



Moderna to Present Preclinical Data at 2019 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting

April 29, 2019

The Company and academic collaborators to share data from seven preclinical studies demonstrating the potential of mRNA-based therapies to treat rare metabolic and genetic disorders

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 29, 2019-- Moderna, Inc., (Nasdaq: MRNA) a clinical stage biotechnology company pioneering messenger RNA (mRNA) therapeutics and vaccines to create a new generation of transformative medicines for patients, today announced that it will present, along with academic collaborators, preclinical data from seven studies that support the potential of mRNA-based therapies to treat the underlying cause of several rare metabolic and genetic disorders. These data, including five oral presentations, will be presented at the 2019 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting taking place April 29 – May 2 in Washington, D.C.

“We are pleased to present these data that show the potential of our mRNA platform and lipid nanoparticle delivery technology to encode for missing or malfunctioning proteins that are at the root of a diverse array of rare diseases,” said Paolo Martini, Ph.D., chief scientific officer of rare diseases at Moderna. “These studies add to our growing body of published preclinical work in rare metabolic and genetic disorders, and build upon our understanding of the potential for mRNA-based treatments to express required proteins or modulate immune responses as we continue our work to bring additional investigational mRNA medicines into the clinic.”

Presentations at the meeting include preclinical data from studies of the following disorders:

- Ornithine transcarbamylase deficiency (OTC), in collaboration with the Gene Therapy Program at the Perelman School of Medicine, University of Pennsylvania (Penn);
- Maple syrup urine disease (MSUD), in collaboration with Penn;
- Arginase-1 (ARG1) deficiency, in collaboration with the University of California, Los Angeles Department of Surgery and Department of Molecular and Medical Pharmacology;
- Factor VIII deficiency (hemophilia A), in collaboration with Seattle Children’s Research Institute;
- Glycogen storage disorder type 1a (GSD1a);
- Adult-onset type II citrullinemia (CTLN2); and
- Progressive familial intrahepatic cholestasis type 3 (PFIC3).

“mRNA medicines have the potential to treat the underlying cause of many metabolic diseases, and may offer important advantages over conventional gene and enzyme replacement therapies for eligible patients,” said James M. Wilson, M.D., Ph.D., director of the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania. “This includes the potential to develop controlled, dose-dependent and transient treatments that may benefit infants and children with these disorders and patients with diseases that are not addressable with current viral-based approaches.”

Abstracts and presentations at ASGCT 2019

- Abstract #27: Liver-Directed Lipid Nanoparticle mRNA Therapy Improves Survival and Reduces Serum Branched Chain Amino Acids in a Mouse Model of Maple Syrup Urine Disease (Greig et al., Oral Presentation, Monday, April 29, 9:00am – 9:15am ET, Heights Courtyard 3 Room)
- Abstract #72: Systemic mRNA Therapy as a Treatment for the Inherited Metabolic Liver Disorder Arginase Deficiency (Truong et al., Oral Presentation, Monday, April 29, 11:45am – 12:00pm ET, Heights Courtyard 2 Room)
- Abstract #382: Immunomodulation of Factor FVIII Inhibitors in Hemophilia A Mice Using Messenger RNA Lipid Nanoparticles (Chen et al., Oral Presentation, Tuesday, April 30, 3:45pm – 4:00pm ET, Lincoln Room)
- Abstract #383: Efficient mRNA Therapy for Treating Ornithine Transcarbamylase Deficiency in Two Mouse Models (Wang et al., Oral Presentation, Tuesday, April 30, 4:00pm – 4:15pm ET, Lincoln Room)
- Abstract #709: mRNA Therapy Improves Metabolic and Behavioral Abnormalities in a Murine Model of Citrin Deficiency (Cao et al., Oral Presentation, Wednesday, May 1, 4:45pm – 5:00pm ET, Heights Courtyard 1 Room)
- Abstract #768: MDR3/ABCB4 mRNA Therapy for Treating Progressive Familial Intrahepatic Cholestasis 3 (PFIC3) (Cao et al., Poster Presentation, Wednesday May 1, 5:00pm - 6:00pm ET, Columbia Hall)
- Abstract #797: mRNA Therapy for the Treatment of Glycogen Storage Disease Type 1a (GSD1a) (Cao et al., Poster presentation, Wednesday May 1, 5:00pm - 6:00pm ET, Columbia Hall)

At present, these research programs are not Moderna development candidates.

