Phase 3 RAINIER Results Supporting Best-in-Class Potential of Povetacicept in IgA Nephropathy Published in the New England Journal of Medicine and Presented at the American Society of Nephrology Kidney Week 2026

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- Vertex is on track for the U.S. FDA Prescription Drug User Fee Act (PDUFA) date of November 30 for potential accelerated approval of povetacicept –

- Data published in NEJM and presented at ASN highlight povetacicept's unique trifecta of efficacy, favorable safety, and the simplicity of dosing at-home every 4 weeks with a low-volume autoinjector –

- Data presented at ASN include a qualitative study examining patient perspectives on current and emerging IgAN therapies, showing that 96% of participants preferred an autoinjector over a prefilled syringe, and 80% of participants preferred dosing every 4 weeks versus weekly dosing -

- New clinical studies of povetacicept underway including a study evaluating patient experience on povetacicept administered via autoinjector -

BOSTON--(BUSINESS WIRE)--Oct. 23, 2026-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the publication of Phase 3 RAINIER results for povetacicept in the *New England Journal of Medicine* (NEJM), presentation of these data at the American Society of Nephrology (ASN) Kidney Week 2026 in Denver, Colorado, and advancement of the povetacicept clinical research program in IgAN. Vertex will also host an investor event during ASN on October 23 at 6:15-7 p.m. MDT (8:15-9 p.m. EDT). Further details are below.

Data being presented include a pre-specified Week 36 interim analysis of the ongoing Phase 3 RAINIER trial of povetacicept, an engineered fusion protein and dual inhibitor of the BAFF (B cell activating factor) and APRIL (a proliferation inducing ligand) cytokines, in immunoglobulin A nephropathy (IgAN). RAINIER met its primary endpoint, with povetacicept achieving a 52.0% reduction from baseline in proteinuria at Week 36 and a statistically significant and clinically meaningful 49.8% reduction versus placebo ($P<0.0001$), while also meeting both secondary endpoints.

The Week 36 interim analysis of RAINIER demonstrated that the depth of proteinuria reduction from baseline was consistent across demographic, clinical and regional subgroups, including an unprecedented 54.2% reduction in urine protein to creatinine ratio (UPCR) versus placebo in North America. Additionally, findings showed that 42.2% of povetacicept-treated participants reached 24-hour UPCR <0.5 g/g, the threshold aligned with current Kidney Disease: Improving Global Outcomes (KDIGO) guidelines, compared with only 6.2% on placebo. Importantly, these findings were observed on top of optimized supportive care in both treatment arms, including sodium-glucose co-transporter 2 (SGLT2) inhibitor use in 65.8% of participants, which is among the highest reported of any contemporary IgAN trial.

"At just 36 weeks of treatment, povetacicept has delivered impressive efficacy results, together with favorable safety and convenient dosing at-home every 4 weeks in a low-volume auto-injector that can fit into the lives of IgAN patients, underscoring the potential of povetacicept to substantially impact and redefine expectations for disease control in IgAN," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. "We are grateful to the patients, investigators, and study teams who made RAINIER the largest and fastest-enrolling contemporary IgAN trial to date and look forward to the November 30th PDUFA target action date."

"What stands out from the RAINIER results is the important clinical validation of simultaneously targeting BAFF and APRIL to address upstream drivers and kidney injury in IgA nephropathy. Povetacicept was specifically designed as a high-potency, high-affinity TACI fusion protein with broad tissue distribution to deliver this dual inhibition in order to target upstream pathogenic immune complex production," said Richard Lafayette, M.D., Professor of Medicine (Nephrology) at Stanford University Medical Center and RAINIER Steering Committee Co-Chair, who presented the data at ASN Kidney Week. "The results observed with povetacicept are among the most compelling I have seen in IgAN and highlight the potential of povetacicept to become an important new treatment option for patients living with this chronic progressive disease."

Vertex also presented findings from a qualitative study examining patient perspectives on the device design and administration of current and emerging IgAN therapies, "Patient Perceptions of Injectable Therapies Among Adults with IgAN: A Qualitative Study" (#TH-PO0473). The study included in-depth, one-on-one interviews with 25 U.S. adults living with IgAN. Participants were shown pictures and short videos of the devices and asked to assess key treatment administration-related attributes. Findings showed that 96% of participants (24 of 25) preferred the characteristics of an autoinjector over a prefilled syringe, citing features such as the needle not being visible and elimination of manual needle insertion. Additionally, 80% of patients (20 of 25) preferred dosing every 4 weeks versus weekly dosing, with the top reasons being convenience and travel or time away from home considerations.

Vertex also announced a new clinical trial underway for povetacicept:

- PAVANT IgAN Patient Experience Study (NCT07890909), A Phase 3b, Open-label, Single-arm, Patient Experience Study of Povetacicept Injection (via Autoinjector) in Adults With Immunoglobulin A Nephropathy

Visit news.vrtx.com/kidney-congress for more information about Vertex's presence at ASN Kidney Week 2026.

Investor Event

Vertex will host an investor event and webcast on October 23 at 6:15-7 p.m. MDT (8:15-9 p.m. EDT) in Denver, Colorado, to discuss the data for povetacicept in IgAN, the Company's program in APOL1- mediated kidney disease (AMKD), and other highlights across its kidney disease portfolio.

