Statement for the Record, U.S. House Energy and Commerce Committee Health Subcommittee Hearing "Made in America: Strengthening Domestic Manufacturing and Our Health Care Supply Chain"

The Alliance for mRNA Medicines (AMM)[1] commends the members of the Health Subcommittee of the House Energy and Commerce Committee for holding this hearing to examine current challenges and opportunities to bolster U.S. manufacturing and foster a resilient health care supply chain. mRNA has and will transform the lives of U.S. patients if the U.S. advances pro-mRNA policies, including those related to manufacturing. Below we present the case for why Congress and the Administration should robustly support mRNA product development as part of the strategy to strengthen domestic manufacturing:

- **As Evidenced by the Recent News on "Baby KJ", mRNA Will Save Lives and Improve the Health of Millions of Americans**
- **mRNA Manufacturing Offers Significant Benefits Over Current Technologies**
- **The U.S. Is Currently a Leader in mRNA Manufacturing**
- **However, Due to Recent State and Federal Government Actions, U.S. Leadership on mRNA Is Under Threat**
- **To Bolster this Innovative Area of Medicine, Congress Should Advance Pro-mRNA Manufacturing Policies in the United States to Help U.S. Patients and Create U.S. Jobs**

I. As Evidenced by the Recent News on "Baby KJ", mRNA Will Save Lives and Improve the Health of Millions of Americans

mRNA is a revolutionary technology with therapeutic applications in serious and complex diseases, with high potential to save lives and improve the health of millions of Americans. It is already being tested in the clinic for patients with diseases that present significant challenges to living a healthy and enriching life. These are American patients who have no effective treatment options – diseases as diverse as pancreatic cancer and melanoma, cystic fibrosis, and cardiovascular, autoimmune, and neurological conditions. mRNA technology is also a critical—and proven—component of America's leadership position in biotechnology and pharmaceutical industries, in academic leadership, and as a key factor in our national security strategy.

Advancements in mRNA technology are improving American lives today, demonstrated by the recent announcement of the successful treatment of an infant, Baby KJ. **Baby KJ successfully**

[1] The Alliance for mRNA Medicines (AMM) is the leading global organization dedicated to advancing and advocating for mRNA and next-generation encoding RNA therapeutics and vaccines for the benefit of patients, public health, and society. Our mission is to propel the future of mRNA medicine, improve patients' lives, and advance scientific knowledge by convening and empowering mRNA industry leaders, innovators, scientists, and other key stakeholders. AMM's membership, which is composed of nearly 80 organizations, consists of biotechs, pharmaceutical companies, contract development and manufacturing organization (CDMOs), suppliers, and academic researchers. Learn more at https://mrnamedicines.org

received a personalized (N of 1) mRNA-CRISPR gene therapy treatment for severe carbamoyl phosphate synthetase 1 (CPS1) deficiency, which (until now) was an incurable, rare disease. This most recent story shows the potential of mRNA technologies and the importance of U.S. mRNA manufacturing to patients. There are many other stories like this in the making, including advances in treating pancreatic cancer, melanoma, breast cancer, and brain cancer. An unprecedented 2022 study using an mRNA technology resulted in an effective cure for some patients with pancreatic cancer (a five-year survival rate). Thus, mRNA technology is transforming health care and saving American lives from previously devastating diseases.

II. mRNA Manufacturing Offers Significant Benefits Over Current Technologies

mRNA manufacturing offers the following benefits over other pharmacological technologies:

- **Scalable and Modular Manufacturing:** the same process can be used for multiple products and using modular systems enabling fast implementation and multiple scales (n of 1 or billions)
- **Quality and Consistency:** cell-free system simplifies and shortens production process; it also reduces risk of contamination
- **Smaller Footprint, Lower Infrastructure Needs:** mRNA facilities require less space and lower capital investment
- **Rapid Development and Production:** from sequence to manufacture moves much faster than other modalities – new mRNAs can be made in weeks vs. years for other technology
- **Continuous Manufacturing Potential:** fosters real-time quality control and thus efficiency and higher quality
- **Hospital-Based / Point-of-Care Manufacturing:** enables individualized cancer treatments for patients
- **Lower Cost of Goods:** allows for broader implementation
- **Easily Adaptable for New Indications:** fosters agility which will help in rare disease, infectious disease, and cancer, among other diseases

III. The U.S. Is Currently a Leader in mRNA Manufacturing

As with many of the most innovative areas in medicine, the U.S. has led the way in mRNA product development and manufacturing, which creates jobs in the U.S. and supply chain security. Just a few of the many examples of U.S. leadership in manufacturing include:

- **New England Biolabs (NEB)** has pioneered the discovery and production of innovative products tailored for molecular biology research for over 50 years. NEB domestically manufactures enzymes for use in basic and applied research, molecular diagnostics, and nucleic-acid therapeutics manufacturing. In addition to their Ipswich, MA, headquarters, in 2019 NEB opened a facility in Rowley, MA, capable of large-scale mRNA synthesis enzyme manufacturing with the quality systems required for use in GMP mRNA production. NEB employs over 500 full time employees in the US.
- **NTx Bio** is strengthening America's pharmaceutical supply chain by manufacturing distributed mRNA production systems, critical RNA precursors, and high-purity RNA entirely in the United States. NTx Bip's benchtop NTxscribe® platform is actively being

used by U.S. cancer centers, CDMOs, and pharma companies to reduce dependence on foreign suppliers and accelerate domestic biomanufacturing for research and critical medicines. NTx instruments and consumables are assembled in New Mexico, with expansion underway in Plano, Texas. Critical raw materials for mRNA are traditionally sourced through complex global supply chains and several key materials are fully dependent on China. To protect U.S. interests and offer consistent and stable supply to customers, NTx developed a line of proprietary biomaterials —including n1-methylpseudouridine triphosphate, enzymes, and polymerases— that are produced in the U.S. using 100% U.S.-sourced materials. NTx's technology enables rapid, distributed manufacturing to bolster U.S. supply chain resilience and reduce reliance on overseas production.

- **ReciBioPharm's** Advanced Bio business operates an 80,000 sq. ft. development and manufacturing facility in the Greater Boston Area that manufactures mRNA therapeutics and live biotherapeutic microbiome products for clinical trials and commercial use. The site employs between 150 and 200 people ranging from interns and technicians to seasoned scientists and manufacturing operations professionals. All of which bolster the U.S. as a leading manufacturer of the most advanced therapeutic products for rare and chronic illnesses.
- **Maravai Lifesciences** helps life sciences companies overcome their biggest development and manufacturing challenges, to streamline and scale from research through clinical trials to commercialization. Maravai does all its manufacturing in the San Diego, California area, including RNA research in all clinical phases and commercial manufacturing.

IV. However, Due to Recent State and Federal Government Actions, the U.S. mRNA Leadership, Including Manufacturing, Is Under Threat.

Recent policy developments, at both the federal level and in certain states. jeopardize the progress that has been made on mRNA innovation. Researchers and industry are already experiencing the impacts of overall funding cuts to research, targeting of mRNA, and the negative policy climate. Taken together, these represent a critical threat to the biomedical leadership of the U.S., as they would:

- cause U.S. research and manufacturing jobs to move to Europe and Asia
- delay therapeutic advances in cancer, rare disease, and other diseases by years
- force the loss of billions in potential healthcare savings
- forfeit U.S. biomedical leadership to Europe and Asia, and
- put U.S. National Security in the hands of other countries.

The consequences were demonstrated in a recent AMM survey of 106 industry leaders where 81% of the respondents expressed concern that anti-mRNA policies would cause manufacturing, research, and related jobs to leave the U.S. in favor of pro-innovation environments in Europe and Asia. Key findings of the survey included:

- **66% of mRNA-related jobs are located in the U.S**. and up to **45% of those U.S. positions** are potentially at risk, with **21%** of organizations indicating *all* such U.S. roles could be eliminated under hostile policy conditions, based on survey results.
- **48% of surveyed mRNA organizations have already experienced direct impacts from recent policy disruptions and funding cuts**. Those impacts include:
  - 54% were forced to cut back on mRNA R&D and postponed studies
  - 46% experienced budget freezes and reductions
  - 46% cancelled collaborations
  - 46% delayed capital investments
  - 30% initiated hiring freezes or layoffs among specialized scientific personnel
  - 20% are relocating projects, divisions or entire company, particularly moving from the U.S. to Europe or Asia

V. Congress Should Advance Pro-mRNA Manufacturing in the United States to Help U.S. Patients and Create U.S. Jobs

U.S. biomedical leadership in mRNA was catalyzed by U.S. government support, including investments from DARPA over a decade ago to support from President Trump in his first term. Continuing U.S. policy support is critical to sustaining this leadership, helping U.S. patients, and creating U.S. jobs. Congress can continue this support by advancing these policies:

- Continue funding mRNA research at NIH, BARDA, ARPA-H, and NSF
- Incenting infrastructure investment in the U.S.
- Investing in production of high-quality raw materials in the U.S.
- Requiring FDA to further advance the regulatory framework for platform technologies
- Enhancing FDA's regulatory capacity for mRNA products
- Supporting workforce development

VI. Conclusion

AMM thanks the Members of the Committee for holding this hearing to explore this important issue.