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Precigen Receives FDA Orphan Drug Designation for PRGN-2012 AdenoVerse™ Immunotherapy in Patients with Recurrent Respiratory Papillomatosis (RRP)

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- RRP is a rare, difficult-to-treat and sometimes fatal neoplastic disease of the upper and lower respiratory tracts caused by human papillomavirus type 6 (HPV 6) or HPV type 11 (HPV 11) -

GERMANTOWN, Md., March 18, 2021 /PRNewswire/ -- Precigen, Inc., a biopharmaceutical company specializing in the development of innovative gene and cell therapies to improve the lives of patients, today announced that the US Food and Drug Administration (FDA) has granted orphan drug designation (ODD) for PRGN-2012, a first-in-class, investigational off-the-shelf (OTS) AdenoVerse™ immunotherapy for recurrent respiratory papillomatosis (RRP). In January 2021, Precigen announced the [clearance of the IND](#) to initiate a Phase I study of PRGN-2012 AdenoVerse™ immunotherapy in adult patients with RRP (clinical trial identifier: [NCT04724980](#)). PRGN-2012 is under development through a Cooperative Research and Development Agreement (CRADA) with the Center for Cancer Research (CCR) at the National Cancer Institute (NCI), which is part of the National Institutes of Health (NIH).



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Orphan drug designation is granted by the FDA to drugs intended to treat rare diseases or disorders that affect fewer than 200,000 people in the US. The sponsor of a drug that receives the designation may qualify for a number of incentives that help to expedite and reduce the cost of development, approval and commercialization, such as grants, clinical design support, FDA fee waivers, tax incentives, and seven years of marketing exclusivity.

"This regulatory designation underscores the critical medical need for new therapies to treat this devastating and sometimes fatal disease," said Helen Sabzevari, PhD, President and CEO of Precigen. "As the first regulatory designation for our proprietary AdenoVerse platform, this orphan drug designation will help to advance PRGN-2012 with important incentives that support delivery of this medicine as rapidly as possible to patients suffering from RRP."

PRGN-2012 is an innovative therapeutic vaccine with optimized antigen design that uses gorilla adenovector technology, part of Precigen's proprietary AdenoVerse platform, to elicit immune responses directed against cells infected with HPV 6 or HPV 11. Gorilla adenovectors have numerous advantages, including the ability for repeat administration, the inability to replicate *in vivo*, which may improve safety, and the ability to deliver large payload capacity. In preclinical models, PRGN-2012 has demonstrated strong and specific immune response against HPV 6 and HPV 11.

For patients interested in enrolling in this clinical study, please call NCI's toll-free number 1-800-4-Cancer (1-800-422-6237) (TTY: 1-800-332-8615), email NCIMO_Referrals@mail.nih.gov, and/or visit the website: trials.cancer.gov.

About Recurrent Respiratory Papillomatosis (RRP)

Recurrent respiratory papillomatosis (RRP) is a rare, difficult-to-treat and sometimes fatal neoplastic disease of the upper and lower respiratory tracts that is caused by infection with HPV 6 or HPV 11.¹⁻⁴ RRP is classified based on age of onset as juvenile or adult. Juvenile-onset disease has an incidence of 4 per 100,000 and adult-onset RRP has an incidence of 2 to 3 per 100,000. There is no cure for RRP and the current standard-of-care is repeated endoscopic debulking with ablation or excision of papillomatous lesions.^{3,4} Recurrence of papilloma after surgical removal is very common and repeated procedures are required to debulk and monitor the disease, which exposes patients to anesthetic and surgical risks, and emotional distress. RRP morbidity and mortality results from the effects of papilloma mass on the vocal cords, trachea, and lungs, which may cause voice changes, stridor, airway occlusion, loss of lung volume, and/or post-obstructive pneumonia.⁵ Although rare, one to three percent of RRP cases can transform into invasive squamous cell carcinoma.^{6,7}

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 unique therapies toward clinical proof-of-concept and commercialization. For more information about Precigen, visit www.precigen.com or follow us on Twitter [@Precigen](https://twitter.com/Precigen) and [LinkedIn](https://www.linkedin.com/company/precigen).

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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, discovery programs and related milestones, the promise of the Company's portfolio of therapies, and in particular its CAR-T therapies, and the Company's refocus to a healthcare-oriented business. 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, including the possibility that the timeline for the Company's clinical trials might be impacted by the COVID-19 pandemic, 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.

References

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