



Moderna Announces Presentation of Interim Data from Phase 1 Study of mRNA Personalized Cancer Vaccine at 2019 ASCO Annual Meeting

June 1, 2019

Data show the potential of using neoantigens identified from an individual's tumor to elicit an immune response to cancer mutations

Tolerability and immunogenicity data support the randomized Phase 2 study of mRNA-4157 in combination with pembrolizumab

Conference call to be held on Monday, June 3 at 8:00 a.m. ET

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 1, 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 interim data from an ongoing Phase 1 clinical study in patients with both resected (adjuvant) and unresected (advanced) solid tumors. The data showed that the Company's mRNA personalized cancer vaccine (PCV) mRNA-4157, given alone or in combination with Merck's pembrolizumab (KEYTRUDA[®]), was well-tolerated at all doses tested and elicited neoantigen-specific T-cell responses. There were no vaccine-related serious adverse events (SAEs) reported for the PCV when administered to patients as a monotherapy or in combination with pembrolizumab.

Presented today at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting, the study demonstrates the immunogenicity of Moderna's mRNA platform for developing PCVs. In addition, clinical activity was observed in some patients receiving mRNA-4157 in combination with pembrolizumab. These safety, tolerability and immunogenicity data and the initial clinical activity observed support Moderna's randomized Phase 2 study investigating pembrolizumab in combination with a 1 mg dose of mRNA-4157, compared to pembrolizumab alone, for the treatment of high-risk adjuvant melanoma.

"We are encouraged by these interim data from our personalized cancer vaccine program, which involves designing and manufacturing a unique vaccine for each patient based on their specific tumor," said Tal Zaks, M.D., Ph.D., chief medical officer at Moderna. "This study demonstrates the ability of Moderna's mRNA personalized cancer vaccine to elicit T-cells that are specific to the cancer mutations. We also observed early signs of clinical activity of our personalized cancer vaccine in combination with pembrolizumab, including in two patients previously treated with checkpoint inhibitors. We look forward to building on these learnings about tolerability and immunogenicity by assessing activity in a randomized Phase 2 study for the treatment of adjuvant melanoma."

"For decades, the cancer community has been working on the concept of developing medicines that can be personalized down to the individual patient level," said Howard A. "Skip" Burris III, M.D., president, clinical operations & chief medical officer at Sarah Cannon Research Institute, and a principal investigator of the mRNA-4157 Phase 1 study. "We know that cancer mutations are rarely shared between patients, so it's encouraging to see individualized, personalized cancer vaccines like mRNA-4157 eliciting immune responses. We're pleased to be a part of a study that aims to advance the science of immunotherapy through mRNA vaccines, and deliver a novel approach that is customized for each patient."

About the Data

[**Abstract 2523: A phase 1 multicenter study to assess the safety, tolerability and immunogenicity of mRNA-4157 alone in patients with resected solid tumors and in combination with pembrolizumab in patients with unresectable solid tumors.**](#)

Presented by: Howard A. Burris, M.D., FACP, FASCO, Sarah Cannon Research Institute
(Poster Session, Saturday, June 1, 8:00 a.m. - 11:00 a.m. CT followed by a Poster Discussion at 1:15 p.m. - 2:45 p.m. CT)

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In this dose-escalation study, 13 patients with resected solid tumors (melanoma, colon and lung cancers) received mRNA-4157 as adjuvant monotherapy after resection of their primary tumor. An additional 20 patients with metastatic, unresected solid tumors (melanoma, bladder, lung, colon, prostate, head and neck and endometrial cancers) received at least one dose of mRNA-4157 in combination with pembrolizumab.

