Eloxx PHARMACEUTICALS

A precision approach to rare kidney disease

Small molecule based genetic therapy

Corporate Presentation | October 2026



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Company highlights

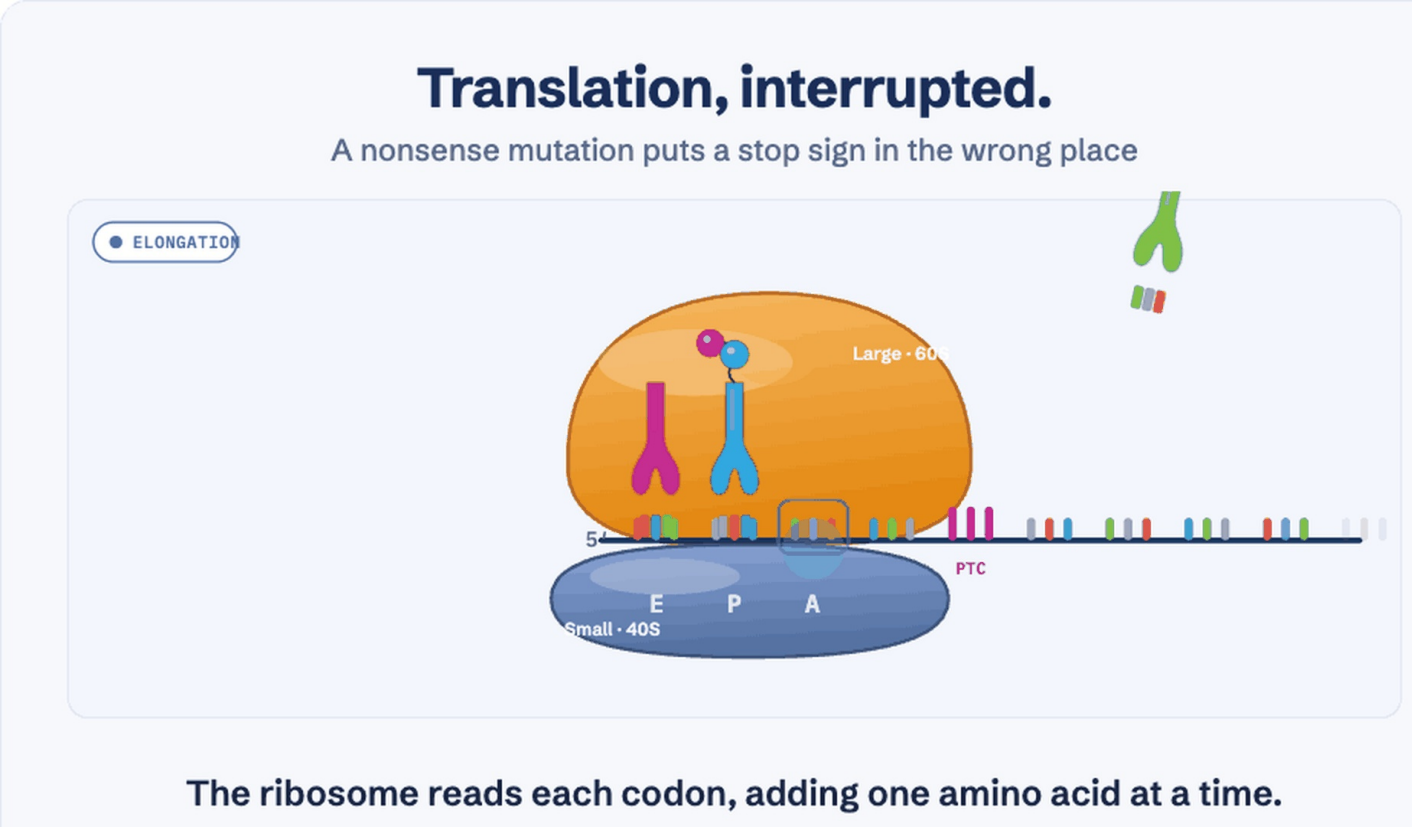
Disease-modifying potential across genetic renal diseases
Exaluren reads through premature termination codons to restore full-length protein

Phase 2 data in **Alport** (NMAS) demonstrated **structural improvement**
Phase 2b ongoing, with 16-week topline data expected in mid-2027

ADPKD provides the larger opportunity
IND allowed for 52-week EXPRESS Phase 2; topline expected 1H 2029

June 2026: **$66M public offering** and Nasdaq uplisting

Exaluren: novel synthetic aminoglycoside restoring full-length protein in nonsense mutations by PTC readthrough



Up to 9-fold greater gene-agnostic readthrough than gentamicin*

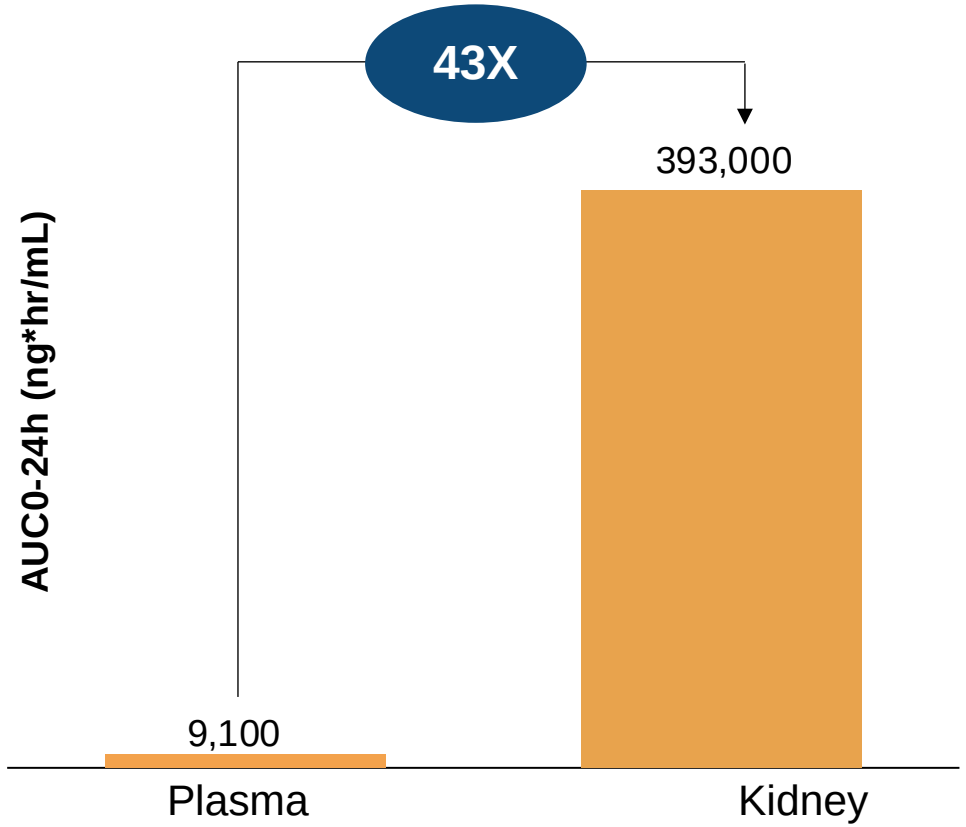
No evidence of readthrough of native stop codon (NSC)

Partially functional full-length proteins are expected to be clinically meaningful

*In reporter assays with a PTC

Exaluren naturally accumulates in kidney

Estimated human exposure in plasma and kidney at 0.75 mg/kg



- Exaluren, like other aminoglycosides, binds to the LRP2 protein megalin
- Megalin is found in the glomerulus and proximal tubules in the kidney, the inner ear and the eye

Estimation based on allometric scaling of mouse, rat and dog plasma and kidney data plus plasma PK from SAD and MAD studies

Eight weeks of exaluren restored collagen IV and improved kidney structure in all three patients

FIRST-IN-HUMAN PROOF OF CONCEPT · n=3

Phase 2a (EL-014): 3 UK patients with COL4A4 nonsense mutation · 8 weeks of exaluren 0.75 mg/kg SC daily · open-label · 12 weeks follow-up

1. Structure improved

Podocyte and GBM morphology improved

Blinded assessment showed reduced foot process effacement and improved GBM width variability

2. Protein restored

+72% collagen α3(IV)/α4(IV) restored

Collagen IV trimer precursor formed

3. Well tolerated

Mild AEs; no kidney or hearing signal

High UPCR variability; stable blood pressure

3 / 3	+72%	0
patients improved kidney structure	mean collagen α3/α4 (IV)	kidney or hearing AEs

EL-014: NCT05448755

Extensive safety database supports development

Exaluren clinical and nonclinical data: no dose-limiting toxicities and no serious AEs were attributed to exaluren

145

subjects treated with exaluren

— 89.4 subject-months of clinical exposure

— No changes in renal biomarkers (KIM-1, clusterin)

No detected

readthrough of native stop codons

— Native termination accuracy >99%, set by stop-codon context

— No aberrant proteins in Phase 1; normal VEGF-A unchanged

~10×

exposure margin at target dose of 0.75 mg/kg

— Chronic tox NOAEL margins: rat 26-week, dog 39-week

— ~52× margin in 4-week rat ototoxicity studies

Crawford et al., JPET 2020; Leubitz et al., Clin Pharmacol Drug Dev 2019; internal Eloxx data

Two Phase 2 programs with data readouts in 2027 and 2029

Alport Syndrome: NMAS

- Phase 2b ongoing. First patient 3Q 2026
- Primary Endpoint: Change in FSD in kidney biopsy
- Readout: Mid-2027
- Prevalence: ~7% of AS (~4,200 in US)

Alport Orphan Drug Designation: US, EMA

NM-ADPKD

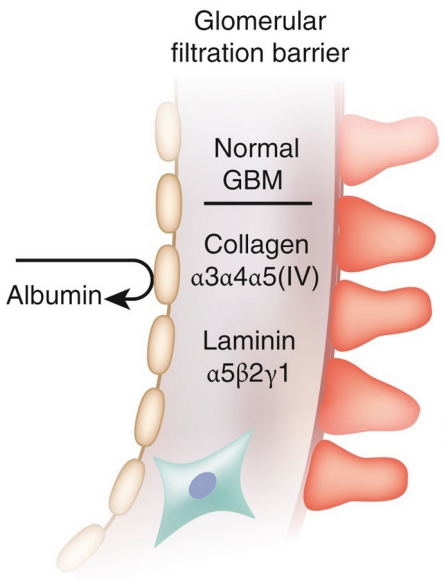
- Phase 2 IND allowed. First patient 3Q 2027
- Primary Endpoint: Change in htTKV growth (MRI)
- Readout: 1H 2029*
- Prevalence: ~26% of ADPKD (~40K-50K in US)

ADPKD Orphan Drug Designation: US, EMA

NMAS: Nonsense mutation Alport syndrome, NM-ADPKD: Nonsense mutation Autosomal Dominant Polycystic Kidney Disease
*52-week topline data. Investigational New Drug application allowed by the FDA.

