



VHL Alliance

(formerly VHL Family Alliance, VHLFA)

STRATEGIC PLAN AND UPDATE

FY13-15

Strategic Plan

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VISION STATEMENT

The VHL Alliance envisions a cure for VHL

MISSION STATEMENT

The VHL Alliance is dedicated to research, education, and support to improve diagnosis, treatment, and quality of life for those affected by VHL.

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BACKGROUND

VHL ALLIANCE (VHLA)

The VHL Alliance was incorporated by the Commonwealth of Massachusetts on April 28, 1993 and received 501(c)(3) nonprofit status on July 1, 1993. The organization was begun by Joyce Graff, Peggy Marshal, and Susan Warnick with the realization of the need and importance of creating a VHL community for patients and families. At a time preceding the Internet, meeting or speaking with another person or family members affected by VHL was difficult and unlikely. In addition, the rarity of VHL makes diagnosis and treatment uncommon. This in turn, meant that as a whole, the medical community was not – and unfortunately continues not to be – as familiar with or aware of von Hippel-Lindau. Few were aware of the multi-system impact of VHL and little information was known about the VHL gene, which was yet to be identified.

Today the VHL Alliance is the pre-eminent resource for patients, caregivers, researchers, and the medical community. The organization is an international network of family support groups serving an estimated 15,000 people worldwide, in 108 countries. It is estimated that, through associated International alliances many of which were spurred by the VHL Alliance, the organization is directly or indirectly connected to approximately 90% of patients and families with a known diagnosis of VHL, world-wide.

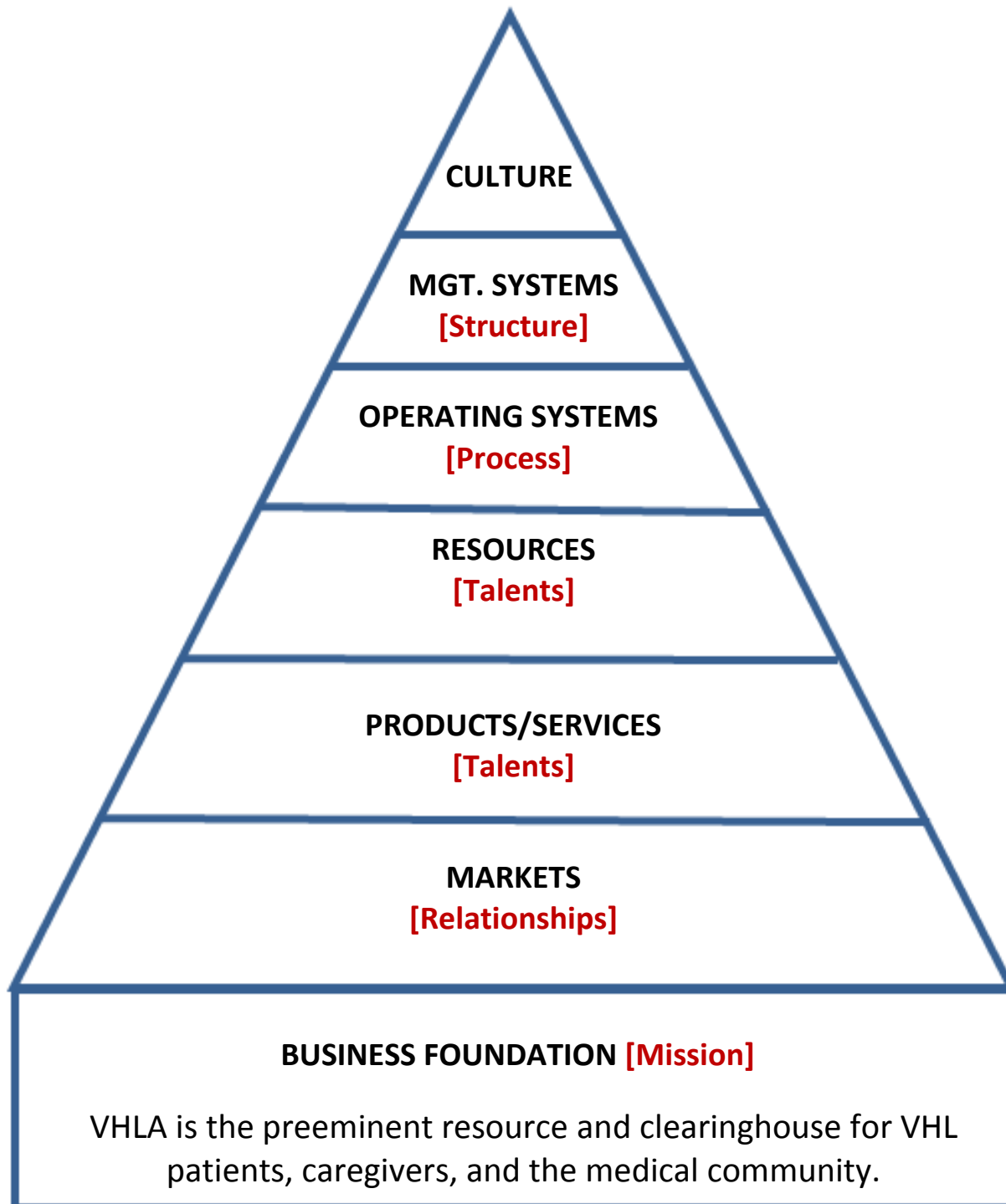
In the fall of 2011, the VHL Alliance initiated its first major transition in leadership. As VHLA's Founding Director Joyce Graff began to contemplate retirement, together with the Board of Directors, a decision was made to recruit and hire a new Executive Director. At Ms. Graff's suggestion, a new staff role was created for her, Director of Wellness, enabling the organization and its constituency continued benefit from her extensive knowledge of VHL diagnosis and treatment, unequalled by anyone in the field.

