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**VHL
ALLIANCE
SPRING
NEWSLETTER**

Letter from the Executive Director



Building Strong VHL Communities requires engineering opportunities for collaboration

By Janet Thompson

The 16th International VHL Medical Symposium held in Milan last October was the first in-person gathering of leading VHL researchers in six years. The excitement of being together again among participants was palpable and the event inspired exceptional thought exchange and high esprit de corps.

It is a central mission for the VHL Alliance to create these opportunities for connection with intention and purpose. Not only within, but across, the three primary groups we serve: Families who navigate VHL, Clinicians who care for them, and Researchers who seek innovative treatments on their behalf.

This year, we will host traditional face-to-face events (Like the Young Adult Retreat recently held with Mayo Clinic in Scottsdale, AZ) and launch new ones that will blend our communities, inspire new ideas, and cultivate collaborative partnerships between patients and medical professionals. We will build community in a common location (Family Meeting in August) as well as cultivating gatherings where we can be together, apart (May Awareness Walk). In every venue, we look forward to bringing together patients, clinicians, and researchers for authentic engagement that will confront VHL with heart, mind, and soul.



Exciting Initiatives for 2025

The VHL Alliance is proud to unveil a series of new initiatives aimed at advancing care, education, and support for individuals and families affected by von Hippel-Lindau disease.

A New VHL-focused CME for Clinicians

In 2025, the VHL Alliance plans to launch a Continuing Medical Education (CME) program, designed to raise awareness of von Hippel-Lindau disease among primary care providers and specialists. This program will equip clinicians with essential knowledge about VHL while offering CME credits, fostering a deeper understanding of early diagnosis, appropriate referrals, and awareness of VHL disease.

By targeting clinicians within and beyond our Clinical Care Center (CCC) network, the program aims to boost referrals to CCCs, increase clinician engagement, and cultivate a future generation of VHL medical experts.

Empowering Volunteers: The VHL Ambassador Program

The VHL Alliance is proud to relaunch the VHL Ambassador Program in 2025, a volunteer initiative designed to extend our reach and strengthen connections within the VHL community. Ambassadors are individuals with lived VHL experience and their family members, trained to share VHLA resources, support newly diagnosed patients in their regions, and lead local engagement opportunities such as VHL Days or VHL Walks.

Advancing Collaboration: The VHL Research Forum

The VHL Alliance is thrilled to announce the launch of the VHL Research Forum, a transformative retreat designed to foster collaboration and innovation within the VHL research community. This initiative aims to create a dynamic, three-day gathering of top researchers.

Scheduled for November 2025, the retreat will bring together VHL leading experts and a cohort of multidisciplinary researchers for a weekend of collaboration, innovation, and mentorship.

Reducing the Cost of VHL Care

The cost of traveling to and from medical appointments, navigating the complexities of insurance, and ensuring that you continue to receive high quality care without breaking the bank is an ongoing challenge for any individual with VHL.

Utilize the VHL Programs available through the National Institutes of Health

The National Institutes of Health (NIH) has a variety of studies available to VHL patients. As the studies are free, you avoid the costs associated with private consultations or additional VHL specialist visits. The NIH does not require or bill insurance. After your first visit to the NIH, you are also eligible for partial travel reimbursements. To learn more about clinical trials and eligibility please contact Lidenys O'Brien, RN, BSN at lidenys.o'brien@nih.gov.



Leverage Angel Flight NE for Free Treatment Transport

Traveling to VHL clinical care centers can be a significant expense, especially if you live far from these facilities. Angel Flight NE offers a solution by providing free air transportation for patients in need. To arrange flights you can apply directly through Angel Flight NE's website: www.angelflightne.org



Access the Free Resources available at the EveryLife Foundation for Rare Diseases

The EveryLife Foundation has a variety of high quality resources & webinars geared towards young adults with rare diseases, including care navigation guides, insurance resources, and more! Learn more on their website at everylifefoundation.org

Explore The Free Resources on the VHL Alliance Website

The VHL Alliance provides a wealth of free resources designed to support patients and their families. These resources can help you manage your care more effectively and reduce costs. Contact the Alliance by email at info@vhl.org to learn more.

Milan Medical Symposium

More than 200 scientists, clinicians, and patient advocates attended the Medical Symposium, sharing valuable insights and research advancements aimed at improving the lives of people living with VHL disease.



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New Treatment Options in Development Give Fresh Hope

Belzutifan is just one approach in a larger effort to find effective treatments for VHL disease. Researchers at the symposium discussed promising new options, including small interfering RNA (siRNA) therapies, protein degradation techniques, and synthetic lethality strategies.

siRNA Therapies: Shutting Down Problem Genes

Dr. James Brugarolas shared insights into using siRNA (small interfering RNA) molecules, which can “silence” specific genes, like HIF-2 α , that drive tumor growth. This approach would involve delivering the siRNA directly to tumor cells by carrying them in microscopic particles. Though more studies are needed to resolve the side effects that were observed in early clinical trials, this technique could potentially prevent VHL-related tumors from forming or growing by switching off the problematic genes.