NMAS: Loss of collagen trimer leads to structural damage

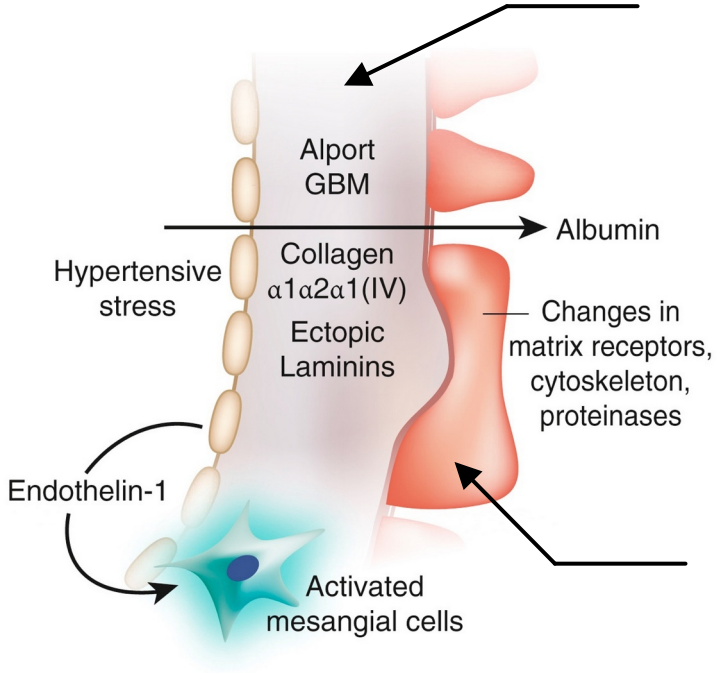
Healthy Kidney



COL4A3, COL4A4 or COL4A5 nonsense mutation

Loss of Collagen α3α4α5 (IV) trimer

Alport Kidney



GBM irregularities (lamellation)

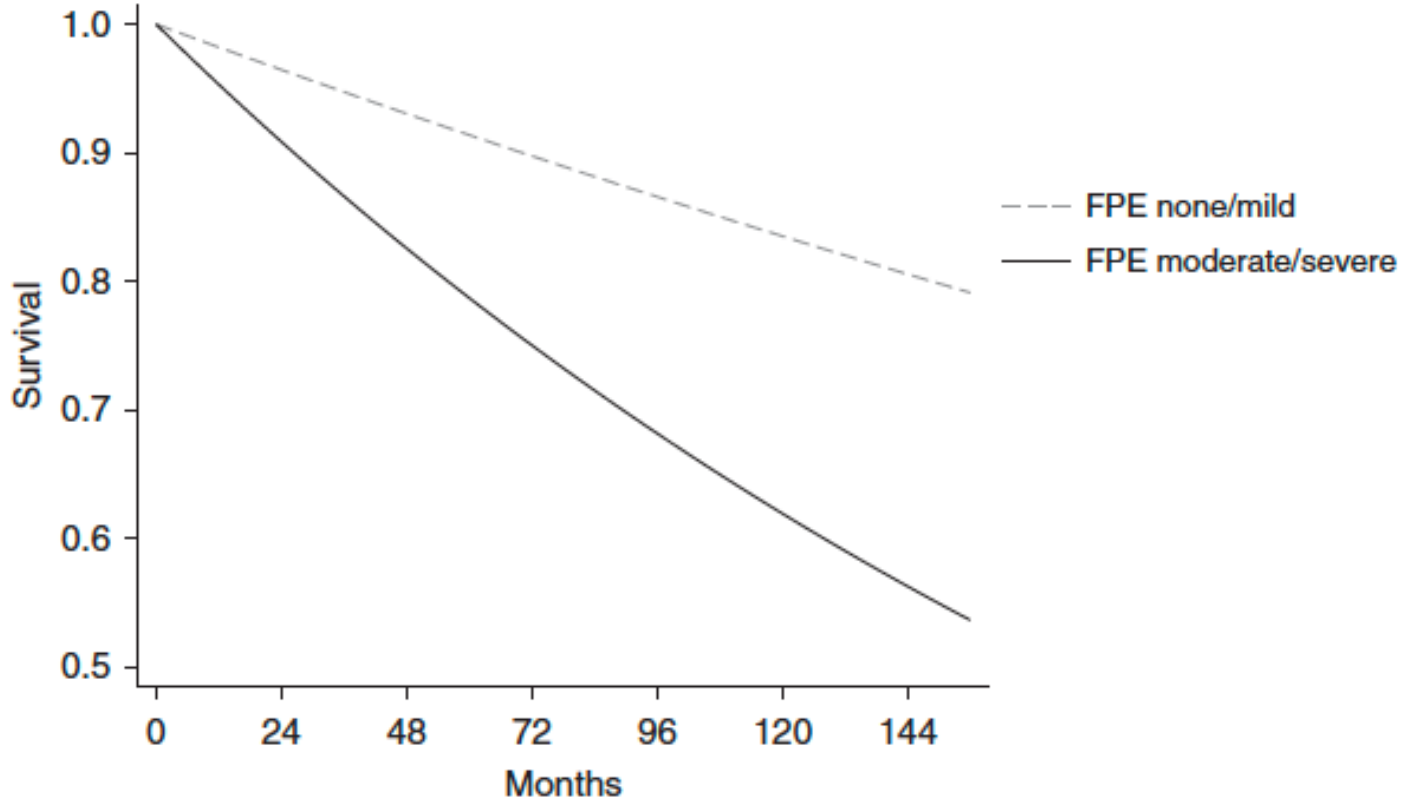
Protein loss (proteinuria), irreversible progression to ESRD

Foot process effacement (FPE) and podocyte loss

Irreversible progression to ESRD, median age ~23 years (vs ~40 years for missense-mutation AS). No approved therapies

Structural damage of foot processes (FPE) is an independent prognostic marker for future kidney failure

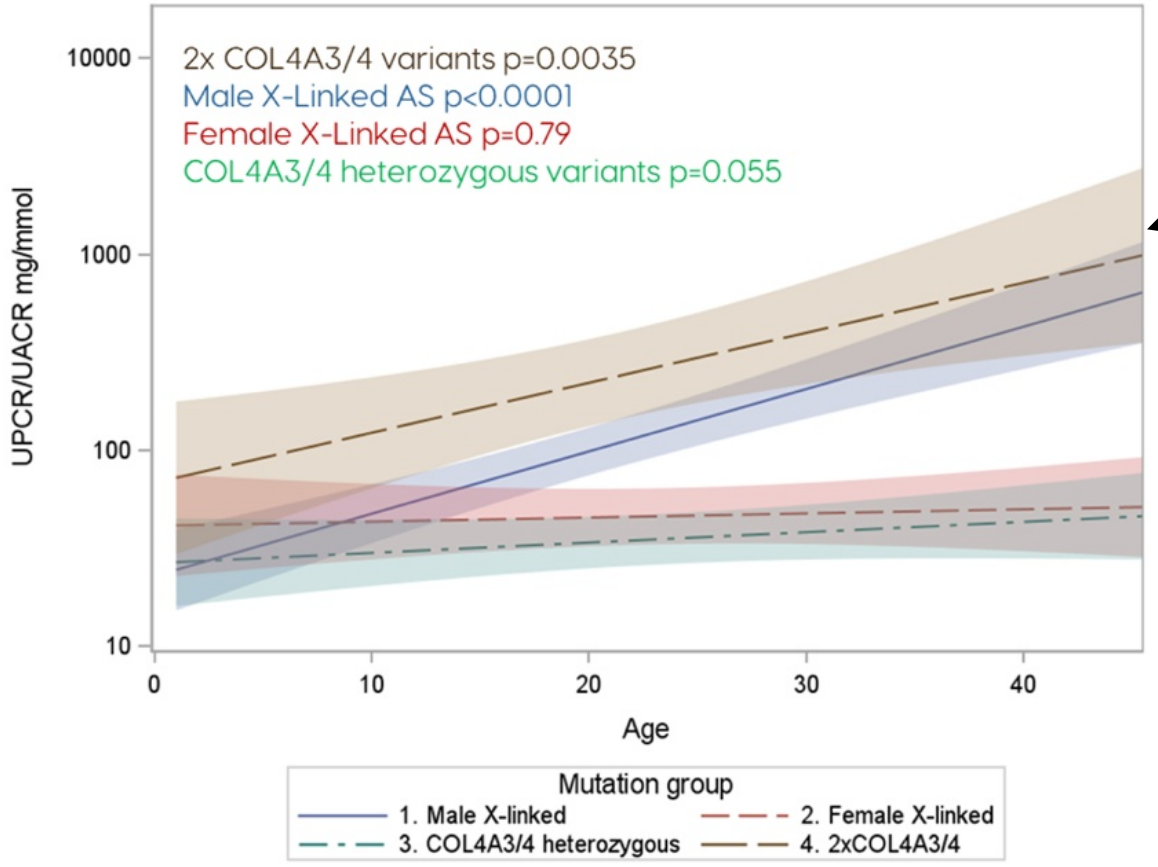
Adjusted survival curve for association between FPE and time-to-kidney failure[1]



[1] Wittig M, Verma A, Bellavia A, et al. The Associations of Foot Process Effacement with Kidney Histopathologic Lesions and Disease Progression. Kidney360. 2026;7(1):72-80. doi:10.34067/KID.0000000894.
Boston Kidney Biopsy Cohort: 813 patients, 48-month median follow-up, diverse kidney diseases

NMAS patients are rapid progressors with increasing UPCR trajectory

RaDaR natural history data



* Results from linear mixed model testing for difference from zero slope

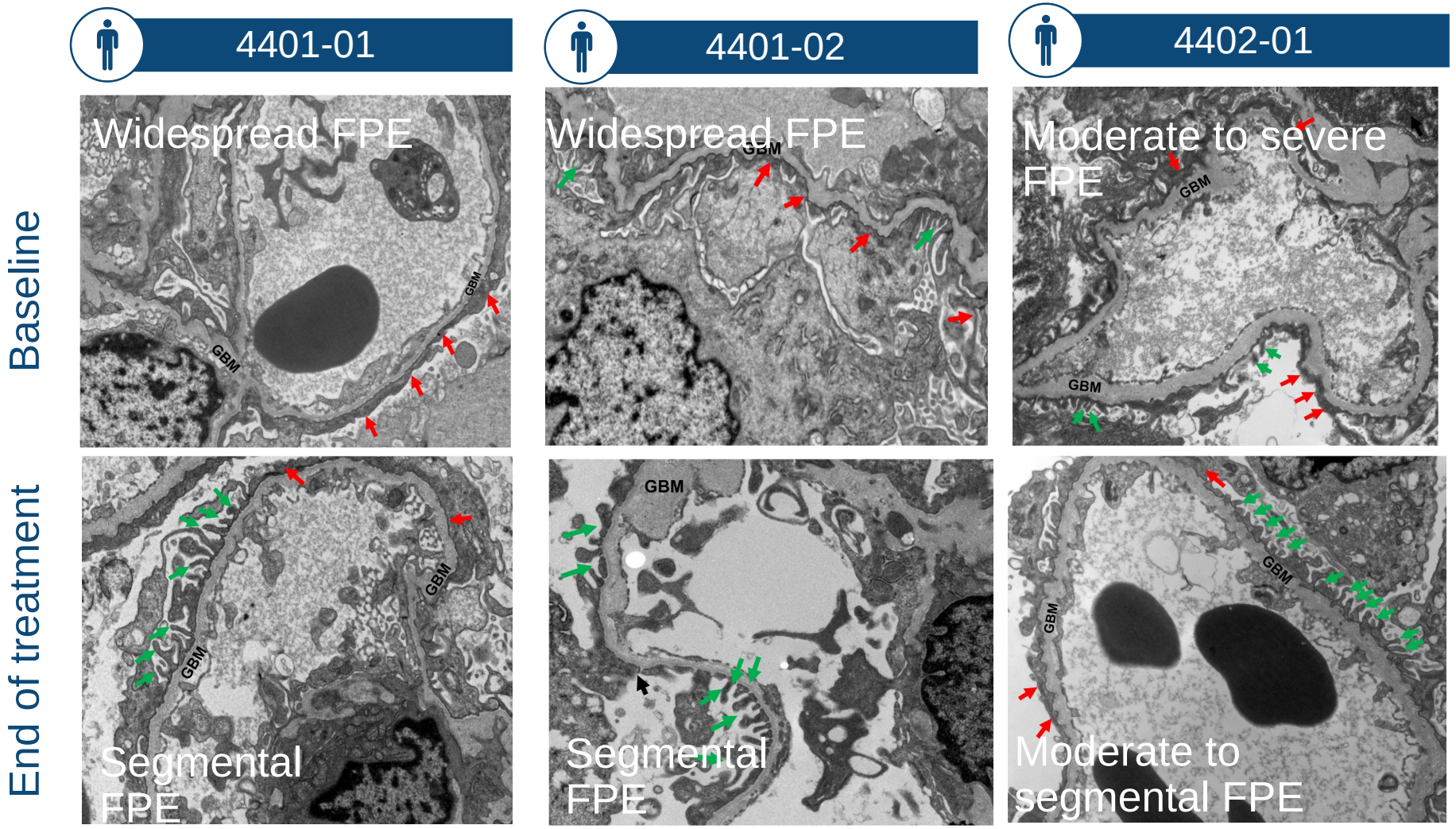
Rapid progressors:

Nonsense mutations are a subset of X-linked male and Autosomal recessive AS patients

Wong et al. 2026

Phase 2a: First-in-human proof of structural improvement in NMAS patients after 8 weeks exaluren treatment

Foot process effacement (FPE) and GBM changes in Phase 2a trial (Representative TEM images)*



Foot process

Effaced foot process

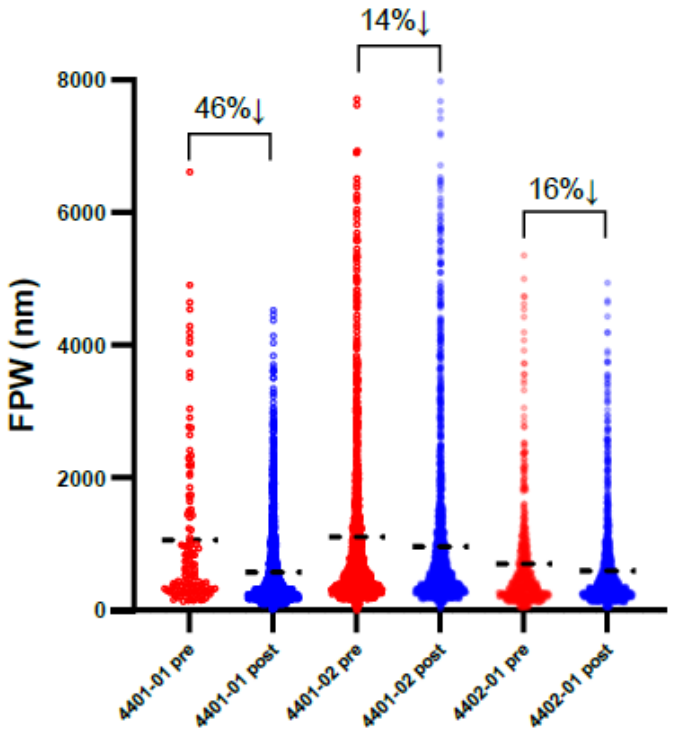
Images independently reviewed by two pathologists blinded to treatment sequence

* Phase 2a (EL-014): 3 UK patients with COL4A4 nonsense mutation · 8 weeks of exaluren 0.75 mg/kg SC daily · open-label · 12 weeks follow-up. TEM: Transmission electron microscopy

Phase 2a: Improvements in FPW and GBM width confirmed structural improvement

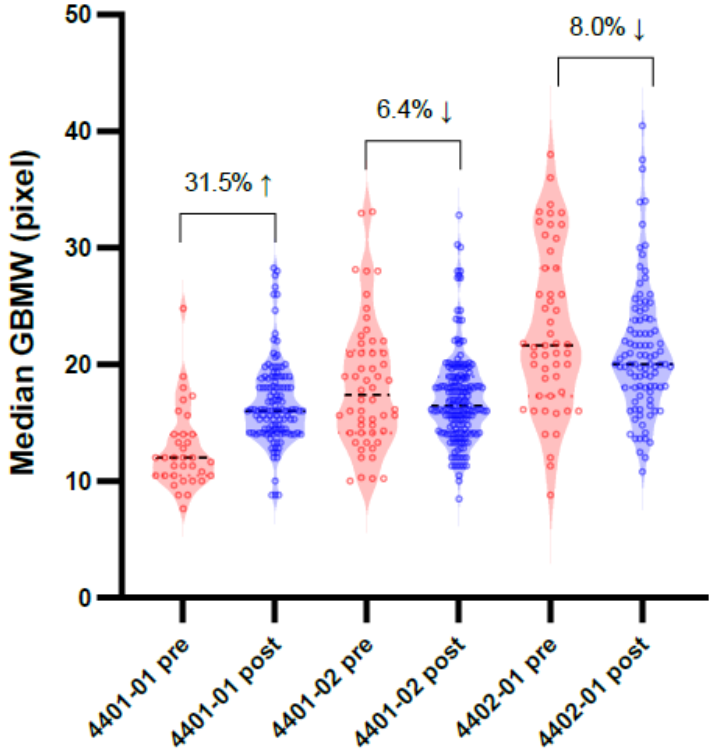
TEM Assessment using Systematic Uniform Random Sampling (analysis blinded to treatment order)

FPW: Reduced in all patients



Average FPW change: -26%

GBM width: Improved relative to baseline



Thin membrane increased while thicker membrane at baseline reduced

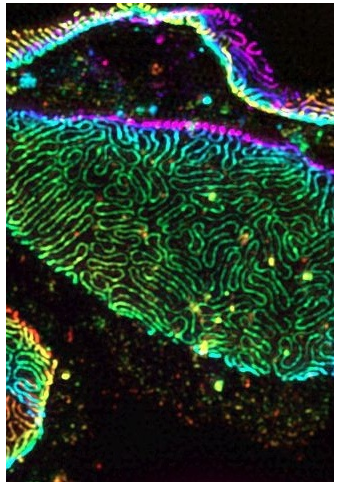
GBM width CV change	−20.5%	−23.9%	−13.1%

Conducted by Behzad Najafian, University of Washington

FPE assessed by change in Filtration Slit Density (FSD)

Unbiased assessment of filtration slit density

Immuno-fluorescence staining*



AI based glomeruli selection in FFPE tissue

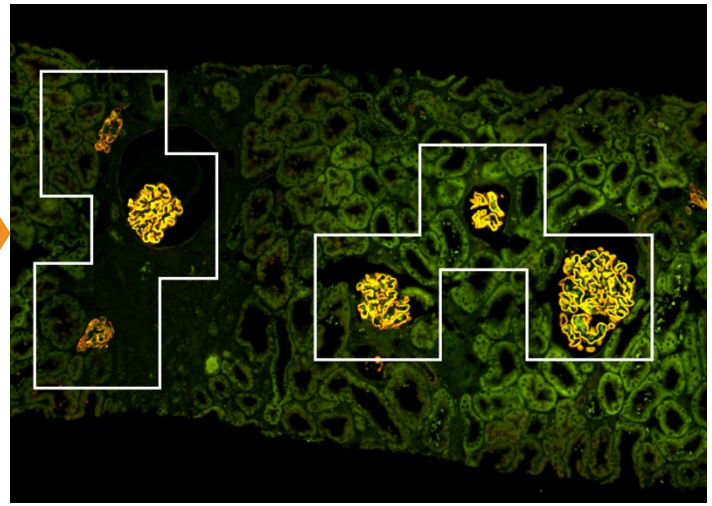
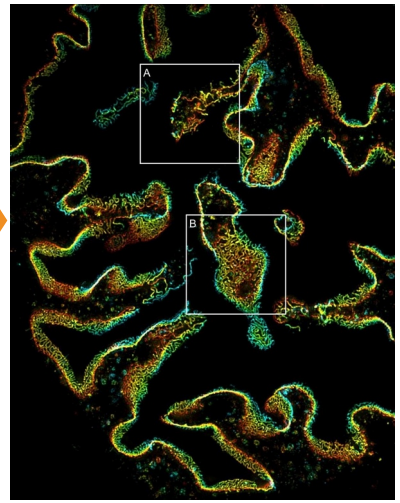
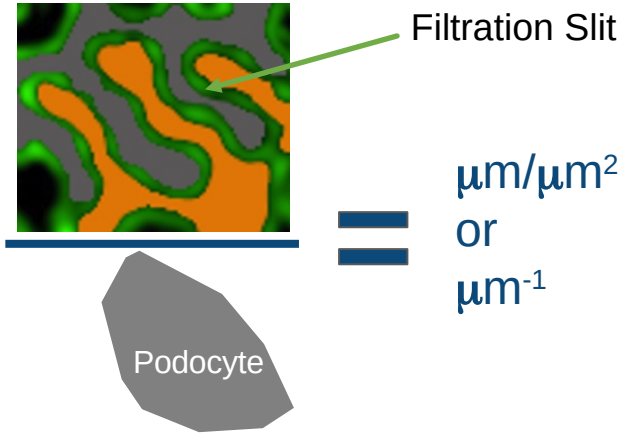


Image acquisition with 3D-Structured Illumination Microscopy (3D-SIM)



