



A precision approach to rare kidney disease

Small molecule based genetic therapy

Corporate Presentation | September 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

Phase 2 planned, with topline data expected in mid-2028



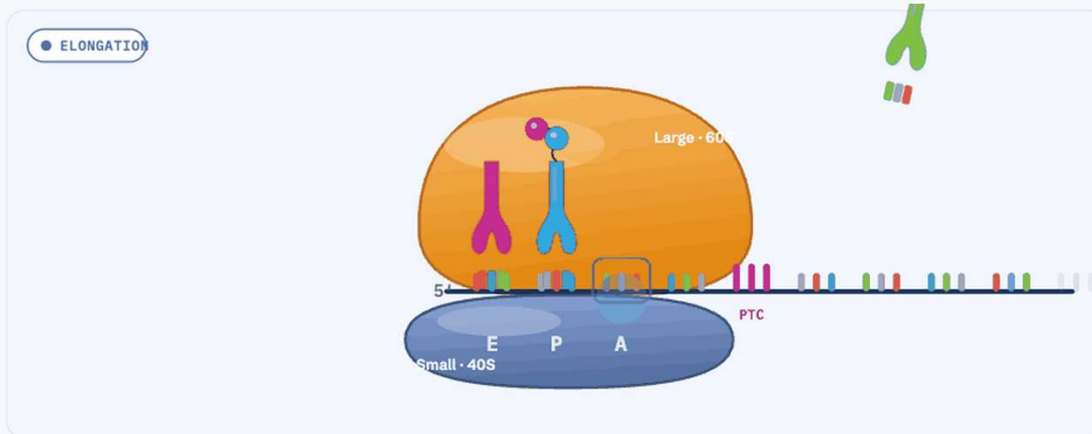
June 2026 offering **expected to fund the company through topline read-outs** in both indications



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

Translation, interrupted.

A nonsense mutation puts a stop sign in the wrong place



The ribosome reads each codon, adding one amino acid at a time.



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



No evidence of readthrough of native stop codon (NSC)

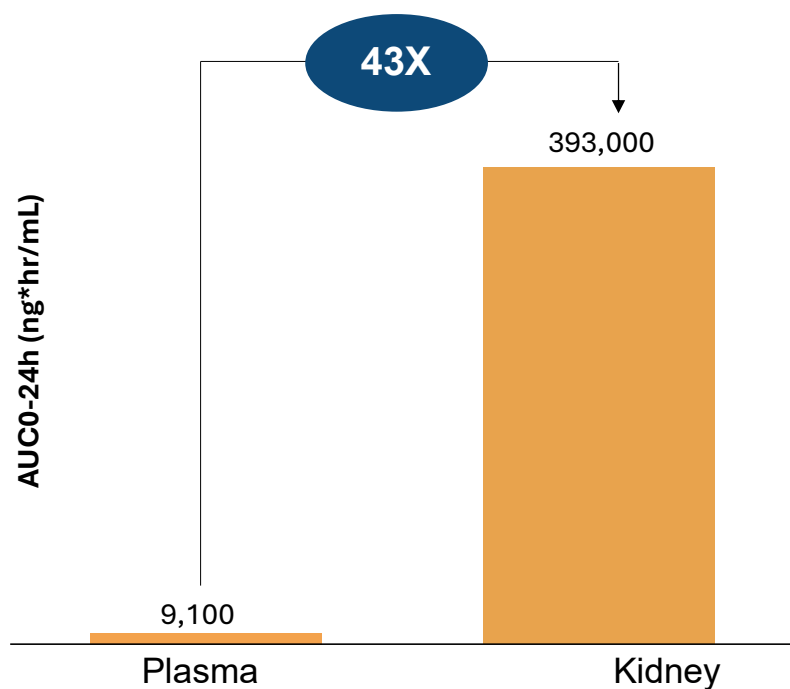
Partially functional full-length proteins are expected to be clinically meaningful

*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 LRP2 protein megalin
- Megalin is found in the glomerulus and proximal tubules in the kidney, the inner ear and eye



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

3 / 3

patients improved kidney structure

2. Protein restored:

+72% collagen $\alpha 3(\text{IV})/\alpha 4(\text{IV})$ restored

Collagen IV trimer precursor formed

+72%

mean collagen $\alpha 3/\alpha 4$ (IV)

3. Well tolerated

Mild AEs; no kidney or hearing signal

High UPCR variability; stable blood pressure

0

kidney or hearing AEs



Extensive safety database supports development

Exaluren clinical and nonclinical data: no dose-limiting toxicities and no serious AE's 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

9.7–10×

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



Two Phase 2 programs with data readouts over 24 months

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



Expected Phase 2 study start in 2026



Primary Endpoint: Change in htTKV growth (MRI)



Readout: Mid-2028*



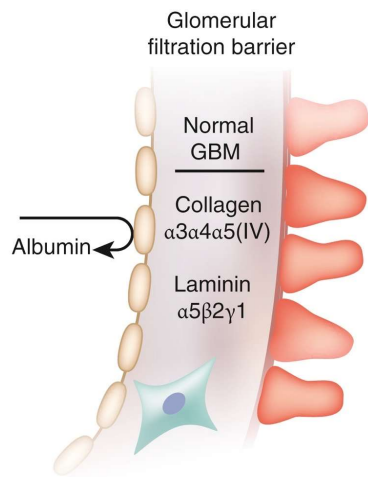
Prevalence: ~26% of ADPKD (~40K-50K in US)

ADPKD Orphan Drug Designation: EMA

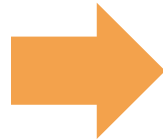


NMAS: Loss of collagen trimer leads to structural damage

Healthy Kidney

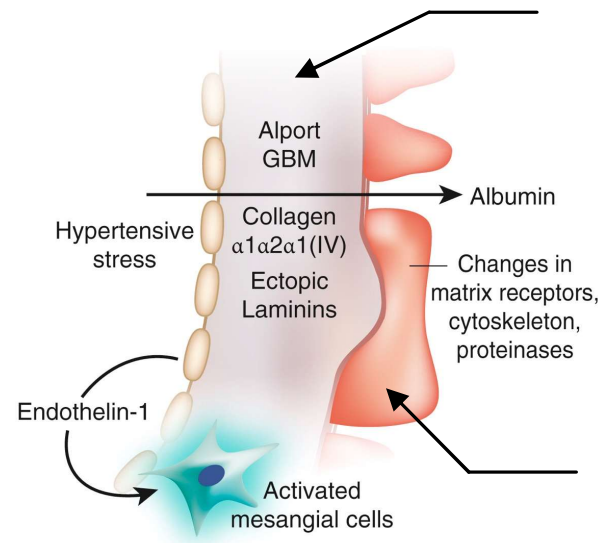


COL4A3, COL4A4
or COL4A5
nonsense mutation



Loss of Collagen
 $\alpha3\alpha4\alpha5(IV)$
trimer

Alport Kidney



GBM irregularities
(lamellation)