About Moderna

Moderna is advancing messenger RNA (mRNA) science to create a new class of transformative medicines for patients. mRNA medicines are designed to direct the body’s cells to produce intracellular, membrane or secreted proteins that can have a therapeutic or preventive benefit and have the potential to address a broad spectrum of diseases. Moderna’s platform builds on continuous advances in basic and applied mRNA science, delivery technology and manufacturing, providing the Company the capability to pursue in parallel a robust pipeline of new development candidates.

Moderna is developing therapeutics and vaccines for infectious diseases, immuno-oncology, rare diseases and cardiovascular diseases, independently and with strategic collaborators.

Headquartered in Cambridge, Mass., Moderna currently has strategic alliances for development programs with AstraZeneca, Plc. and Merck, Inc., as well as the Defense Advanced Research Projects Agency (DARPA), an agency of the U.S. Department of Defense; the Biomedical Advanced Research and Development Authority (BARDA), a division of the Office of the Assistant Secretary for Preparedness and Response (ASPR) within the U.S. Department of Health and Human Services (HHS). Moderna has been ranked in the top ten of Science's list of top biopharma industry employers for the past four years. To learn more, visit www.modernatx.com.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended including, but not limited to, statements concerning: the potential of mRNA-based therapies to treat rare metabolic and genetic disorders in humans, including the potential to offer advantages over existing therapies, the potential to develop controlled, dose-dependent, and transient mRNA-based treatments that benefit infants and children, and the potential to provide an option for patients with diseases that are not addressable with current viral-based approaches; the potential of Moderna's mRNA platform and lipid nanoparticle delivery technology to encode for missing or damaged proteins for a diverse array of rare diseases; the potentially broad applicability of Moderna's platform technology; and the potential that preclinical research programs may lead to additional Moderna development candidates. In some cases, forward-looking statements can be identified by terminology such as "will," "may," "should," "expects," "intends," "plans," "aims," "anticipates," "believes," "estimates," "predicts," "potential," "continue," or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. The forward-looking statements in this press release are neither promises nor guarantees, and you should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties and other factors, many of which are beyond Moderna's control and which could cause actual results to differ materially from those expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others: whether preclinical research programs will lead to additional Moderna development candidates; whether preclinical results will be predictive of future clinical study results; whether mRNA-based treatments could be unsafe or intolerable during preclinical and clinical studies; preclinical and clinical development is lengthy and uncertain, especially for a new class of medicines such as mRNA, and therefore our preclinical programs may be delayed, terminated, or may never advance to the clinic; no mRNA drug has been approved in this new potential class of medicines, and may never be approved; mRNA drug development has substantial preclinical, clinical and regulatory risks due to the novel and unprecedented nature of this new class of medicines; and those risks and uncertainties described under the heading "Risk Factors" in Moderna's most recent Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (SEC) and in subsequent filings made by Moderna with the SEC, which are available on the SEC's website at www.sec.gov. Except as required by law, Moderna disclaims any intention or responsibility for updating or revising any forward-looking statements in this press release in the event of new information, future developments or otherwise. These forward-looking statements are based on Moderna's current expectations and speak only as of the date hereof.

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Source: Moderna, Inc.

Investors:

Lavina Talukdar
Head of Investor Relations
617-209-5834
lavina.talukdar@modernatx.com

Media:

Jason Glashow
Head, Corporate Communications
617-674-5648
Jason.glashow@modernatx.com