Precigen Announces Full FDA Approval of PAPZIMEOS (zopapogene imadenovec-drba), the First and Only Approved Therapy for the Treatment of Adults with Recurrent Respiratory Papillomatosis

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- *PAPZIMEOS approval marks a historic milestone for the RRP patient community as the first and only FDA-approved therapy for the treatment of adults with RRP*
- *PAPZIMEOS received full approval from the FDA for the treatment of adults with RRP*; *a confirmatory clinical trial is no longer required*
- *RRP is a rare, debilitating, and potentially life-threatening disease caused by chronic HPV 6 or HPV 11 infection, which results in recurrent benign tumors in the respiratory tract; RRP affects an estimated 27,000 adult patients in the US*
- *PAPZIMEOS is a non-replicating adenoviral vector-based immunotherapy designed to generate an immune response directed against papilloma cells expressing HPV 6/11—the first and only approved therapy to treat the root cause of RRP*
- *Precigen will host a conference call on Monday, August 18 at 8:00 AM ET*

GERMANTOWN, Md, Aug. 15, 2025 /PRNewswire/ -- Precigen, Inc. (Nasdaq: PGEN), a biopharmaceutical company specializing in the advancement of innovative precision medicines to improve the lives of patients, today announced that the US Food and Drug Administration (FDA) has approved PAPZIMEOS ™(zopapogene imadenovec-drba) for the treatment of adults with recurrent respiratory papillomatosis (RRP). PAPZIMEOS is the first and only FDA-approved therapy for the treatment of adults with RRP. Precigen completed submission of the rolling Biologics License Application (BLA) in December 2024 under an accelerated approval pathway; however, the FDA has granted PAPZIMEOS full approval, which does not require a confirmatory clinical trial. PAPZIMEOS is a non-replicating adenoviral vector-based immunotherapy designed to express a fusion antigen comprising selected regions of human papillomavirus (HPV) types 6 and 11 proteins—the root cause of RRP. PAPZIMEOS is delivered via four subcutaneous injections over a 12-week interval.

RRP is a rare, debilitating, and potentially life-threatening disease of the upper and lower respiratory tract caused by chronic HPV 6 or HPV 11 infection. RRP can lead to severe voice disturbance, a compromised airway, and recurrent post-obstructive pneumonias. Management of RRP has primarily consisted of repeated surgeries, which do not address the root cause of the disease and can be associated with significant morbidity as well as significant patient and health system burden.

"For more than a century, since RRP was first recognized as a distinct disease, patients have had to rely on repeated surgeries to manage this relentless condition. Today marks a historic turning point. With the landmark FDA approval of PAPZIMEOS and broad label, all adult RRP patients are now eligible for access to the first and only approved therapy that targets the root cause of the disease," said Helen Sabzevari, PhD, President and CEO of Precigen. "This milestone affirms the power of our AdenoVerse platform and the exceptional capabilities of our team to rapidly advance a wholly novel therapy from discovery to approval considerably faster than industry benchmarks. We are profoundly grateful to the NIH clinicians, the FDA, and—most importantly—the patients and families who made this breakthrough possible. We look forward to swiftly delivering PAPZIMEOS to the RRP community and ushering in a new era of treatment that targets the underlying cause of the disease rather than just managing its symptoms."

"This long-awaited FDA approval represents a momentous milestone for the RRP community," said Kim McClellan, President of the Recurrent Respiratory Papillomatosis Foundation. "For the first time, adult patients with RRP have access to an FDA-approved therapy that offers the potential to reduce—or even eliminate—endless repeated surgeries. This breakthrough brings long-overdue hope to patients and families who have endured so much. We are deeply grateful to the teams at Precigen and the NIH, and above all, to the patients and caregivers whose courage, advocacy, and perseverance have made this historic moment possible."

The approval is supported by data from the open-label, single-arm, pivotal study in adult patients with RRP:

- The pivotal study successfully met its primary safety and pre-specified primary efficacy endpoints.
- 51% (18 out of 35) of study patients achieved Complete Response, requiring no surgeries in the 12 months after treatment with PAPZIMEOS. These Complete Responses remained durable for over 12 months. Of the 18 patients with a Complete Response in the ongoing study, 15 patients evaluated at 24 months demonstrated continued Complete Response.
- PAPZIMEOS was well-tolerated with no dose-limiting toxicities and no treatment-related adverse events greater than Grade 2.
- PAPZIMEOS induced HPV 6/11-specific T cell responses in RRP study patients with a significantly greater expansion of peripheral HPV-specific T cells in responders compared with non-responders.

The pivotal study was led by lead investigators, Clint T. Allen, MD, and Scott M. Norberg, DO, at the National Institutes of Health. Pivotal data were presented at the 2024 American Society of Clinical Oncology (ASCO) annual meeting and published in *The Lancet Respiratory Medicine*.

Precigen will begin promoting PAPZIMEOS immediately and is committed to helping patients with RRP access the therapy. Precigen has established Papzimeos SUPPORT, a comprehensive patient support program offering personalized services, including insurance navigation, financial assistance, and ongoing access support, which can be accessed by calling 866-827-8180. Healthcare professionals interested in learning more about PAPZIMEOS or accessing provider support services are encouraged to visit www.PAPZIMEOS.com.

