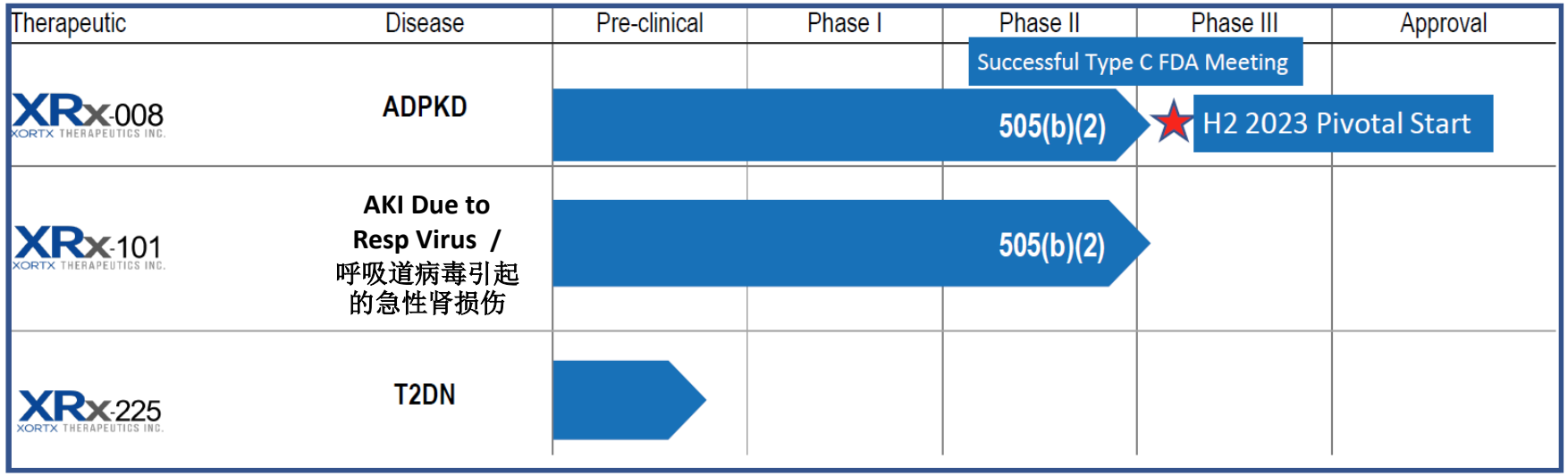
First-in-class Product Candidate Ready for Ph3 Clinical Trials of Xanthine Oxidase Inhibitor for ADPKD / 首创的候选产品，已准备好进行黄嘌呤氧化酶抑制剂治疗 ADPKD 的 3 期临床试验

XORTX Therapeutics, Inc.

XORTX Therapeutics is a publicly traded company (NASDAQ: XRTX) with a proprietary Xanthine Oxidase (XO) inhibitor pipeline targeting serious progressive kidney diseases via reduction of uric acid levels

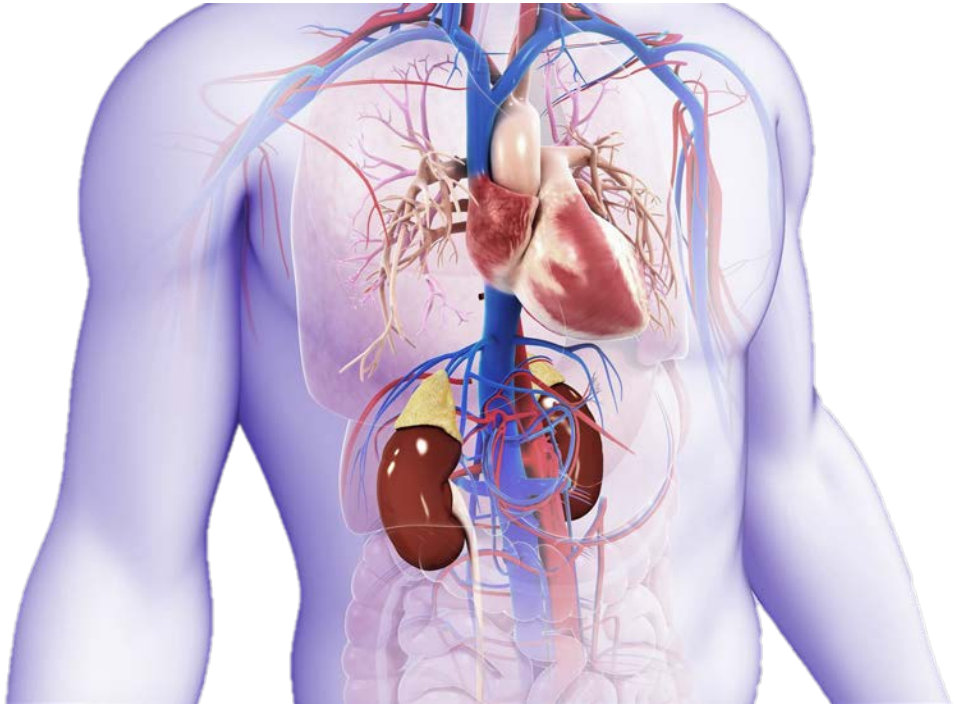
XORTX's pipeline targets areas of high unmet medical need, including Autosomal Dominant Polycystic Kidney Disease (ADPKD), Acute Kidney Injury (AKI) due to COVID-19, and Type 2 Diabetic Nephropathy (T2DN).

End-stage Renal Disease (ESRD) market valued at **$75B globally** in 2020 with a **CAGR of 13%** from 2021-2028[1]



Management team was responsible for Oxypurinol development in prior ventures and company has engaged leading renal key opinion leaders on Clinical Advisory Board (see Appendix)

Common ADPKD Progression & Symptoms /
常见的ADPKD 进展和症状



Pain and Discomfort / 疼痛和不适
Cysts put pressure on abdomen and imping of organs /
囊肿压迫腹部并撞击器官

Liver cysts (40%); / 肝囊肿（40%）；
10X increase in Neurologic Anurisms; / 神经性无尿症增加 10 倍；
Increased Cardiovascular disease / 心血管疾病增加

Hypertension – High Blood Pressure / 高血压 – 高血压

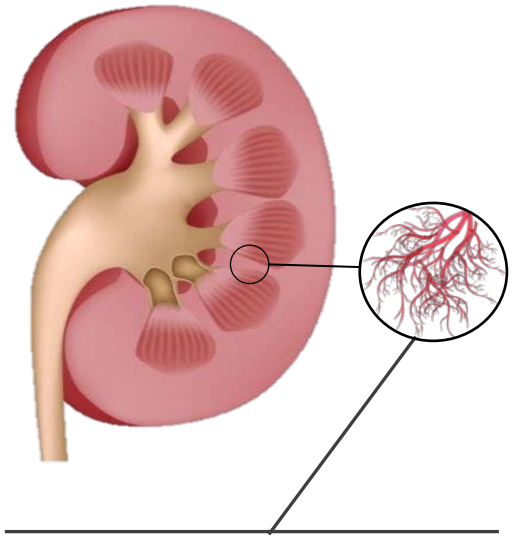
Kidney Stones / 肾结石

Declining Renal Function "GFR" → End Stage Renal Disease (ESRD) /
肾功能"肾小球滤过率"下降→ 终末期肾脏病（ESRD）

