



FOR IMMEDIATE RELEASE

Contact: Heidi Leone
VHL Alliance
1.617.277.5667 | Heidi.Leone@vhl.org

VHL COMMUNITY CELEBRATES HISTORIC TREATMENT *FDA Approval of Breakthrough Treatment Brings Welcome Hope*

Boston, MA, Aug 16, 2021 – The US Food and Drug Administration (FDA) has approved the first-ever pharmaceutical treatment for patients with von Hippel-Lindau (VHL) disease, a genetic condition putting patients at risk of developing several cancers and other types of tumors. Belzutifan (Welireg, Merck), a novel HIF-2 α inhibitor, was granted Breakthrough Therapy designation in March 2021 and approved by the FDA on August 13, 2021. Its development can be traced through Nobel Prize-winning research and is in further trials for patients with kidney, pancreatic, and other cancers.

Von Hippel-Lindau disease is a genetic disorder characterized by tumors that can occur at any time throughout a patient's lifetime in up to ten different areas of the body, including the brain, spine, kidneys, pancreas, adrenal glands, eyes, and ears. Until now, the only way to treat VHL was with regular surveillance (MRIs and other tests), followed by timely surgical removal of tumors before they can cause permanent damage. Most VHL patients face multiple, high-risk and expensive surgeries over their lifetimes. Systemic therapy has long been the hope of patients and their families.

"For VHL patients, the long-held hope and belief that this battle can be won is now a reality. Thank you to the patients that have participated in clinical trials and researchers who have worked tirelessly for this breakthrough. It is because of your hard work and sacrifice that VHL-related surgeries may now be a thing of the past. We are grateful for this evidence that proves that we must remain persistent in our pursuit of Curing Cancer through VHL," said Chandra Clark, Executive Director of the VHL Alliance.

The FDA has approved the use of belzutifan (Welireg, Merck) for adult patients with von Hippel-Lindau (VHL) disease who require therapy associated with renal cell carcinoma (RCC), central nervous system (CNS) hemangioblastomas, or pancreatic neuroendocrine

tumors (pNET), not requiring immediate surgery. The approval was based on the results of a phase 2 trial for the treatment of VHL-associated RCC that showed an overall response rate (ORR) of 49% in VHL-related RCC, 63% in VHL-related CNS hemangioblastomas, and 83% in VHL-related pNETs.

“The approval of belzutifan for treatment of VHL-related renal cancers, hemangioblastomas, and neuroendocrine tumors of the pancreas is a game-changer in the treatment of VHL patients,” said Othon Iliopoulos, MD, Director, VHL Comprehensive Clinical Care Center at Massachusetts General Hospital and Harvard Medical School, Associate Professor of Medicine at Harvard Medical School. “We now have a well-tolerated pill with the efficacy to treat a disease for which surgery was previously the only option. The resulting impact on patient quality of life will be immense. I would now like to see us initiate clinical trials designed to help us understand the optimal time to start the medication and to determine the appropriate length of therapy.”

For further information on belzutifan (Welireg), please refer to Merck's press release: <https://bit.ly/3k1zTI8>

About VHL

VHL, or von Hippel-Lindau disease, is the result of a mutation of the *VHL* gene. While it is generally inherited, 20% of patients are *de novo*, or first in the family. VHL patients face a lifetime battle of unpredictable tumors, both malignant and benign, in ten parts of their body including the brain, spine, eyes, ears, lungs, kidney, pancreas, liver, adrenal glands, and reproductive organs. The *VHL* gene controls the major feeding pipeline of every tumor. Curing VHL brings us 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 \$2.6 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.

###