Protein loss (proteinuria),
irreversible progression to
ESRD

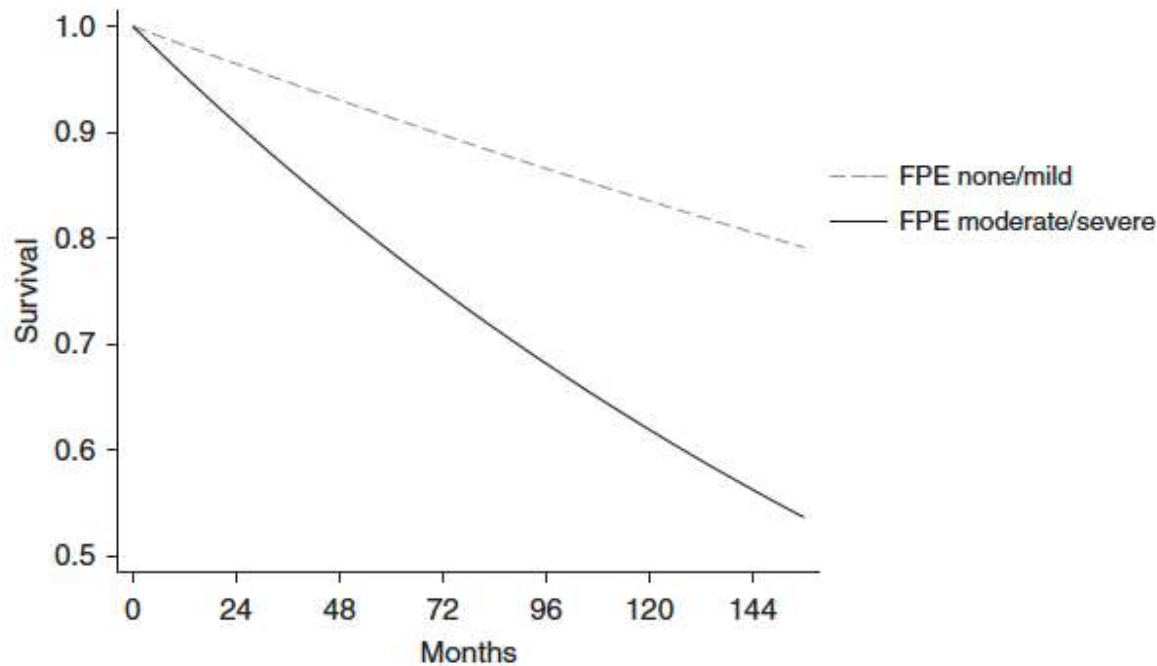
Foot process
effacement (FPE)
and podocyte loss

Irreversible progression to ESRD, median age ~20 years (vs ~40 years for missense-mutation AS). No approved therapies



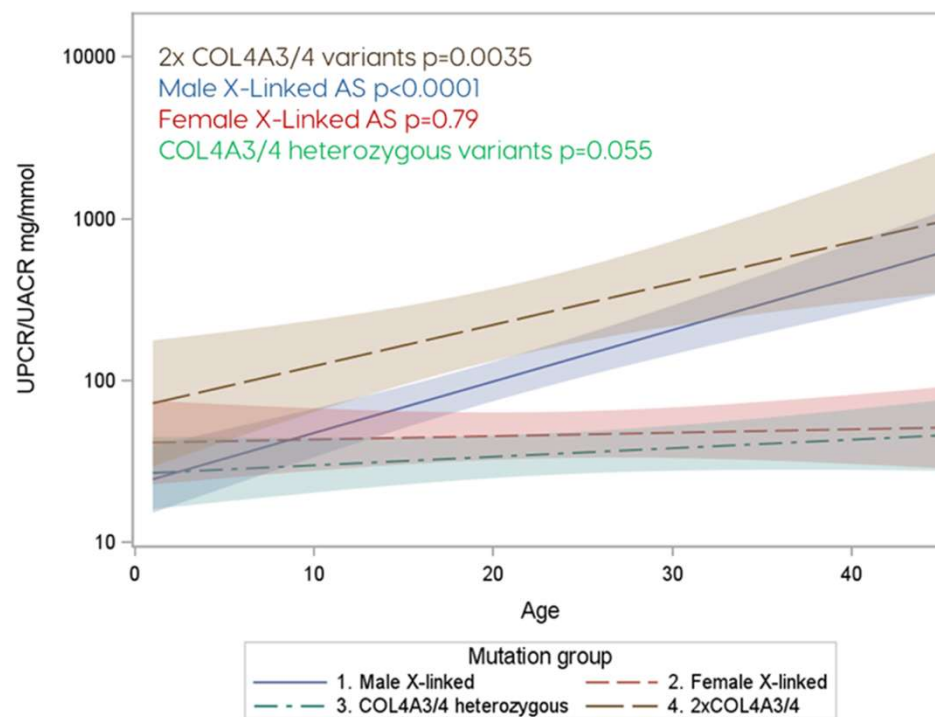
Structural damage of foot processes (FPE) is an independent prognostic marker for future kidney failure

Adjusted survival curve for association between FPE and time-to-kidney failure¹



NMAS patients are rapid progressors with increasing UPCR trajectory

RaDaR natural history data



Rapid progressors:

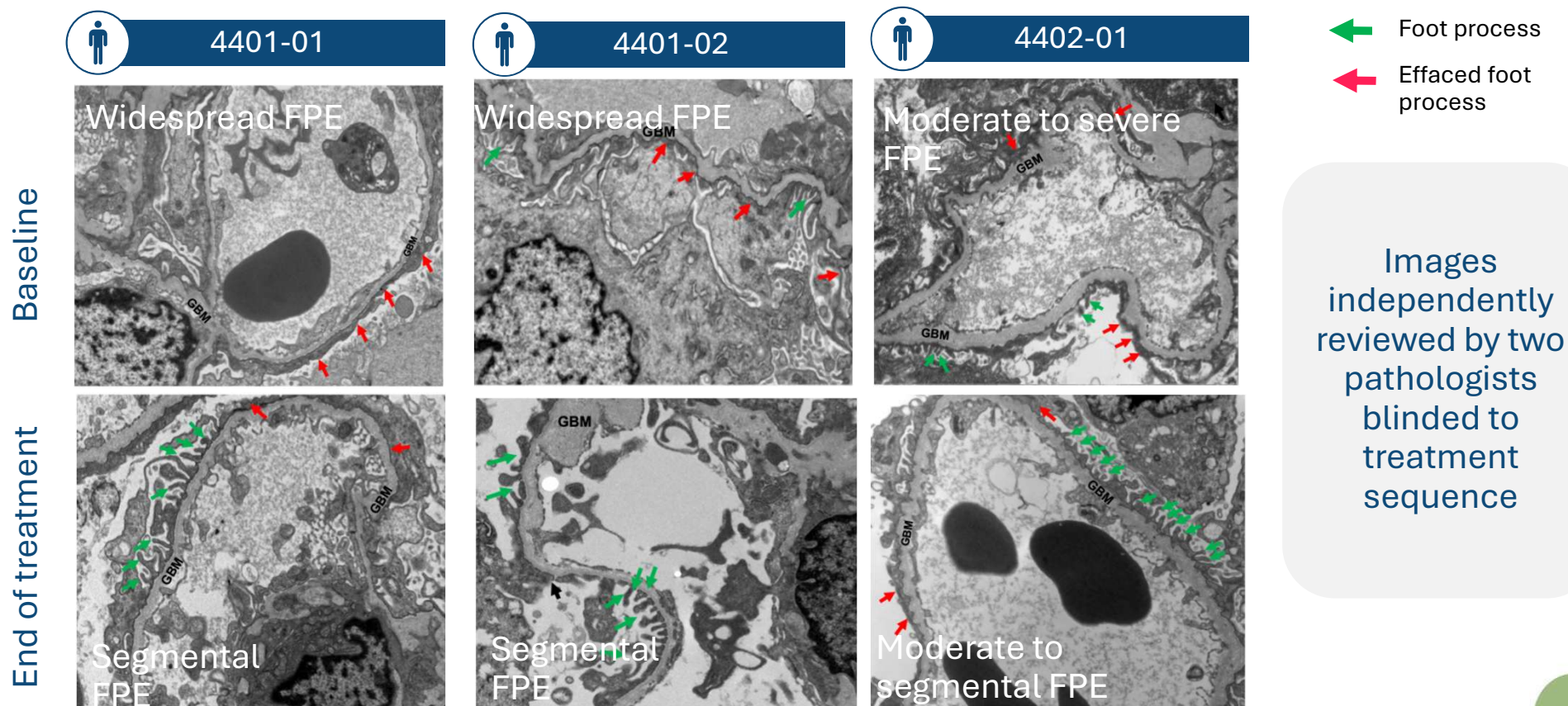
Nonsense mutations are a subset of X-linked male and Autosomal recessive AS patients

