Phase II study may lead to first systematic therapy for VHL disease

Boston, MA – VHLA Board member and trial principal investigator Dr. Eric Jonasch presented important results of the MK-6482 (PT-2977) Phase II trials today at the 2020 American Society of Clinical Oncology Annual Meeting. MK-6482 is a small molecule inhibitor of hypoxia-inducible HIF-2α, and builds directly on years of research by 2019 Nobel Medicine Prize co-winner William G. Kaelin, Jr., MD, of Harvard’s Dana-Farber Cancer Institute.

“A tremendous unmet need for VHL patients is a treatment that can decrease the size and number of VHL related tumors, while maintaining good quality of life. The results of this clinical trial are very encouraging: we are observing a reduction in size of tumors in the kidney and in the brain, with acceptable toxicity. We hope to make this treatment available for patients with VHL disease” said Eric Jonasch, M.D., professor of Genitourinary Medical Oncology at the University of Texas MD Anderson Cancer Center. “This agent could profoundly change the way we manage lesions in patients with VHL disease.”

Dr. Jonasch, a principal investigator in the international Phase II trial of MK 6482, shared results today in an oral presentation at the 2020 American Society of Clinical Oncology Annual Meeting. Dr. Jonasch has also served as a Board member of the VHL Alliance since 2016, and chairs VHLA’s Clinical Research Council.

Merck & Co, Inc., which acquired Peloton Therapeutics, previously announced the Phase II trial results on May 13. The international trial was led by researchers at the University of Texas MD Anderson Cancer Center and included the US National Institutes of Health and teams at 10 designated VHL Clinical Care Centers in the US and in Europe.

The trial, for treatment of VHL disease-associated clear renal cell carcinoma, enrolled 61 VHL patients. 30% of patients showed an objective response rate in clear cell renal cell carcinoma tumors and 86.9% of patients had a decrease in the size of their target lesions.
VHLA Executive Director Dr. Ilene Sussman said that “the positive data coming from the MK-6482 Phase 2 trial means far more than scientific success: it holds out hope for a therapeutic option for patients who currently have surgery – and multiple and high-risk surgery – as their only option.”

She continued, “We are still far from having this drug available as a prescribed treatment option to VHL patients. But MK-6482 is the first time that those fighting this disease – which is like having cancer again, and again, and again- have the hope of a therapeutic intervention.”

Von Hippel-Lindau disease is a genetic mutation characterized by tumors at any time throughout a patient's lifetime in up to ten different areas, including the brain, spine, kidneys, pancreas, adrenal gland, eyes and ears. Renal cell carcinoma is a leading cause of mortality for those with the VHL mutation, affecting approximately 40% of patients.

For an abstract of Dr. Jonasch's presentation: https://meetinglibrary.asco.org/record/185945/abstract


**About VHL**

VHL or von Hippel-Lindau disease is a genetic form of cancer. VHL patients battle a series of tumors in multiple organs throughout their lives. The VHL gene controls the major feeding pipeline of every tumor. Curing VHL is one step closer to curing many other forms of cancer.

**About the VHL Alliance**

The VHL Alliance (VHLA) is the preeminent resource and clearinghouse for those affected by von Hippel-Lindau disease, including patients, caregivers, researchers, and the medical community. VHLA is a 501(c)(3) non-profit organization founded in 1993, which is dedicated to research, education, and support to improve awareness, diagnosis, treatment, and quality of life for those affected by VHL. VHLA is the leading funder of VHL research,
funding over $2.3 million in grants to support studies designed to find a cure. The VHL Alliance's vision is Curing Cancer through VHL.

For information about VHL and the VHL Alliance, please visit vhl.org.

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