



## AstraZeneca and Moderna Announce Filing of First Clinical Trial Application in Messenger RNA Therapeutics™ Collaboration

July 26, 2016

**CAMBRIDGE, Mass., and LONDON, UK, July 26, 2016** — AstraZeneca and Moderna Therapeutics today announced that AstraZeneca has filed a Clinical Trial Application (CTA) with the Paul Ehrlich Institute and the German Federal Ministry of Health to initiate a Phase 1 clinical trial of AZD8601. The program is part of a collaboration between AstraZeneca and Moderna to discover, develop and commercialize messenger RNA (mRNA) Therapeutics™ to treat serious cardiovascular, metabolic and renal diseases as well as cancer. It marks the first program resulting from the collaboration to progress towards clinical trials.

AZD8601 is an investigational mRNA-based therapy that encodes for vascular endothelial growth factor-A (VEGF-A). Its discovery and development has also involved collaboration with Kenneth Chien, M.D., Ph.D., Professor of Cellular and Molecular Biology and Medicine and his team at the Integrated Cardio-Metabolic Centre at Karolinska Institutet in Stockholm, Sweden.

Marcus Schindler, Vice President, Innovative Medicines & Early Development at AstraZeneca said, “Developing regenerative therapies for the treatment of cardiometabolic disease is a hugely exciting and innovative area. We believe that using mRNA Therapeutics to initiate a strong, local and transient surge of VEGF-A expression could help us overcome challenges associated with previous approaches to regulate this protein in tissues. AZD8601 could one day provide a unique regenerative treatment option for patients with heart failure, diabetic wound healing and other ischemic vascular diseases.”

Stéphane Bancel, Chief Executive Officer, Moderna said, “This marks a significant milestone for both Moderna and AstraZeneca as our first partnered mRNA program reaches the clinic. It is a validation of our shared vision to harness the potential of mRNA Therapeutics to address serious unmet needs with the goal of improving patients’ lives. This has been a highly collaborative partnership since its inception, and I want to recognize the tremendous work of all involved, including the AstraZeneca team, my Moderna colleagues and Dr. Chien and his team. Together, I believe we will continue to make significant strides that will push new boundaries in the treatment of cardiovascular and metabolic diseases.”

Dr. Chien said, “It has been rewarding working as an integrated academic partner with AstraZeneca in its joint work with Moderna to generate a complete package of strong pre-clinical data over the past three years since our initial publication in mouse studies, and we are thrilled that the clinical trial application has been filed.”

### About AZD8601

mRNA is responsible for carrying genetic instructions transcribed from DNA, which cells then translate to produce proteins. Proteins are responsible for directing the body’s biological functions. Moderna’s pioneering mRNA Therapeutics are designed to trigger the cellular machinery to produce specific proteins. In this application, AZD8601 may enable the delivery of genetic instructions to spur the production of VEGF-A.

The AZD8601 program is built upon a decade of pioneering research on heart stem cells in cardiovascular development conducted by Dr. Chien, including the finding that VEGF-A can act as a cell fate switch for cardiac progenitors.<sup>i ii</sup>

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i Lui KO, Zangi L, Silva EA, Bu L, Sahara M, Li RA, Mooney DJ, Chien KR. Driving vascular endothelial cell fate of human multipotent Isl1+ heart progenitors with VEGF modified mRNA. *Cell Res.* 2013 Oct;23(10):1172-86.

ii Zangi L, Lui KO, von Gise A, Ma Q, Ebina W, Ptaszek LM, Später D, Xu H, Tabebordbar M, Gorbатов R, Sena B, Nahrendorf M, Briscoe DM, Li RA, Wagers AJ, Rossi DJ, Pu WT, Chien KR. Modified mRNA directs the fate of heart progenitor cells and induces vascular regeneration after myocardial infarction. *Nat Biotechnol.* 2013 Oct;31(10):898-907.

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### About AstraZeneca

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialization of prescription medicines, primarily for the treatment of diseases in three therapy areas – Respiratory and Autoimmunity, Cardiovascular and Metabolic Diseases, and Oncology. The company is also active in inflammation, infection and neuroscience through numerous collaborations. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information please visit: [www.astrazeneca.com](http://www.astrazeneca.com).

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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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