



Moderna Announces Recent Progress in Its Immuno-Oncology and Rare Disease Programs and Highlights Corporate Objectives

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Immuno-Oncology

- *IND amendment submitted to the FDA for a Phase 2 cohort of OX40L (mRNA-2416) to treat advanced ovarian carcinoma*
- *First patient dosed in the Phase 1 study of OX40L + IL23 + IL36γ (Triplet) (mRNA-2752) for advanced or metastatic solid tumor malignancies or lymphoma*
- *Planning for a Phase 2 study of personalized cancer vaccine (mRNA-4157) initiated together with strategic collaborator Merck*

Rare Diseases

- *IND submitted to the FDA for a Phase 1/2 study of methylmalonic acidemia (mRNA-3704)*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 8, 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 recent updates to several of its immuno-oncology and rare disease programs and outlined its 2019-2020 corporate objectives. Moderna has 21 mRNA development candidates in its [pipeline](#), with 11 programs now in clinical development.

"This year we are focused on making significant advances to our pipeline as we work to bring multiple programs into Phase 2 clinical trials, move programs within our rare disease portfolio toward the clinic and leverage our mRNA platform to create both new development candidates and potential new modalities where we believe there is an opportunity to develop therapies for a broad range of diseases," said Stéphane Bancel, Moderna's Chief Executive Officer. "I am pleased with the continued progress of our pipeline, our ability to now manufacture mRNA for clinical development at our new site in Norwood and the relentless work of our team. I believe we are better positioned than we have ever been to deliver on the promise of our science to bring forward mRNA medicines that have the potential to improve the lives of patients."

Mr. Bancel will present a company overview and strategy update today at 10:30am PST at the 37th Annual J.P. Morgan Healthcare Conference in San Francisco. A live webcast of today's presentation can be found at investors.modernatx.com.

Detailed program updates:

Intratumoral Immuno-oncology: *These programs aim to drive anti-cancer T cell responses by injecting mRNA therapies directly into tumors.*

- **OX40L (mRNA-2416):** Based on previously reported clinical observations in two patients with advanced ovarian carcinoma in its Phase 1 study, Moderna has submitted an Investigational New Drug (IND) amendment to the U.S. Food and Drug Administration (FDA) and to the study's clinical research sites to commence a Phase 2 cohort of mRNA-2416 as a monotherapy in advanced ovarian carcinoma within its current Phase 1 study. Thus far, 28 patients have been dosed in the ongoing Phase 1 trial for mRNA-2416, an open-label, multicenter study of repeated intratumoral injections of mRNA-2416 in patients with advanced relapsed/refractory solid tumor malignancies and lymphomas. Initial data from the Phase 1 study were presented in a poster session at the Annual Meeting of the Society for Immunotherapy of Cancer in November 2018.
- **OX40L + IL23 + IL36γ (Triplet) (mRNA-2752):** Moderna has dosed the first patient in the Phase 1 study of mRNA-2752, an intratumoral injection comprising three mRNAs encoding for OX40L + IL23 + IL36γ for the treatment of advanced or metastatic solid tumor malignancies or lymphoma. The open label, multi-center study is evaluating the safety and tolerability of mRNA-2752 as a single agent and in combination either with AstraZeneca's durvalumab or tremelimumab, and will assess anti-tumor activity, protein expression in tumors and pharmacokinetics, and exploratory endpoints that include assessment of immunological response.

Cancer Vaccines: *These programs focus on stimulating a patient's immune system to tumor-related antigens to enable the immune system to elicit a more effective antitumor response.*

- **Personalized Cancer Vaccine (PCV) (mRNA-4157):** Moderna and Merck are planning a randomized Phase 2 study comparing PCV and KEYTRUDA® against KEYTRUDA alone. To date, interim Phase 1 PCV study data from 24 patients showed no dose limiting toxicities up to 0.39 mg (the third of four dose levels). Interim Phase 1 immunogenicity data have also been collected in certain patients dosed with mRNA-4157 as a monotherapy, and potential antigen-specific T cell responses have been detected. The Phase 1 study continues in the dose-escalation phase of the protocol.
- **KRAS vaccine (mRNA-5671):** Merck will lead an open-label, multi-center, dose-escalation and dose-expansion Phase 1 study to evaluate the safety and tolerability of mRNA-5671 administered as an intramuscular injection both as a monotherapy and in combination with KEYTRUDA. KRAS is a frequently mutated oncogene in epithelial cancers, primarily in non-small cell lung, colorectal and pancreatic cancers. The IND for a KRAS vaccine was originally submitted by

Moderna and included an mRNA for the membrane protein STimulator of INterferon Gene (STING) to help promote antitumor activity. That IND was transferred to Merck which now will move the program forward under the same IND with KRAS as the sole mRNA. Merck may choose to include STING mRNA in later clinical development of the KRAS vaccine.

