Ocugen, Inc. Announces Investigational New Drug Application in Effect After Review by FDA to Initiate Phase 1 Clinical Trial Evaluating First-in-Class OCU500 Inhaled Vaccine Candidate for COVID-19

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- *OCU500 will be administered via inhalation and as a nasal spray*
- *COVID-19 remains a substantial public health threat in the U.S. and around the world*
- *Phase 1 clinical trial is anticipated to start in 2Q 2025*

MALVERN, Pa., Jan. 27, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (“Ocugen” or the “Company”) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines, today announced that the U.S. Food and Drug Administration (FDA) has reviewed the Company’s Investigational New Drug (IND) application and it is in effect. This is a critical step toward the initiation of the Phase 1 clinical trial for OCU500—an inhaled mucosal vaccine for COVID-19. The National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, will sponsor and conduct the Phase 1 trial to assess the safety, tolerability, and immunogenicity of OCU500 administered via two different routes, inhalation into the lungs and intranasally as a spray.

“We are grateful for our ongoing collaboration with NIAID and pleased to bring our novel vaccine technology into the clinic through Project NextGen,” said Mike Shine, Senior Vice President, Commercial at Ocugen. “COVID-19 remains a real public health concern, and an increasing number of studies are showing the benefit of mucosal vaccines that attack the virus where it enters the body—through the nose and mouth—to give better and longer protection. We look forward to this important next step in potentially providing a more durable and safer option to help prevent infection and transmission of COVID-19 regarding various variants of concern.”

Even though the pandemic has ended, COVID-19 still presents a significant burden in the U.S. The Centers for Disease Control (CDC) estimates that from October 1, 2024 to January 11, 2025, there were 4.4 to 7.9 million COVID infections, resulting in 120,000 to 210,000 hospitalizations and 14,000 to 25,000 deaths.

The Phase 1 trial would enroll 80 adult subjects aged 18 to 64 years. Forty (40) subjects would be assigned to the low-dose group, and 40 subjects would be assigned to the high-dose group. Within each group, 20 subjects would receive the inhalation form of the vaccine, and the other 20 subjects would receive the intranasal form. The primary aim of the study would be to determine safety, while secondary and exploratory endpoints include antibody production and the number of breakthrough COVID-19 infections.

OCU500 is based on a novel chimpanzee adenovirus-vectored (ChAd36) technology. Earlier clinical studies to prevent COVID-19 that employed a similar technology administered via inhalation demonstrated increased mucosal and systemic antibodies and a durable immune response up to one year using one-fifth the dose compared to the same vaccine administered intramuscularly. Ocugen intends to expand this mucosal platform to address other serious respiratory threats including seasonal influenza, bird flu, and respiratory syncytial virus (RSV).

The original ChAd36 vector that makes the Ocugen vaccine unique was licensed from Washington University in St. Louis.

"We are delighted to see the progress of the ChAd36 vector encoding a SARS-CoV-2 spike antigen that was originally designed and tested at Washington University in St. Louis," said Dr. Michael Diamond, Professor of Medicine and Co-Director of the Center for Vaccines and Immunity to Microbial Pathogens at Washington University School of Medicine. "We believe this vector is ideal for mucosal administration and can be designed to carry COVID-19 strains as well as influenza and antigens from other respiratory viruses," added Dr. David Curiel, Professor of Radiation Oncology, Washington University School of Medicine, co-collaborator on the vector design.

Project NextGen is a $5 billion multi-government agency initiative to develop the next generation of vaccines and therapeutics to combat the spread of COVID-19. NIAID, with funding from Project NextGen, will cover the full cost of the Phase 1 clinical trial, including operations and related analysis. Ocugen is providing clinical trial materials and, upon completion, will have full right of reference to the findings, which Ocugen believes will provide clinical evidence to support the further development of the Company’s lead mucosal vaccine candidate.

"Ocugen further advanced the vector technology, enabling the incorporation of single/multiple antigens into a single vector. This innovation offers significant manufacturing flexibility in responding to emerging variants within one hundred days of identifying a circulating variant of concern," said Dr. Arun Upadhyay, Chief Scientific Officer at Ocugen. "This vector technology, combined with mucosal delivery, has the potential to enable rapid development of respiratory vaccines in response to future outbreaks including bird flu."

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines that improve health and offer hope for patients across the globe. We are making an impact on patient’s lives through courageous innovation —forging new scientific paths that harness our unique intellectual and human capital. Our breakthrough modifier gene therapy platform has the potential to treat multiple retinal diseases with a single product, and we are advancing research in infectious diseases to support public health and orthopedic diseases to address unmet medical needs. Discover more at www.ocugen.com and follow us on X and LinkedIn.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing clinical trials, anticipated regulatory filings and anticipated development timelines, which are subject to risks and uncertainties. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks, and uncertainties that may cause actual events or results to differ materially from our current expectations, including, but not limited to, the risks that preliminary, interim and top-line clinical trial results may not be indicative of, and may differ from, final clinical data; the ability of OCU500 to perform in humans in a manner consistent with nonclinical or preclinical study data; that unfavorable new clinical trial data may emerge in ongoing clinical trials or through further analyses of existing clinical trial data; that earlier non-clinical and clinical data and testing of may not be predictive of the results or success of later clinical trials; and that that clinical trial data are subject to differing interpretations and assessments, including by regulatory authorities. These and other risks and uncertainties are more fully described in our periodic filings with the Securities and Exchange Commission (SEC), including the risk factors described in the section entitled "Risk Factors" in the quarterly and annual reports that we file with the SEC. Any forward-looking statements that we make in this press release speak only as of the date of this press release. Except as required by law, we assume no obligation to update forward-looking statements contained in this press release whether as a result of new information, future events, or otherwise, after the date of this press release.

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