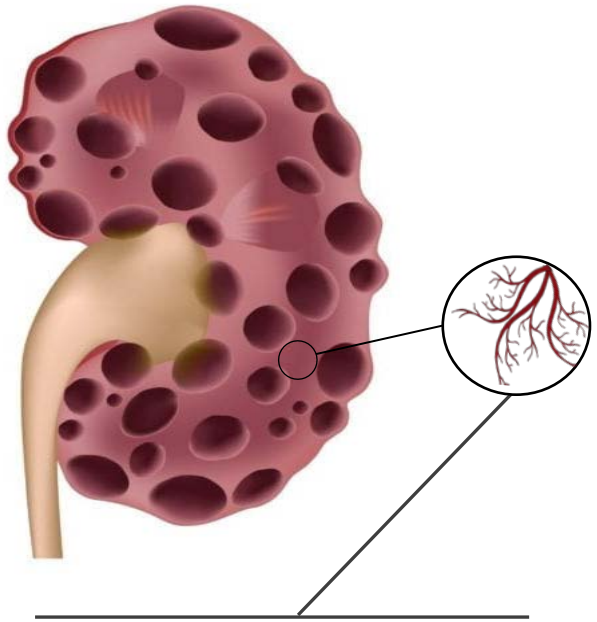
ADPKD's Disease Progression Driven by Growth of Renal Cysts, Tissue Uric Acid and Loss of Glomeruli / 肾囊肿生长、组织尿酸和肾小球损失导致 ADPKD 疾病进展

Insoluble XO and UA effects - Mechanism of Injury 2 of 2 / 不溶性黄嘌呤氧化酶和尿酸反应 - 损伤机制 2/2

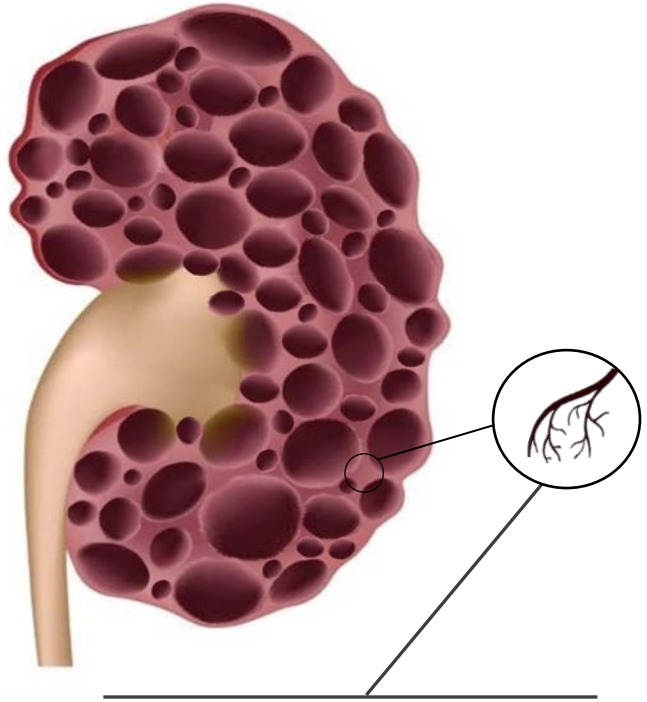
NORMAL / 正常 → IMPAIRMENT / 损害 → FAILURE → DIALYSIS or Transplant / 失败→透析或移植



Healthy Kidney / 健康肾脏
Capillaries: Normal
Kidney Size/Volume: Normal - 100%
Cysts: Minimal to None
毛细血管：正常
肾脏大小/体积：正常 - 100%
囊肿：最小至无



Impaired Kidney / 受损肾脏
Capillaries: Constricting and Shortening
Kidney Size/Volume: Enlarging - 150%
Cysts: Increasing
毛细血管：收缩和缩短
肾脏大小/体积：增大 - 150%
囊肿：增加



Kidney Failure / 肾功能衰竭
Capillaries: Constricted and Shortened
Kidney Size/Volume: Engrossed - 200%
Cysts: Covering the Entire Kidney
毛细血管：收缩和缩短
肾脏大小/体积：占据 - 200%
囊肿：覆盖整个肾脏

XORTX THERAPEUTICS INC.

XORTX Technology: Xanthine Oxidase Inhibitor for Aberrant Purine Metabolism and Increased Uric Acid / XORTX 技术：黄嘌呤氧化酶抑制剂，可抑制嘌呤代谢异常和尿酸升高

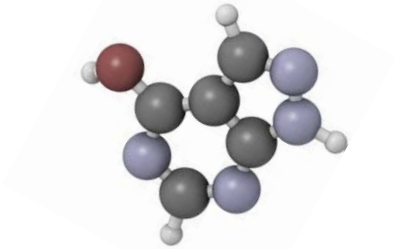
Platform Technology / 平台技术

Proprietary pipeline-in-a product technology / 专有的产品管线技术

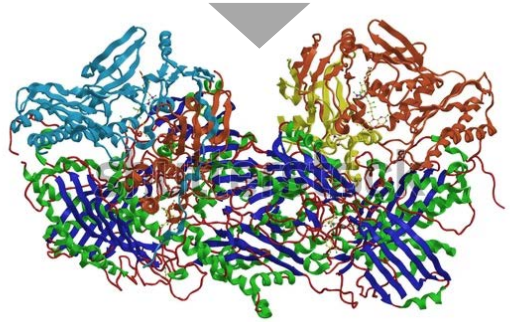
New Oral Rx / 新口服处方

Proprietary and highly scalable CMC approach / 专有且高度可扩展的 CMC 方法

Oxypurinol / 奥昔嘌醇



>90% Inhibition of Xanthine Oxidase / >90% 黄嘌呤氧化酶抑制



Xanthine Oxidase / 黄嘌呤氧化酶

Clinical Data / 临床数据

Robust data supporting therapeutic potential of approach / 支持该方法治疗潜力的强有力数据

Clinical Trials / 临床试验

Ready to start Phase III Pivotal Trial in 2023/24 – Accelerated Approval Confirmed by FDA / 准备于 2023/24 年启动三期关键试验——FDA确认加速批准

XORTX THERAPEUTICS INC.

Designed to Slow the Decline in Renal Function
旨在减缓肾功能衰退

XRx-008
XORTX THERAPEUTICS INC.

Product Candidate Summary / 候选产品摘要

- XORLO™ - Novel, proprietary, well tolerated oral formulation of Oxypurinol. / XORLO™ - 新颖、专有、耐受性良好的奥昔嘌醇口服制剂。
- Oxypurinol is a past recipient of New Drug Application (NDA) Approvable Letter – greater than 750 patients clinical experience. / 奥昔嘌醇曾获得新药申请 (NDA) 批准函—— 超过 750 名患者的临床经验。

Differentiation / 与众不同之处

- Oxypurinol is minimally metabolized and excreted unchanged. Few Liver Toxicity → better compliance on drug. / 奥昔嘌醇被最低程度地代谢，排泄物未发生变化。 对肝脏毒性更小→更好的药物依从性。
- Combined extracellular and intracellular action of XRx-008 is fundamental. / XRx-008 的细胞外和细胞内联合作用至关重要。
- Potential to avoid toxicity problems associated with tolvaptan, the only approved drug for ADPKD. / 可以避免托伐普坦（唯一批准用于治疗 ADPKD 的药物）相关的毒性问题。
- Potential to modify underlying disease pathology supported by third-party phase 1 and 2 clinical trials in over 750 patients with no reported serious adverse events unique to oxypurinol. / 第三方 1 期和 2 期临床试验支持改变潜在疾病病理学的潜力，该试验对超过 750 名患者进行，未报告奥昔嘌呤特有的严重不良事件。
- Next Milestone complete GMP manufacturing of DP and API, start Ph3 in 2023/24 – subject to FDA approval. / 下一个里程碑：完成制剂和活性药物成分的 GMP 生产，并于 2023/24 年启动三期——须经 FDA 批准。

Designed to Slow the Decline in Renal Function
旨在减缓肾功能衰退

XRx-008
XORTX THERAPEUTICS INC.

