The world is changing before our eyes. Our priorities are becoming more clear. It is important to appreciate the reality in which we find ourselves as we realize that things will be “new” and not “different.” “Different” seems negative, “new” feels like a more encouraging and an upbeat way to approach this time.

Although this time is uncertain and scary, there is a sense of normalcy in our community. All of us who are patients and caregivers are experts at uncertainty and fear. The rest of the world is just catching up to what we have already embraced. Fear does not stop us, uncertainty in life is an expectation, and, through this, we understand what we need more than what we want. I am proud to know so many of you and to have heard your stories and listened to your struggles and triumphs.

Crisis, such as this pandemic, result in unintended consequences, both positive and negative. The outcome of how VHL Alliance has handled the world crisis over the past weeks has been remarkable. More patients and caregivers are connecting via webinars and conference calls than at any time in the past. The facilitation of these interactions is creating relationships that may not have otherwise been discovered. VHLA is committed to continuing to do all that we can do, to assure that we will grow and endure after this crisis is over.

During the pandemic, the work of VHLA’s staff and volunteers have exemplified how to turn a crisis into a wonderful opportunity. Existing programs have been adapted and new programs have been created to connect and meet the immediate needs of our constituency. I strongly urge you to learn more and participate in the new “Surviving and Thriving Together” series.

VHLA’s mission continues; we remain committed to research, education, and support to improve awareness, diagnosis, treatment, and quality of life for those affected by VHL. Our vision of Curing Cancer through VHL will not change.

Executive Director Ilene Sussman is retiring later this year. Her leadership and knowledge have been impressive and served all of us well. We have started the search for her successor and are confident in finding a new Executive Director that will move the organization forward with the same growth we experienced under Ilene’s guidance. Thank you, Ilene, for everything and thank you to your team for all of the hard work that gets done each day.

We do not know what our changing world means for the finances of VHLA. But we do know that we will not let anything deter our tremendous momentum from the past five years, such as continuing to support patients and caregivers, facilitating high-quality coordinated care through our Clinical Care Centers, and honoring Nobel Laureate Dr. William G. Kaelin, Jr. As our world finds its new normal, we hope to continue this path that has allowed VHLA to fund research critical for accomplishing our vision of Curing Cancer through VHL.
VHL AWARENESS

VHLA AND RARE DISEASE DAY

by Jennifer Galenkamp, VHLA Board Member

The VHL Alliance Chairman Doug Karle and I joined approximately 800 other rare disease advocates in Washington, DC, February 25-28. The week-long event, organized by Rare Disease Legislative Advocates (RDLA, rareadvocates.org), included a briefing by the Congressional Rare Disease Caucus. This all-day conference addressed the issues of concern to the rare disease community, and culminated with an Advocacy Day on Capitol Hill. The week also included an event at the FDA and a separate event at NIH, which were free and open to all. Participation in this annual event is opened to anyone and each year the numbers continue to grow as the rare disease community unites.

Doug noted that, “by combining forces, rare disease organizations like VHLA can expand their influence not only in size but also in access to resources and information.”

Some of the issues for which RDLA has been advocating include: expanding incorporation of patient experience data into the FDA's regulatory review process (through S. 1052, Support the Better Empowerment Now to Enhance Framework and Improve Treatments (Benefit) Act); increasing funding for the FDA Orphan Products Clinical Trial Grants Program and the Natural History Grants Program (like MyVHL, vhl.org/MyVHL), to keep up with both increased costs of clinical trials, and a large number of quality grant applications, most of which go unfunded. Another initiative will support the creation of the Rare Disease Center of Excellence at the FDA, following the success of its Oncology Center of Excellence.

These were long, emotion- and hope-filled days. But the positive response to the requests of the Rare Disease community, including to VHLA’s request for expansion of support for VHL research through NIH grants, was heartening. Most people do not know that [2019 Nobel Laureate in Medicine] Dr. William Kaelin, whose research is on VHL, is an NIH External grant recipient.

Meanwhile, out in Nevada, a newly diagnosed VHL patient, 14-year-old Ysa, took the initiative to advocate in her state. Since the shock of her de novo diagnosis, Ysa said, “my family and I have been researching a lot. My mom, Amanda, got into contact with the Nevada Rare Action Network Ambassador, and we attended a Rare Disease Day 2020 event that she was hosting. This event was attended by members of Congress, such as Steven Horsford, as well as motivational speakers. I had the chance to share my story and was invited to speak at the podium. We need to increase awareness of rare diseases in all spaces including advocating for all to receive proper medical treatment.”

Advocacy – making our case to lawmakers – is, to be sure, a new area for VHLA. We will be working in the coming months to provide resources and encouragement for each one of you to get involved with Rare Disease advocates at your State level, and to begin to plan for a large VHL delegation in Washington next year. We are looking for people like Ysa and her mother who want to get involved and are willing to share their stories to make a difference. If you are interested in learning more, please email Ilene at ilene.sussman@vhl.org.