The hiring of a new Executive Director, Ilene Sussman, initiated an evolution from a grassroots organization to one with a more professional environment. The transition provided an opportunity for VHLA's leadership to reassess the organization's vision, mission, and strategies. A strategic planning committee, comprised of patients and family members including members of the Board of Directors, Chapter Leaders, and other volunteers was convened charged to develop a blueprint that would expand on current programmatic efforts and provide direction for the organization as it entered its third decade.

The resulting Strategic Plan, described within, is considered a guide for the VHL Alliance; it is in no way to be considered unalterable. During implementation of this Strategic Plan, modification will become inevitable. As such, the plan is a "living" document.

FIGURE 1: ASSESSING THE VHL ALLIANCE

Primary Client: Patients



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ASSESSING THE VHL ALLIANCE

BUSINESS FOUNDATION

A Business Foundation is the keystone an organization on which it can achieve its defined mission. The business foundation is the underpinnings of the organization's programmatic efforts on which the organizational structure is determined. A clear and agreed upon definition is essential when initiating a strategic planning process.

Business Foundation of the VHL Alliance

VHLA is the preeminent resource and clearinghouse for VHL patients, caregivers, and the medical community.

MARKETS

The **patient** is the VHL Alliance's **primary client**. At the same time, there are multiple support communities, stakeholders, or markets on which the patient relies for support, care, diagnosis, treatment, and advancements toward a cure. As such, it is in the best interest of VHL patients that the VHL Alliance broadens its programmatic efforts to include these additional important stakeholders.

VHL Alliance Markets

- **Patients**, primary client
- **Caregivers**, including family members and loved ones
- **Researchers**, advance diagnosis and treatment and, ultimately discover a cure
- **Medical Community**, provide clinical diagnosis and treatment
- **Pharmaceutical Industry**, develop and market new forms of treatment
- **Imaging Industry**, develop and market new forms of diagnostic tools
- **General Community**, through general awareness and VHL and the involvement of the VHL in other forms of cancer research toward a cure will be expanded

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ORGANIZATION CULTURE

An organization's culture reflects its internal values as well as its external image. Programmatic efforts and organizational structure need to be designed and implemented to support the organization's Culture. Programs and structure that are not aligned with the envisioned Culture can derail an organization from reaching its goals.

