



## Moderna Therapeutics Provides Mid-Year Corporate Update, Announces Continued Clinical Development Progress and Pipeline Acceleration

July 26, 2016

**Eleven mRNA-based development candidates nominated to date across ventures and partners**

- **Two Phase 1 studies underway for mRNA-based infectious disease vaccines**
- **CTA filed for Phase 1 study of AZD8601, AstraZeneca's investigational mRNA-based Therapeutic that encodes for VEGF-A**
- **Eight candidates advancing through IND-enabling studies**

**Several additional clinical studies planned to initiate in 2016**

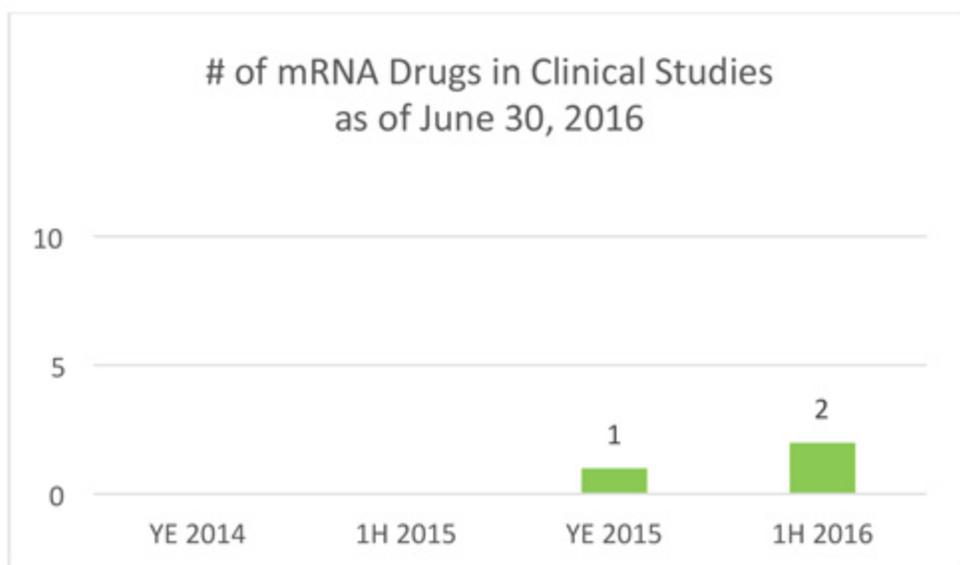
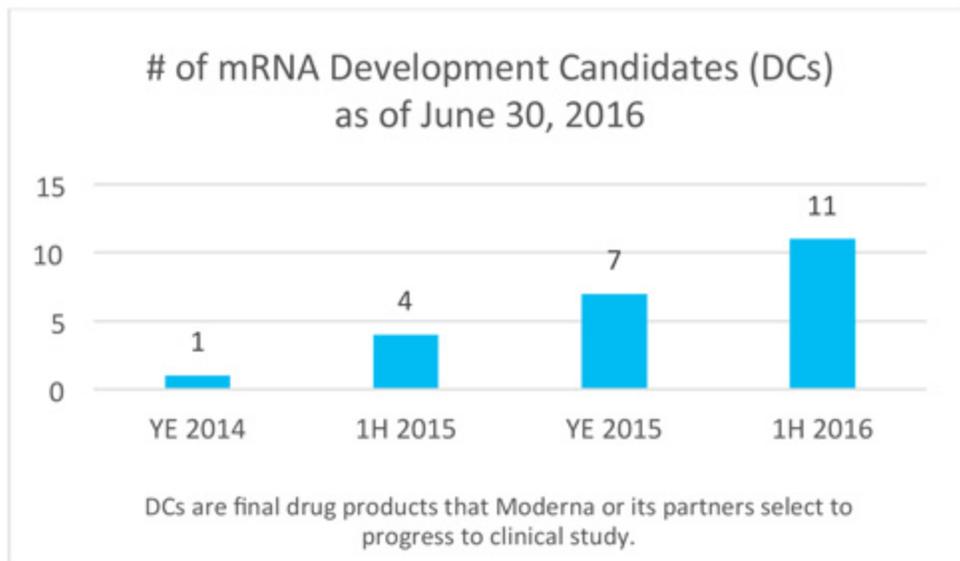
**CAMBRIDGE, Mass., July 26, 2016** – Moderna Therapeutics, a clinical stage biotechnology company that is pioneering messenger RNA (mRNA) Therapeutics™ to create a new generation of transformative medicines for patients, today announced a mid-year corporate update, highlighting continued clinical development progress and acceleration of its pipeline. A Phase 1 study for mRNA 1851 is now underway in the U.S.; this is Moderna's second clinical program. A Phase 1 study for mRNA 1440 continues enrollment in Europe. mRNA 1440 and mRNA 1851 are infectious disease vaccine candidates for undisclosed targets and indications that were developed utilizing Moderna's proprietary mRNA vaccine technology. More than 200 healthy volunteers have been dosed to date across the two ongoing studies.