Protein Degradation: Clearing Out Harmful Proteins

Another promising area of research is protein degradation, which essentially flags harmful proteins in tumor cells for destruction. Dr. Qing Zhang explained how this approach might be used to selectively degrade cancer-promoting proteins. This method could work in combination with other treatments to disrupt tumor growth from multiple angles, potentially reducing the likelihood of resistance.

Synthetic Lethality and Synthetic Essentiality: Targeting Cancer-Specific Weaknesses

Dr. Qing Zhang’s research on synthetic lethality and Dr. Xin Lu’s work on synthetic essentiality showed how targeting specific weaknesses in cancer cells with defective (mutant) versions of the VHL protein could selectively kill these cells without harming normal ones. For example, using a drug that blocks a protein important to VHL mutant cells but is not important to cells with normal versions of VHL may cause only the mutant cells to die, sparing healthy tissue.

Why This Matters for You: New treatment strategies like siRNA therapies, protein degradation, and synthetic lethality are designed to target VHL-related tumor cells more specifically, giving hope that more treatment options will eventually be available for VHL.

Exciting Belzutifan Research



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Managing VHL can be challenging, but recent studies on Belzutifan are providing new hope.

Finding the Right Dose of Belzutifan

This LITESPARK-013 study is a phase 2 clinical trial that compared two doses of Belzutifan (120 mg and 200 mg) in patients with advanced clear cell renal cell carcinoma (ccRCC) whose disease had progressed after prior systemic therapies, including programmed death receptor (or ligand)-1 (PD-(L)1) inhibitors. The primary goal was to determine if a higher dose would offer greater efficacy without increasing adverse effects, thus optimizing the dose for treatment.

What This Means for You: For patients with VHL-related kidney cancer, these findings suggest that the standard 120 mg dose of Belzutifan is sufficient and does not need to be increased to achieve better outcomes. This supports the use of the lower dose to minimize side effects while still effectively managing tumor growth.

Belzutifan's Promise in Treating Eye Tumors

The LITESPARK-004 study investigated the effectiveness of Belzutifan, a HIF-2 α inhibitor, in treating retinal hemangioblastomas associated with VHL disease. Retinal hemangioblastomas are common in VHL patients and can lead to vision loss due to exudation (fluid accumulation), hemorrhage (bleeding), and retinal detachment. This study focused on evaluating Belzutifan's potential to manage these eye tumors non-invasively.

What This Means for You: For patients with VHL-related eye tumors, Belzutifan may offer a promising non-surgical option that can effectively reduce tumor size and manage disease progression over the long term. This could mean less reliance on invasive procedures and a greater potential for preserving vision.

Combining Belzutifan with Other Treatments

This study discussed innovative approaches for using Belzutifan in treating metastatic renal cell carcinoma (mRCC). It explored how combining Belzutifan with other therapies might overcome resistance mechanisms and improve treatment outcomes for patients with advanced RCC.

What This Means for You: For patients with advanced RCC, the combination of Belzutifan with other targeted therapies could offer a more powerful treatment approach, especially in cases where the cancer has become resistant to Belzutifan alone. These new strategies are being tested in clinical trials and could soon provide more comprehensive options for managing metastatic RCC.

Introducing VHL Connect

VHL Alliance is excited to launch VHL Connect, a new local education and engagement event designed to empower families with VHL resources and information. Contact info@vhl.org for more info on the following events!

- 📍 May 3, MD Anderson (Houston, TX)
- 📍 May 16, UPenn (Philadelphia, PA)
- 📍 May 31, Huntsman Cancer Institute (Salt Lake City, UT)
- 📍 June 7, Mass General Brigham (Boston, MA)

Join us in Michigan!

We are excited to invite you to the Family Weekend 2025, taking place from August 1-3, 2025, at the University of Michigan in Ann Arbor, MI. This annual event offers a unique opportunity to connect with others impacted by von Hippel-Lindau (VHL) disease & learn from leading VHL clinicians! Scan the QR code to secure your spot today.



Walk with us in May

Every step you take helps fund vital programs, advance treatments options, and provide critical resources for patients and families navigating VHL.

What: Fundraise to support our patient programs, fund VHL research, and more!

When: May 17th, 2025

Where: Your home turf!

Sign up today to raise awareness, support groundbreaking research, and stand with those affected by von Hippel-Lindau (VHL) disease by visiting vhl.org/walk



Thank you

At the VHL Alliance, we are dedicated to improving the lives of those affected by VHL through research, education, and support. Your generosity fuels groundbreaking research, provides critical resources for patients and families, and ensures that no one faces VHL alone.

Together, we can drive progress and bring hope to the VHL community.

Scan the QR code or mail a check via the enclosed envelope to support our mission today!

Visit vhl.org/ways-to-give to learn about donating real estate, vehicles, stock, or setting up your own fundraiser!



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