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



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)*



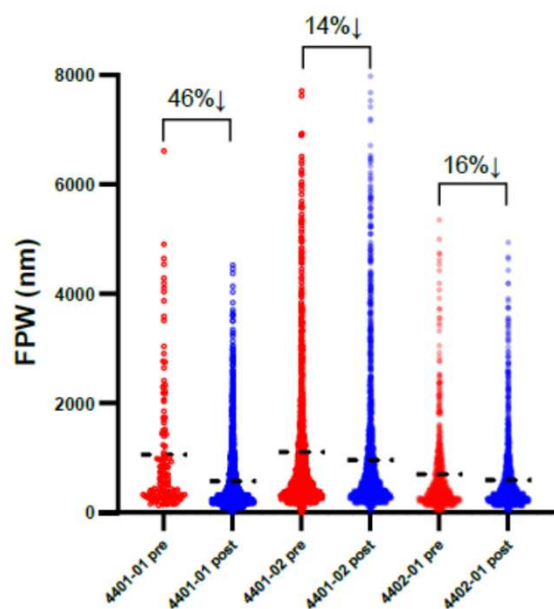
* Phase 2a (EL-014): 3 UK patients with COL4A4 nonsense mutation · 8 weeks of exaluren 0.75 mg/kg SC daily · open-label · 12 weeks follow up. TEM: Transmission electron microscopy



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

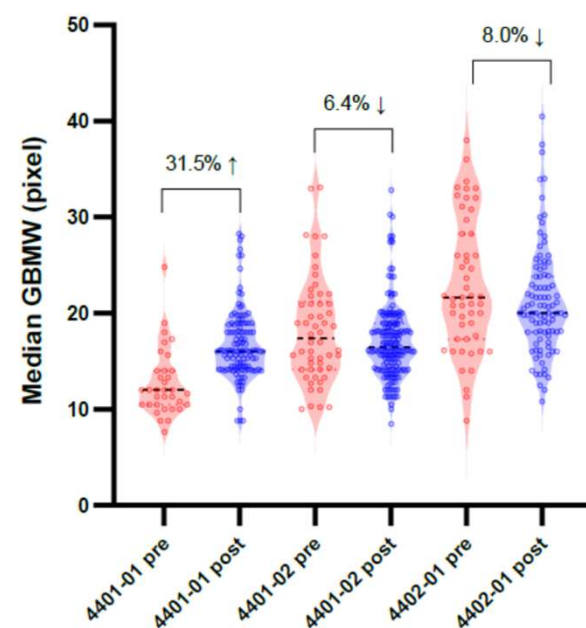
TEM Assessment using Systematic Uniform Random Sampling (analysis blinded to treatment order)

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%

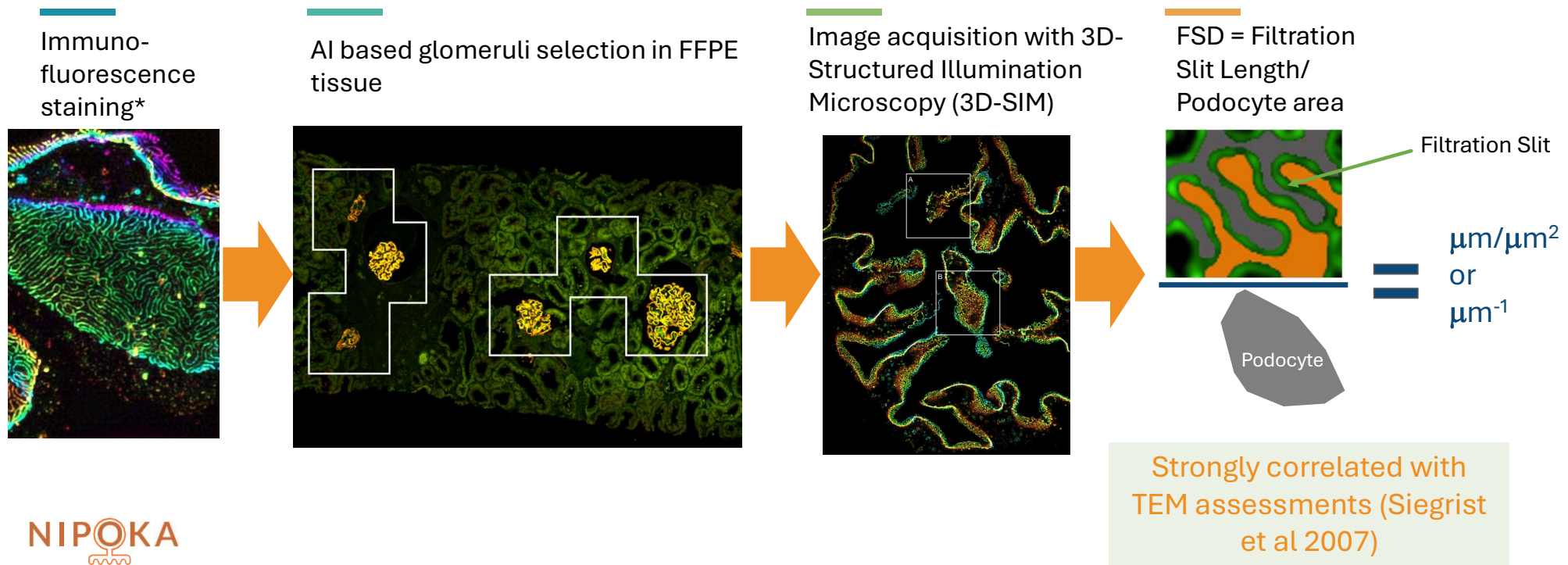
-23.9%

-13.1%



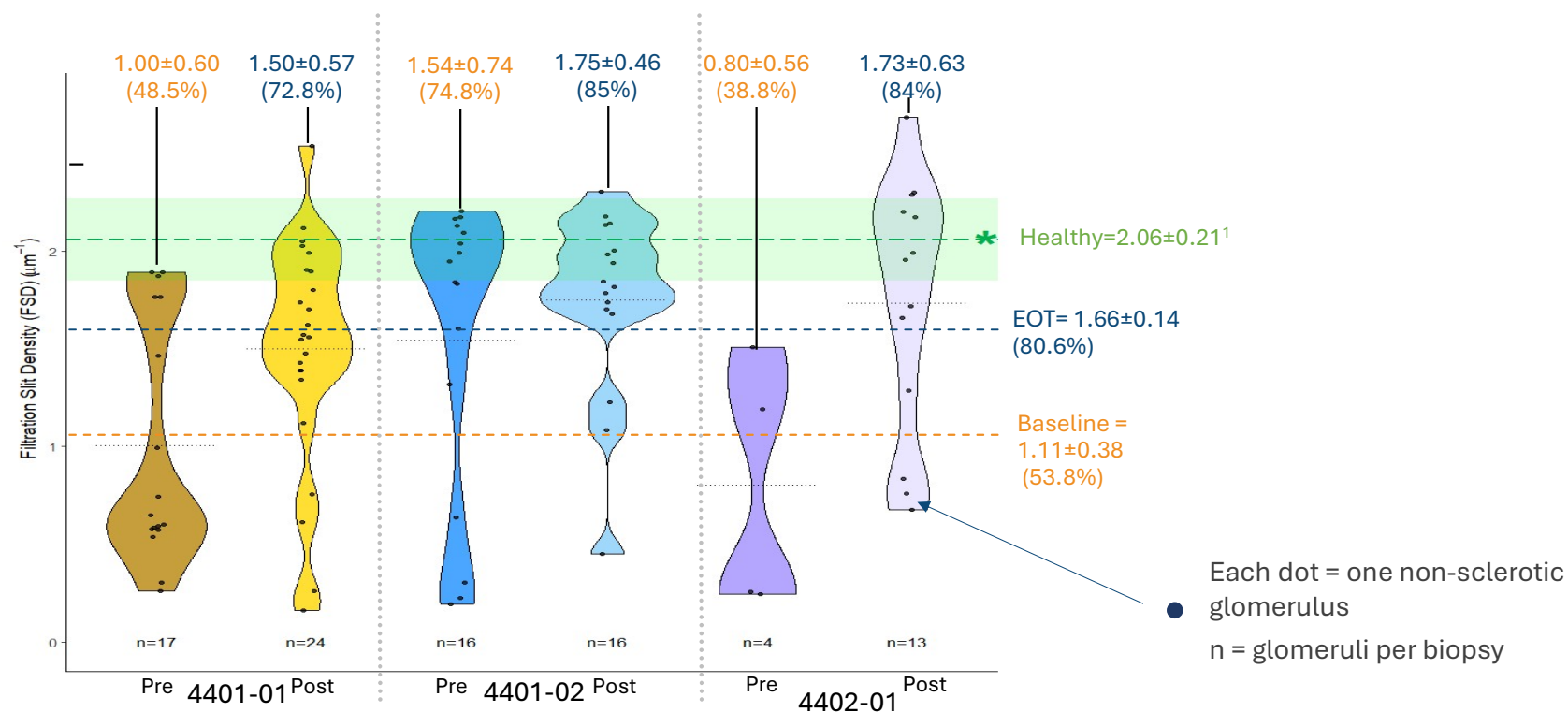
FPE assessed by change in Filtration Slit Density (FSD)