- XRx-008 in progressive kidney disease designed to decrease uric acid & attenuate loss of filtering capacity of kidneys. / XRx-008 用于治疗进行性肾脏疾病，旨在降低尿酸并减轻肾脏过滤能力的丧失。
- A potential therapeutic option to maintain and extend kidney health can redefine kidney disease treatment in the future. / 维持和延长肾脏健康的潜在治疗选择，可以重新定义未来肾脏疾病的治疗。
- JYNARQUE® effect focuses on minimizing cyst/kidney volume. The FDA approvable endpoint is based upon slowing loss of filtering capacity of kidneys. / JYNARQUE® 效果侧重于最大限度地减少囊肿/肾脏体积。FDA 批准的终点是基于减缓肾脏过滤能力的丧失。

ASN American Society of Nephrology

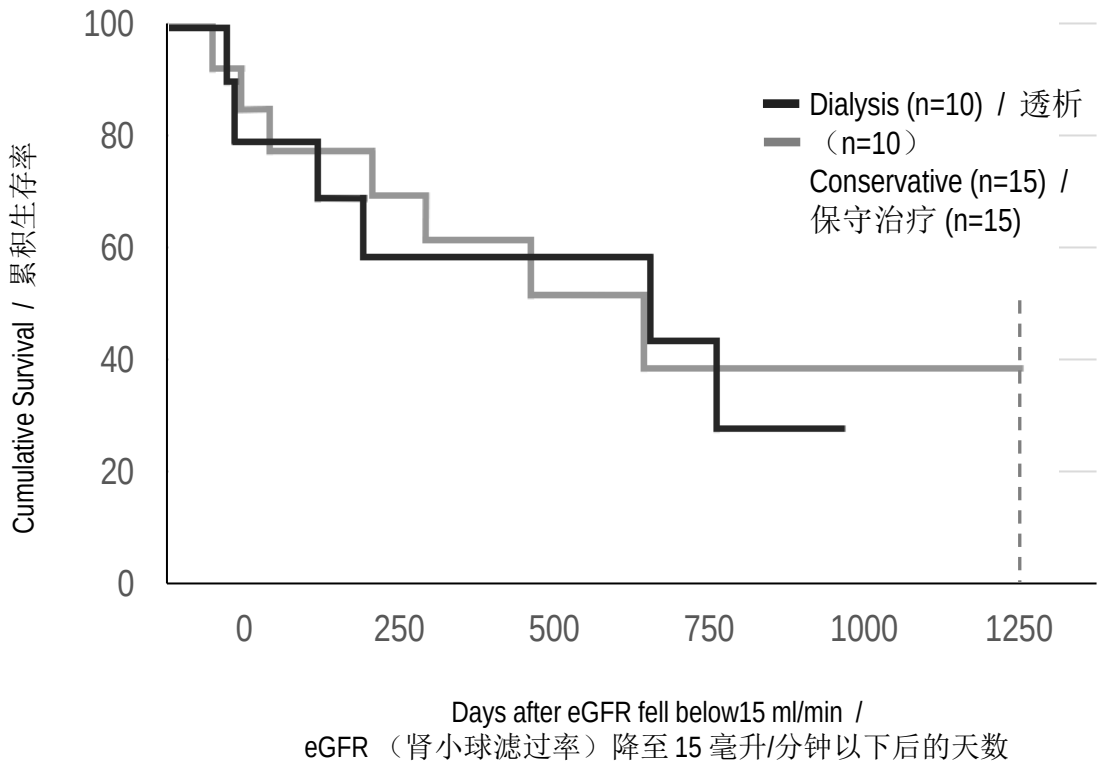
End-Stage Renal Disease (ESRD) A Life Altering Event / 终末期肾脏病 (ESRD) 改变生活的事件

- 4 hour dialysis 3 times per week / 每周透析3次，每次4小时
- Loss of ability to work full time / 失去全职工作的能力
- Dependence on family / 依赖家庭
- Pain and declining health are constant burden / 疼痛和健康状况恶化是持续的负担
- Shortened survival – only 50% of patients survive two years / 生存期缩短——只有 50% 的患者能生存两年

Onset Delay of ESRD May Improve Quality of Life and Longevity

/ 延迟终末期肾病（ESRD）的发病可能会提高生活质量和寿命

Source: Curr Opin Nephrol Hypertens – 22(2): 185-192, 2013 / 资料来源： Curr Opin Nephrol Hypertens – 22(2): 185-192, 2013



