VHL Alliance

FALL 2023
NEWSLETTER

30 Years Of Service in the VHL Community

Three decades ago, the VHL Alliance was born from compassion and shared experiences. Founded by Joyce Graff, Susan Warnick, and Peggy Marshall, our mission of connection and support began.

From the moment we started in 1993—coinciding with the discovery of the VHL gene—we've grown into a global family, spanning 108 countries and bringing together over 15,000 individuals worldwide.

Our journey has been about more than years; it's been about milestones. We've expanded programs, empowered research, and embraced growth. And now, as we enter our 30th year, we're thrilled to execute on our new strategic plan, guiding us toward a future of even greater impact.

Together, with our devoted team and your unwavering support, we're shaping a tomorrow where VHL patients, families, and caregivers find enhanced well-being and brighter prospects.

Join us in celebrating this incredible milestone at our 30th Anniversary Gala on October 6th in Philadelphia! This will be a night of inspiration, connection, and hope. Let's come together to make it an unforgettable evening.

Here's to 30 years of resilience, unity, and endless optimism.

VHL|30TH ALLIANCE

1993
VHL Family Alliance (VHLFA) is incorporated and the VHL gene is identified

1994
First International Research Symposium held in Germany

1996
First VHL Family Weekend takes place in California

1998
VHL Family Alliance launches the research grant program

2009
VHL Kids Handbook is released

2012
VHL Clinical Care Center initiative begins

2013
The 'VHL Family Alliance' becomes 'The VHL Alliance'

2014
The Cancer in Our Genes International Patient (CGIP) Databank launches

2015
First ever VHL May Awareness Month

2018
Phase 2 clinical trial using belzutifan begins

2018
Dr. William G. Kaelin, Jr., Sir Peter J. Ratcliffe, and Dr. Gregg L. Semenza win the 2019 Nobel Prize for their work on VHL

2021
Welireg (belzutifan) approved by the FDA to treat VHL patients and the VHL Virtual Tumor Board launches

2023
We celebrate 30 years of service in the VHL Community!

The VHL 30th Anniversary Gala is Coming!

A celebration like no other and a chance to raise funds towards advancements in research, treatment, and a cure for VHL.

This year's event will be a very special celebratory evening highlighting 30 years of great work of the VHL Alliance and honoring the incredible contributions of Dr. Eric Jonasch and his tireless commitment to working on behalf of the entire VHL community for so long. The evening will begin with a reception and move to dinner and celebration at The Franklin Institute, Philadelphia PA, on Friday, October 6, 2023. For more information on how to join in the celebration and/or become engaged as a sponsor, we invite you to visit our website here.

We look forward to having our community and supporters together in this gala celebration!

If you're unable to attend the gala in person, you can still celebrate our 30th Anniversary by making a contribution at vhl.org/donate

2023 VHL Family Weekend

With your help, we made the 2023 Annual VHL Family Weekend one of our best. This year's celebration included dynamic speakers and presentations from the talented Vanderbilt University team and VHL experts from around the country, impromptu karaoke sessions, and a constant exchange of ideas, inspiration, and more.

Events like this underscore the importance of connection and collaboration in achieving our mission. Oftentimes a negative aspect of a rare disease is feeling isolated—events like Family Weekend help us build community through our unique shared experience. Gathering in person has been especially powerful coming on the heels of the COVID years when we were not able to assemble face to face.

We're already working on our location for VHL Family Weekend in 2024. Keep your eyes peeled for more information in the coming months!

Bringing Awareness to the VHL Cause in May

While any of us can confirm that the effects of VHL are not limited to one month each year, May Awareness Month is an important time for the VHL community. Not only do we celebrate our strengths as a community and the many advances in treating this rare disease, but we also come together in May to shine a strong, focused light on VHL.