Unbiased assessment of filtration slit density



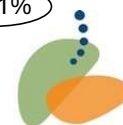
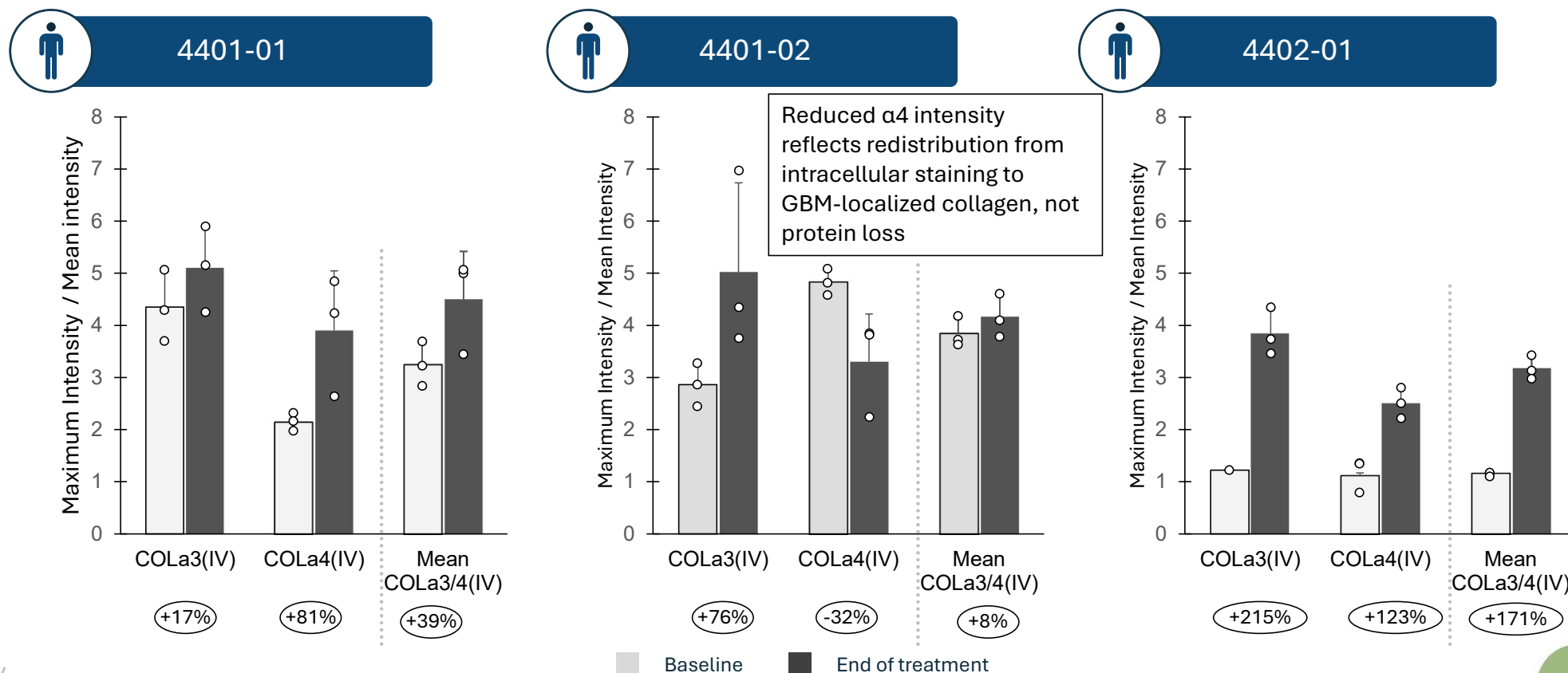
Phase 2a: FSD increased consistent with FPE reduction

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



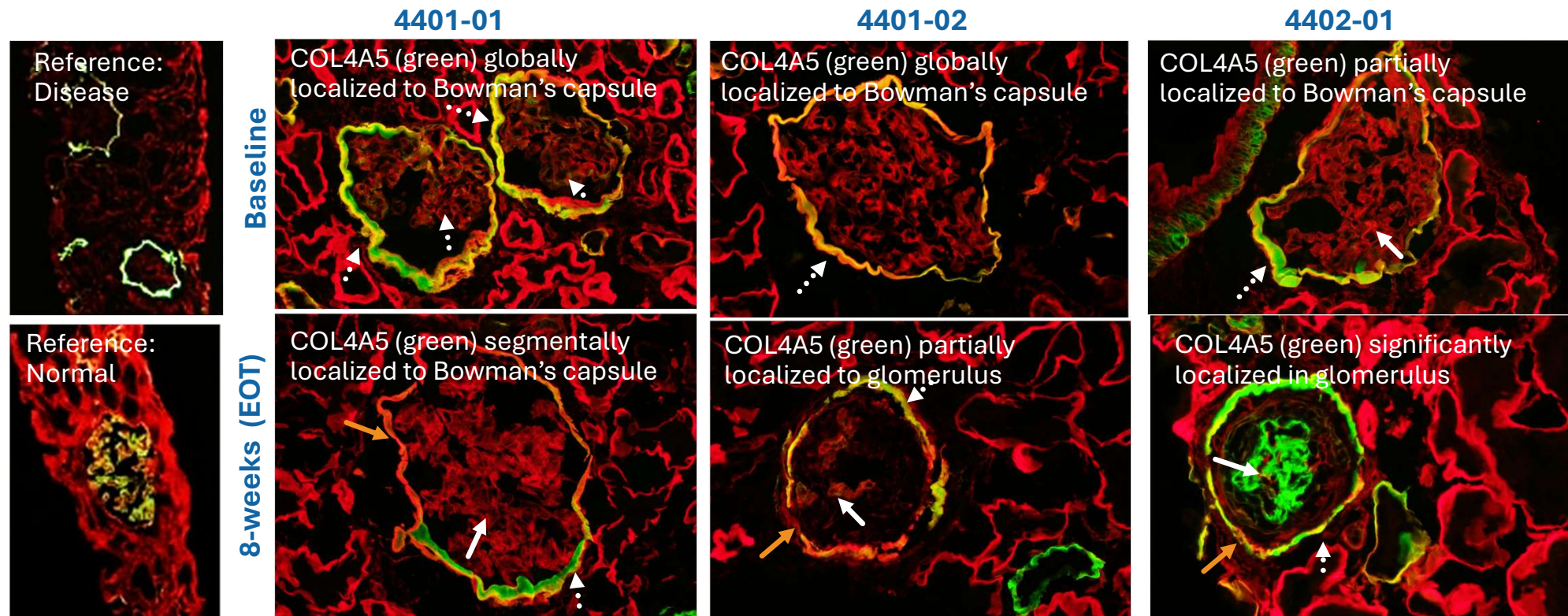
Phase 2a: Mean 72% increase in average collagen $\alpha 3$ (IV) and $\alpha 4$ (IV) across all patients