FSD = Filtration Slit Length/ Podocyte area



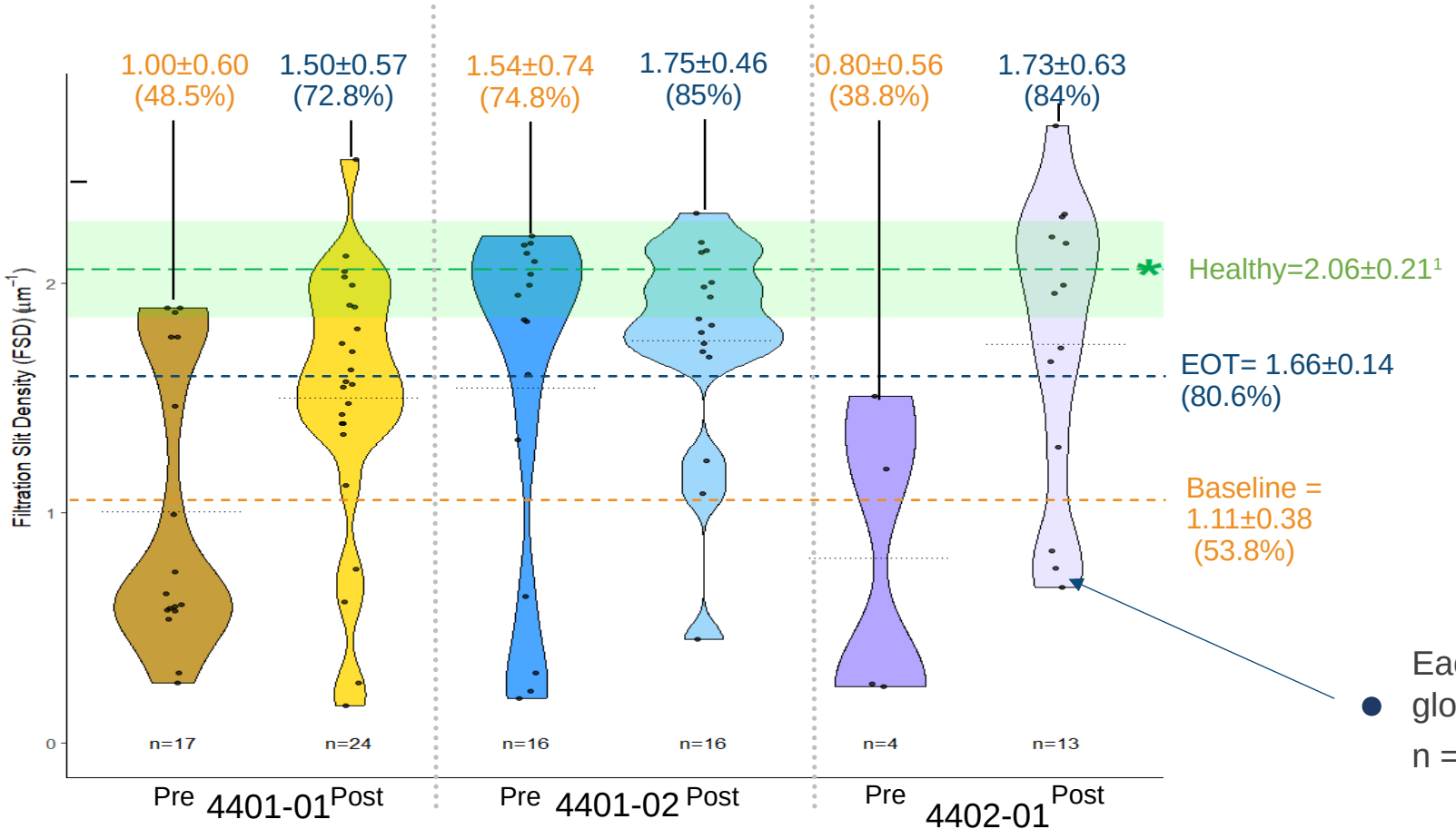
Strongly correlated with TEM assessments (Siegrist et al. 2007)

NIPOKA

*Immunostaining with podocin (filtration slits) and synaptopodin (foot process area)
NIPOKA will be conducting FSD measurements for Phase 2b clinical trial in Alport syndrome using its Podocyte Exact Morphology Measurement methodology

Phase 2a: FSD increased consistent with FPE reduction

Mean and distribution of FSD in Phase 2a clinical trial patients, μm^{-1} (% of healthy) after 8 weeks of treatment

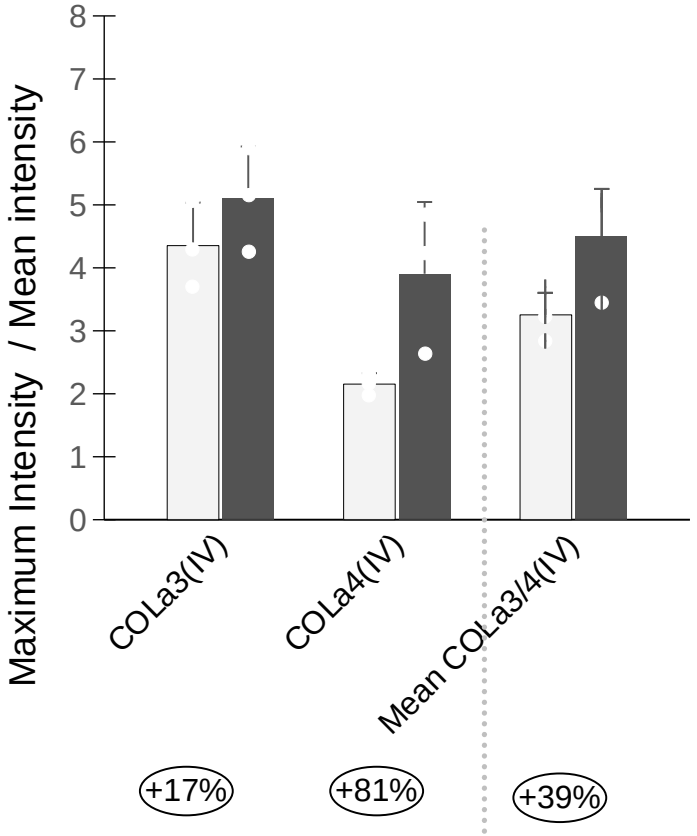


[1] from prospective NURTuRE study. Raw data provided by Evotec from TH-PO786 ASN 2023

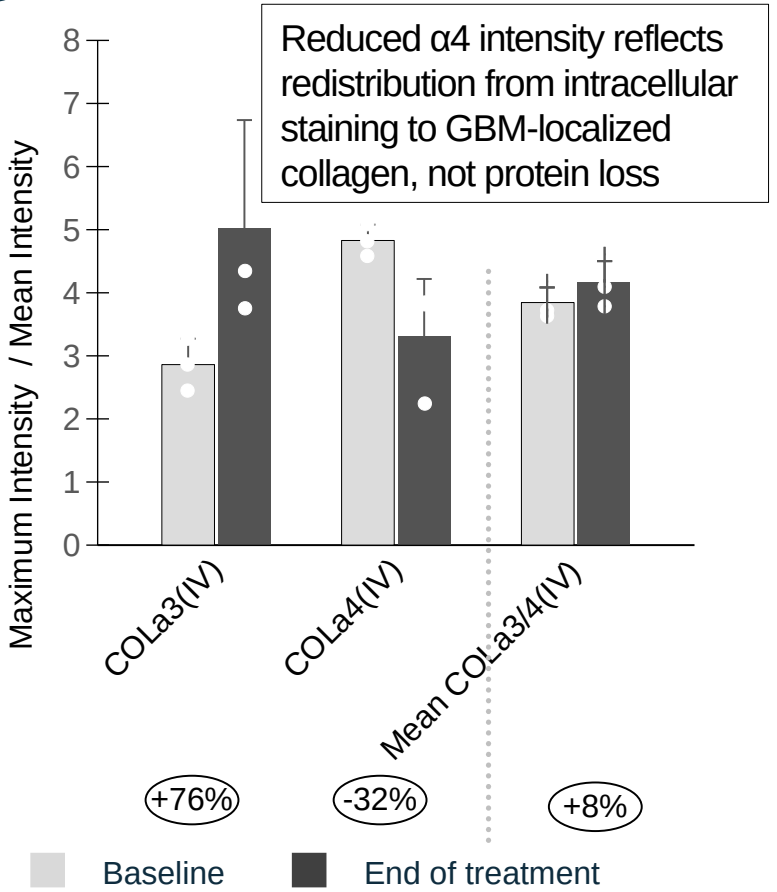
Phase 2a: Mean 72% increase in collagen α3 (IV) and α4 (IV) across all patients

Change in collagen 4 expression in Phase 2a trial (blinded assessment)