US FDA Orphan Drug Status & Benefits / 美国 FDA 孤儿药资格和福利

Orphan Drug Grant: Key Criteria for Approval & Validation / 孤儿药补助：批准和验证的关键标准

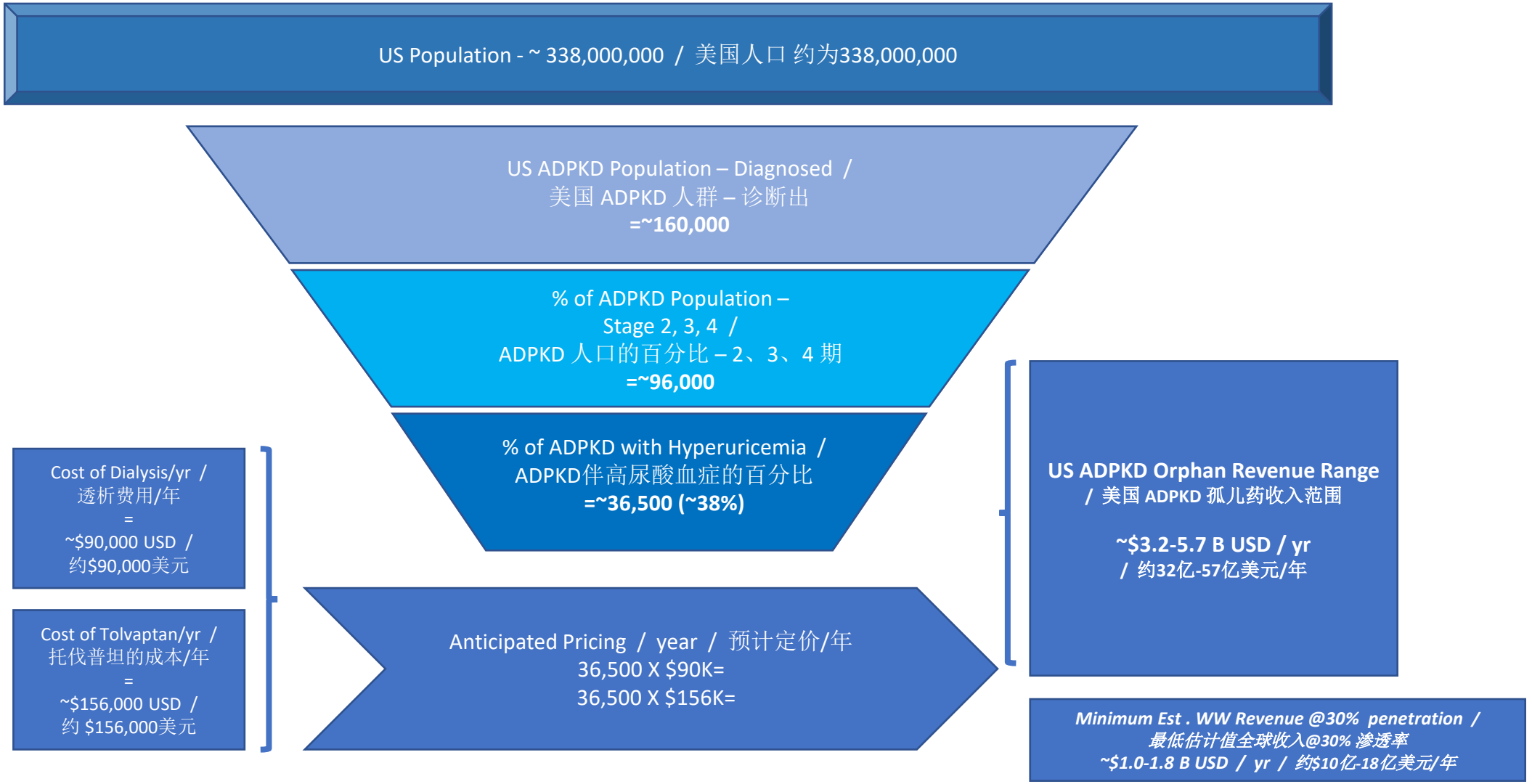
	Demonstrated / 已证实
I. **Mechanism of Injury (MOI)** exists in models of Autosomal Dominant Polycystic kidney disease. / **损伤机制（MOI）**存在于常染色体显性多囊肾病模型中。	
a) Xanthine Oxidase enzyme expression in Kidney / 肾脏中黄嘌呤氧化酶的表达	✓ Demonstrated – Kidney Over Expression of XO / 已证实 – 肾脏过度表达黄嘌呤氧化酶
b) Hyperuricemia accelerates disease progression / 高尿酸血症加速疾病进展	✓ Demonstrated – Hyperuricemia Accelerates TKV & GFR decline / 已证实 – 高尿酸血症加速 TKV 和肾小球滤过率下降
II. The identical drug formulation inhibits the MOI in a substantial and statistically significant manner. / 相同的药物制剂对损伤机制有显著的抑制作用，并有统计学上的意义。	✓ Demonstrated – XOI attenuates both TKV and GFR progression. / 已证实 – 黄嘌呤氧化酶抑制剂可减弱TKV和肾小球滤过率的进展。

Benefits of Orphan Drug Designation / 孤儿药资格的好处

- Seven years market exclusivity post-approval + premium pricing / 批准后七年市场独占权+溢价定价
- Tax credits of 25% off the clinical drug testing cost awarded upon approval / 经批准可享受临床药物测试费用 25% 的税收抵免
- Waiver of FDA User Fees / 免除 FDA 用户费用
- Clinical development grants / 临床开发补助金

XORTX THERAPEUTICS INC.

ADPKD → ESTIMATED ADDRESSABLE MARKET IN THE US / ADPKD在美国的预计潜在市场



ADPKD – Only One Therapy is Approved While Suboptimal Treatment Options Remain / ADPKD – 只有一种疗法获得批准，但治疗方案仍然不理想

- 160,000 patients diagnosed with ADPKD in the US / 美国诊断出 160,000 名 ADPKD 患者 (1)
- ADPKD is the largest kidney disease market with a genetic origin / ADPKD 是最大的遗传性肾脏疾病市场
- A majority of ADPKD patients require dialysis or kidney transplantation / 大多数 ADPKD 患者需要透析或肾移植
- Otsuka's JYNARQUE® (tolvaptan) was approved in 2018 for the treatment of ADPKD with black box warning – "for risk of serious liver injury". / 大冢制药的 JYNARQUE®（托伐普坦）于 2018 年被批准用于治疗 ADPKD，并带有黑框警告——"存在严重肝损伤的风险"。
- Annual Treatment Cost of JYNARQUE® (tolvaptan) ~156,000 USD. Otsuka reported 2022 sales of tolvaptan of $962 m USD. More than ~7,000 ADPKD patients have been treated with tolvaptan / JYNARQUE®（托伐普坦）的年度治疗费用约为 $156,000 美元。大冢制药报告称，2022 年托伐普坦销售额为 9.62亿美元。超过7000 名 ADPKD 患者已接受托伐普坦治疗(2)
- 95% of ADPKD patients can't take or tolerate JYNARQUE® (tolvaptan) / 95% 的 ADPKD 患者无法服用或耐受 JYNARQUE®（托伐普坦） (3)

Sources: /资料来源： (1) Vicente E. Torres March 2021 AJKD, (2) A.G.P./Alliance Global Partners Research, (3) Wim Van Biesen, Jan 2019, NDT