THE VHL ALLIANCE WOULD LIKE TO INVITE YOU TO AN EVENING HONORING

William G. Kaelin, Jr., MD
2019 NOBEL LAUREATE IN MEDICINE

WEDNESDAY, SEPTEMBER 2, 2020
HARVARD CLUB OF NYC
vhl.org/NobelDinner

New Date!
ADVOCATING FOR FEDERAL SCIENTIFIC RESEARCH DOLLARS

The National Institutes of Health’s (NIH) extramural grant program ($32 billion/year) is the largest funder of scientific research in the US. While that sounds like an extraordinary number, the reality is that less than 20% of the greater than 50,000 submitted grants are funded. In other words, the competition for NIH research funding is fierce. The areas of research of submitted and approved grants are vast. In general, there is an unspoken bias to fund research that will impact the greatest number of people. This is why the funding of research for rare conditions is more challenging. It is for the same reason that VHL researchers tend to focus their grant submission on the kidney, renal cell carcinoma (RCC). Sporadic RCC is currently one of the fastest-growing of all cancers. This explains why our understanding of kidney tumors far surpasses that of other VHL manifestations.

Is there a way to push the needle in the direction of more dollars for VHL research? A major first step is to ask Congress to recognize that finding a treatment and cure for VHL is a key to treating and curing many other forms of cancer and to strongly encourage NIH to support additional internal research and extramural grants on VHL.

Making the case to our lawmakers was one of VHLa’s efforts during February and March. Late winter is a peak time for the U.S. House of Representative’s appropriations process and it is important to remind our Congressional leaders, of the importance and impact of VHL research. Members of the House and Senate appropriations committees are key people in this process.

Another source of scientific research funding is the Department of Defense (DoD). In 1992, the DoD created the Congressionally Directed Medical Research Program (CDMRP). With an annual budget of approximately $1.2 billion, researchers who meet the strict criteria have a less than 15% chance of being funded by the DoD.

There are three distinct areas of funding in the CDMRP program.

• **Peer-Reviewed Medical Research Program** (PRMRP, $360 million): This “catchall” area includes 49 conditions ranging from diabetes to lung injury or disease and includes pancreatitis. The budget is not equally distributed among all the diseases specified in the program.

• **Peer-Reviewed Program** (PRMRP, $110 million): This is a pool of 15 cancers including Rare Cancers ($7.5 million) under which grants related to VHL can be submitted.

• **Disease-Specific Line Items** (approximately $730 million): There are 30 disease-specific line items. Breast cancer research is the largest recipient at $150 million. Kidney cancer research is also included with a funding level of $40 million.

Only grants addressing the pre-specified disease can be submitted to any of these 3 programs. This means, for example, a grant on CNS hemangioblastomas can be submitted to the Cancer Research Program as a rare cancer, while a grant related to RCC can be submitted under the kidney cancer line item. With an even lower percentage of grants being funded through the DoD than through NIH, many excellent VHL-related grants are not being funded.

What can VHLa and its constituency do to improve the likelihood of getting VHL included as a specific line item? There are two ways.

The first approach is to work to have VHL specifically included in the Cancer Research Program. Advocacy to members of the Senate is key since only they have the authority to ask for a specific disease to be included. Connecting to senators who sit on the defense appropriations subcommittee is critical.

The second approach is to work with congressional leaders to make VHL a Disease

...your story is impactful... help us advocate for federal dollars to expand VHL scientific research.”

continued on page 4
A MESSAGE FROM THE VHLA CLINICAL ADVISORY COUNCIL

Dear VHL Community,

As you are well aware, we are all experiencing significant changes in response to the COVID-19 pandemic. Hospitals around the world are quickly developing strategies to treat an influx of coronavirus patients, while at the same time making sure that the care of existing patients is minimally disrupted.

While there is no question that surveillance is one of the most important tools for managing VHL, in light of the current situation, each person must weigh the risks and benefits of attending an in-person office appointment versus temporarily delaying a scan. This is something that only you, in conjunction with your medical team, can decide. Therefore, if you are scheduled for routine follow up or surveillance examinations or scans in the near future, please contact your VHL provider to decide if the timing should be changed. Telemedicine is a great option, when possible and appropriate. In the event of an emergency or the development of new VHL-related symptoms, do not hesitate to seek advanced medical care.

Please note that there is currently no evidence to suggest that VHL patients are at greater risk of contracting COVID-19 compared to the general public. If you have any concerns regarding your specific situation, please contact your VHL provider.

During this crisis, the VHL Alliance will remain fully functioning. Feel free to reach out if you have any questions or need any assistance. We are continuing to monitor the rapidly changing circumstances and will update our recommendations as needed.

Stay safe!
The VHL Alliance Clinical Advisory Council

For More Information on COVID-19 and VHL
Visit vhl.org/coronavirus

Learn more and join VHLA’s “Surviving and Thriving Together” Series
vhl.org/together
The VHL Alliance has a lot of updates to our Clinical Care Center (CCC) Program. We are excited to announce Levine Cancer Institute in Charlotte, NC and Northwestern Memorial Hospital in Chicago, IL have been recognized as the newest VHL Clinical Care Centers! Additionally, the VHLA Clinical Advisory Council has renewed recognition for the following CCCs: MD Anderson, UVA, Vanderbilt, Mayo Scottsdale, MSKCC, UCLA, UC Denver, UMN and WashU.