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



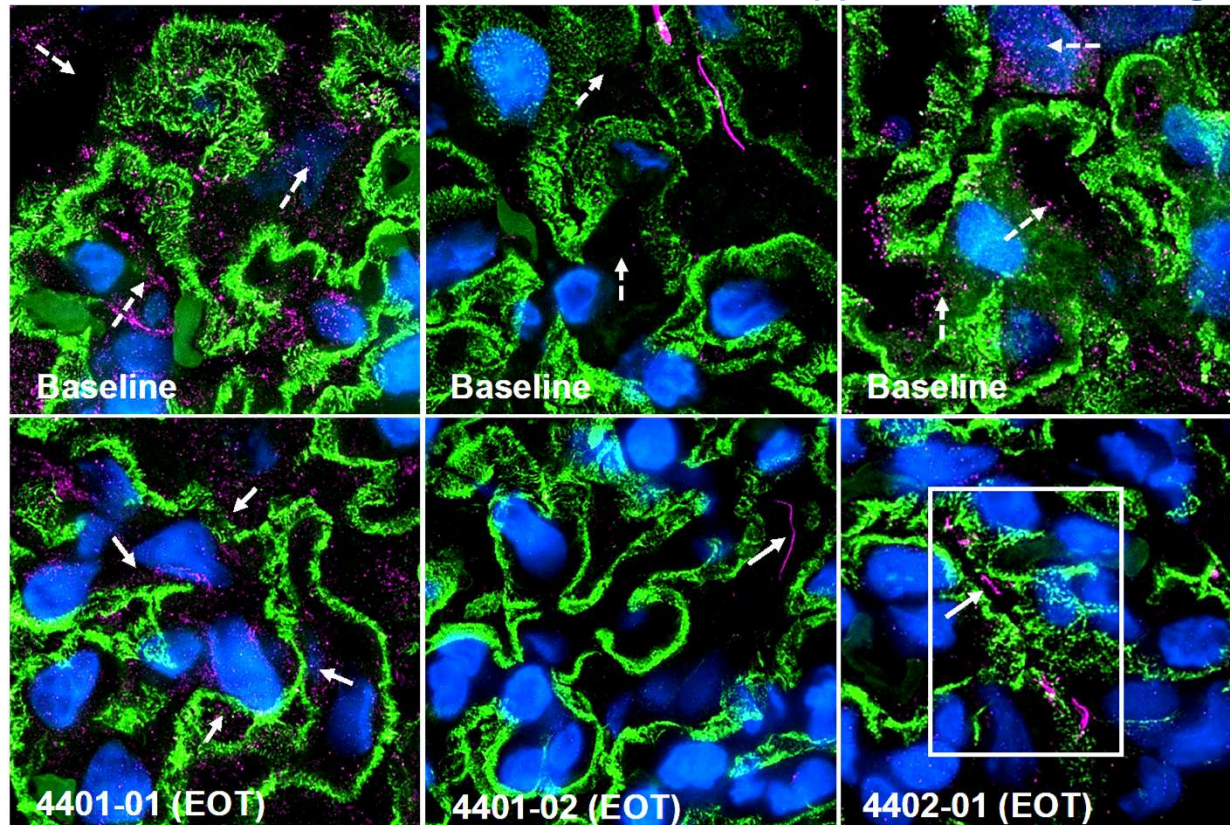
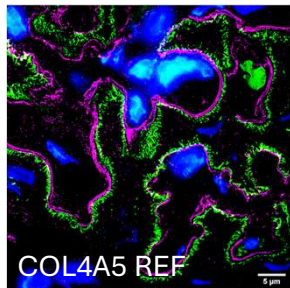
Phase 2a: Collagen $\alpha 5(\text{IV})$ GBM localization consistent with collagen IV formation

COL4A5 immunofluorescence staining in fresh frozen tissue biopsies

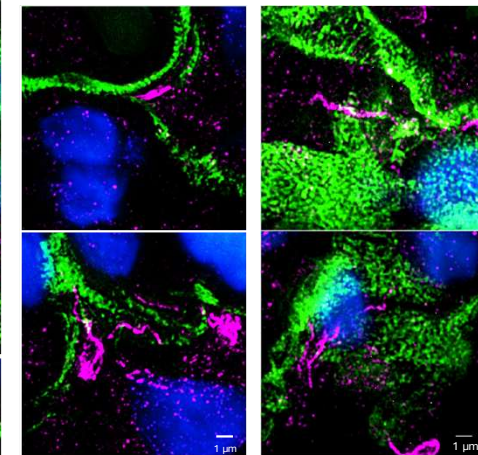


Phase 2a: Collagen $\alpha 5(\text{IV})$ localization and evidence of trimer formation consistent after eight weeks (n=3)

Collagen $\alpha 5(\text{IV})$ 3D-Structured Illumination Microscopy immunostaining



4402-01 (EOT) - Detail



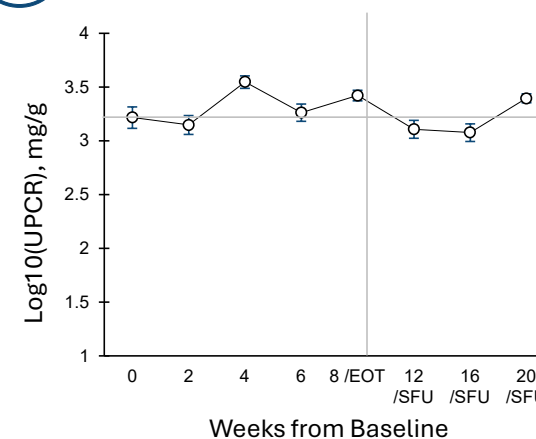
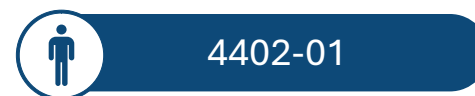
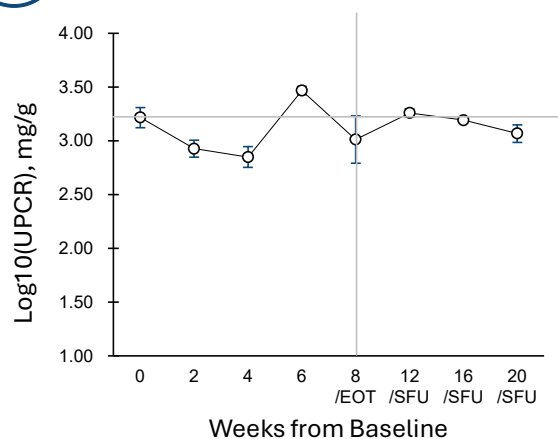
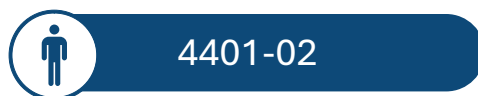
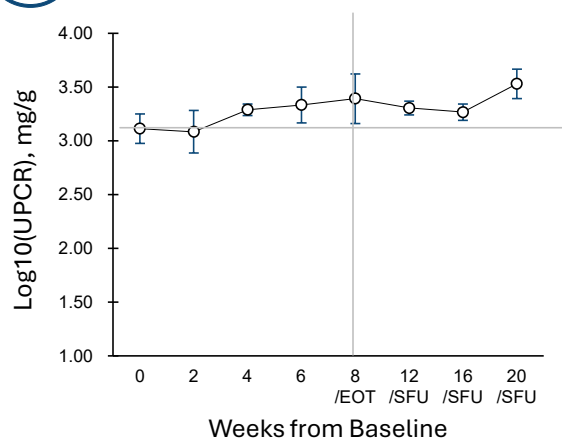
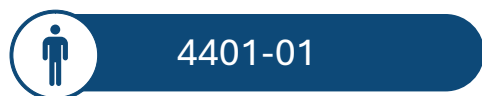
NIPOKA