Conference Call
The Company will host a conference call on Monday, August 18 at 8:00 AM ET to provide additional details regarding the approval, including key aspects of the label and commercialization. Event details can be found on Precigen's website in the Events & Presentations section at investors.precigen.com/events-presentations.

About RRP
RRP is a rare, debilitating, and potentially life-threatening disease of the upper and lower respiratory tract caused by chronic HPV 6 or HPV 11 infection. RRP can lead to severe voice disturbance, compromised airway, and recurrent post-obstructive pneumonias. Although rare, RRP has the potential for transformation to malignant cancer and can be fatal. Management of RRP has primarily consisted of repeated surgeries, which do not address the underlying cause of the disease and can be associated with significant morbidity as well as significant patient and health system burden. As the number of lifetime surgeries increases, the risk for irreversible iatrogenic laryngeal injury increases with each surgery, and patients may undergo hundreds of these surgeries over their lifetimes. RRP can impact patients' work and social lives, financial stability, and mental health. Patients with RRP can experience substantial impacts to daily living with decreased quality of life and high health care utilization. Based on an internal analysis of claims data and electronic health records, there are approximately 27,000 adult RRP patients in the US.

About PAPZIMEOS ™ (zopapogene imadenovec-drba), for subcutaneous injection only
PAPZIMEOS is the first and only FDA-approved therapy for the treatment of adults with RRP and the first and only approved therapy to address the root cause of RRP. PAPZIMEOS is a non-replicating adenoviral vector-based immunotherapy designed to express a fusion antigen comprising selected regions of human papillomavirus (HPV) types 6 and 11 proteins. PAPZIMEOS is designed to generate an immune response directed against HPV 6 and HPV 11 proteins in patients with RRP. Discovered and designed in Precigen's labs using Precigen's proprietary AdenoVerse therapeutic platform, PAPZIMEOS represents a new therapeutic paradigm for RRP.

Indication and Important Safety Information

What is PAPZIMEOS?
PAPZIMEOS is a type of immunotherapy used to treat a condition called recurrent respiratory papillomatosis (RRP) in adults.

What is the most important information I should know about PAPZIMEOS?
Some people may have a reaction to the shot. Signs and symptoms may include redness, pain, swelling, itching, or warmth where the shot was given. After your first treatment, your healthcare provider will watch you for at least 30 minutes to make sure you're feeling okay.

Please contact your doctor immediately if you develop an infection, the reaction to your shot worsens, or you experience any of the below symptoms, which may indicate a systemic allergic reaction:

- Difficulty breathing
- Widespread rash
- Facial swelling

Thrombotic events (blood clots that block your blood vessels) may occur after your PAPZIMEOS shot. Please notify your doctor immediately if you have the following symptoms:

- Shortness of breath
- Chest pain
- Leg swelling
- Persistent abdominal pain
- Severe or persistent headaches
- Blurred vision

What should I know before taking PAPZIMEOS?
Before taking PAPZIMEOS, tell your healthcare provider about all of your medical conditions, including:

- If you are pregnant or plan to become pregnant because it is not known if PAPZIMEOS will harm the unborn baby.
- If you are breastfeeding or plan to breastfeed. It is unknown if PAPZIMEOS is present in breast milk, or how it affects the breastfeeding child or milk production. Talk to your healthcare provider about the best way to feed your baby during treatment with PAPZIMEOS.

What are the most common side effects of PAPZIMEOS?
The most common side effects include:

- Pain, redness, or swelling where the shot was given
- Feeling tired
- Chills
- Fever
- Muscle aches
- Nausea (feeling sick)
- Headache
- Increased heart rate
- Diarrhea
- Vomiting
- Sweating a lot

These are not all of the possible side effects of PAPZIMEOS. Call your healthcare provider for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088. You may also report side effects to Precigen, Inc. at 1-855-PGE-NRRP (1-855-743-6777).

Please see full Prescribing Information**.**

Precigen: Advancing Medicine with Precision®
Precigen (Nasdaq: PGEN) is a biopharmaceutical company specializing in the advancement of innovative precision medicines to address difficult-to-treat diseases with high unmet patient need. Precigen is dedicated to advancing scientific breakthroughs from proof-of-concept through commercialization. With a strong commitment to innovation, Precigen is developing a robust pipeline of differentiated therapies across its core therapeutic areas of immuno-oncology, autoimmune disorders, and infectious diseases. 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
This press release contains "forward-looking" statements within the meaning of the safe harbor provisions of the US Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "anticipate," "intend," "plan," "goal," "seek," "believe," "project," "estimate," "expect," "strategy," "future," "likely," "may," "should," "will" and similar references to future periods. These statements are subject to numerous risks and uncertainties that could cause actual results to differ materially from what the Company expects. Examples of forward-looking statements include, among others, information relating to the Company's business and business plans, the success of efforts to commercialize PAPZIMEOS™ (zopapogene imadenovec-drba) for the treatment of recurrent respiratory papillomatosis (RRP) in adults, the Company's ability to successfully obtain foreign regulatory approvals for PAPZIMEOS, expectations about the safety and efficacy of PAPZIMEOS and the Company's other product candidates, the timing of clinical trials and their results, the Company's ability to commence clinical studies or complete ongoing clinical studies, and the ability of PAPZIMEOS to treat RRP. 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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