ARM and NIIMBL Release Project A-Gene to Bring Quality by Design Principles to Gene Therapy Manufacturing

Washington, DC – June 24, 2021

Four-year effort addresses challenges to the manufacturing scale-up of life-changing gene therapies

The Alliance for Regenerative Medicine (ARM) and the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) today released *Project A-Gene*, a multistakeholder collaboration to incorporate Quality by Design (QbD) principles into a manufacturing case study of a viral vector commonly used in gene therapies.

A-Gene brings best practices and a standard methodology for Chemistry, Manufacturing and Controls (CMC) to the burgeoning gene therapy field, where the science is rapidly advancing but manufacturing scale-up has often become an obstacle to regulatory approval and commercialization. Many of the hurdles to the streamlined, cost-effective manufacture of cell and gene therapy products derive from a lack of standardized methodologies and training around CMC programs.

A-Gene, a four-year effort, emulates previous QbD efforts that were applied to the manufacturing of monoclonal antibodies (A-Mab) and vaccines (A-Vax). Each of those technologies faced similar hurdles when developers sought to advance from small-batch manufacturing for clinical trials to full-scale commercial production. A-Mab and A-Vax helped to lower barriers to technology transfer and to better prepare new entrants to the industry. It is commonly noted that gene therapy manufacturing is at a similar stage of development as monoclonal antibody manufacturing was 15-20 years ago.

"With the help of A-Mab, industry made tremendous progress on the manufacture of monoclonal antibodies," said Michael Lehmicke, ARM's senior director for science and industry affairs and lead on the *A-Gene* project. *"We think A-Gene will similarly help gene therapy developers standardize their processes and build the necessary CMC capabilities to deliver durable and potentially curative treatments for a range of serious and sometimes fatal diseases."*

More than 50 industry experts from over 20 leading therapeutic developers — all of them ARM member organizations — as well as the Food and Drug Administration and the Standards Coordinating Body contributed to *A-Gene*. In order to maximize its usefulness as a case study, *A-Gene* focuses on a specific application: *in vivo* gene therapy delivered using an adeno-associated virus (AAV), which is the most frequently used viral vector in gene therapy.

QbD is a systematic approach to development that begins with predefined objectives and emphasizes product and process understanding and process control, based on sound science and quality risk management.

Kelvin Lee, Institute Director for NIIMBL, shared, *"We are very excited about the project because gene therapies have demonstrated a transformational impact on patients and now is the time to have the biopharmaceutical manufacturing community share best practices to accelerate access to these treatments for patients in need."*

ARM and NIIMBL are making the *A-Gene* report publicly available via ARM's website. ARM will host a five-part live webinar series highlighting specific *A-Gene* chapters, the first of which will be held June 24, 2021, exclusive to ARM members. The remaining four sessions, held monthly through October, will be open to non-members and the public. The series will be followed by a half-day virtual workshop in November focusing on the best practices for the gene therapy sector as shared in *A-Gene*.

Media inquiries

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About the Alliance for Regenerative Medicine

The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization dedicated to realizing the promise of regenerative medicines and advanced therapies. ARM promotes legislative, regulatory, reimbursement and manufacturing initiatives to advance this innovative and transformative sector, which includes cell therapies, gene therapies and tissue-based therapies. Early products to market have demonstrated profound, durable and potentially curative benefits that are already helping thousands of patients worldwide, many of whom have no other viable treatment options. Hundreds of additional product candidates contribute to a robust pipeline of potentially life-changing regenerative medicines and advanced therapies. In its 11-year history, ARM has become the global voice of the sector, representing the interests of 390+ members worldwide, including small and large companies, academic research institutions, major medical centers and patient groups. To learn more about ARM or to become a member, visit http://www.alliancerm.org.

About NIIMBL

The National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) is a public-private partnership whose mission is to accelerate biopharmaceutical innovation, support the development of standards that enable more efficient and rapid manufacturing capabilities, and educate and train a world-leading biopharmaceutical manufacturing workforce, fundamentally advancing U.S. competitiveness in this industry. NIIMBL is part of Manufacturing USA®, a diverse network of federally-sponsored manufacturing innovation institutes, and is funded through a cooperative agreement with the National Institute of Standards and Technology (NIST) in the U.S. Department of Commerce with significant additional

support from its members. To learn more about NIIMBL or to become a member, please visit www.niimbl.org.