UPCR during treatment period

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

..... Baseline UPCR



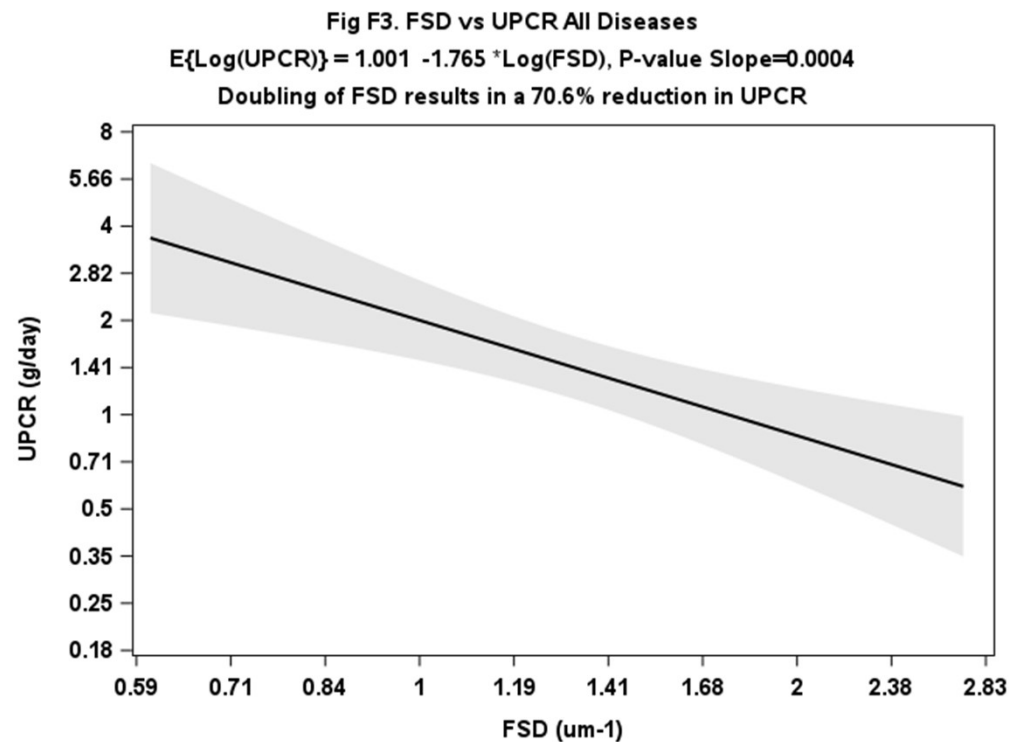
* Urine sample assessments based on the collection of second void at each day after discarding the first morning void. UPCR measurements taken on 1-day prior to and visit day during study period. Baseline values based on UPCR values from screening, baseline and Day 1 visits. Averages based on geometric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

** UPCR at 48 weeks in placebo arm of 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



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

ESTABLISHED PRECEDENT

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



ASSENT → Alport

OUR OPPORTUNITY

- 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



The EXACT Study: 2b placebo-controlled NMAS study planned with FSD primary endpoints

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



Phase 2b

Randomized, double-blind

Placebo-controlled · delayed-start

N = 24 · age ≥ 12

▲ Kidney biopsy

PART 1 — 16 weeks · randomized 2:1

PART 2 — 16 weeks · all exaluren

Arm A

Screening
≤6 wks

Exaluren 0.75 mg/kg SC daily (n=16)

Exaluren (all n=24)

Follow-up
4 wks/OLE

Arm B

Placebo (n=8)

Exaluren

Screening

Week 16

Week 32

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

Key secondary endpoint
UPCR and additional clinical / safety
measures.



Advancing exaluren in nonsense mutation Autosomal Dominant Polycystic Kidney Disease

Healthy Kidney



Healthy kidney

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PKD1 or PKD2
nonsense mutation



Loss of polycystin
complex

ADPKD Kidney



Polycystic kidney

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Structural consequence

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

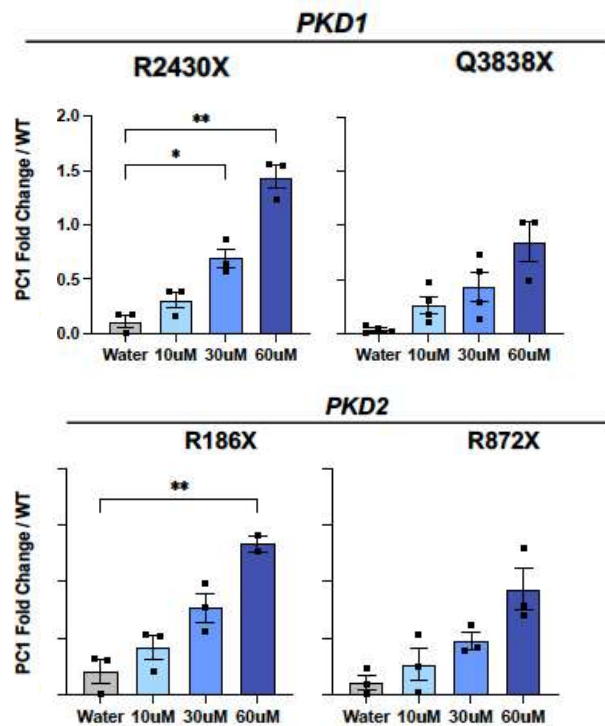
Irreversible progression to ESRD, median age ~55 to 80 years (20 years longer for missense-mutation ADPKD) • No approved gene targeted therapies



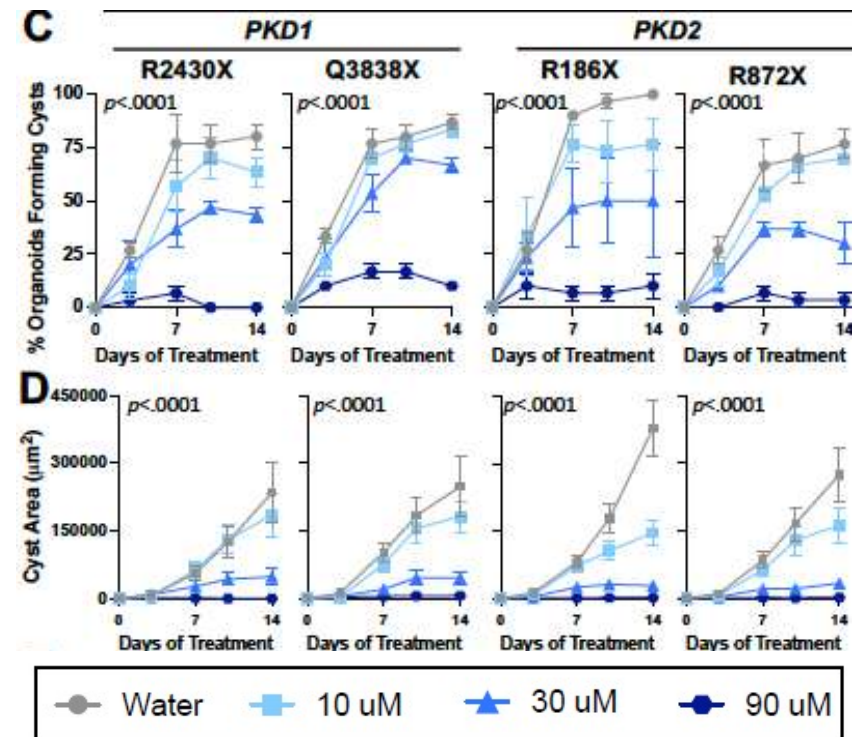
Exaluren restored 10 to 75% of PC1 and reduced cysts by ~50% in kidney organoids

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 ELX-02 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.