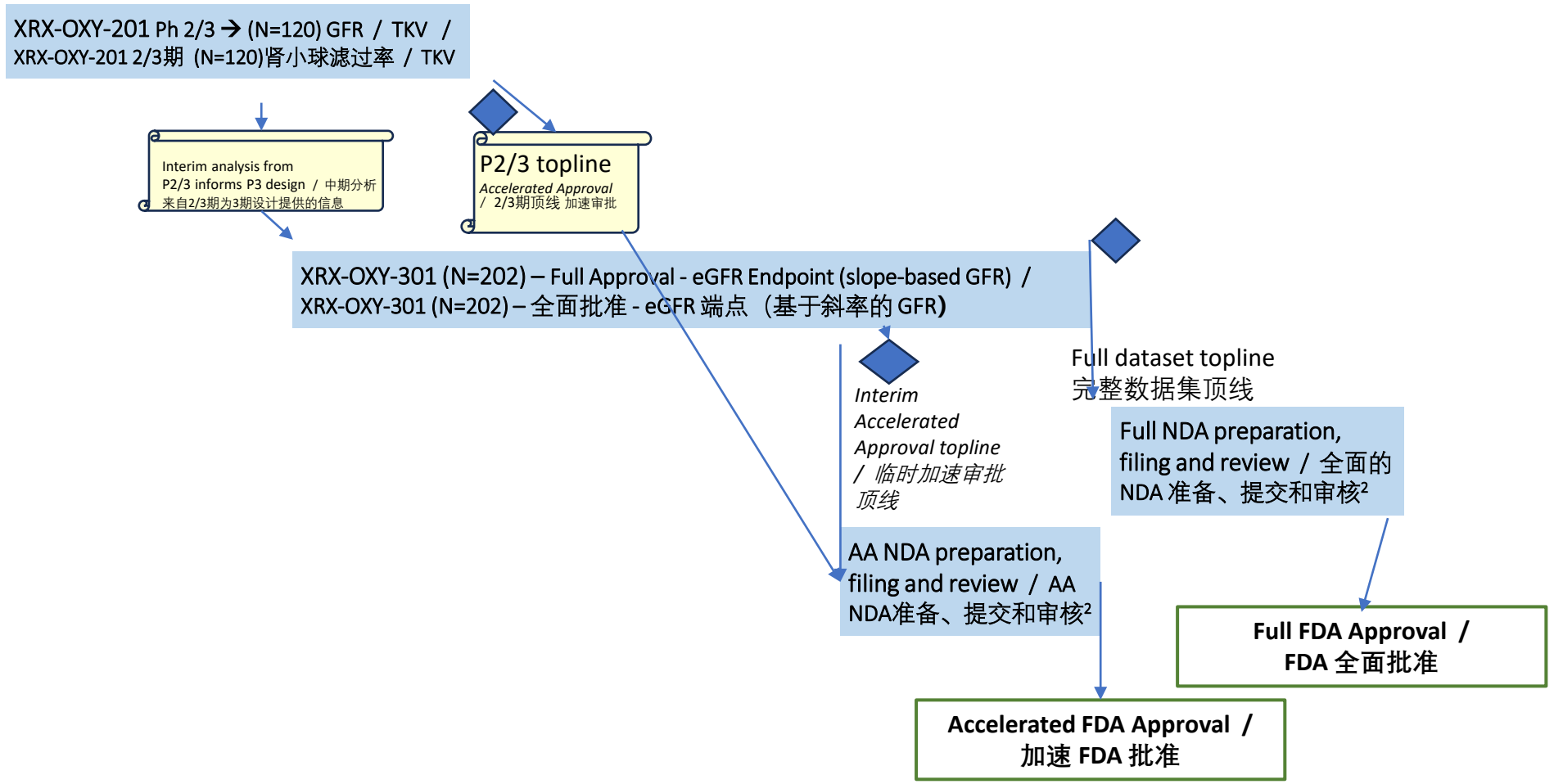
Competitive Landscape / 竞争格局

DRUG / 药品	STATUS / 状况
Tolvaptan / 托伐普坦 (oral vasopressin V2 antagonist) / （口服加压素V2拮抗剂） Otsuka Pharma /大冢制药	**FDA-APPROVED / FDA 批准** Black Box Warning – Liver toxicity / 黑框警告 – 肝脏毒性 Extremely low usage du to tolerability / 由于耐受性，使用率极低
Lixivaptan / 利昔普坦 (oral vasopressinV2 antagonist) / （口服加压素V2拮抗剂） Centessa Pharma	**ABANDONED / 放弃** Phase III / 三期
Bardoxolone (oral Nrf2 activator) / Bardoxolone （口服 Nrf2 激活剂） Reata Pharmaceuticals Inc.	**ABANDONED / 放弃** Phase III / 三期
GLPG2737 (a Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) inhibitor) / （囊性纤维化跨膜电导调节因子（CFTR）抑制剂） Galapagos	Phase II / 二期
XRx-008 XORTX THERAPEUTICS INC. (xanthine oxidase inhibitor) / （黄嘌呤氧化酶抑制剂） XORTX Therapeutics Inc.	**IN DEVELOPMENT / 开发中** Preparing to Enter Phase III / 准备进入三期

XRx-008 - Clinical Development Plan / XRx-008 - 临床开发计划

A single, 2-part pivotal trial strategy allows for staging of investment and early accelerated approval / 单一的、由两部分组成的关键试验策略，可分期投资和早期加速批准

2023				2024				2025				2026				2027				2028			
Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4



Milestone Timeline *(Milestones in Black Text are Anticipated / Planned)* / 里程碑时间表（*黑色文本中的是预期/计划中的里程碑*）

XRx-008
XORTX THERAPEUTICS INC.

	2023 H1	2023 Q3	Q4			2024 Q1			Q2		
CMC – Mfg CMC – 制造 Drug Substance & Drug Product 原料药及药品			Clinical Trial Drug Supply mfg / 临床试验药物供应制造 →						Commercial Drug Supply & Mfg / 商业药品供应和制造 →		
Clinical / 临床	XRx-OXY-101 - +ve topline reported / XRx-OXY-101 - + 已报告顶线										
FDA & EMA Regulatory / FDA & EMA监管	US FDA + ODD grant + Type D – Accel Approval / 美国FDA + 获得孤儿药认定+ D型 – 加速批准	EMA- ODD application / 欧洲药品管理局-孤儿药申请	XRX-OXY-201 - *FDA submission* - / *XRX-OXY-201 - FDA 提交* -	XRX-OXY-301 Special Protocol Assessment (SPA) / XRX-OXY-301特殊方案评估（SPA）		XRX-OXY-301 – FDA submit / XRX-OXY-301 – FDA 提交					
XRx-008 ADPKD		XRX-OXY-201 protocol finalization / XRX-OXY-201 方案最终确定	XRX-OXY-301 protocol finalization / XRX-OXY-301 方案最终确定				XRX-OXY-201 - 1st Patient / XRX-OXY-201- 第 1 位患者	Ph3 Site Initiation / 三期现场启动	US FDA & EMA Protocol reviewed / 评估美国 FDA 和 欧洲药品管理局的方案		XRx-OXY-301 – 1st Patient XRx-OXY-301- 第 1 位 患者
XRx-225 T2DN		XO expression Studies in ADPKD / ADPKD 黄嘌呤氧化酶表达研究	T2DN (NCE) / 2 型糖尿病肾病(新化学实体) →								
Licensing 许可		Ongoing - - - Global Licensing Discussions / 全球许可讨论进行中									
Conferences 会议		i) Singular Ii) Sidoti iii)Wainwright iv) Rare&Gen Conf	AmSocNephrol / 美国肾脏学会								

Consistent Clinical & Regulatory Progress - *Milestones for Value Creation* / 一致的临床和监管进展 - *创造价值的里程碑*

2023/2024 - continued progress coming over the next 4 quarters / 2023/2024 - 未来 4 个季度持续取得进展

- **Dosing of PK Bridging Clinical Trial – Completed → Topline Results released; / PK桥接临床试验的给药剂量 – 已完成重要结果发布；**
- **Type D meeting with FDA - Accelerated Approval confirmed (May 1, 2023); / 与 FDA 举行的 D 类会议 - 加速批准已确认（2023 年 5 月 1 日）；**
- **Orphan Drug Designation (ODD) – GRANTED (April 20, 2023); / 孤儿药资格 (ODD) 认定– 已批准（2023 年 4 月 20 日）；**
- **European Medicines Agency – Orphan Drug Designation H2 2023 / 欧洲药品管理局 – 2023 年下半年孤儿药认定**
- **Initiation of XRX-OXY-201 –** *near term readout Interim end of 2024 / topline 2025 / 启动 XRX-OXY-201 – 2024 年中期的近期读数 / 2025 年顶线*
- **Initiation of XRX-OXY-301 Registration Trial for XRx-008 in individuals with ADPKD (accelerated approval); / 在 ADPKD 患者中启动 XRx-008 的 XRX-OXY-301 注册试验（加速批准）；**
- **Special Protocol Assessment ("SPA") - TBD and, / 特别方案评估（"SPA"）- 待定以及**
- **Potential significant global licensing deal with Big Pharma (non-dilutive financing); / 与大型制药公司可能达成重大全球许可协议（非稀释性融资）；**
- **Novel Patent Filings *in preparation* – ADPKD discoveries; Dose ranging; New NCE Drug Candidates / *正在准备*新颖专利申请– ADPKD 发现；剂量范围； 新化学实体（NCE） 候选药物**

Capitalization Table / 资金表

Capitalization as of March 31, 2023 / 截至2023年3 月 31 日的资金

	# of Shares 股份数量	WAEP	C$ Value 加元价值	% of Fully Diluted 完全稀释的百分比
Common Shares Outstanding (Insiders): 已发行普通股（内部人士）：	1,541,987			5.19%
Common Shares Outstanding (Other): 已发行普通股（其他）：	16,481,700			55.34%
Warrants Outstanding: 已发行认股权证：	10,579,796	C$3.38	C$35,787,353	35.59%
Options Outstanding: 已发行期权：	1,154,319	C$2.42	C$2,793,452	3.88%
Fully Diluted Shares : 完全摊薄后股份：	**29,723,802**			**100.00%**

CASH as of 30st June 2023: ~$6.2M USD

截至 2023 年 6 月 30日的现金：约 620 万美元

XORTX THERAPEUTICS INC.