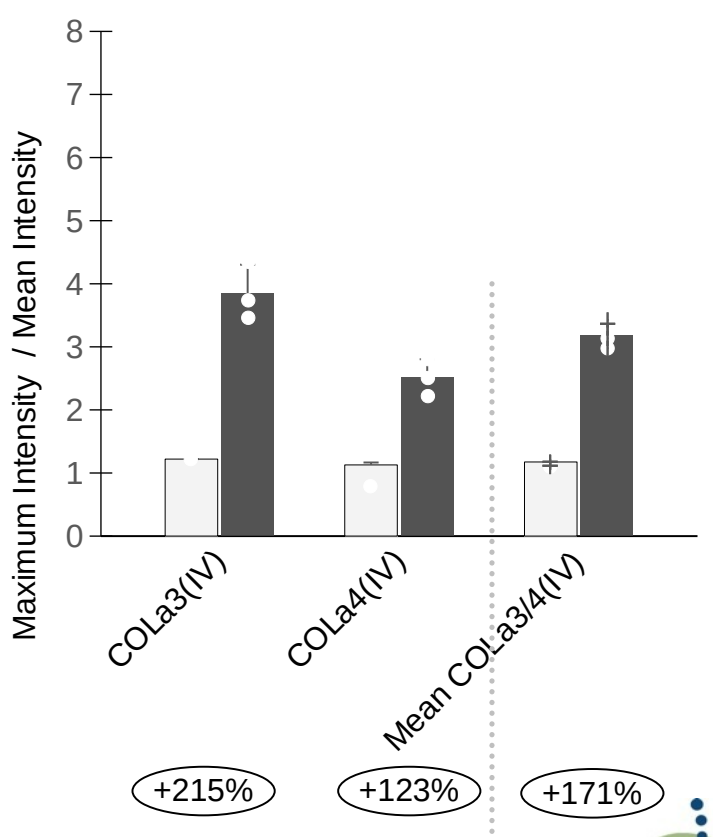
4401-01



4401-02



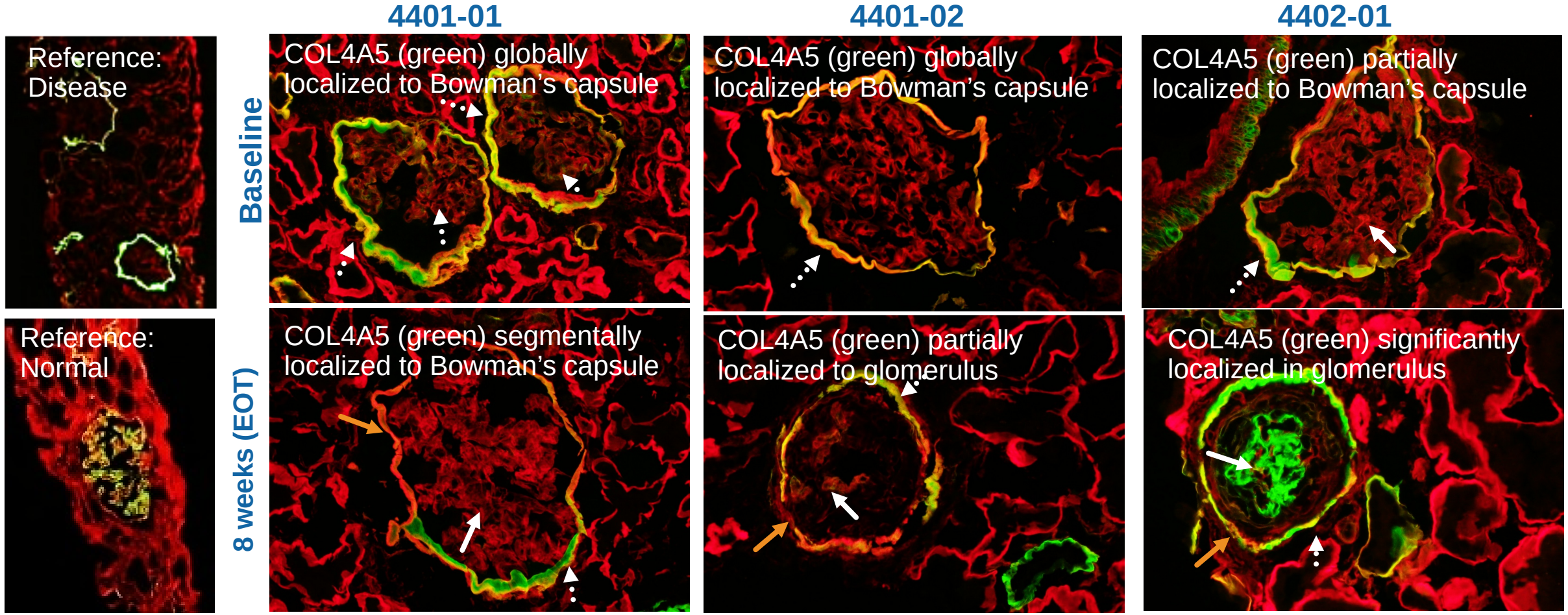
4402-01



Error bars represent standard deviation

Phase 2a: Collagen α5(IV) GBM localization consistent with collagen IV formation

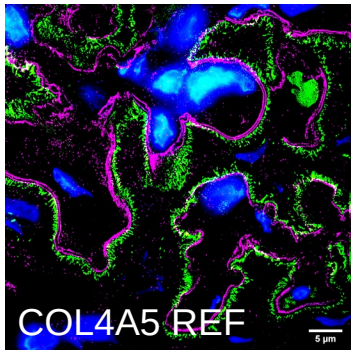
COL4A5 immunofluorescence staining in fresh frozen tissue biopsies

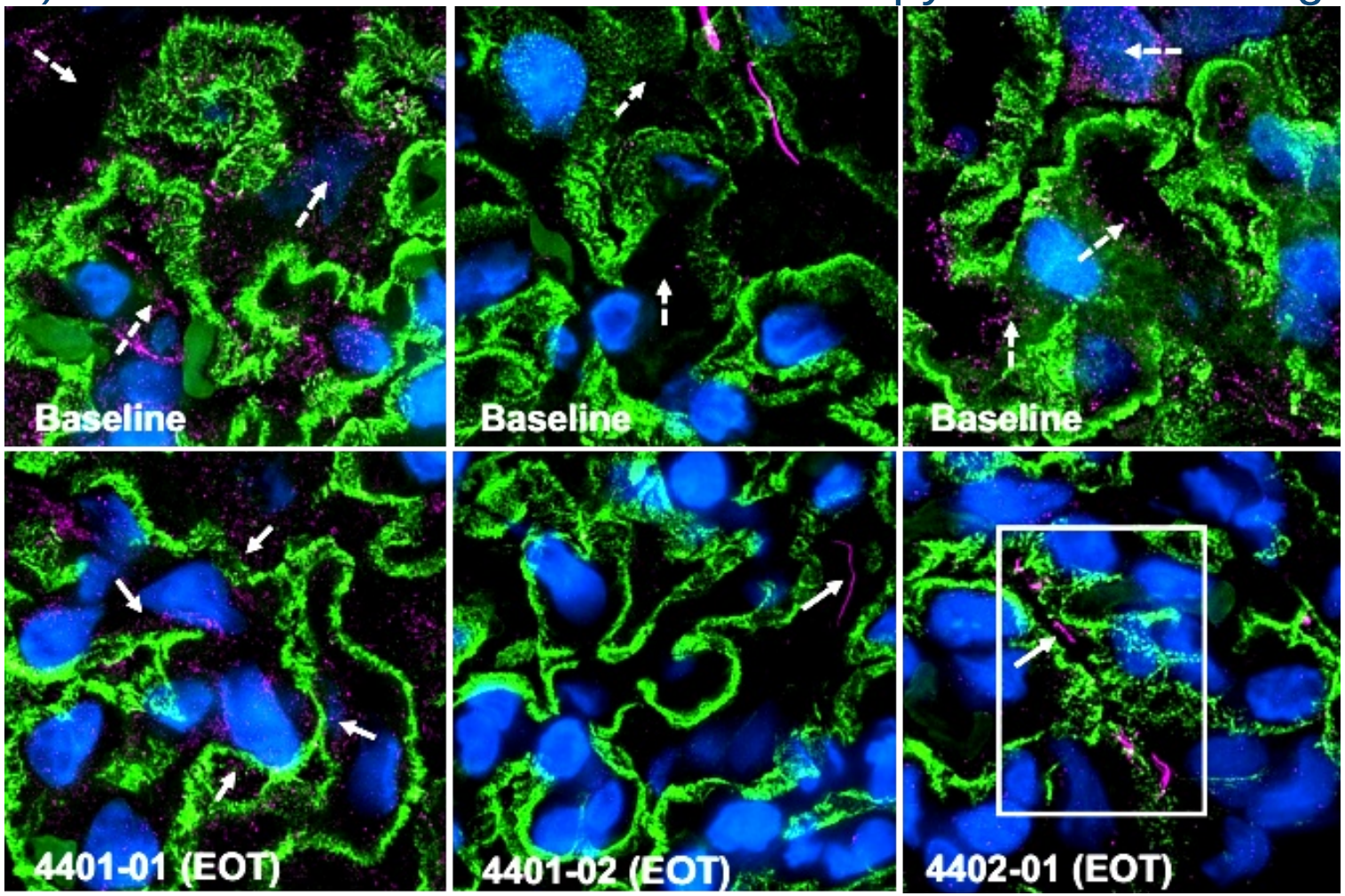


Reference for COL4A5 staining in ARAS patient and healthy kidney (Said et al. 2017). Baseline (Top): Collagen α5(IV) localized to the Bowman's capsule (dashed white arrows); EOT (Bottom): Collagen α5(IV) staining in glomerular tuft (solid white arrows), with reduced signal in the Bowman's capsule (solid orange arrow). Staining: Collagen α5(IV) IF (green) COL4A2 (red). Scale bar is 50 µm.

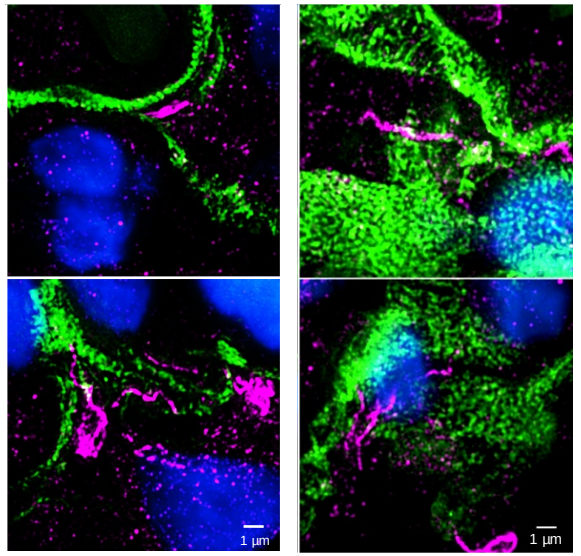
Phase 2a: Collagen α5(IV) localization and evidence of trimer formation consistent after eight weeks (n=3)

Collagen α5(IV) 3D-Structured Illumination Microscopy immunostaining





4402-01 (EOT) - Detail



NIPOKA

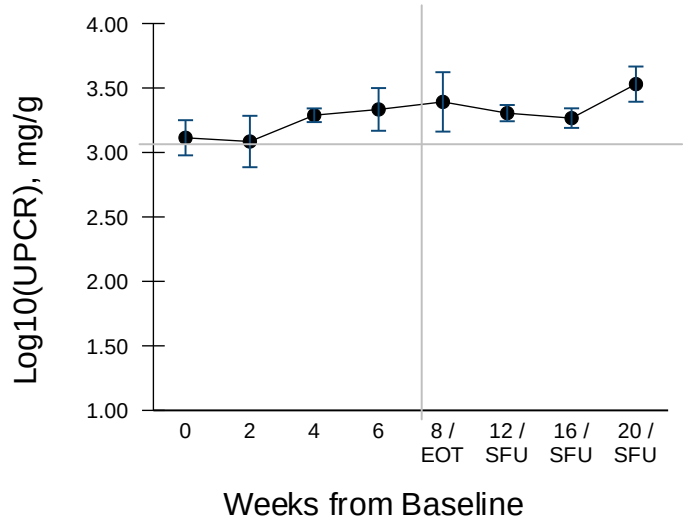
Collagen α5(IV) in 3D-SIM: Baseline (Top): Diffuse and low abundance non-specific intracellular α5(IV) staining is observed in all Participants (dashed white arrows) most notably in Participant 4402-01. A bright magenta band in Participant 4401-02 was determined by the pathologist to be an imaging artifact. EOT (Bottom): 4401-01: Partial relocalization of α5(IV) to foot processes (solid arrows). 4401-02: Emergence of faint linear α5(IV) band (solid arrow) along foot processes. 4402-01: Prominent linear α5(IV) staining (solid arrows) along foot processes in the boxed region. Stains: Collagen α5(IV) (magenta); synaptopodin/foot process (Green); DAPI (Blue). Scale bar is 5 µm

UPCR during treatment period

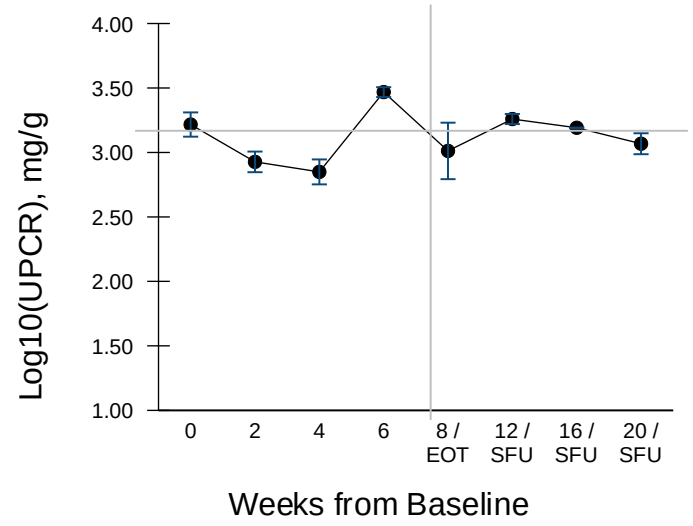
UPCR trends in Phase 2a trial, log (mg/g)*