Today, Moderna and AstraZeneca announced the filing of a Clinical Trial Application (CTA) in Europe for a Phase 1 study of AZD8601, an investigational mRNA-based therapy that encodes for vascular endothelial growth factor-A (VEGF-A). AZD8601 is part of the collaboration [announced](#) between Moderna and AstraZeneca in 2013 to discover, develop and commercialize mRNA Therapeutics to treat serious cardiovascular, metabolic and renal diseases as well as cancer. AZD8601 is the first program from the collaboration to progress towards clinical trials. AZD8601 could one day provide a unique regenerative treatment option for patients with heart failure, diabetic wound healing and other ischemic vascular diseases.

Eight additional nominated mRNA development candidates are advancing through IND-enabling studies across a pipeline composed of Moderna's internally led programs and partnered programs. Several of these candidates are on track to enter the clinic in 2016.

"In the first half of 2016, our team continued to make significant strides to advance our goal to deliver a new generation of transformative medicines for patients across a broad spectrum of diseases. In the five years since Moderna's founding, we have initiated two clinical trials, filed a CTA for a third program and moved another eight development candidates into IND-enabling studies. We have amassed an incredibly talented and dedicated team and forged key partnerships with notable biopharma leaders who are dedicated to advancing mRNA Therapeutics to meet serious unmet medical needs," said Stéphane Bancel, CEO of Moderna. "We look forward to a busy and productive second half of 2016 as we work to progress several additional candidates to the clinic."

There are more than 90 discovery programs advancing across Moderna's ecosystem of internal ventures and external partners. Current therapeutic areas of focus include infectious diseases, rare diseases, oncology, immuno-oncology and cardiovascular disease, among others.



### 1H 2016 Highlights

#### Expansion of Ecosystem to Advance mRNA Therapeutics for Unmet Needs

In 1H 2016, Moderna continued to expand its ecosystem and collaborate with key strategic partners to advance mRNA research and broaden the potential reach and impact of mRNA Therapeutics across various disease areas. Highlights include:

- Immuno-Oncology Collaboration with AstraZeneca.** In January, Moderna [announced](#) a new collaboration with AstraZeneca to discover, co-develop and co-commercialize immuno-oncology mRNA therapeutic candidates. The collaboration is in addition to the exclusive agreement announced by the companies in 2013 to develop mRNA Therapeutics for the treatment of cardiovascular, metabolic and renal diseases as well as selected targets in oncology.
- Inclusion of New Infectious Disease Vaccine Program with Merck.** In January, Moderna [announced](#) that Merck licensed a vaccine program against an undisclosed viral target, including mRNA 1566 and a set of related novel vaccine candidates, as part of the ongoing collaboration between the companies. The inclusion of this new program, which was not part of the original collaboration agreement, follows the rapid progress made in the first year of the collaboration.
- Global Health Partnership with the Bill & Melinda Gates Foundation.** Moderna also [announced](#) in January a partnership with the Bill & Melinda Gates Foundation to advance the development of a novel, affordable combination of mRNA-based antibody therapeutics to help prevent human immunodeficiency virus (HIV) infection. The global health partnership has the potential for follow-on projects to develop additional mRNA-based projects for various infectious diseases.