Systemic Intracellular Therapeutics: *These programs aim to deliver mRNA into cells within target organs as a therapeutic approach for diseases caused by a missing or defective protein.*

- **Methylmalonic Acidemia (MMA) (mRNA-3704):** An IND application has been submitted to the FDA for mRNA-3704, Moderna's development candidate for MMA. If approved, this will be Moderna's first rare disease program to advance into clinical trials. The Company plans to conduct an open-label, multi-center, dose escalation Phase 1/2 study of multiple ascending doses of mRNA-3704 in pediatric patients with isolated MMA due to MUT enzyme deficiency. The objectives of the study are to evaluate safety and tolerability. mRNA-3704 has received Rare Pediatric Disease Designation by the FDA and Orphan Drug Designation by both the FDA and the European Medicines Agency.
- **Propionic Acidemia (PA) (mRNA-3927):** mRNA-3927 was granted Orphan Drug Designation by the FDA in December 2018 and Rare Pediatric Disease Designation by the FDA in January 2019. PA is a rare, life-threatening, inherited metabolic disorder due to a defect in the mitochondrial enzyme propionyl-CoA carboxylase, or PCC. It primarily affects the pediatric population and there is no approved therapy. Moderna is continuing to advance mRNA-3927 in pre-clinical studies. Moderna also continues to enroll patients in a global natural history study of MMA and PA (MaP Study) designed to identify and correlate clinical and biomarker endpoints for these disorders. This is a global, multi-center, non-interventional study for patients with confirmed diagnosis of MMA due to methylmalonyl-CoA mutase (MUT) deficiency or PA.

More than 760 subjects have been dosed with a therapeutic or vaccine candidate developed with Moderna's mRNA technology.

Information about each program in Moderna's pipeline, including those discussed in this press release, can be found on our investor relations page of our website www.modernatx.com.

Corporate Objectives:

Moderna shared its corporate objectives for 2019 - 2020, which include:

1. Generate human proof-of-concept data for multiple medicines
2. Execute on current development pipeline
3. Create new development candidates in existing modalities
4. Invent new modalities

Corporate Updates:

Continued growth across organization: Moderna ended 2018 with approximately 735 full time employees. The Company ended 2017 with 535 employees.

Continued strong cash position: We expect our cash, cash equivalents, and investments in marketable securities as of December 31, 2018 to be approximately \$1.7 billion (unaudited), as compared to \$902 million (audited) as of December 31, 2017. The year over year increase includes approximately \$564 million (unaudited) in net proceeds from our initial public offering completed in December 2018, approximately \$661 million in net proceeds from our preferred stock issuances in 2018, and a \$13 million (unaudited) premium associated with the 2018 amended and restated personalized cancer vaccines agreement with Merck & Co.

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 Moderna 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, and Merck & Co., 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: Moderna's 2019-2020 corporate objectives; Moderna's plans to move multiple programs into Phase 2 clinical trials and programs within its rare disease portfolio toward the clinic; Moderna's plans to create new development candidates; Moderna's plans to develop therapies in new modalities to treat a broad range of diseases; the potential of intratumoral immuno-oncology to drive anti-cancer T cell responses with mRNA medicines; the commencement of a Phase 2 cohort for mRNA-2416; the potential for Systemic Intracellular Therapeutic programs to deliver mRNA into cells within target organs as a therapeutic approach; the advancement of mRNA-3704 into clinical trials; and Moderna's expectations regarding its cash, cash equivalents, and investments in marketable securities as of December 31, 2018. 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: preclinical and clinical development is lengthy and uncertain, especially for a new category of medicines such as mRNA, and therefore our preclinical programs or development candidates may be delayed, terminated, or may never advance to or in the clinic; no mRNA drug has been approved in this new potential category of medicines, and may never be approved; mRNA drug development has substantial clinical development and regulatory risks due to the novel and unprecedented nature of this new category of medicines; and those described in Moderna’s Prospectus filed with the U.S. Securities and Exchange Commission (SEC) on December 7, 2018 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.

Media:

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

Investors:

Lorence Kim
Chief Financial Officer
617-209-5849
lorence.kim@modernatx.com