Baseline UPCR

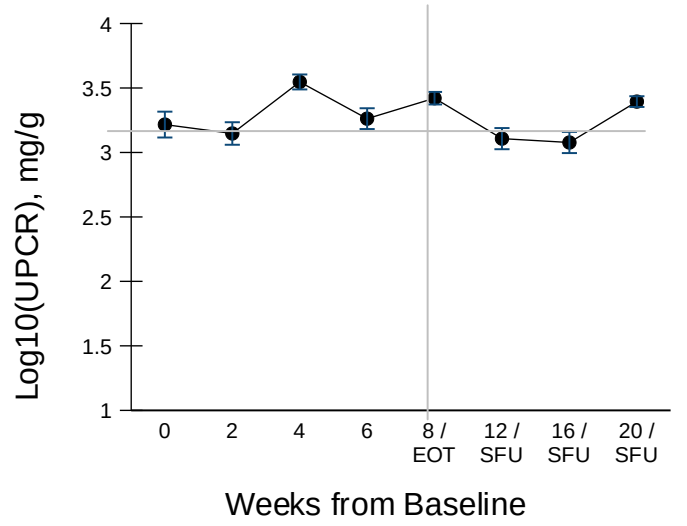
4401-01



4401-02



4402-01

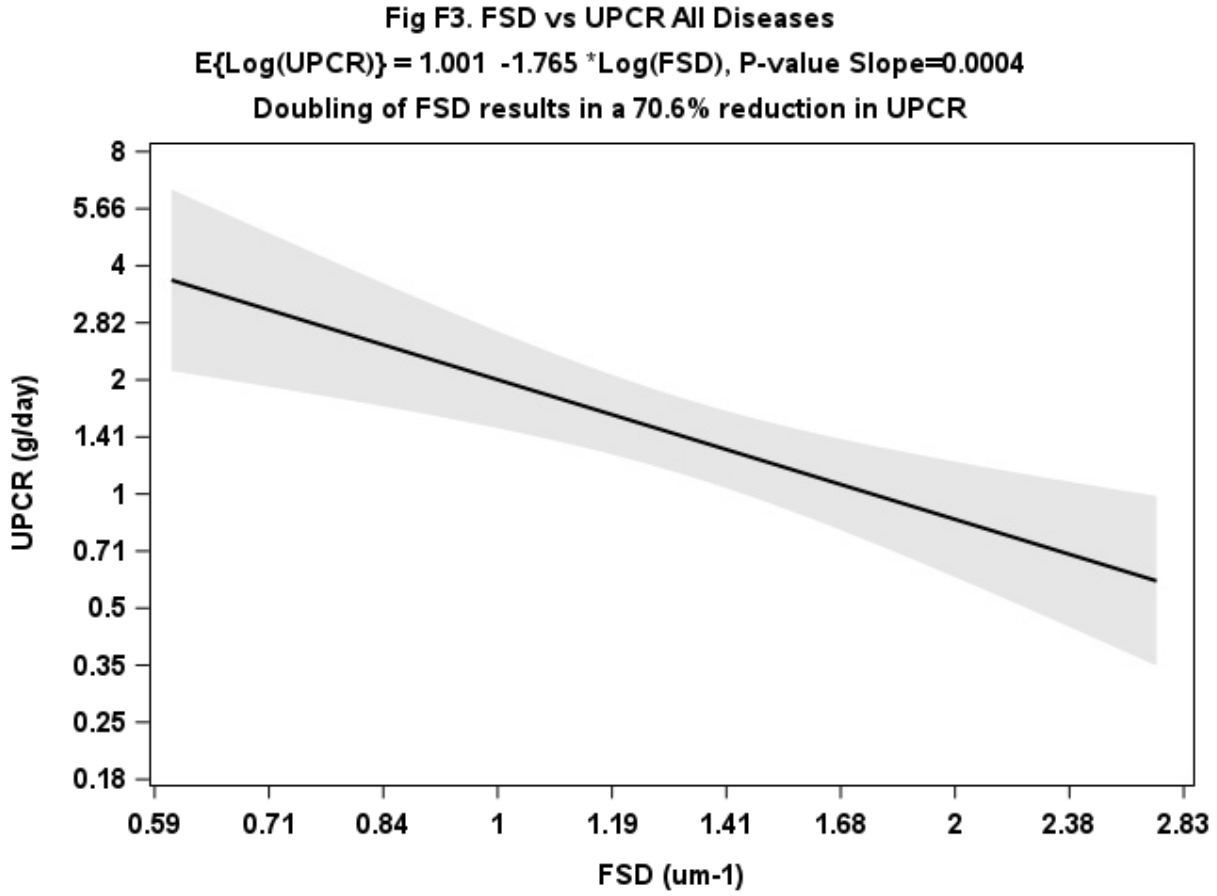


* Urine sample assessments based on the collection of second void at each day after discarding the first morning void. UPCR measurements taken 1 day prior to and on visit day during study period. Baseline values based on UPCR values from screening, baseline and Day 1 visits. Averages based on geometric means

** UPCR in the placebo arm of the HERA trial increased by 12.6% after 48 weeks. In the CARDINAL study, at 24 weeks and 36 weeks, eGFR adjusted UACR in placebo patients increased by 28% and UACR in subset of placebo adolescent patients increased by ~50% at 24 and 36 weeks. Baseline age and eGFR in HERA and CARDINAL were 31/40 years and 57/63 ml/min per 1.73 m2 indicating significantly less severe patient population than NMAS

FSD increases predicted to reduce UPCR

Analysis of published FSD and UPCR correlations across kidney diseases



Eloxx analysis based on data from Kim et al., J. Am. Soc. Nephrol. 1995; 5: 1659-1668; Data replotted from Kim et al. Age range 4-26 years. FSD assessment using TEM image analysis of kidney biopsies. FSD in Alport patients = 1.21±0.28 (n=15, mean age 13.3) and in healthy controls = 1.75±0.31 (n=10, mean age 13.2). TH-PO786 ASN 2023; Unpublished data provided by Evotec

FDA supported working group “ASSENT” launched to explore biopsy and clinical endpoints for Alport

An established FDA endpoint playbook for approvals in rare kidney disease

PARASOL → FSGS

1. FDA-backed consortium (2023)
2. Proteinuria accepted as FSGS surrogate endpoint for full approval
3. Enabled sparsentan approval — first FSGS therapy

ASSENT → Alport

1. FDA-initiated working group for Alport approval endpoints (Dec 2025)
2. Evaluating biopsy and clinical endpoints; FDA-requested expert input
3. Could expand regulatory pathways for exaluren

PARASOL (FDA, NephCure, ISGD, Kidney Health Initiative, NKF) established proteinuria as a surrogate endpoint in FSGS, supporting FDA approval of sparsentan (FILSPARI). ASSENT: Alport Syndrome endpoint working group with FDA engagement, initiated December 2025.

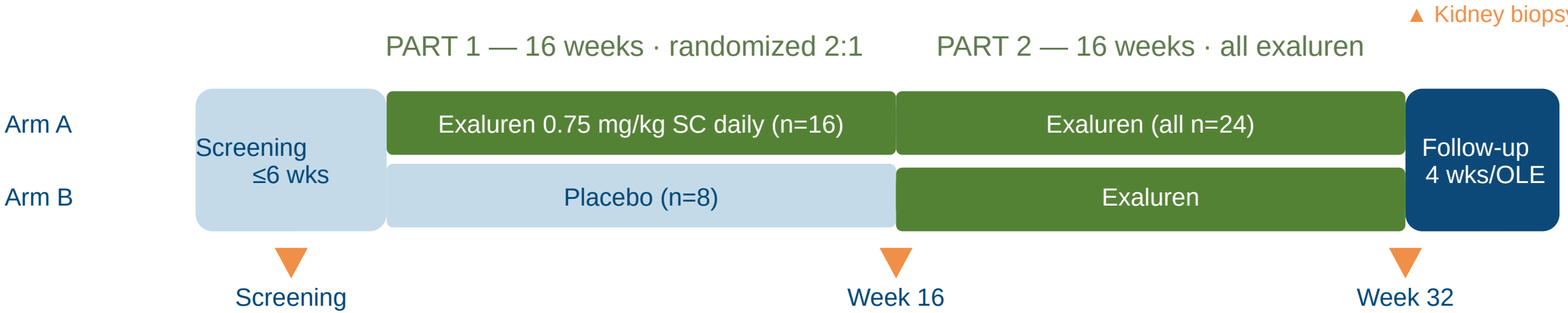
The EXACT Study: ongoing Phase 2b placebo-controlled NMAS study with FSD primary endpoint

Randomized, double-blind, placebo-controlled, delayed-start Phase 2b study.

EXACT Clinical Study

Phase 2b | Randomized, double-blind | Placebo-controlled · delayed-start | N = 24 · age ≥ 12

▲ Kidney biopsy



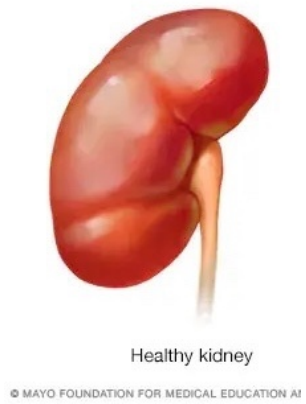
Primary endpoint
Change in filtration slit density (FSD) in kidney biopsy.

Key secondary endpoint
UPCR and additional clinical / safety measures.

NCT07523581

Advancing exaluren in nonsense mutation Autosomal Dominant Polycystic Kidney Disease

Healthy Kidney



PKD1 or PKD2 nonsense mutation

Loss of polycystin complex

ADPKD Kidney



Structural consequence

- Ciliary pathways disrupted
- Increased epithelial-cell proliferation
- New cyst formation and growth
- Total kidney volume growth and eGFR decline

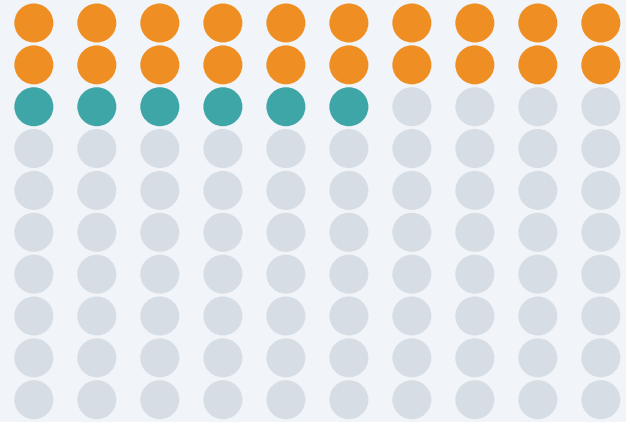
Irreversible progression to ESRD, median age ~55.6 for PKD1 truncating vs. 67.9 for non-truncating PKD1 • No approved gene targeted therapies

Nonsense-mutation ADPKD: a meaningful commercial opportunity

NONSENSE MUTATION FREQUENCY

≈ 1 in 4*

Illustrative 100 patients



ESTIMATED US + EU GENETIC POPULATION**

US: 40-50K, EU: 30-50K

Patients with a PKD1 or PKD2 nonsense mutation

US Annual Total Addressable Market

$9.2–$11.5B to **$15.6–$28.3B**

Assuming Jynarque (tolvaptan)-like pricing ($229,000 per patient per year)

Assuming Trutkana, Voyxact, Fabhalta-like pricing ($390K to $566K per patient per year)