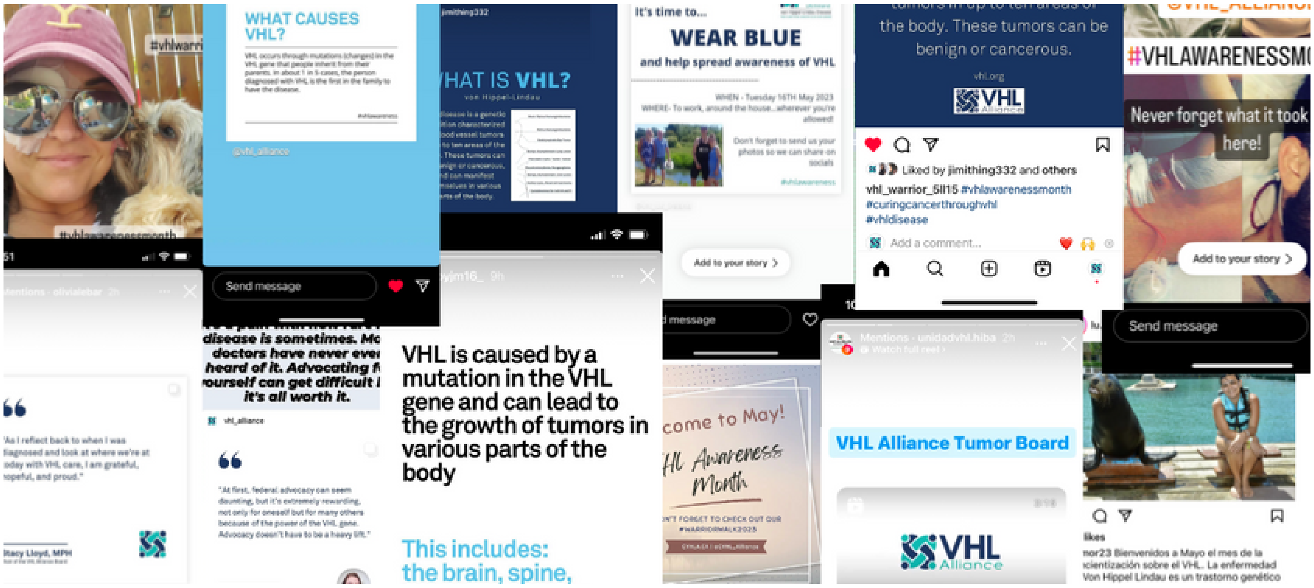
You may remember a time when you learned what happens when sunlight is focused through a magnifying glass onto a single leaf or a blade of grass. That magnified, focused light has a powerful impact. This is our goal each year with VHL Awareness Month.

We are fortunate to work with a large and growing network of partners who help us focus that light each May. One of these partners is the National Health Council who offered us a guest blog post on their website in May of this year. National Organization for Rare Disorders (NORD) also provided opportunities to get the word out about VHL.

There are many folks behind the scenes at these organizations that care about our community and we especially feel that when we ask for their support to bring awareness each May.

VHL Awareness Month is a communication effort. We communicate in a variety of ways, from wearing our favorite VHL t-shirt and talking to people about VHL in the grocery store to sharing the #vhlawareness hashtag on social media. One VHL patient, Isaiah Lee, even brought our message to the national stage on American Ninja Warrior! No matter which way we choose to communicate, the goal is the same: **Tell our stories.**

This year, our community showed up in a big way to give the world a view into our own. Thank you to every single community member, partner, and advocate who helped raise awareness and tell our story!



Senate Committee Removes Research Restrictions on DoD-Funded VHL Research

The VHL Alliance, led by its Advocacy Working Group, successfully lobbied the Senate Committee on Appropriations to remove restrictions on Department of Defense (Dod) funded research on VHL. Specifically, on July 27, the committee approved its version of the fiscal year 2024 Defense Appropriations Act, which makes VHL research eligible for funding from the Peer Reviewed Medical Research Program (PRMRP), a $370 million competitive grant program.

Unlike previous years, however, the Senate Committee this year included the designation of "Von Hippel-Lindau syndrome" without also including limitations of where the condition manifests (e.g., kidney, brain, spine, etc). Last year's funding bill, the fiscal year 2023 Defense Appropriations Act, included the designation as "Von Hippel-Lindau Syndrome Benign Manifestations", which effectively includes any research proposals that seek to study the cause of VHL or anything related to cancerous aspects of the disease. If this year's designation becomes law, VHL researchers will be able to submit applications to the PRMRP for a much broader scope of VHL research topics.

In a related development, the Senate Committee on Appropriations on July 27 also approved the fiscal year 2024 Labor, Health and Human Services, Education Appropriations (LHHS) bill, which included language directing the National Institutes of Health to "continue to support research on VHL disease and biology, seeking both pharmacological and gene therapy treatments for VHL and other cancer patients." The Committee in its report accompanying the bill also recognizes that "finding a treatment and cure for VHL disease... is key for treating and curing not only the rare disease of VHL but also many other forms of cancer."

These important legislative accomplishments would not have been possible without advocacy efforts of VHL families around the country and the hard work of two of our Congressional champions, Senators Cory Booker (D-NJ) and Ed Markey (D-MA).

The full Senate is expected to vote on both the Defense and Labor-HHS Appropriations bills in September after it returns from its August recess.

American Society of Clinical Oncology (ASCO) Annual Meeting

A research poster entitled “The impact of surgery on patients with VHL-associated tumors: An international patient survey” was presented at the 2023 American Society of Clinical Oncology (ASCO) Annual Meeting that took place on June 2-6 in Chicago, IL. The poster described research led by a team including VHLA Research Council Chair Othon Iliopoulos, M.D., VHLA Director of Health Joshua Mann, and others.

Data collected included demographics, impact of most recent surgery on aspects of condition, treatment goals, and patient preference in treatment. Patients were presented with two scenarios: “watch and wait” and potentially need surgery, or “take a once-daily pill” to delay a potential surgery.