The CCC Spotlight Program has been going great! We have learned more about VHL medical professionals from all over the country. Check out our CCC Spotlight Series for more.

In light of most non-urgent appointments being postponed or conducted remotely via telemedicine due to COVID-19, we have distributed to all VHL Clinical Care Centers an AMA quick guide for expediting the implementation of telemedicine.

Most importantly, we want to thank the medical professionals and support staff for everything that they do, not just for VHL patients, but for all patients. We sincerely appreciate their hard work, devotion, and sacrifice, especially those on the frontlines protecting us during this crisis.
WHAT TO DO IF DENIED INSURANCE COVERAGE

Unfortunately, one of the most frequent questions we are asked is “What do I do? My insurance denied coverage for my VHL care.” When a VHL patient or family gets that news, it can be overwhelming. But fear not; there are some steps that you can take. You should know that you have a right to challenge and appeal any decision made by your health insurance company denying coverage.

If you find yourself in this situation, here are some steps that you can take:

Follow the process for filing the appeal as outlined by your insurance company in your explanation of benefits package and write a letter. Make sure to include your name, policy number, policy holder’s name, contact information, date of the denial letter, what was denied, the reason given for the denial, why the service should be covered, and the name and contact information for your doctor. Ask your doctor to write their own appeal letter on your behalf; ask them to include any relevant journal articles or surveillance/treatment guidelines.

If your appeal is denied, you still have a right to file a formal grievance asking for an external review. It is then up to third party clinical reviewers to decide whether the denial was made in the best interest of the patient’s health. External reviews may take up to 45 days, however they can be expedited to no more than 72 hours in cases of medical urgency.

Finally, you can file a complaint with your state’s Department of Insurance, who might be able to help make sure your claims are addressed properly. If you have any questions or need assistance, please call your state’s Consumer Assistance Program (CAP or Department of Insurance).

The whole process can be extremely tedious and stressful. You and your doctor will be your strongest and most important advocates. Working with VHL expert specialists at CCCs will help strengthen your case. Information is power, so make sure to also back up your claims with information from the VHL Handbook and the VHL Surveillance Guidelines. Your perseverance and resilience will hopefully pay off with a decision reversal.

If you have any questions, please feel free to reach out to us at the VHL Alliance. Our website also has helpful non-VHLA financial aid resources.

MyVHL:

The VHL Patient Natural History Study

Do you want to help find a cure?

PARTICIPATE NOW!

Learn more at: vhl.org/MyVHL

Pay it forward.

Tell your story to help build the data for a cure.
The VHL Alliance is grateful to anyone who hosts a fundraiser! These events are essential to our ability to support VHL research and education. We love to see communities coming together to encourage those they hold dear and contribute to our cause. We want to share with you two recent events.

The Montclair Kimberley Academy community was inspired by an esteemed graduate who had recently been diagnosed with VHL. Their creativity led them to hold a Student-Faculty basketball game in support of their beloved classmate while raising $8,000 for VHLA. We love to see communities coming together to encourage those they hold dear and contribute to our cause.

Imagine 48 hours of non-stop running to benefit VHL and honor their friend and inspiration, Shawn Mastrantonio. This is exactly what the LUNAR Running Festival was about. As Shawn said so eloquently, “this was very humbling because of the thoughtfulness, support, and love shown, not just for me, but for all the VHL WARRIORS and everybody who participated. That in itself is truly a gift and such a blessing. I recently read a quote by Ernest Hemingway, ‘We’re all broken. That’s how the light gets in.’ I believe that it works both ways. We get light but we also give it.” Combined, the 150 participants ran nearly 600 miles in 48 hours and raised over $7,000.

A shout out to the Montclair Kimberley Academy community and those involved in the LUNAR Running Festival, especially Meghan Brown, Don Kelly, Bob Kelly, and others for all they did to make these events so successful.

We hope events like this will continue as a way of inspiring, supporting, and bringing awareness to the VHL community. If you are interested in doing an event to honor someone you love to benefit the VHL Alliance, please reach out to Heidi at heidi.leone@vhl.org.

**Take your May Awareness event virtual!**

Host a virtual Coffee Against Cancer, book club, paint & sip, happy hour, or game night.

So many ideas to bring people together! Take just a few minutes to tell them about VHL - and share your story.

#VirtualVHL #CuringCancerthroughVHL #VHLCoffeeAgainstCancer #VHLAwareness
We’ve Gone Virtual!

2020 Julie Flynn Hope Retreat

Register now at: vhl.org/yar

June 13, 2020

Are you a young adult with VHL? If so, join other young adults for an afternoon of fun, discussion and information!

Biennial International VHL Medical/Research Symposium

Register now at: vhl.org/symposium

Amsterdam, the Netherlands

October 29-31, 2020

2020 Annual Family Weekend

Register now at: vhl.org/familyweekend

Salt Lake City, UT

October 23-25

14th International VHL Medical/Research Symposium

Designed by Annika Marshall