Vertex Announces US FDA Acceptance of Biologics License Application for Accelerated Approval of Povetacicept in IgA nephropathy

June 1, 2026

- FDA assigns Prescription Drug User Fee Act (PDUFA) target action date of November 30, 2026 –

BOSTON--(BUSINESS WIRE)--Jun. 1, 2026-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the U.S. Food and Drug Administration (FDA) has accepted its Biologics License Application (BLA) submission for povetacicept, an investigational engineered fusion protein and dual inhibitor of the BAFF (B cell activating factor) and APRIL (a proliferation inducing ligand) cytokines, in adults with immunoglobulin A nephropathy (IgAN).

The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of November 30, 2026. If approved, povetacicept will become the first commercialized therapy in Vertex's emerging nephrology franchise.

"The Phase 3 RAINIER trial is the largest conducted in IgAN and achieved full enrollment faster than any contemporary IgAN trial, reflecting the significant unmet need in IgAN and our urgency to bring povetacicept to patients with this serious disease," said Nia Tatsis, Ph.D., Executive Vice President and Chief Regulatory and Quality Officer at Vertex. "With today's FDA acceptance of the BLA, we are one step closer to our goal of transforming the care of patients living with IgAN given povetacicept's potential best-in-class clinical profile, including every 4-week dosing delivered in a low-volume autoinjector."

As announced in March, the submission is supported by positive data from a pre-specified Week 36 interim analysis of the ongoing Phase 3 RAINIER trial of povetacicept in IgAN, demonstrating a statistically significant and clinically meaningful reduction in proteinuria, a key marker of kidney disease progression, compared to placebo. The RAINIER trial met its primary objective: patients treated with povetacicept achieved a 52.0% reduction from baseline in urine protein to creatinine ratio (UPCR) at Week 36, with a statistically significant and clinically meaningful 49.8% UPCR reduction compared to placebo (P<0.0001). The reduction in proteinuria was consistent across all pre-specified subgroups.

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. There were no serious adverse events (SAEs) related to povetacicept. As expected, anti-drug antibodies (ADAs) were observed; these ADAs had no impact on efficacy or the risk profile.

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

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 autoimmune 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.

Povetacicept was previously studied in IgAN in RUBY-3, an ongoing, multiple-ascending dose, multi-cohort, open label, Phase 1/2 basket study. As reported at the American Society of Nephrology Kidney Week 2025 Annual Meeting, at 48 weeks, key efficacy findings for the povetacicept 80mg cohort showed a 64% decrease from baseline in mean 24-hour UPCR, estimated glomerular filtration rate (eGFR) stabilization with change from baseline in eGFR (mean±SE) of +3.3±3.1 mL/min/1.73m^2, 90% (9/10) of participants achieving hematuria resolution (defined as a decrease to negative or small levels of urine blood in participants with baseline levels of urine blood of moderate or large by dipstick), and 53% of participants achieving clinical remission (defined as UPCR <0.5 g/g, negative hematuria, and <25% reduction in eGFR vs. baseline).

Povetacicept has received FDA Breakthrough Therapy Designation for the treatment of IgAN. It is the only dual BAFF+APRIL inhibitor in pivotal trials for multiple kidney diseases, with the ongoing Phase 2/3 OLYMPUS trial in primary membranous nephropathy (pMN). Expansion into additional indications for povetacicept is advancing with the recently initiated ETNA Phase 2 trial in generalized myasthenia gravis (gMG).

Povetacicept is an investigational agent and has not been approved by health authorities.

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 RAINIER

RAINIER (NCT06564142) is a global Phase 3 randomized, double-blind, placebo-controlled pivotal trial of povetacicept 80 mg administered subcutaneously every four weeks vs. 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 urine protein to creatinine ratio (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.

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 and acute pain, 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, 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 Nia Tatsis, Ph.D., statements regarding povetacicept becoming the first commercialized therapy in Vertex's emerging nephrology franchise, expectations for the anticipated benefits of povetacicept, including povetacicept's best-in-class potential, and Vertex's plans regarding the anticipated launch of povetacicept, the clinical status of and expectations for the OLYMPUS Phase 2/3 trial in pMN and the recently initiated ETNA Phase 2 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 regulatory approvals may not occur on the anticipated timeline, or at all,, that 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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