# FDA Grants Priority Review to Precigen's BLA for PRGN-2012 for the Treatment of Adults with Recurrent Respiratory Papillomatosis with PDUFA Target Action Date Set for August 27, 2025

Feb 25, 2025

*– Priority review reduces the BLA review timeline to 6-months and is granted to therapies that, if approved, would provide significant improvements in the treatment, diagnosis or prevention of serious conditions –*

*– If approved, PRGN-2012 would be the first and only available FDA-approved therapy for eligible patients with RRP, a rare and devastating chronic disease for which the current standard-of-care is repeated surgeries –*

GERMANTOWN, Md., Feb. 25, 2025 /PRNewswire/ -- Precigen, Inc. (Nasdaq: PGEN), a biopharmaceutical company specializing in the development of innovative gene and cell therapies to improve the lives of patients, today announced the US Food and Drug Administration (FDA) has accepted the company's biologics license application (BLA) for PRGN-2012 (nonproprietary name: zopapogene imadenovec†), an investigational AdenoVerse® gene therapy for the treatment of adults with recurrent respiratory papillomatosis (RRP). The FDA granted priority review to the BLA and set a Prescription Drug User Fee Act (PDUFA) target action date of August 27, 2025. The FDA has indicated that they are not currently planning to hold an advisory committee meeting to discuss this application.

PRGN-2012 is designed to elicit immune responses directed against cells infected with human papillomavirus (HPV) 6 or HPV 11. PRGN-2012 received Breakthrough Therapy Designation, Orphan Drug Designation, and an accelerated approval pathway from the FDA, and Orphan Drug Designation from the European Commission.

If approved, PRGN-2012 would be the first and only FDA-approved therapeutic for the treatment of adults with RRP. RRP is a rare, difficult-to-treat, lifelong neoplastic disease of the upper and lower respiratory tracts caused by infection with HPV 6 or HPV 11 that can be fatal. Currently, there is no cure for RRP and the current standard-of-care is repeated surgeries, which do not address the underlying cause of disease and are associated with significant morbidity. As a result, the cycle of recurrence and surgery continues and patients can require hundreds of lifetime surgeries.[1-7] The cumulative risk of laryngeal injury increases with each RRP surgery, particularly with patients requiring five or more lifetime surgeries.[8] There is high unmet need for a therapeutic alternative to prevent these irreversible surgery-related injuries.

The BLA is supported by data from the pivotal Phase 1/2 clinical study (NCT04724980), which were presented at the 2024 American Society of Clinical Oncology (ASCO) annual meeting and published in *The Lancet Respiratory Medicine*. The pivotal study met its primary safety and efficacy endpoints, with more than 50% of patients achieving Complete Response and more than 85% of patients experiencing a decrease in surgical interventions in the year after PRGN-2012 treatment compared to the year prior to treatment. PRGN-2012 was well-tolerated with no dose-limiting toxicities and no treatment-related adverse events greater than Grade 2. Primary endpoints included safety and Complete Response rate defined as the percentage of patients who require no RRP surgeries in the 12-month period after PRGN-2012 treatment completion. Key secondary endpoints included HPV-specific immune responses, extent of papilloma growth as measured by Derkay scoring, and quality of life as measured by Vocal Handicap Index-10 (VHI-10).

"The priority review designation is a testament to the FDA's recognition of the significant unmet need for the RRP patient population. RRP patients have never had an FDA-approved therapy, relying instead on repeated surgeries to alleviate the symptoms of RRP without addressing the underlying disease," said Helen Sabzevari, PhD, President and CEO of Precigen. "Treatment with PRGN-2012 has shown significant, durable clinical benefit. We have patients treated with PRGN-2012 who have been surgery-free for more than three years now, bringing hope for an alternative to the cycle of repeated surgeries, which carry immense risk for irreversible damage and significant morbidity. We look forward to working with the FDA over the coming months during their BLA review and hope to introduce the first FDA-approved therapeutic option to the RRP patient population, estimated at

more than 27,000 adults in the US, later this year."

**AdenoVerse®**
Precigen's AdenoVerse platform utilizes a library of proprietary adenovectors for the efficient gene delivery of therapeutic effectors, immunomodulators, and vaccine antigens designed to modulate the immune system. Precigen's gorilla adenovectors, part of the AdenoVerse library, have potentially superior performance characteristics as compared to current competition. AdenoVerse gene therapies have been shown to generate high-level and durable antigen-specific T-cell immune responses as well as an ability to boost these responses via repeat administration. Superior performance characteristics and high yield manufacturing of AdenoVerse vectors leveraging UltraVector® technology allows Precigen to engineer cutting-edge investigational gene therapies to treat complex diseases.

**Precigen: Advancing Medicine with Precision ™**
Precigen (Nasdaq: PGEN) is a dedicated discovery and clinical stage biopharmaceutical company advancing the next generation of gene and cell therapies using precision technology to target the most urgent and intractable diseases in our core therapeutic areas of immuno-oncology, autoimmune disorders, and infectious diseases. Our technologies enable us to find innovative solutions for affordable biotherapeutics in a controlled manner. Precigen operates as an innovation engine progressing a preclinical and clinical pipeline of well-differentiated therapies toward clinical proof-of-concept and commercialization. For more information about Precigen, visit www.precigen.com or follow us on LinkedIn or YouTube.

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**Cautionary Statement Regarding Forward-Looking Statements**
Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon the Company's current expectations and projections about future events and generally relate to plans, objectives, and expectations for the development of the Company's business, including the timing and progress of preclinical studies, clinical trials, regulatory approvals, commercial launches and related milestones, the promise of the Company's portfolio of therapies, and in particular its CAR-T and AdenoVerse therapies. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release. The Company has no obligation to provide any updates to these forward-looking statements even if its expectations change. All forward-looking statements are expressly qualified in their entirety by this cautionary statement. For further information on potential risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Company's most recent Annual Report on Form 10-K and subsequent reports filed with the Securities and Exchange Commission.

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†zopapogene imadenovec is the nonproprietary name for the investigational therapeutic known as PRGN-2012. Zopapogene imadenovec has not been approved by any health authority in any country for any indication.

**References**
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