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

Washington, DC – July 26, 2022

Effort to address challenges to the manufacturing scale-up of cell-based therapies follows release of Project A-Gene for gene therapy in 2021

The Alliance for Regenerative Medicine (ARM) and the National Institute for Innovation in Manufacturing BioPharmaceuticals (NIIMBL) today released Project A-Cell, a multistakeholder collaboration to incorporate Quality by Design (QbD) principles into a manufacturing case study of a Chimeric Antigen Receptor T-cell (CAR-T) therapy.

A-Cell brings best practices and a standard methodology for Chemistry, Manufacturing and Controls (CMC) to the cell-based therapy field, which has produced six FDA-approved CAR-T therapies to treat blood cancers but still faces challenges to manufacturing scale-up. 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-Cell emulates previous QbD efforts that were applied to the manufacturing of monoclonal antibodies (A-Mab), vaccines (A-Vax), and more recently, gene therapies (A-Gene). Antibodies and vaccines faced similar hurdles to those now faced by the cell and gene therapy sector 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. Educational institutions, including the University College of London, are already using A-Gene as an educational tool, while more than 500 representatives from ARM members attended a series of webinars to share the report's findings. The A-Gene document has been viewed more than 5,000 times on ARM's website.

"A-Gene is already making a significant impact as an educational and workforce development tool for the gene therapy sector," said Michael Lehmicke, ARM's vice president for science and industry affairs and lead on the A-Gene and A-Cell projects. *"We think A-Cell will similarly advance the cell therapy field and help to deliver durable and potentially curative treatments for a range of serious cancers and other diseases."*

More than 50 industry experts from over 30 leading therapeutic developers, as well as regulatory experts and the Standards Coordinating Body, contributed to A-Cell. A-Cell focuses on an autologous CAR-T therapy because of early regulatory successes and significant investor interest in this therapeutic approach. But many of the concepts from this study are applicable and beneficial to a broader profile of cell-based therapy products, including mesenchymal stem/ stromal cell therapies and pluripotent stem cell-based therapies.

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.

"We are very excited about the project because cell therapies have demonstrated a transformational impact on patients," said Gene Schaefer, NIIMBL senior fellow. *"Now is a critical 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-Cell report publicly available via ARM's website. ARM will host a webinar series in 2022 and early 2023 highlighting specific A-Cell chapters, the first of which will be held in September.

Media inquiries
For more information or for media requests, please contact Stephen Majors, Senior Director of Public Affairs for ARM, at smajors@alliancerm.org.

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 12-year history, ARM has become the global voice of the sector, representing the interests of 400+ 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.