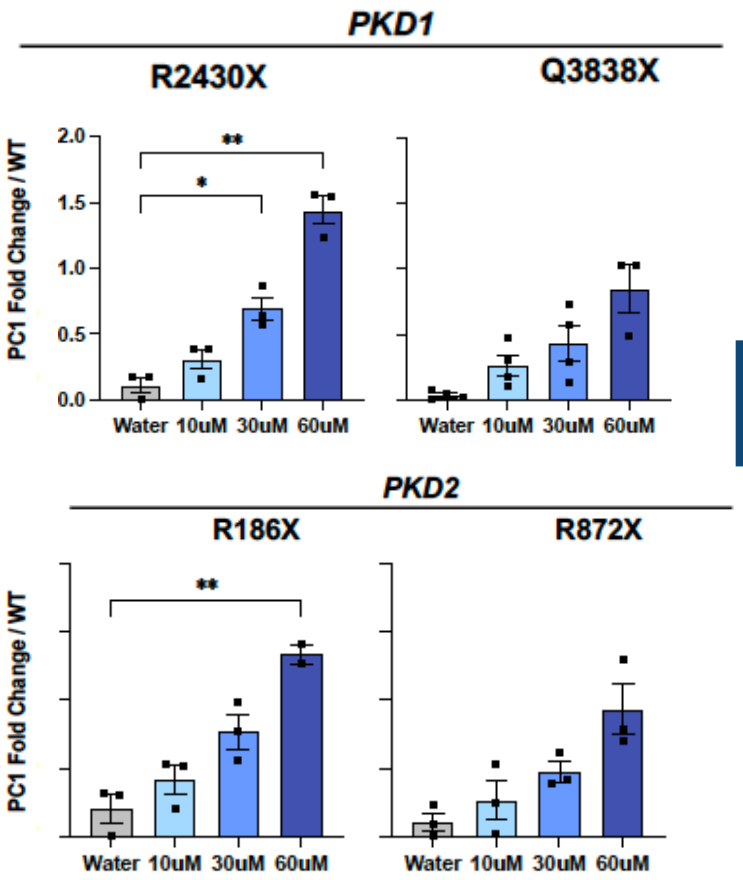
ADPKD precedent: Jynarque (tolvaptan) had ~$1.47B estimated US sales in 2024 targeting ~7% of patients despite poor tolerability and compliance

* Nonsense mutations represent approximately 23-28% of PKD1 cases and 33-54% of PKD2 cases, equating to approximately 25-33% of all ADPKD.
** US ADPKD estimated at 160,000-200,000. Willey et al., 2019; Suwabe et al. 2020; Aung et al. 2021; Neumann et al. 2013; Solazzo et al., 2018; Cornec-Le Gall et al., 2013; Rossetti et al., 2007; Heyer et al., JASN 2016; Kataoka et al., 2020

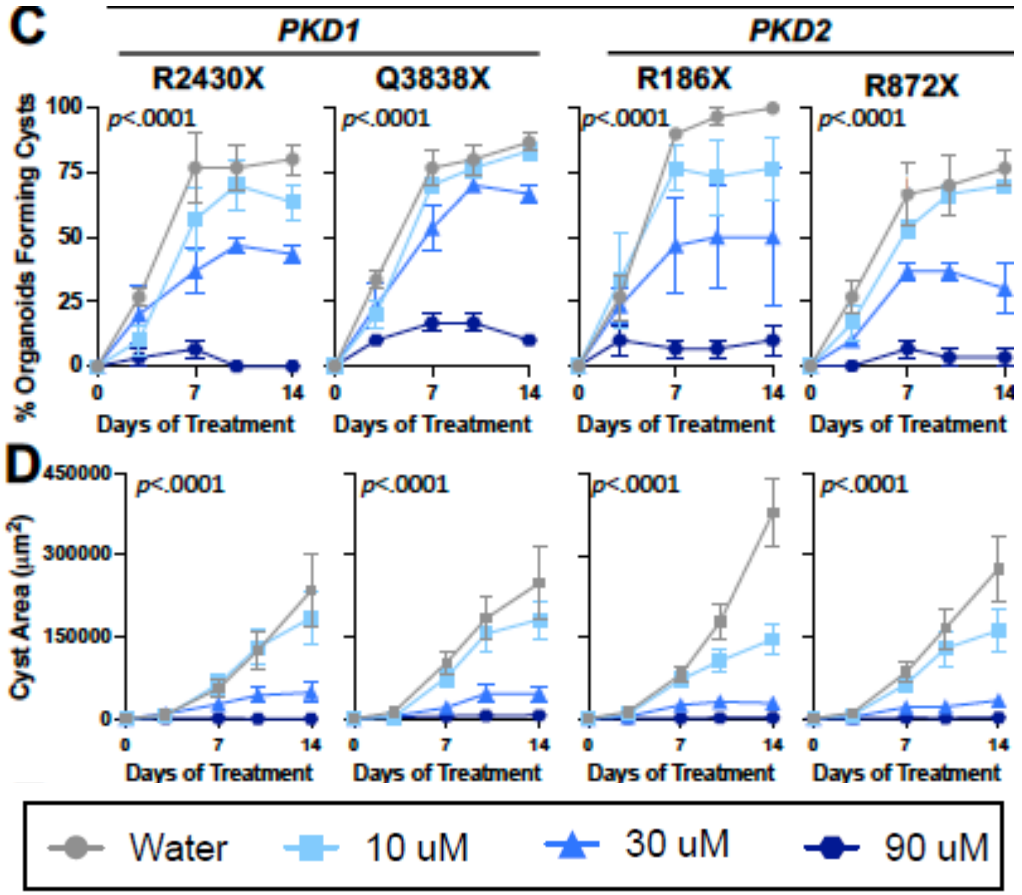
Exaluren restored 10 to 75% of PC1 and reduced cysts by ~50% in kidney organoids

PC1 expression and new cyst formation in organoids treated with exaluren for 14 days

PC1 expression in iPSC organoids, % WT*



Cyst prevention in iPSC organoids**



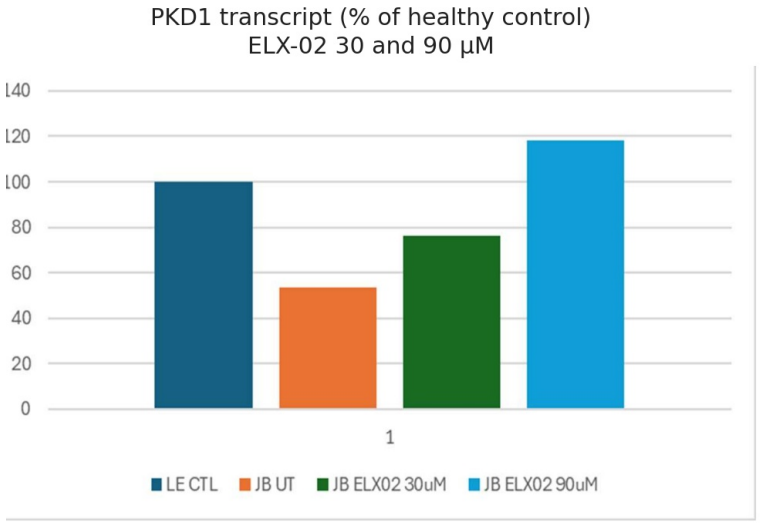
* Quantification of immunoblot PC1 fold change following treatment with exaluren relative to WT PC1 abundance for each targeted PKD mutation. n=3 independent biological replicates per genotype; Welch's ANOVA with Dunnett's T3 multiple comparisons test where *p<.05 and **p<.01. Mean±SEM.

**C) Fold change in cyst area relative to starting cyst area at day 0. n=30 organoids pooled from 3 independent trials each with 10 organoids per condition. Mean±SEM. Nonlinear regression extra-sum-of-squares F test p-values as indicated; D) Percentage of cysts with fold change less than 1. n=3 independent trials each with 10 organoids per condition. Mean±SEM. Nonlinear regression extra-sum-of-squares F test p-values as indicated.

Exaluren increased PKD1 transcript expression and corrected two ciliary abnormalities in patient primary epithelial cells

72-hour treatment of primary PKD1 Tyr1599Ter patient derived HuREC cells*

PKD1 mRNA expression

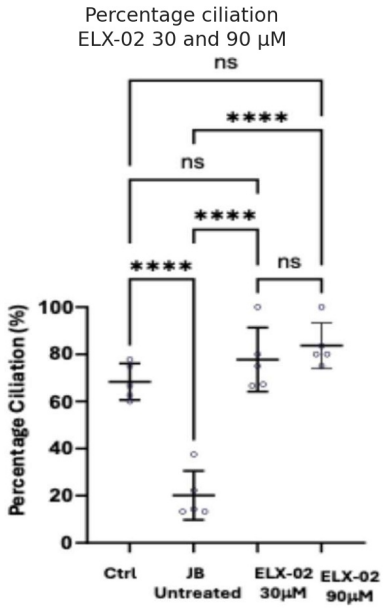


~50% → ~120%

of healthy-control mRNA (90 µM)

Cilia present

% of cells with an “antenna”

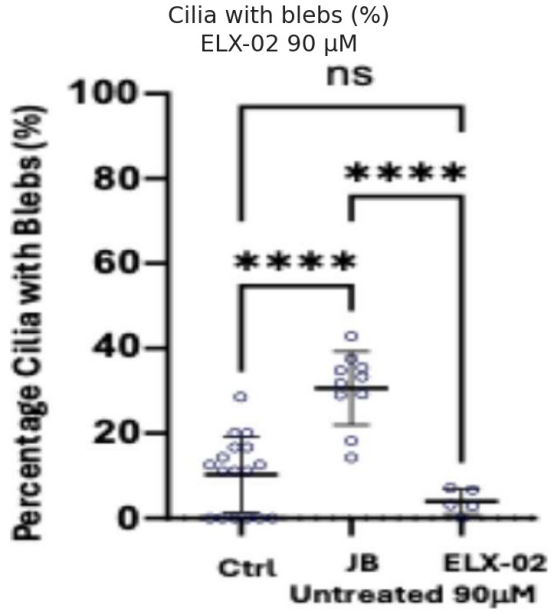


~20% → ~82%

More cells formed cilia | p<0.0001

Abnormal cilia

% cilia with abnormal bulges



~35% → <10%

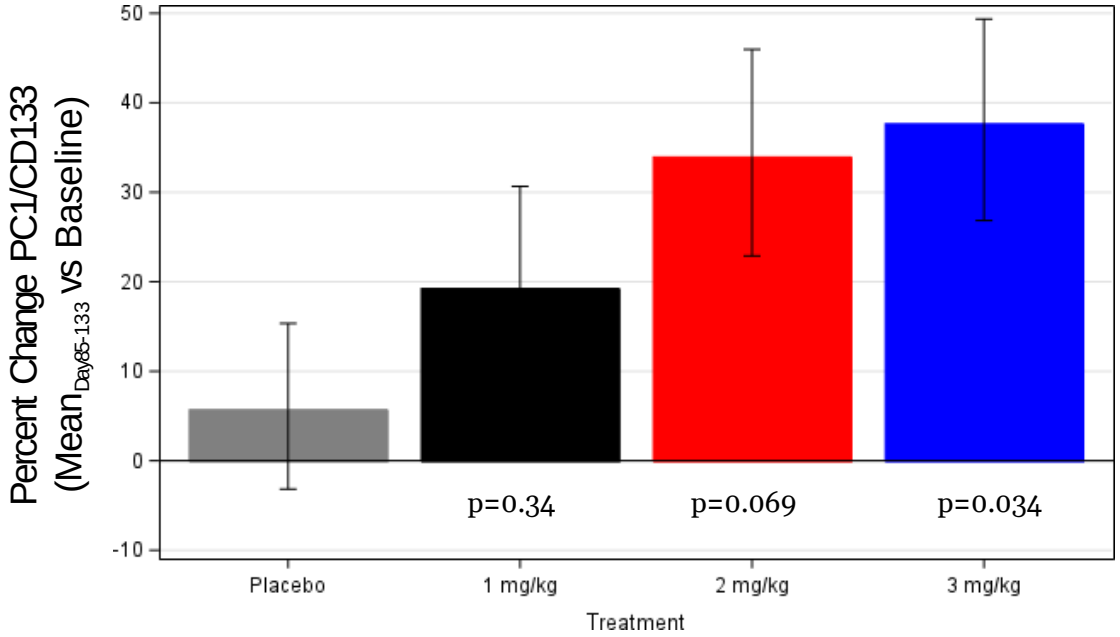
Fewer cilia with abnormal bulges | p<0.001

