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Precigen Receives Breakthrough Therapy Designation for PRGN-2012 AdenoVerse™ Immunotherapy for the Treatment of Recurrent Respiratory Papillomatosis

Jun 20, 2023

- First FDA Breakthrough Therapy Designation granted for AdenoVerse immunotherapy platform; designation prioritizes PRGN-2012 as a potential treatment for RRP –
- Designation based on positive Phase 1 clinical data that showed 50% of patients were "surgery-free" (Complete Response) after PRGN-2012 treatment with a minimum follow up of 12 months post-treatment –

GERMANTOWN, Md. , June 20, 2023 /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 that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for the first-in-class investigational PRGN-2012 AdenoVerse immunotherapy for the treatment of recurrent respiratory papillomatosis (RRP).



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FDA's [Breakthrough Therapy Designation](#) expedites the development and review of medicines which are intended to treat serious or life-threatening diseases, and in which preliminary clinical evidence demonstrates substantial improvement on clinically significant endpoints over available therapies.

"This Breakthrough Therapy Designation is the first for Precigen's AdenoVerse platform and recognizes the immense potential of PRGN-2012 to change the lives of patients with RRP," said Helen Sabzevari, PhD, President and CEO of Precigen. "Standard-of-care for RRP consists of repeated surgical interventions, and there are currently no approved therapeutics. The potential of PRGN-2012 to reduce surgical interventions and improve outcomes for these patients makes us incredibly proud to receive the FDA's Breakthrough Therapy Designation. This designation will enable our direct engagement with senior leadership at the FDA regarding the most efficient product development pathway, including eligibility for rolling and priority review of a BLA to support a potential PRGN-2012 registration."

PRGN-2012 incorporates optimized antigen design that uses gorilla adenovector technology, part of Precigen's proprietary AdenoVerse platform, to elicit immune responses directed against cells infected with human papillomavirus type 6 (HPV 6) or HPV type 11 (HPV 11). Gorilla adenovectors have numerous advantages, including the ability for repeat administration, the inability to replicate *in vivo*, and the ability to deliver a large genetic payload. PRGN-2012 has previously been granted [Orphan Drug Designation](#) in patients with RRP by the FDA.

The Breakthrough Therapy Designation was informed by the clinical evidence for PRGN-2012 generated in the Phase 1 study ([NCT04724980](#)), which was presented at [Precigen's most recent R&D day](#), and showed strong response at the recommended phase 2 dose (RP2D) in patients who had an average of 5.8 RRP surgeries (range 3 – 10) in the year prior to PRGN-2012 treatment. PRGN-2012 treatment resulted in 50% of patients (6 out of 12) in Complete Response, requiring no post-treatment surgeries with a minimum follow up of 12 months. PRGN-2012 treatment resulted in a reduction of surgeries in 83% (10 out of 12) patients in the 12 months following treatment. PRGN-2012 induced robust *de novo* HPV-specific T-cell immune response in RRP patients. PRGN-2012 was well-tolerated with no dose-limiting toxicities and no treatment-related adverse events greater than Grade 2. The Phase 1 clinical evidence demonstrating safety and efficacy of PRGN-2012 for the treatment of RRP patients, who have no approved therapeutic option, provides the foundation for this Breakthrough Therapy Designation.

PRGN-2012 is currently being evaluated in a Phase 2 study in adult patients with RRP. Precigen completed enrollment in the Phase 2 study with 23 patients dosed, bringing the total number of enrolled patients to 35 at the RP2D. Patient follow up is ongoing as are discussions with the FDA regarding potential rapid development paths to enable a future submission of a Biologics License Application (BLA).

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

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