Vitalgen Completes Phase III Enrollment for VGR-R01 Gene Therapy Targeting Bietti Crystalline Dystrophy

Company on track for BLA submission in H2 2026 for potential first-in-class treatment

SHANGHAI, SHANGHAI, CHINA, May 27, 2025 /EINPresswire.com/ -- Vitalgen BioPharma Co., Ltd., a clinical-stage gene therapy company, today announced the completion of patient enrollment in March 2025 for its Phase III pivotal trial (VGR-R01-301) evaluating VGR-R01, an investigational AAV-based gene therapy for Bietti Crystalline Dystrophy (BCD).

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>
> *Dr. David Wu, Business Development Director*

The multicenter trial, conducted across five leading clinical institutions in China, will evaluate the efficacy and safety of VGR-R01 in BCD patients. The therapy has received Breakthrough Therapy Designation from China's Center for Drug Evaluation (CDE) and Orphan Drug Designation from the U.S. FDA. With these regulatory designations, VGR-R01 is positioned to potentially become both a first-in-class and best-in-class treatment, with a Biologics License Application (BLA) submission targeted for the second half of 2026.

"Completing enrollment for our Phase III trial represents a pivotal milestone in bringing the first potential treatment to BCD patients who currently have no therapeutic options," said Dr. David Wu, Business Development Director at Vitalgen. "The strong safety profile and efficacy demonstrated in our Phase I/II trials, combined with our regulatory designations, underscore VGR-R01's potential to transform the treatment landscape for this devastating disease."
Positive Phase I/II Results Support Advancement

Phase I/II clinical data demonstrated that VGR-R01 improved best-corrected visual acuity (BCVA) and functional vision in low-luminance conditions. The therapy showed benefits in patients with disease duration exceeding 10 years and reported no VGR-R01-related serious adverse events.

VGR-R01 delivers a functional copy of the CYP4V2 gene to retinal pigment epithelial cells, addressing the underlying metabolic dysfunction that causes progressive vision loss in BCD patients. By restoring lipid metabolism in the retina, the therapy aims to prevent structural and

functional deterioration, offering hope for halting or slowing disease progression.

About Bietti Crystalline Dystrophy

BCD is a rare inherited retinal disorder primarily affecting East Asian populations, particularly in China, Japan, and Korea, with an estimated 80,000+ patients globally. The disease causes progressive vision loss, night blindness, and visual field constriction. No approved treatments currently exist, representing a significant unmet medical need.

About Vitalgen

Vitalgen BioPharma Co., Ltd. is a clinical-stage biotechnology company developing gene therapies for rare and common diseases. Founded in 2020 and headquartered in Shanghai, the company operates GMP-compliant facilities meeting both Chinese and U.S. regulatory standards. For more information, visit www.vitalgen.com.

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Forward-Looking Statements

This press release contains forward-looking statements regarding VGR-R01's development, including expectations for regulatory submissions and the therapy's potential benefits. These statements are based on current expectations and are subject to risks and uncertainties that could cause actual results to differ materially. Factors include, but are not limited to, clinical trial outcomes, regulatory review processes, and manufacturing capabilities. Vitalgen undertakes no obligation to update these forward-looking statements except as required by law.

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