

## PRESS RELEASE

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Scientists at The Wistar Institute and Stanford University Report Encouraging Results on First-in-Human Clinical Trial for an Investigational Drug to Treat Epstein-Barr Virus Cancer

**PHILADELPHIA** — (January 20th, 2025) — In newly reported clinical trial results, The Wistar Institute and Stanford University have successfully tested an investigational drug for the treatment of Epstein-Barr Virus (EBV)-positive nasopharyngeal cancer (NPC) in humans. The drug VK-2019 was tested in a first-in-human clinical trial by a research team led by Wistar research associate professor **Troy Messick**, **Ph.D.**, and Stanford professor of medicine <u>A. Dimitrios Colevas</u>, <u>M.D.</u> VK-2019 was discovered and developed at The Wistar Institute in the **Lieberman lab**.

Findings were published in the paper, "First-in-Human clinical trial of a small molecule EBNA1 inhibitor, VK-2019, in patients with Epstein-Barr positive nasopharyngeal cancer, with pharmacokinetic and pharmacodynamic studies," in the journal <u>Clinical Cancer Research</u>, a journal of the American Association for Cancer Research.

"It is very encouraging that the drug is well-tolerated in patients with advanced cases of NPC," said Dr. Messick. "Now that the safety of the drug has been tested, we are well prepared to advance VK-2019 to further testing, including in other cancers or diseases that are caused by EBV."

In a cohort of 23 participants with cases of NPC that had exhausted all available treatment options, the clinical trial team administered oral doses of VK-2019 that increased from 60 to 1800 milligrams. Even at high doses, most patients tolerated the drug well with only minor side effects. Only one adverse event deemed attributable to VK-2019 was reported, which was successfully reversed.





Analysis of a limited number of patient biopsies before and during treatment with VK-2019 showed that levels of viral DNA could be reduced. One patient showed an anti-tumor response, even at this early stage of clinical testing with the drug's yet-to-be-optimized dosing schedule.

Armed with robust evidence that even high doses of VK-2019 are safe for cancer patients, the researchers plan to expand the number of participants in clinical trials to evaluate the drug's clinical efficacy against cancer.

"The results of the trial indicate a robust safety profile for VK-2019 in NPC patients," said Dr. Colevas. "Now knowing the drug's safety, we should be able to conduct trials with larger numbers of participants to better understand and optimize its pharmacological properties to fight cancer."

Working alongside <u>Paul M. Lieberman, Ph.D.</u>, Wistar Hillary Koprowski, M.D., Endowed Professor, Dr. Messick co-invented the small-molecule VK-2019 for clinical testing. Dr. Paul Lieberman, an expert in EBV and its role in causing diseases, discovered EBNA1's potential as a clinical target for treating EBV-associated conditions. Preclinical testing from the Lieberman lab has demonstrated that VK-2019 blocks the ability of the virus to replicate and thereby significantly inhibits the growth of tumors dependent on EBV.

More than 90% of people globally carry EBV. After an initial infection, EBV persists typically in a latent form without symptoms, but researchers have found that latent EBV infection can cause certain cancers, particularly nasopharyngeal cancer (NPC). NPC, in its more advanced stages, can often be fatal despite treatment.

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**Clinical Trial information:** The clinical trials are registered on clinicaltrials.gov under NCT04925544 and NCT03682055.

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The Wistar Institute is the nation's first independent nonprofit institution devoted exclusively to foundational biomedical research and training. Since 1972, the Institute has held National Cancer Institute (NCI)-designated Cancer Center status. Through a culture and commitment to biomedical collaboration and innovation, Wistar science leads to breakthrough early-stage discoveries and life science sector start-ups. Wistar scientists are dedicated to solving some of the world's most challenging problems in the field of cancer and immunology, advancing human health through early-stage discovery and training the next generation of biomedical researchers. wistar.org.

