Charles River and ASC Therapeutics to Scale Manufacturing of Second-Generation Gene Therapy for Hemophilia A

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Expanded relationship will build upon established processes initiated in 2019 to meet ASC Therapeutics clinical program

WILMINGTON, Mass.--(BUSINESS WIRE)--Jun. 13, 2022-- Charles River Laboratories International, Inc. (NYSE: CRL) and ASC Therapeutics, a privately held biopharmaceutical company pioneering the development of transformative *in vivo* gene replacement, gene editing and allogeneic cell therapies, today announced they have agreed to manufacture ASC618, a second-generation gene therapy for hemophilia A.

Hemophilia A is caused by the lack of the blood clotting factor VIII (FVIII), a protein whose instructions are provided by F8, a gene that is mutated in people with the disease. ASC618 is designed to deliver a shortened, but optimized version of the gene to liver cells. ASC Therapeutics has received IND clearance, as well as key regulatory designations in the U.S. and Europe for its ASC618 program.

Viral Vector Manufacturing Services

Since 2019, ASC Therapeutics has worked with Charles River, previously Vigene Biosciences, to leverage the Company's industry-leading expertise in Good Manufacturing Practice (GMP)-virus manufacturing, and established processes for AAV production and purification. Together, Charles River and ASC Therapeutics have established a high-yield upstream process for AAV8 production, optimized the downstream purification methods to result in a more predictable drug product output, and fine-tuned a scalable manufacturing process in both upstream and downstream.

The collaboration will take advantage of Charles River's end-to-end CDMO capabilities, building on the Company's acquisitions of Cognate BioServices, Cobra Biologics, and Vigene Biosciences in 2021 that expanded its comprehensive cell and gene therapy portfolio to span each of the major CDMO platforms – cell therapy, viral vector and plasmid DNA production.

Approved Quotes

- "We are proud to work with ASC Therapeutics as they progress their second-generation gene therapy program for hemophilia A to clinical stage. For three years, our collaboration has relied upon a strong and transparent relationship to support a seamless transition between process development and GMP production, and we're excited to expand that work." – Kerstin Dolph, Senior Vice President, Global Biologics, Charles River
- "Our work with Charles River has a strong foundation of trust—strengthening our shared outlook for the future and building confidence in our internal processes and oversight of external capabilities. As we moved toward the next phase of our therapeutic development, we are excited to work with Charles River to further maximize our manufacturing know-how." -- Ruhong Jiang, PhD, Chief Executive Officer, ASC Therapeutics

About Charles River

Charles River provides essential products and services to help pharmaceutical and biotechnology companies, government agencies and leading academic institutions around the globe accelerate their research and drug development efforts. Our dedicated employees are focused on providing clients with exactly what they need to improve and expedite the discovery, early-stage development and safe manufacture of new therapies for the patients who need them. To learn more about our unique portfolio and breadth of services, visit www.criver.com.

About ASC Therapeutics

ASC Therapeutics is a biopharmaceutical company pioneering the development of gene replacement therapies, *in vivo* gene editing and allogeneic cell therapies for hematological, metabolic, and other rare diseases. Led by a management team of industry veterans with significant global experience in gene and cell therapy, ASC Therapeutics is developing multiple therapeutic programs based on four technology platforms: 1) In-vivo gene therapy of inherited blood clotting disorders, initially focusing on ASC618, second generation gene therapy for hemophilia A, for which U.S. FDA IND clearance, Orphan Drug and Fast Track Designations were received; 2) In-vivo gene therapy in metabolic disorders, initially focusing on Maple Syrup Urine Disease, in collaboration with the Universities of Massachusetts and Pennsylvania; 3) In-vivo gene editing, initially focusing on ASC518 for hemophilia A; and 4) Allogeneic cell therapy, the first indication with a Decidua Stromal Cell-based therapy for steroid-refractory acute Graft-versus-Host Disease, for which U.S. FDA IND clearance and Orphan Drug Designation were received. To learn more please visit https://www.asctherapeutics.com/.

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