Clinical Advisory Board / 临床顾问委员会

Dr. Charles Edelstein, M.D., Ph.D. / Charles Edelstein博士、医学博士、哲学博士
Professor of Medicine and Nephrologist at the University of Colorado. Dr. Edelstein is board certified in Nephrology and has a doctoral degree (PhD) in Internal Medicine. He did his Internal Medicine residency and Nephrology fellowship at University of Stellenbosch and University Cape Town Medical School, respectively. His academic research focuses on both therapeutic studies in animal models of polycystic kidney disease (PKD) as well as acute kidney injury (AKI) and biomarkers of AKI. Dr. Edelstein is a world leader in PKD research and PKD care and has received an award for the WSCI Outstanding Investigator Award and is a former president of the Western Section of the American Federation of Clinical Research and International Society of nephrology member and American Society of Nephrology Advisory Committee Member. / 科罗拉多大学医学教授及肾脏病专家，获得肾脏病学会认证，并拥有内科医学博士学位。他分别在斯泰伦博斯大学和开普敦大学医学院完成了内科住院医师实习和肾脏病学研究。学术研究重点是多囊肾病(PKD)和急性肾损伤(AKI)动物模型的治疗研究以及AKI和AKI的生物标志物。是世界上PKD研究和PKD护理领域的领军人物，曾获得WSCI杰出研究者奖，并且曾担任美国临床研究联合会西部分会主席、国际肾病学会会员和美国肾病学会顾问委员会成员。

Dr. Petter Bjornstad, M.D. / Petter Bjornstad博士、医学博士
University of Colorado Denver School of Medicine Barbara Davis Center. / 科罗拉多大学丹佛医学院巴巴拉·戴维斯中心

Dr. Richard Johnson, M.D / Richard Johnson博士、医学博士
Professor of Medicine and the Chief of the Renal Division and Hypertension at the University of CO. / 科罗拉多大学医学教授兼肾脏科和高血压科主任

Dr. Anjay Rastogi, M.D., Ph.D. / Anjay Rastogi博士 、医学博士、哲学博士
Professor and Clinical Chief of Nephrology at the David Geffen School of Medicine at UCLA, Los Angeles, CA. / 加利福尼亚州洛杉矶加州大学洛杉矶分校大卫格芬医学院肾脏病学教授兼临床主任。

Dr. Federico Maese, M.D. / Federico Maese博士、医学博士。
Cardiology Specialist in Red Oak, TX. / 德克萨斯州雷德奥克心脏病学专家。

Dr. Henk ter Keurs, M.D. / Henk ter Keurs博士、医学博士
Professor of Cardiac Sciences, Medicine at the University of Calgary. / 卡尔加里大学心脏科学、医学教授。

Investment Highlights / 投资亮点

- **Developing drug-based therapies for serious progressive kidney diseases** with a high unmet medical need, including Autosomal Dominant Polycystic Kidney Disease (ADPKD), Diabetes Type-2 Nephropathy (T2DN) and Acute Kidney Injury (AKI). / **开发**医疗需求未得到满足的**严重进行性肾脏疾病的药物疗法**，包括常染色体显性多囊肾病（ADPKD）、2 型糖尿病肾病 (T2DN) 和急性肾脏受伤（AKI）。

- **Three patent families derived from our proprietary pipeline-in-a-product technology** with broad therapeutic claims. Multiple third-party studies with well-understood Oxypurinol have characterized the mechanism of action (MoA) in over 750 patients treated, including multiple Ph2 studies. Patent protection until 2034. / **三项专利系列源自我们专有的产品管线技术**，具有广泛的治疗功效。 多项针对 750 多名治疗患者的众所周知的奥昔嘌醇第三方研究已描述了其作用机制 (MoA)，其中包括多项二期研究。 专利保护期至 2034 年。

- **Clear focus on XRx-008 program for ADPKD with a $1.0-1.8 Billion USD per year revenue potential for XRTX in the US alone:** strong unmet medical need; only one drug approved subject to a black box warning with market exclusivity expiring in 2025; Orphan Drug Designated granted by FDA; path to accelerated approval confirmed; potential for early revenue in 2026 just in time to replace Tolvaptan by Otsuka. / **明确关注 ADPKD 的 XRx-008 项目，仅在美国，XRTX 每年就有 10亿-18 亿美元的收入潜力：**强烈的未满足医疗需求；只有一种药物获批但有黑框警告，市场独占权将于 2025 年到期； FDA授予孤儿药资格； 加速审批路径得到确认； 有可能在2026年提前获得收入，及时取代大冢制药的托伐普坦。

- **Short-term catalyst for the share price: signing of a global licensing deal in H2/2023 may provide non-dilutive funding before start of a Ph3 pivotal registration clinical trial with approximately 200 patients. / 股价的短期催化剂：2023 年/下半年签署的全球许可协议可能会在约 200 名患者的 3 期关键注册临床试验之前提供非稀释性资金**

- **Strong cash position** of $7.9 Million USD vs. market cap of $12.7 Million USD - runway for 16 months. / **现金状况强劲**，达$790 万美元，而市值为 $1270 万美元， 可持续运营16 个月

- **Senior team led by CEO Allen W. Davidoff, Ph.D was responsible for Oxypurinol development** in prior ventures and knows how to build shareholder value (Cynapsus Therapeutics was acquired for $624 Million USD and Trillium Therapeutics was bought for $2.2 Billion USD by Pfizer). / **由首席执行官Allen W. Davidoff 博士领导的高级团队在之前的企业中负责奥昔嘌醇的开发**并且深谙如何为股东创造价值（Cynapsus Therapeutics 被辉瑞以 $6.24 亿美元收购 ， Trillium Therapeutics 被辉瑞以 $22 亿美元收购 ）。

Contact Us / 联系我们

XORTX Therapeutics Inc.

Redefining Kidney Disease
重新定义肾脏疾病

XRx-008 XORTX THERAPEUTICS INC.

XRx-101 XORTX THERAPEUTICS INC.

XRx-225 XORTX THERAPEUTICS INC.

XORTX THERAPEUTICS INC.