Results:

- mRNA-4157 was well-tolerated at all dose levels studied with no dose-limiting toxicities or grade 3/4 adverse events (AEs) or SAEs reported when administered as a monotherapy or in combination with pembrolizumab. The most common grade 2 adverse events were fatigue, soreness at the injection site, colitis and myalgias.
- A cohort of patients at the top dose level (1 mg) are undergoing apheresis and deeper characterization of immunogenicity responses. Data from one such patient was available at the data cutoff and showed neoantigen-specific CD8 T-cell responses were detected to 10 out of 18 class I neoantigens after the 4th dose of the vaccine (compared to 0/18 at baseline).
- Clinical responses (one complete response + five partial responses) at doses ranging from 0.04-1.0 mg were observed in 6 out of 20 patients receiving at least one dose of mRNA-4157 in combination with pembrolizumab. The complete response occurred to pembrolizumab monotherapy before mRNA-4157 was administered. Of the five partial responses, two were seen in patients previously treated with a checkpoint inhibitor.
- Of the 13 patients who received adjuvant mRNA-4157 monotherapy, all patients have completed a full course of vaccination per the study protocol. Eleven patients remained disease free up to 75 weeks on study.

Additionally, the National Cancer Institute (NCI) presented early data today from its Phase 1 study of PCV mRNA-4650 as a monotherapy for patients with advanced metastatic cancers. The NCI program uses Moderna's mRNA technology but uses a different neoantigen selection process and study design than Moderna's Phase 1 mRNA-4157 study.

[Abstract 2643: A Phase 1/2 study to assess the immunogenicity and tolerability of personalized mRNA vaccine mRNA-4650 encoding defined neoantigens expressed by the autologous cancer.](#)

Presented by: Gal Cafri, Ph.D., Postdoctoral Fellow, National Cancer Institute Surgery Branch
(Poster Session, Saturday, June 1, 8:00 a.m. - 11:00 a.m. CT)

Conference Call

Moderna will host a conference call and webcast on Monday, June 3 at 8:00 a.m. ET to discuss these mRNA-4157 data. Participants are invited to listen by dialing (866) 922-5184 (domestic) or (409) 937-8950 (international) and providing conference ID 3016438 or join the live webcast by going to the ["Events and Presentations"](#) area on the Investors page of the Company's website, www.modernatx.com. An archived webcast of the conference call can also be accessed through the Company's website and a replay of the call will be available there for four weeks after the call.

About Moderna's Immuno-Oncology Programs

Moderna's oncology programs are currently focused on two main areas: cancer vaccines and intratumoral immuno-oncology (I/O) therapies. Moderna is developing these potential mRNA treatments as monotherapies and/or in combination with checkpoint inhibitors from our strategic collaborators Merck and AstraZeneca. The company currently has five I/O programs in development, including two programs advancing into Phase 2 trials.

An advantage of Moderna's mRNA platform is that it allows for investigational medicines that combine in a single mRNA therapy several different approaches to activate the immune system to attack cancer, either with mRNA encoding for common tumor proteins found across cancer types or multiple mRNAs encoding for various immunomodulatory proteins.

Moderna's investigational PCVs are designed to use neoantigens identified from an individual's tumor to program the body's immune system to elicit a more effective anti-tumor response. Upon sequencing the tumor, Moderna's proprietary algorithms predict the neoantigens (antigens encoded by tumor-specific mutated genes) most likely to trigger the immune system to attack a particular cancer. Today, mRNA encoding up to 34 unique neoantigens can be delivered in a single vaccine. Moderna develops and manufactures these investigational PCVs at its personalized vaccines unit within its Norwood, Mass. manufacturing facility.

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, and 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 and follow on Twitter at [@moderna_tx](https://twitter.com/moderna_tx).

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 for Moderna's investigational PCVs to use neoantigens identified from an individual's tumor to program the body's immune system to elicit a more effective antitumor response; and the planned Phase 2 study investigating pembrolizumab in combination with mRNA-4157, compared to pembrolizumab alone, in high-risk adjuvant melanoma. 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 Phase 1 results for mRNA-4157 and mRNA-4650 will be predictive of any future clinical studies; whether mRNA-4157 and mRNA-4650 will be shown to be unsafe or intolerable during future clinical studies; clinical development is lengthy and uncertain, especially for a new class of medicines such as mRNA, and therefore our clinical programs or development candidates may be delayed, terminated, or may never advance; no mRNA drug has been approved in this new potential class 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 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.

Moderna Contacts:

Investors:

Lavina Talukdar

Head of Investor Relations

617-209-5834

lavina.talukdar@modernatx.com

Media:

Krysta Pellegrino

Krysta@healthandcommerce.com

650-255-6142