#### Strong Financial Position Affords Several Years of Runway

As of June 30, Moderna had approximately \$1 billion in cash, pro forma for the cash payments connected with the Merck and Vertex collaborations.

This affords Moderna several years of runway to support its continued growth and pipeline acceleration.

Moderna also reported the following financial results for 1H 2016:

- GAAP revenues of \$36 million
- Cash inflows of approximately \$80 million, consisting of milestone payments and reimbursements
- Investments of \$135 million to continue to advance its platform and pipeline

#### **Establishment of Internal GMP Manufacturing Capabilities and Laboratory Expansion**

As part of its efforts to support the accelerated output of its mRNA research and enable simultaneous clinical trials across various therapeutic areas, Moderna has expanded both its manufacturing capacity and its overall footprint in Cambridge, Mass. Highlights since January include:

- Establishing a wholly owned and validated GMP manufacturing facility in Cambridge, Mass., that is producing clinical-grade product.
- Increasing the square footage of office, lab and manufacturing space from 115,000 sq. ft. at the beginning of 2016 to 200,000 sq. ft. as of June 2016.

#### **Growth of the Moderna Team**

To support its clinical progress and discovery engine, Moderna has continued to grow its internal team. Highlights include:

- Increasing employee headcount from 320 in January to 440 by the end of June.
- Appointing Saqib Islam, former Executive Vice President and Chief Strategy and Portfolio Officer of Alexion Pharmaceuticals, as Moderna's Chief Business Officer.
- Appointing Dr. Michael Watson, MB ChB, MRCP, AFPM, former Global Head of Vaccination Policy and Advocacy at Sanofi Pasteur, as President of Valera, Moderna's infectious disease-focused venture company.

#### **Q2 2016 and Recent Highlights**

- **Personalized Cancer Vaccines Collaboration with Merck.** In June, Moderna [announced](#) a new strategic collaboration with Merck to advance novel mRNA-based personalized cancer vaccines with KEYTRUDA® (pembrolizumab) for the treatment of multiple types of cancer. The collaboration will leverage Moderna's rapid cycle time, small-batch manufacturing to supply vaccines tailored to individual patients within weeks. Clinical study of the personalized cancer vaccine is anticipated to begin in 2017.
- **Research Collaboration with Vertex in Cystic Fibrosis.** In July, Moderna [announced](#) an exclusive research collaboration and licensing agreement with Vertex Pharmaceuticals to discover and develop mRNA Therapeutics for the treatment of cystic fibrosis. The three-year collaboration will focus on the use of mRNA therapies, administered via pulmonary delivery, to treat the underlying cause of CF by enabling cells in the lungs to produce functional copies of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which is known to be defective in people with CF.

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#### **About Moderna Therapeutics**

Moderna is a clinical stage pioneer of [messenger RNA Therapeutics™](#), an entirely new *in vivo* drug technology that produces human proteins, antibodies and entirely novel protein constructs inside patient cells, which are in turn secreted or active intracellularly. This breakthrough platform addresses currently undruggable targets and offers a superior alternative to existing drug modalities for a wide range of diseases and conditions. Moderna is developing and plans to commercialize its innovative mRNA drugs through its own ventures and its strategic relationships with established pharmaceutical and biotech companies. Its current ventures are: [Onkaido](#), focused on oncology, [Valera](#), focused on infectious diseases, [Elpidera](#), focused on rare diseases, and [Caperna](#), focused on personalized cancer vaccines. Founded by [Flagship Venture Labs™](#), Cambridge-based Moderna is privately held and currently has strategic agreements with [AstraZeneca](#), [Alexion Pharmaceuticals](#), [Merck](#) and [Vertex Pharmaceuticals](#). To learn more, visit [www.modernatx.com](http://www.modernatx.com).

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