220 VHL patients participated in the survey. Of those, 205 people had experienced surgery at least once and 171 people had experienced multiple surgeries. The results showed that surgery negatively impacts the lives of patients, leading to a worsening in their fatigue, mental health, and ability to go about daily life. Almost half of patients would like a treatment that reduces the number of surgeries, and over a third would like a treatment that delays the need for surgery. Nearly three quarters of patients would prefer to take a pill that might delay the need for surgery. It was concluded that treatment options that reduce the need for surgery would benefit patients with VHL.

Kidney Cancer Research Summit

The 2023 Kidney Cancer Research Summit (KCRS), organized by VHLA advocacy partner KidneyCan, took place on July 13-14 in Boston, MA. The two-day summit brought together clinicians, researchers, advocates, industry, and others to discuss efforts for collaboration in the field of kidney cancer research. It featured panel discussions and lectures by the world’s leading experts in the field, many of whom work very closely with The VHL Alliance and the VHL community.

Some of the more VHL-relevant lectures included “Biomarkers in Kidney Cancer”, “Clinical and Scientific Updates in Kidney Cancer”, “Expert Insights on the Research Funding Landscape”, and a wonderful keynote lecture by Nobel Laureate and VHL researcher, Dr. William G. Kaelin, Jr., entitled “New Directions in Basic Kidney Cancer Research”. In it, he described how approaches to treating kidney cancer have changed drastically over the years as a result of robust research in the field. He predicted that in the future, there will be better treatment options with more genetically validated targets and that cancer may one day be cured through research into advanced combination therapies.

VHL Research Update

Amit Tirosh, MD, Sheba Medical Center
Clinically, several important reports have been published. Tamura and team published a comprehensive analysis of the spectrum of genetic changes in the *VHL* gene in the Japanese population, including an impressive number of 206 families. The thorough analysis shed more light on rare genetic changes in the *VHL* gene, which can lead to VHL disease.

Bond and team performed an important work on the emotional burden of VHL. The study was published in *Family Cancer*, and assessed the attitude of young adults with VHL, towards dating and to disclosing VHL diagnosis to a dating partner. The author reports that diagnosis with VHL contributes to fear of rejection due to decreased desirability. This concern was exacerbated by the unfamiliarity of the partners to the disease. However, some of the participants suggested that the reveal of VHL diagnosis strengthened the relationship by deepening trust. Importantly, participants discussed a desire for healthcare providers to offer support in this context. The clinical implications of this research are direct and can be implemented immediately, by discussing dating with our patients and raising the option to explain more about the disease to the partner.

Finally, very recently the collaborative effort of VHL experts from all around the world, led by the VHL Alliance and Dr. Anthony Daniels from Vanderbilt University Medical Center, was finalized and published. The guidelines, fruit of parallel work in subgroups focusing on VHL-related issues, delineate the diagnosis, surveillance and key management issues in patients with VHL.

VHL Research Funding Available Now

Every year, the VHL Alliance deploys nearly $200k in grant funding to support VHL focused medical research worldwide.

We're pleased to announce that applications for our 2024 grant program are open now! Projects should further the understanding of VHL disease and may address VHL cell and molecular biology, the development and/or application of VHL-relevant animal models, or the diagnosis and treatment of VHL patients.

Letters of Intent are due on October 6, 2023, and can be submitted to research@vhl.org.

More information can be found at www.vhl.org/research/grants.

VHL Alliance Experience Survey Coming Soon

Your voice matters! Get ready to influence the future of the VHL Alliance. Look out for an email with the VHL Alliance Experience Survey—it's your chance to help us improve our programs and services.

What's Inside:
Share your insights on your VHL journey and interactions with us. Your feedback will guide us in creating a stronger, more engaged community.

Why Participate:

- Help shape our programs as we enter our 30th year
- Highlight areas for growth and enhancement
- Foster a supportive VHL network

Together, we can make our community thrive. Watch your inbox for the survey link at the beginning of October!

Save the Date

KidneyCan Advocacy Days
September 18-19, 2023
We're proud to support our partners at KidneyCan.org as they continue to advocate for life saving research funding. Click here to learn more!

VHL Alliance 30th Anniversary Gala
October 6th, 2023
Join us as we celebrate our 30th Anniversary in style at the Franklin Institute in Philadelphia. Click here to register now!

VHL Veterans Panel
November 16, 2023
Join us for a special webinar geared towards newly diagnosed patients and family members. A panel of VHL veterans will be available to answer your VHL related questions!

Young Adult Retreat
Feb 23-25, 2024
We're excited to announce that the VHL Alliance Young Adult Retreat will be returning to Washington, D.C. in February. Do you know a VHL patient between the ages of 18-35 who wants to attend? Scholarships to cover travel expenses are available to those who need. Send us an email at info@vhl.org for more information.