



Vertex Announces Positive Results From Phase 2b AMPLIFIED Study of Inaxaplin in Additional Populations of People With APOL1-Mediated Kidney Disease (AMKD) and Completion of Enrollment in the Phase 2/3 AMPLITUDE Trial

September 22, 2026

- In people with AMKD with modest proteinuria, treatment with inaxaplin led to a reduction in urine albumin to creatinine ratio (UACR) of -42.7% at Week 13 compared to baseline –
- In people with AMKD and type 2 diabetes, treatment with inaxaplin led to a reduction in UACR of -17.3% at Week 13 compared to baseline –
- Inaxaplin was generally safe and well tolerated in both cohorts –
- Vertex has completed enrollment in the AMPLITUDE trial and remains on track for interim analysis results in early 2027, which, if positive could support potential accelerated approval in the US -

BOSTON--(BUSINESS WIRE)--Sep. 22, 2026-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced positive results from the Phase 2b AMPLIFIED study of inaxaplin in people with APOL1-mediated kidney disease (AMKD) and modest proteinuria and in people with AMKD and type 2 diabetes.

In this study, inaxaplin was evaluated as a 45 mg once-daily dose on top of optimized standard of care. Forty-one people were enrolled and dosed in two cohorts: 1) people with AMKD with modest proteinuria (UACR ≥ 0.1 g/g to < 0.42 g/g) were eligible for cohort 1 (N=23), and 2) people with AMKD, type 2 diabetes and proteinuria (UACR ≥ 0.1 g/g to < 6 g/g) were eligible for cohort 2 (N=18). The primary endpoint in the study was mean percent change in UACR at Week 13 compared to baseline, evaluated separately for each cohort. The other endpoints included mean percent change in urine protein to creatinine ratio (UPCR) at Week 13 compared to baseline and safety.

In cohort 1, treatment with inaxaplin administered on top of optimized standard of care led to a reduction of -42.7% (95% CI -58.3%, -21.1%) in UACR at Week 13 compared to baseline. UPCR decreased by -44.7% from baseline at Week 13. These results are in line with the treatment effect previously reported in the Phase 2a study of inaxaplin in AMKD with focal segmental glomerulosclerosis (FSGS), which demonstrated a reduction in UACR of -43.4% and a reduction in UPCR of -47.6% at Week 13 compared to baseline. In this modest proteinuria cohort, most people were not diagnosed with FSGS.

In cohort 2, treatment with inaxaplin administered on top of optimized standard of care led to a reduction in UACR at Week 13 of -17.3% (95% CI -36.3%, 7.2%) compared to baseline. UPCR decreased by -25.4% from baseline at Week 13.

Efficacy Results for AMPLIFIED Phase 2b Study by Cohort

	Cohort 1: AMKD with modest proteinuria N=22*	Cohort 2: AMKD with type 2 diabetes N=17*
Primary Endpoint		
Mean baseline UACR g/g (standard deviation)	0.28 (0.09)	0.67 (0.87)
Mean % change (geometric) of UACR at Week 13 compared to baseline** (95% confidence interval)	-42.7% (-58.3%, -21.1%) #	-17.3% (-36.3%, 7.2%) #
Other Endpoint		
Mean baseline UPCR g/g (standard deviation)	0.46 (0.20)	1.12 (1.51)

Mean % change (geometric) of UPCR at Week 13 compared to baseline** (95% confidence interval)	-44.7% (-60.9%, -21.9%)#	-25.4% (-45.1%, 1.4%)#
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*According to the pre-specified statistical analysis plan, two people, one in each cohort, who were noncompliant with treatment, were not included in the efficacy analyses.

**Analyses include those on treatment who had an assessment at Week 13: 18 and 14 people in cohort 1 and 2, respectively.

#95% CI were generated post-hoc

Inaxaplin was generally safe and well tolerated across both cohorts. There were no serious adverse events (SAEs) related to inaxaplin. All adverse events (AEs) were mild or moderate in severity. The most common AE (occurring in >5% of subjects) was headache (7.3%). Five people had isolated, asymptomatic transaminase elevations, which resolved.