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info@xortx.com
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Nasdaq : XRTX

TMX TORONTO STOCK EXCHANGE TSX VENTURE EXCHANGE :XRTX

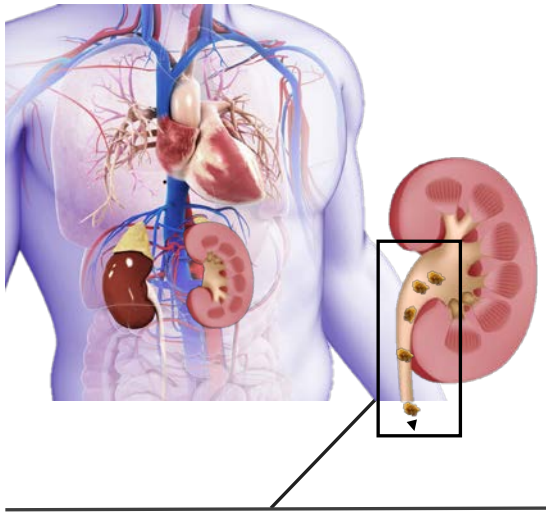
BÖRSE FRANKFURT : ANU

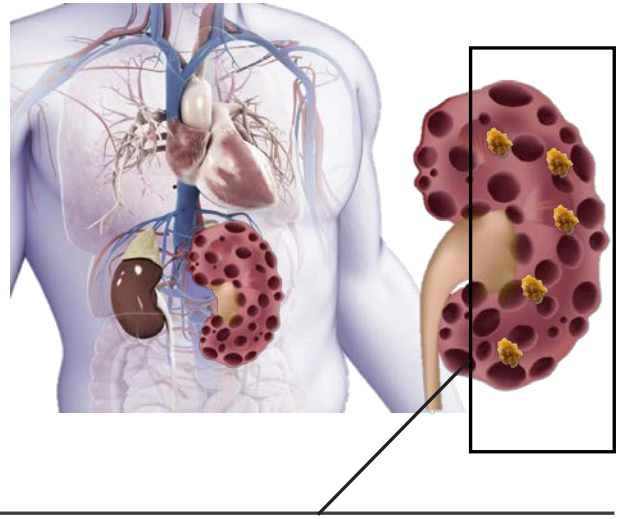
Annex / 附录

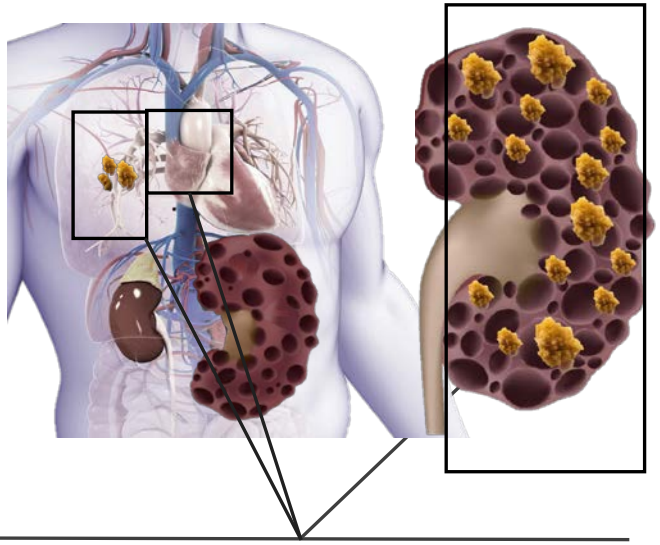
Aberrant Purine Metabolism and Chronically Increased Uric Acid is Associated with Kidney Injury and Failure

Uric Acid Crystals – Mechanism of Injury 1 of 2

NORMAL → IMPAIRMENT → FAILURE → DIALYSIS or Transplant

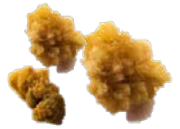






1. Aberrant Purine Metabolism and Dietary Sources of Uric Acid Such As Fructose, Foods Containing Yeast, Oily Fish, Shellfish and Organ meats.

2. Uric Acid Crystals Form in Kidneys. Uric Acid Crystals 2' "seed" Oxalate crystal formation.



3. Acute Kidney Failure is caused by elevated uric acid in plasma and crystal formation in multiple organs including heart, lungs, skin and eyes.

Designed to Slow the Decline in Renal Function

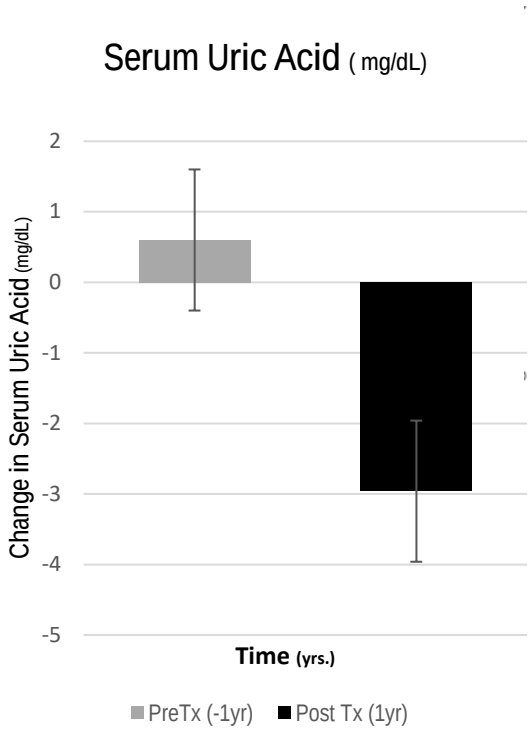
XRx-008
XORTX THERAPEUTICS INC.

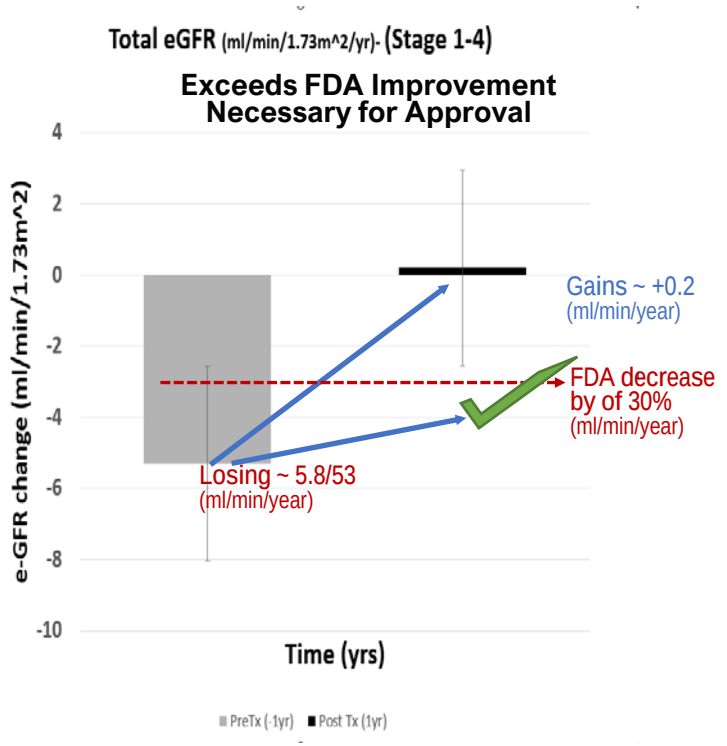
ASN
American Society of Nephrology

- Serum uric acid concentrations are higher in ADPKD patients than in non-ADPKD patients with CKD.
  (Mejias et. Al. Hyperuricemia, Gout, and Autosomal dominant polycystic kidney disease Am. J. Med Sci 1989: 297 145-148)
- Higher SUA is associated with increased kidney volumes, increased End-Stage Renal Disease (ERSD) and ADPKD disease progressions. (Helal, 2013, Han, 2014)
- Increased SUA is strongly associated with endothelial dysfunction via decreased nitric oxide bioavailability. (Khosla, 2005. Zoccali 2006, Kurowska 2002, Portaluppi 2004)
- In early stage ADPKD patients, uric acid levels and eGFR are independent predictors of endothelial dysfunction. (Kocygit et al. Clinical Practice. Nephron Clin Pract 123: 157-164, 2013)