A live webcast of the presentation and Q&A portions can be accessed through the Investor Relations section of Vertex's website at investors.vrtx.com. To ensure a timely connection, it is recommended that participants register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website in the "Investors" section.

About RAINIER

RAINIER (NCT06564142) is a global Phase 3 randomized, double-blind, placebo-controlled pivotal trial of povetacicept 80 mg administered subcutaneously every 4 weeks versus placebo on top of standard of care in 605 adults with IgAN (N=557 in main cohort, N=48 in the exploratory cohort). The trial was designed to have a pre-planned interim analysis evaluating the percent change from baseline in UPCR for the povetacicept arm versus placebo after a pre-specified number of patients reach 36 weeks of treatment. Final analysis will occur at two years of treatment, with a primary endpoint of total estimated glomerular filtration rate (eGFR) slope through Week 104. RAINIER is the largest trial conducted in IgAN and achieved full enrollment faster than any contemporary IgAN trial.

As previously reported, patients treated with povetacicept achieved a 52.0% reduction from baseline in 24-hour UPCR at Week 36, with a statistically significant and clinically meaningful reduction of 49.8% in 24-hour UPCR compared to placebo ($P<0.0001$).

The trial also met its secondary objective. For the first secondary endpoint, patients treated with povetacicept demonstrated a 77.4% reduction from baseline in serum galactose deficient IgA1 (Gd-IgA1) compared to an increase of +9.1% in the placebo group, yielding a reduction of 79.3% compared to placebo ($P<0.0001$). For the second secondary endpoint, in patients with baseline hematuria, 85.1% achieved hematuria resolution in the povetacicept treatment group compared to 23.4% in the placebo group, resulting in hematuria resolution of 61.7% compared to placebo ($P<0.0001$).

Povetacicept was generally safe and well tolerated. The majority of adverse events (AEs) were mild to moderate. The most common events (≥10% of participants in either treatment group) for the povetacicept group versus the placebo group were upper respiratory tract infection (18.3% vs 11.4%), nasopharyngitis (9.7% vs 12.4%), and injection site reactions (14.5% vs 2.2%). There were no serious adverse events (SAEs) related to povetacicept and no deaths in the trial. There were no opportunistic infections, and no discontinuations due to infections. As expected, anti-drug antibodies (ADAs) were observed; these ADAs had no impact on efficacy or the risk profile.

Results from the RAINIER trial support the Biologics License Application (BLA) for povetacicept, which has been accepted by the U.S. Food and Drug Administration (FDA) for accelerated approval in adults with IgAN. The Prescription Drug User Fee Act (PDUFA) target action date is November 30, 2026.

The RAINIER trial continues in a blinded manner until the last patient enrolled completes 104 weeks. Patients in RAINIER who have completed the Week 104 visit are eligible to enroll in the open-label extension study (NCT07807995), "A Phase 3 Open-Label Study of Long-term Treatment With Povetacicept in Adults With Immunoglobulin A Nephropathy," and receive an additional 104 weeks of treatment and follow-up.

About Povetacicept

Povetacicept is a dual inhibitor of the BAFF and APRIL cytokines, which promote B cell activation, differentiation and/or survival, and provides B cell control by inhibiting the ability of BAFF and APRIL to drive the pathogenesis of multiple immune-mediated diseases. Due to its engineered TACI domain, povetacicept has demonstrated improved binding affinity, potency, pharmacokinetics, and/or tissue distribution compared to other APRIL, BAFF, and dual BAFF+APRIL inhibitors in preclinical studies.

If povetacicept is approved by the FDA, Vertex plans to launch povetacicept as a low-volume (<0.5 mL) subcutaneous autoinjector delivered once every 4 weeks via at-home administration.

IgAN is the first of multiple indications that Vertex is exploring for povetacicept given its pipeline-in-a-product potential. There are ongoing studies including the Phase 2/3 OLYMPUS trial (NCT07204275) in primary membranous nephropathy (pMN) and the Phase 2 ETNA trial (NCT07501702) in generalized myasthenia gravis (gMG).

About IgA Nephropathy (IgAN)

IgAN is a serious, progressive, life-threatening kidney disease driven by uncontrolled autoreactive B cell activity and is the most common cause of primary glomerulonephritis, affecting approximately 330,000 people in the United States and Europe and more than 1.5 million globally. IgAN results from the deposition of circulating immune complexes, consisting of immunoglobulins and galactose-deficient immunoglobulin A (Gd-IgA1), in the renal glomerular mesangium, triggering kidney injury and fibrosis.

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, adrenocorticotropic 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 17 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 Richard Lafayette, M.D., and statements regarding expectations for the potential accelerated approval of povetacicept in the U.S., expectations for new clinical trials planned for povetacicept, expectations for the RAINIER trial, expectations for the launch of povetacicept if approved by the FDA, expectations for the anticipated benefits of povetacicept, including its best-in-class potential, and the clinical status of and expectations for the Phase 2/3 OLYMPUS trial in pMN and the Phase 2 ETNA trial in gMG. 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 clinical programs may not support registration or further development in a timely manner, or at all, due to safety, efficacy, and other risks, that the potential accelerated approval and/or the anticipated commercial launch of povetacicept may be delayed, if it occurs at all, 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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Vertex Pharmaceuticals Incorporated

Investors:
InvestorInfo@vrtx.com or
+1 617-341-6108

Media:
mediainfo@vrtx.com

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