"These results add to the evidence base for inaxaplin and its potential as a first- and best-in-class treatment targeting the underlying cause of AMKD. The modest proteinuria cohort, which included patients with and without FSGS, delivered remarkable reductions in proteinuria consistent with the original Phase 2a study in patients with AMKD and FSGS," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "We are also excited with the treatment effect, though smaller in magnitude, in the type 2 diabetes cohort. We look forward to discussing the AMPLIFIED results with regulators in the context of the AMPLITUDE pivotal study."

"These results are very convincing," said Glenn Chertow, M.D., M.P.H., Professor of Medicine, Stanford University School of Medicine, and Chair of the Vertex APOL1 Program Steering Committee. "These patients were already well treated with foundational chronic kidney disease therapies and treatment with inaxaplin led to additional reductions in proteinuria. As someone who treats this rapidly progressing disease where unmet need is high, I am looking forward to the AMPLITUDE interim analysis and am hopeful that we'll soon have a potentially disease-specific and disease-modifying therapy to offer to patients living with AMKD."

Vertex has completed full enrollment in the Phase 2/3 AMPLITUDE study of inaxaplin in people with severe proteinuria and no other kidney disease-causing comorbidities. Vertex is on track to share data from the pre-planned interim analysis (IA) of AMPLITUDE in early 2027 once the IA cohort reaches 48 weeks of treatment. If positive, the data could form the basis for potential accelerated approval in the U.S.

About APOL1-Mediated Kidney Disease (AMKD)

AMKD is a rapidly progressing, proteinuric kidney disease caused by two variants in the *APOL1* gene. It occurs in people of African ancestry. AMKD occurs when inherited *APOL1* genetic variants cause kidney cell injury, cell death and damage to the glomeruli, which filter blood in the kidney. This leads to protein in the urine (known as "proteinuria") and decreased ability of the kidney to function, which can lead in turn to dialysis, transplant or death. AMKD affects approximately 150,000 people in the U.S. and Europe. There are no therapies currently approved for AMKD.

About AMPLIFIED

AMPLIFIED is a Phase 2, single-arm, open-label study evaluating the effect of treatment with a 45 mg once-daily dose of inaxaplin for 13 weeks to reduce proteinuria in people with AMKD with modest proteinuria, and people with AMKD and type 2 diabetes — populations not being studied in the AMPLITUDE trial. The primary endpoint is the percent change of urine albumin to creatinine ratio (UACR) from baseline.

About AMPLITUDE

AMPLITUDE is a global Phase 2/3 clinical trial designed to assess the impact of inaxaplin on kidney function and proteinuria for people living with AMKD. Inaxaplin is being evaluated as a 45 mg once-daily oral dose compared to placebo, on top of standard of care. The primary efficacy endpoint for the final analysis is estimated glomerular filtration rate (eGFR) slope in patients receiving inaxaplin compared to placebo after two years of treatment.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia, acute pain and acromegaly, and it continues to advance clinical and research programs in these areas. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including IgA nephropathy, neuropathic pain, APOL1-mediated kidney disease, primary membranous nephropathy, congenital adrenal hyperplasia, adrenocorticotrophic hormone (ACTH)-dependent Cushing's syndrome, autosomal dominant polycystic kidney disease, type 1 diabetes, generalized myasthenia gravis, and myotonic dystrophy type 1.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 16 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on [LinkedIn](#), [Facebook](#), [Instagram](#), [YouTube](#) and [X](#).

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, the statements made by Carmen Bozic, M.D., and Glenn Chertow, M.D., M.P.H., and statements about sharing data from the pre-planned interim analysis of AMPLITUDE in early 2027, expectations that, if positive, the AMPLITUDE data could form the basis for potential accelerated approval in the U.S., and expectations regarding the eligible patient population. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that data from the company's research and development programs may not support registration or further development of its compounds due to safety, efficacy, and other risks, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report and subsequent quarterly reports filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. You should not place undue reliance on these statements or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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