Xanthine Oxidase Inhibition in ADPKD Patients is Associated with a Reversal of Glomerular Filtration Rate Decline

– an FDA approvable endpoint -



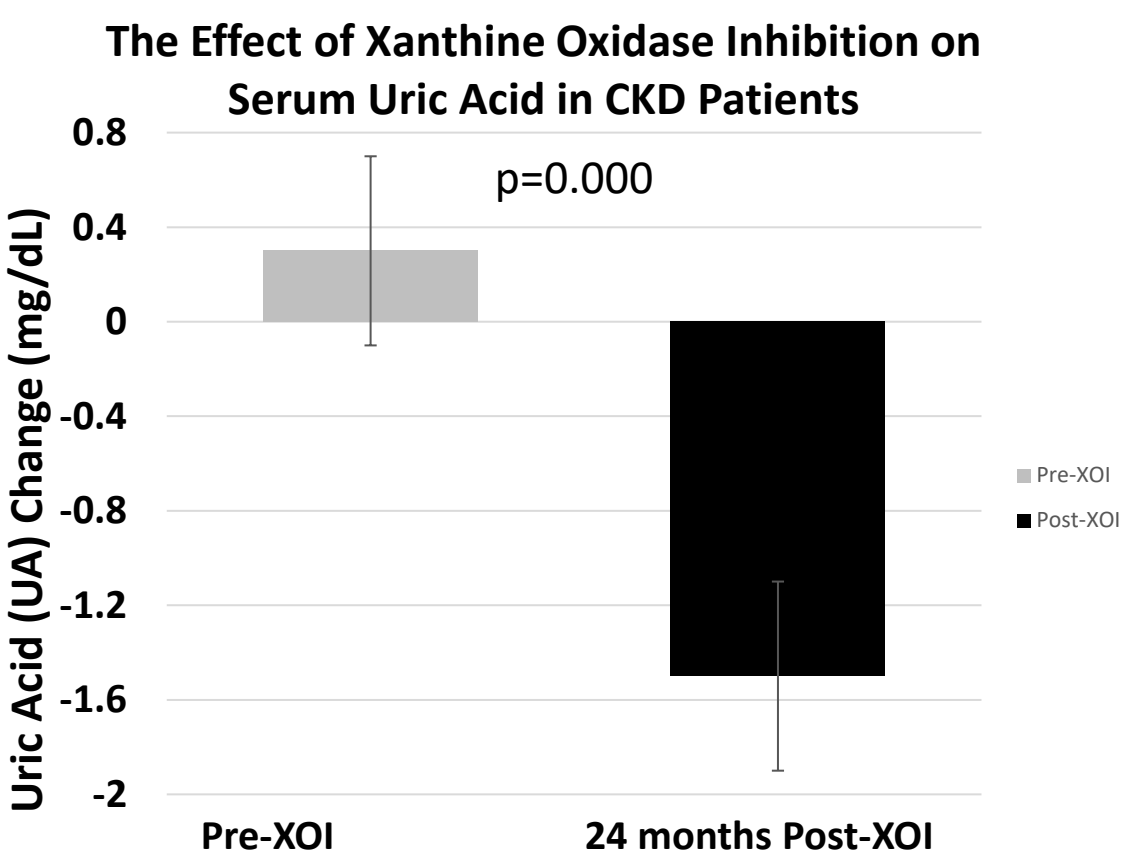


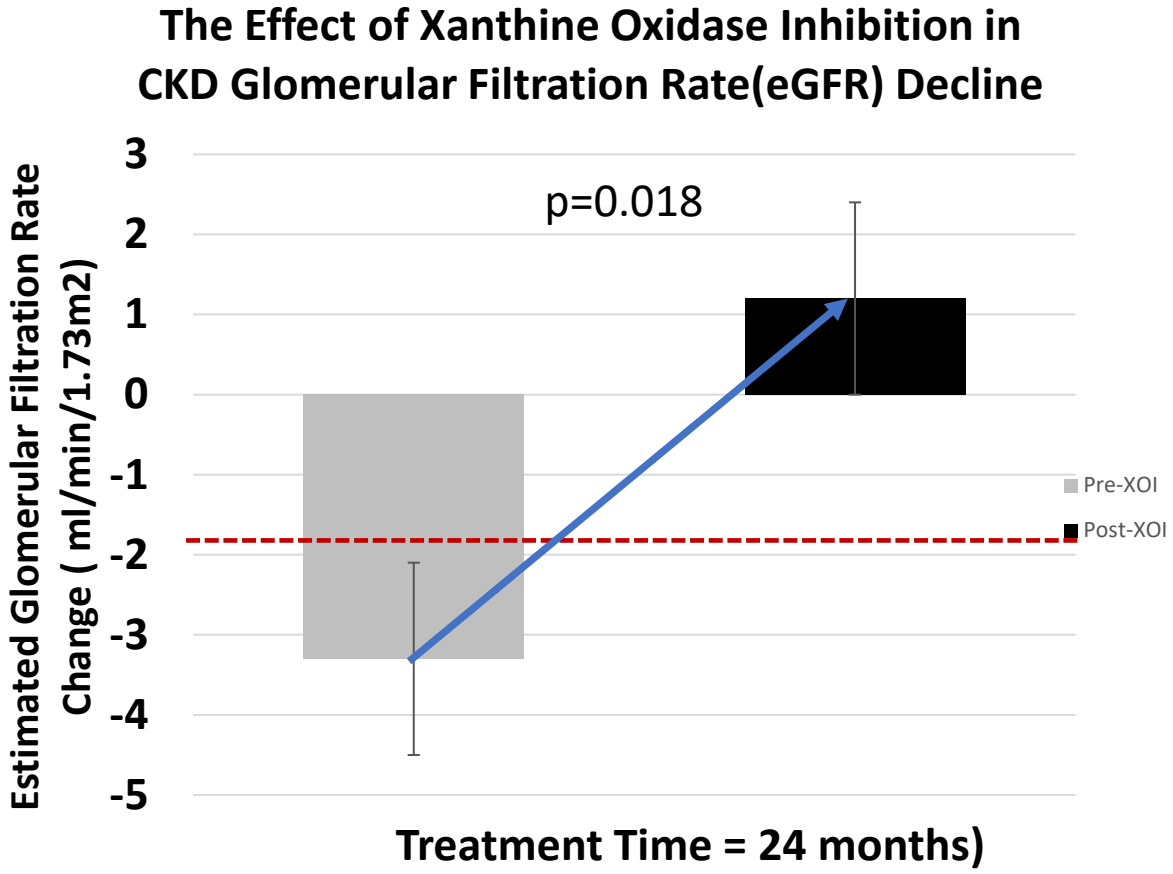
(n=53)

(Han et al, 2014)

The Effect of Xanthine Oxidase Inhibition On Chronic KD Progression

Effect of 2 years XOI treatment, 100 mg daily *(n=113:56;57)*





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Goicoechea, et al, 2010