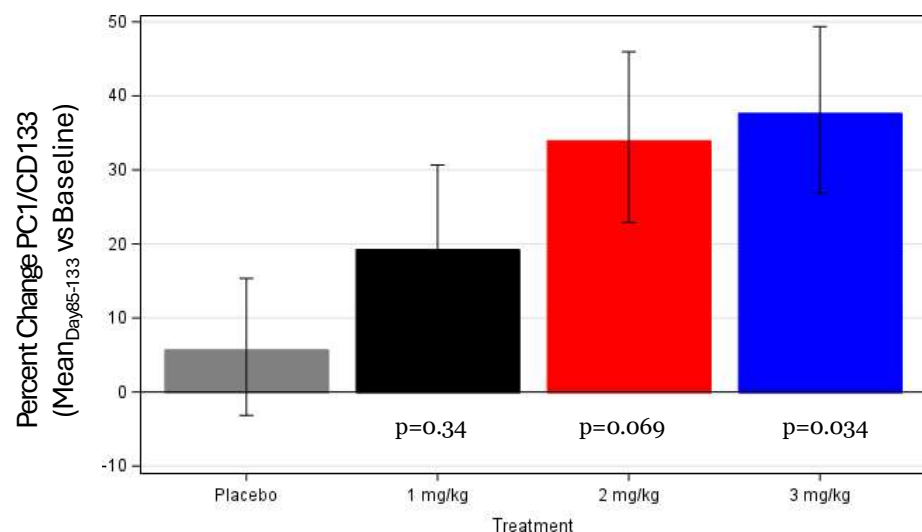
**C) fold-change in cyst area relative to starting cyst area at day 0. n=30 organoids pooled from 3 independent trials each with 10 organoids per condition. Mean±SEM. Nonlinear regression extra-sum-of-squares F test p-values as indicated; D) Percentage of cysts with fold change less than 1. n=3 independent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



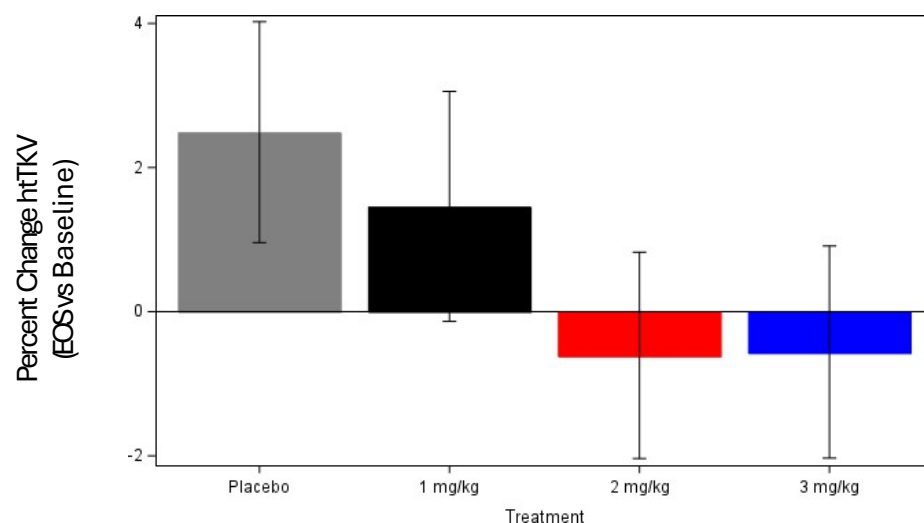
Regulus clinical data link partial PC1 restoration with reduced htTKV growth

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.



Eloxx is the most advanced independent ADPKD asset

Comparison of ADPKD programs in clinical development

Company / asset	Acts on	ADPKD evidence	Development stage	Addressable US population ¹
Novartis (Regulus) farabursen²	Wild-type allele PKD1 and PKD2. Indirect miR-17 inhibition	Clinical Urinary PC1/PC2 increased; htTKV growth reduced Phase 1b, n≈10/arm	Phase 1a/1b	~140K (100%)
PYC PYC-003³	Wild-type allele PKD1 only. Amplifies the remaining WT allele	Preclinical 1.7× PC1 over untreated, one heterozygous line - Reduced cyst volume in patient derived 3D cyst assay	Phase 1a SAD only	>100K (~71%)
Vertex VX-407⁴	Misfolded mutant (PKD1). Small-molecule corrector	Not disclosed	Phase 1 (undisclosed)	~12K (~10%)
Eloxx exaluren⁵	Nonsense mutant (PKD1 + PKD2). PTC Readthrough	Preclinical 10-7% Wild type PC1 in 4 homozygous nonsense kidney organoids; ~50% cyst reduction - Primary patient epithelial cells: phenotype correction Clinical - Proof of mechanism and structural change in Alport trial	145 subjects, Ph 1+2	~40–50K (~29–36%)

¹ US diagnosed ADPKD ~140,000: Willey C et al. Kidney Dis (Basel) 2019;5:107–17.

² Regulus Therapeutics corporate presentation, Nov 2024; WCN26 abstract 7987. Regulus acquired by Novartis — Novartis media release, 30 April 2025 (\$800M upfront plus up to \$900M in contingent value rights).

³ PYC Therapeutics corporate presentation, 13 Jan 2026;

⁴ Vertex press releases and R&D pipeline, 2024–2026;

⁵ Preclinical: Vishy CE et al. Cell Stem Cell 2024 (PKD1 R2430X and Q3838X; PKD2 R186X and R872X; isogenic controls; homozygous nonsense clones; PC1 quantified relative to WT abundance, Primary patient cells: Sayer laboratory data on file, 2026 (patient-derived hURECs). Genotype frequency per Cornec-Le Gall E et al. J Am Soc Nephrol 2013;24:1006–13;.



Shared mechanism with clinical proof of concept supports Alport and ADPKD programs

Summary

Evidence		Nonsense Mutation Alport Syndrome	Nonsense Mutation ADPKD
	Platform	✓ Gene-agnostic PTC readthrough	✓ Same Mechanism
	Protein restoration	✓ +72% Collagen $\alpha3/\alpha4$ (IV). Functional Collagen $\alpha3,\alpha4,\alpha5$ (IV) precursor formed in patients	✓ +10% to +75% of wild-type Polycystin-1 in kidney organoids
	Structural change	✓ Reduced FPE, Improved GBM structure.	✓ Cyst area and growth reduction in kidney organoids after 14 days
	In vivo	✓ COL IV restoration (non-exaluren) improves proteinuria and survival ¹	✓ Restoration of polycystin activity is disease-modifying on a protein dosage dependent basis (non-exaluren) ²
	Clinical	✓ FSD increased to 80.6% of healthy in three treated patients after 8 weeks	✓ 30% to 40% increase in PC1 reduced htTKV growth in farabursen Phase 1b ³

¹ Non-exaluren studies: Yamamura et al. 2020: treatment of Col4a5 mutant mouse with an ASO; LeBleu et al. 2009: stem cell therapies in Col4a3 knockout mice; Lin et al. 2014

