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Andelyn Biosciences Chosen by Hubble Therapeutics to Manufacture Gene Therapy for Rare Childhood Blindness Disorder

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Andelyn's AAV CuratorÃ¢â??Â¢ Platform to power production of clinical-grade vectors for HUB-101, targeting treatment of Leber Congenital Amaurosis 16 (LCA16), a rare genetic disorder leading to early-onset blindness.



Andelyn Biosciences, Inc., a leading and patient-focused cell and gene therapy Contract Development and Manufacturing Organization (CDMO), has been selected by Hubble Therapeutics, LLC, to manufacture adeno-associated virus (AAV) using its suspension AAV Curator™ Platform to manufacture clinical grade AAV for the treatment of Leber Congenital Amaurosis 16 ("LCA16").

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LCA16 is a severe, early-onset retinal dystrophy resulting from mutations in the KCNJ13 gene, which expresses a critical ion channel in the retinal pigment epithelium cells in the retina. Children typically present with visual abnormalities within five years after birth, followed by progressive retinal degeneration during the next two decades of life, ultimately resulting in blindness. HUB-101 has received Rare Pediatric Disease Designation and Orphan Disease Designation from the FDA for this program. Andelyn will optimize and scale-up the HUB-101 process, generate GMP-grade plasmids, and produce GMP vectors that include normal copies of the KCNJ13 gene. From this partnership, Hubble Therapeutcs LLC ("HubbleTx") will be able to conduct Phase 1/2 clinical trials with the intention to demonstrate safety and efficacy in an adult patient population.

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"Over the past 18 months we have identified best-in-class partners including Andelyn Biosciences to establish a gene therapy development program for adult and pediatric patients suffering from Leber Congenital Amaurosis 16 (LCA16),"

said Jeff Sabados, President of Hubble Therapeutics LLC. Sabados continued, “Andelyn Biosciences has become a global leader in gene therapy development for ultra-rare and orphan diseases and we are grateful for their unwavering commitment toward helping Hubble Therapeutics realize our steadfast promise to support families throughout the world impacted by this devastating genetic disease.”

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Matt Niloff, Chief Commercial Officer at Andelyn Biosciences said, “We are extremely grateful for the opportunity to produce the clinical grade material for HUB-101 and be a critical part of the pathway for Hubble Therapeutics to demonstrate clinical benefit for the LCA16 program. We are thrilled that our establishment as a top clinical and commercial CDMO is helping to accelerate so many programs like HUB-101 which offer real hope to patients stricken with debilitating diseases.”

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With exceptional quality and scalable end-to-end development and manufacturing capabilities across its three facilities located in and near Columbus, Ohio, Andelyn continues to partner with premier innovator organizations like Hubble Therapeutics to enable the development of life-altering gene therapies for rare and prevalent diseases.