* HuRECs: Human urine derived renal epithelial cells. Sayer Laboratory, Newcastle University

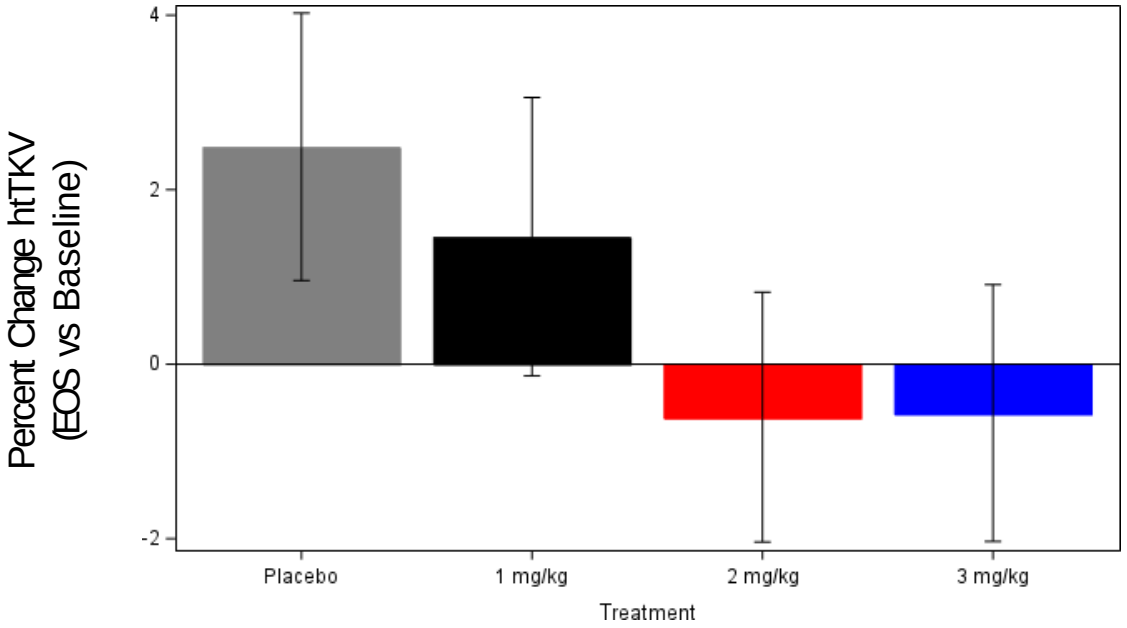
Independent clinical evidence links partial PC1 restoration with reduced htTKV growth

Reported results of farabursen (RGLS8429) anti-miRNA-17 ASO 3-month (Q2W) treatment*

Changes in PC1 expression



Changes in htTKV growth**



Regulus acquired by Novartis in June 2025 for $0.8B upfront plus up to $0.9B CVR***

* Source: Regulus Therapeutics Corporate presentation November 2024
** Geometric least squares mean percent change data and statistical analyses performed on log scale transformation to account for non-normal distribution; EOS, End of study. Subject with severe motion during MRI excluded. MRI data not available for two subjects who received only two (D1 and 15) and three (D1, 15, 29) doses, respectively
*** Novartis press release, April 30, 2025; acquisition completed June 25, 2025.

EXPRESS: Designed for a robust treatment effect and potential Breakthrough Therapy Designation

Global Phase 2 · US and EU orphan designation · Full-year htTKV primary endpoint

PHASE 2	RANDOMIZED · 1:1	DOUBLE-BLIND	N ≈ 60 ADULTS (18-65)

Key Inclusion criteria

- PKD1/ PKD2 nonsense
- Mayo 1B–1E
- eGFR ≥30

Exaluren 60 mg SC daily (n≈30)

Placebo (n≈30)

52 weeks of blinded, placebo-controlled treatment

BASELINE
3Q 2027 FIRST DOSING

WEEKS 28 & 36
INTERIM MRIs

52 WEEKS
1H 2029 TOPLINE

PRIMARY ENDPOINT: annualized htTKV growth

SECONDARY ENDPOINT: eGFR slope · safety · kidney imaging

Experienced leadership in rare disease drug development

Key Eloxx Leadership

Sumit Aggarwal
President and CEO

- Reshaped Eloxx pipeline and executed strategic agreements (Cystic Fibrosis Foundation and Almirall)
- Sold Zikani Therapeutics to Eloxx in 2021 after refocusing to rare diseases

Dr. T.R. Srinivas
SVP, Chief Medical Officer

- Nephrologist; led U.S. Medical Affairs for ADPKD product development and supported the Phase 3 farabursen program
- Novartis, CareDx (CMO), University Hospitals Cleveland, MUSC

Dr. Teji Singh
Head of Clinical Development

- Seven primary product first approvals across rare diseases
- Sanofi-Genzyme, Sarepta Therapeutics, Shire

Dr. Roger Clark
Head of Discovery Sciences

- 1 approved drug (Xerava); 5 additional clinical compounds
- Bayer Pharmaceuticals, Critical Therapeutics, Tetraphase Pharmaceuticals, Zikani Therapeutics

John Woolford
Vice President, Corporate Development

- 30+ years biotechnology advisory experience in financings, IPO and corporate alliances
- ICR (formerly Westwicke), Legg Mason (now Stifel), MedImmune (now AstraZeneca) and Charles River Labs

Demonstrated ability to execute high-value pharma collaborations

Overview of global license agreement with Almirall for ZKN-013 executed in 2024

ZKN-013 global development and commercialization rights to Almirall

Eloxx eligible to receive up to $470 million in development, regulatory and commercial milestones

Potential for Priority Review Vouchers (PRVs) for each indication

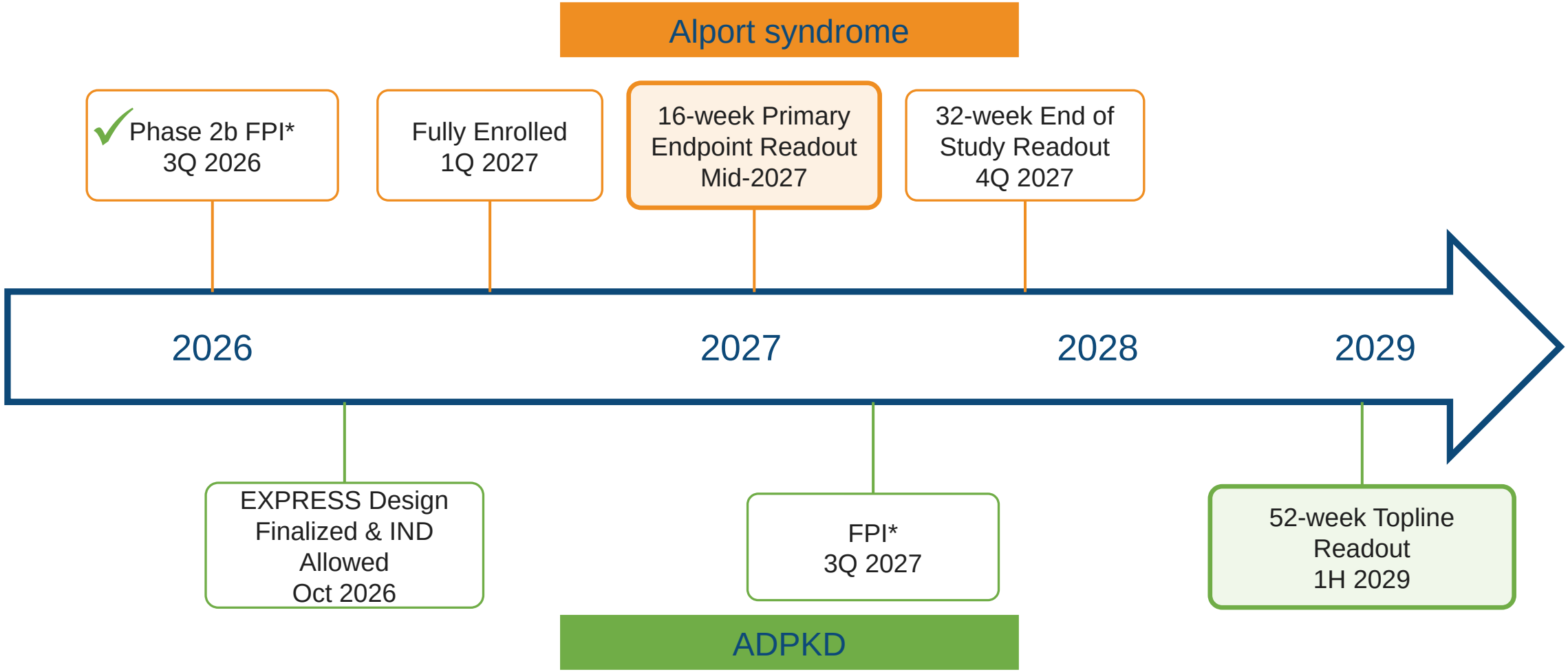


Provides Almirall entry into rare diseases, with initial focus on PTC readthrough in RDEB and FAP

Tiered royalties on sales

Multiple high impact clinical readouts anticipated from 2026 to 2029

Key inflection points: Alport (mid-2027) and ADPKD (1H 2029) readouts



* First Patient In

June 2026 follow-on offering and Nasdaq uplisting

Balance sheet (as of June 30, 2026)

- Cash of $62.0M
- 12.5 million fully diluted shares, including 4.04 million common stock and 7.38 million pre-funded warrants

Transaction

- 2,975,000 shares at $11.00 plus pre-funded warrants for 3,025,000 shares, or 6.0M share equivalents
- Gross proceeds of $66.0M
- Key shareholders: Coastlands Capital, Samsara, Aberdeen, Invus, Stone Pine, EcoR1, Nantahala

Nasdaq uplisting

- Shares began trading on the Nasdaq Capital Market on June 9, 2026

Human proof-of-concept with two clinical rare kidney disease programs

Human proof of mechanism: protein restoration and structural reversal in human kidney biopsies

Same drug, two rare kidney diseases: Expected mid-2027 Alport readout supports both programs. 52-week EXPRESS Phase 2 in ADPKD expected to read out in 1H 2029*

Nasdaq listed following a $66M public offering led by specialist healthcare investors

*First patient expected 3Q 2027. Investigational New Drug application allowed by the FDA.



INVESTOR RELATIONS

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WEB

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