² Non-exaluren studies: Dong et al. 2021; re-expression of Pkd2 in ADPKD mouse; Kurbegovic et al. (2023) Pkd1 gene transfer in Pkd1 null mice; Hopp et al. 2012

³ Non exaluren studies: Regulus Therapeutics Corporate presentation November 2024



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. 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, Tetrphase Pharmaceuticals, Zikani Therapeutics

John Woolford

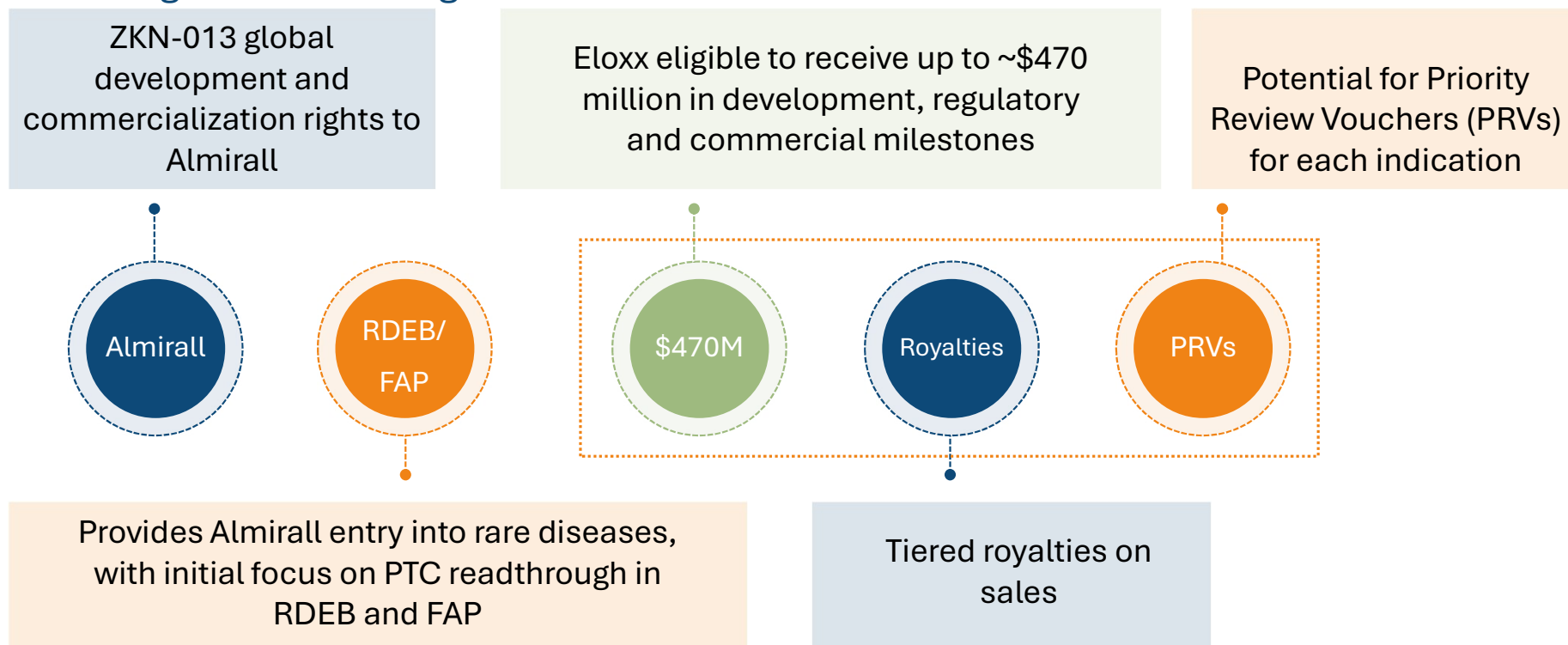
Vice President, Corporate Development

- 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



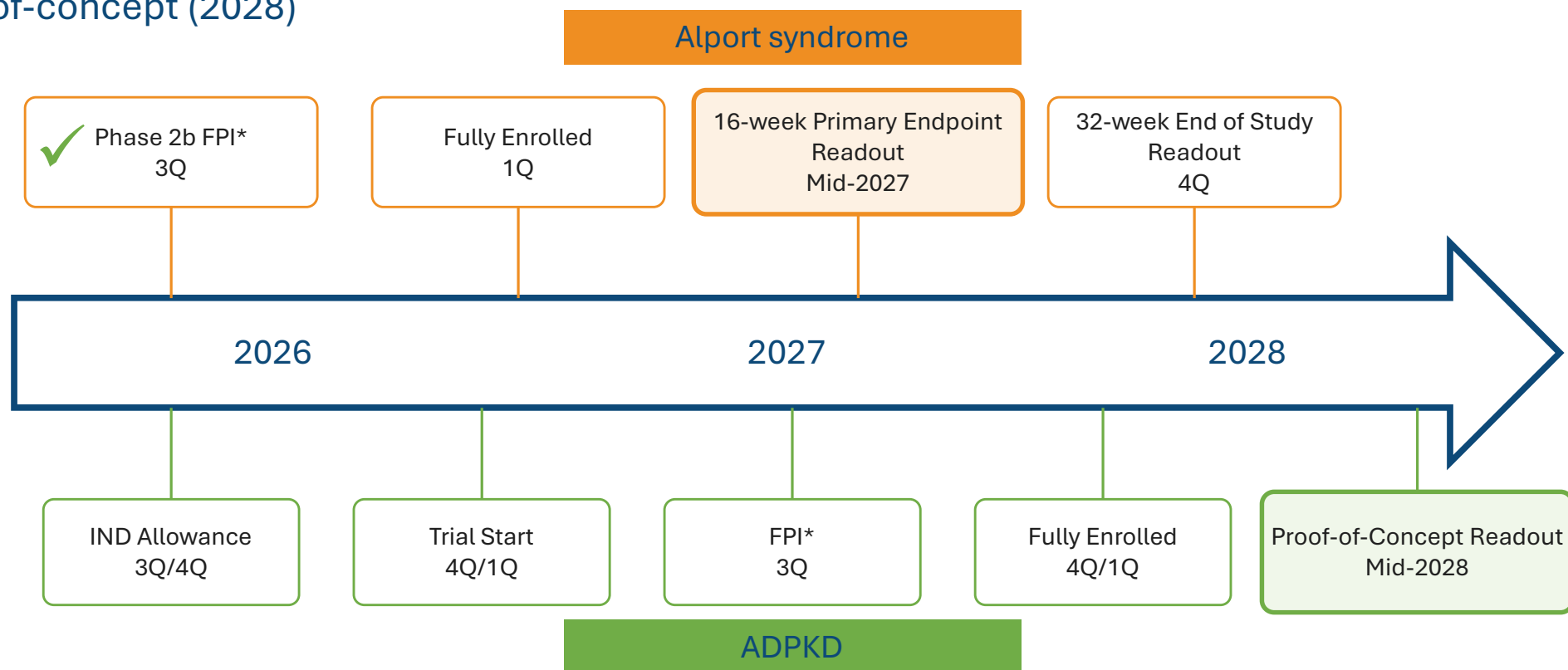
Demonstrated ability to execute high value pharma collaborations

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



Multiple high impact clinical readouts anticipated from 2026 - 2028

Anticipated key value inflection points: Phase 2b primary endpoint (2027) and ADPKD proof-of-concept (2028)



June 2026 follow-on provides expected runway to Alport and ADPKD readouts

Balance sheet (as of June 30, 2026)

- Cash of \$62.0M
- Runway to mid-2028
- 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. Proof-of-Concept Phase 2 in ADPKD readout expected in mid 2028*



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

*Assuming trial enrollment in 2027 and subject to finalization and Investigational New Drug application clearance by the FDA.





INVESTOR RELATIONS

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