

Researchers advancing novel experimental gene-based COVID-19 vaccine, AAVCOVID

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Massachusetts Eye and Ear and Massachusetts General Hospital (MGH), members of Mass General Brigham, today announced progress towards the testing and development of an experimental vaccine called AAVCOVID, a novel gene-based vaccine candidate against SARS-CoV2, the virus that causes COVID-19.

The AAVCOVID vaccine program was developed in the laboratory of Luk H. Vandenberghe, Ph.D., director of the Grousbeck Gene Therapy Center at Massachusetts Eye and Ear and Associate Professor of Ophthalmology at Harvard Medical School. It is currently in preclinical development with a plan to begin clinical testing in humans later this year. Mason Freeman, MD, director and founder of the MGH Translational Research Center is leading the efforts to develop the clinical studies intended to establish safety and efficacy of the experimental vaccine.

The AAVCOVID Vaccine Program is a unique, gene-based vaccine strategy that uses adeno-associated viral (AAV) vector, a clinically established [gene transfer technology](#) leveraging the properties of a harmless viral carrier. AAV is used to deliver genetic sequences of the SARS-CoV-2 Spike antigen so the body can develop an immune response to the coronavirus. AAV technology has been used extensively in the field of gene therapy, and substantial experience and capacity exists for manufacturing and clinical use of AAV-based medicines. Two AAV-based drugs have been approved by U.S. Food and Drug Administration in recent years.

The AAVCOVID vaccine candidate will be administered by an intramuscular injection. Currently, tests are underway in animal models, and initial manufacturing activities have begun. Based on the preclinical findings, one or more candidates will advance into the clinical phase of testing in humans.

While several types of COVID-19 vaccines are in development worldwide, AAV technology offers several distinct advantages, including its adaptability and potential to elicit a beneficial immune response in people. In addition, other versions of AAV technology have been tested in the clinic for more than two decades with a favorable safety record.

"AAV is a superior technology for safe and efficient gene delivery, and the unique technologies we are applying in AAVCOVID support the potential for a potent immunity to be induced to SARS-CoV-2 from a single injection," said Dr. Vandenberghe. "In a crisis, we can harness the power of molecular biology and develop a draft of a vaccine in weeks, and that's what was done here. Now, [clinical studies](#) are needed to establish safety and efficacy of our novel approach," he said.

"While many organizations are engaged in generating vaccine candidates to prevent COVID-19 disease, it is very far from certain what the best approach will be," said Dr. Freeman. "Dr. Vandenberghe's unique vaccine method brings an elegant, novel and extremely creative approach to meeting our goal: to protect our most vulnerable patients as well as the healthcare workers who care for them during this and future viral outbreaks."

The team is advised by experts at Massachusetts General Hospital and the Mass General Brigham Innovation Fund and includes deep involvement of experts from industry with experience in vaccine development, regulatory affairs, and manufacturing. The research is funded by philanthropic support led by Wyc Grousbeck, Boston Celtics

lead owner and CEO and Mass. Eye and Ear Chairman, his wife Emilia Fazzalari, CEO of Cinco Spirits Group LLC, the Grousbeck family, and others.

"This is what innovation looks like. It is a combination of both the scientific insight of Dr. Vandenberghe and his team as well as the nimble and collaborative spirit of the institutions and donors who have come together to move this program from idea to promising [vaccine candidate](#) at lightning speed," said Joan W. Miller, MD, Chief of Ophthalmology at Mass. Eye and Ear, Massachusetts General Hospital, and Brigham and Women's Hospital, and Chair of Ophthalmology and David Glendenning Cogan Professor of Ophthalmology at Harvard Medical School.

"We are deeply grateful to all involved in this collaboration, and especially to the donors who generously stepped up to spearhead the initial funding of this program," said John Fernandez, President of Mass. Eye and Ear.

"The unprecedented coronavirus pandemic has challenged us all, and overcoming it demands the best thinking and the most creative ideas from our scientific and clinical teams supported and strengthened by our philanthropic communities," said Peter L. Slavin, MD, President of MGH. "This collective spirit of innovation, resolve and generosity is the most powerful weapon we have to fight this formidable virus."

About the AAVCOVID Technology

The AAVCOVID Vaccine Program is a gene-based vaccine strategy that seeks to deliver genetic sequences of the SARS-CoV-2 using an adeno-associated virus (AAV) vector, a harmless virus that serves as a delivery vehicle into the body.

Vaccination with AAVCOVID delivers the gene code for antigens from

the SARS-CoV-2 Spike protein following a low dose intramuscular injection, which is designed to elicit an immune response to prevent infection. This approach is supported by extensive experience with the safety of the AAV technology platform in other diseases, including the use of AAVs in two FDA-approved medicines, and preclinical studies on immunogenicity.

Dr. Vandenberghe and his laboratory began work on the vaccine in mid-January following the Wuhan outbreak and the first publication of genetic sequences of the new [coronavirus](#). Using a specific AAV with desirable vaccine properties, the program seeks to induce immunity to prevent infection and or disease in healthy populations.

AAV is also a rapidly adaptable technology. If a new strain of the SARS-CoV-2 virus emerges, the genetic code inside the AAVCOVID vaccine can be exchanged for an updated genetic code and processed into an updated [vaccine](#) in weeks, according to the researchers.

More information: For more information on AAVCOVID vaccine and the researchers' biographies, see masseyandear.org/covid-19/vaccine

Provided by Massachusetts General Hospital

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