Culture of the VHL Alliance

- Active/Engaged
- Altruistic
- Collaborative
- Community
- Compassionate
- Creative
- Empathetic
- Entrepreneurial
- Generous
- Patient focused
- Philanthropic
- Responsive
- Resourceful
- Supportive
- Uplifting

ASSESSING EXISTING SERVICES, RESOURCES, AND SYSTEMS

As a successful organization with a 20 year history, the VHL Alliance has developed excellent programmatic efforts - Products/Services - to reach out, support, and educate the defined markets. Resources through which the services are provided along with Operating Services to deliver the services exist. Management Systems are in place to guide and implement the work of the VHL Alliance.

Existing Products/Services of the VHL Alliance

- Clinical Care Centers
- Clinical Trials Task Force
- Educated Consumers
- Family Meetings
- Hotline Volunteers
- Internet Communications
- International VHL Affiliate Support
- Newsletter
- Online Communities
- Patient Registry
- Patient Registry Task Force
- Personal Education and Support
- Regional Chapter Leaders
- Relationship with other rare genetic forms of Kidney Cancer
- Research Community
- Research Grants
- Research Meetings
- Support Groups
- Tissue Bank
- VHL Handbooks
- Website
- Written Material

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Resources

- Board of Directors
- Clinical Care Centers
- Committees & Task Forces
- Expertise in VHL
- Medical Advisory Board
- Newsletter
- Office Staff
- Online Communities
- Patients
- Research Advisory Board
- Volunteers
- Website

Operating Systems

- Board of Directors
- Chapters
- Clinical Care Centers
- Committees
- Hotline
- Medical Advisory Board
- Office Staff
- Research Advisory Board

Management Systems

- Board of Directors
- Committees
- Medical Advisory Board
- Office Staff
- Policies
- Research Advisory Board

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APPROACH

SMART GOALS

Creation of SMART Goals¹ is a management tool often used for personal development. This technique was adapted and applied to creating organizational goals for the VHL Alliance.

Letter	Major Term	Explanation
S	Specific	Clear, unambiguous, easily understood What is expected? Why is it important? Who's involved? Where is it going to happen? Which attributes are important?
M	Measurable	Defines the answer to how will the team know when it has accomplished its goal, including what should be accomplished and what is the goal Covers at least one measure of quality metric, quantity, time and/or cost effectiveness
A	Attainable	Achievable In the organization's ability and resources to achieve; May stretch a team but are neither out of reach nor below standard performance
R	Realistic	Practical, sensible, reasonable Within reach to meet expectation; achievable
T	Timely	Time bound with defined target date A commitment to a deadline provides focus of efforts to complete the goal on or before the due date

¹ Doran, G. T. (1981). There's a S.M.A.R.T. way to write management's goals and objectives. *Management Review*, Volume 70, Issue 11(AMA FORUM), pp. 35–36.

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OUTCOME

BOARD STRUCTURE

Based on the defined SMART Goals, the FY13 Board of Directors created seven Committees to advance the ambitious objectives identified through the Strategic Planning Structure. At least one member of the Board members will be responsible for each committee and will serve to report back to the Board on the committee's progress.

Board Committees

1. Build Resources Committee
2. Strengthen the Organization Committee
3. Enhance Programs Committee
4. Finance Committee
5. Nominating Committee
6. Clinical Advisory Council (formerly named Medical Advisory Council)
7. Research Council (formerly named Research Board)

WORK OF COMMITTEES, COUNCILS, AND STAFF

BUILD RESOURCES COMMITTEE - CHAIR, GAYLE GREENE

- Enhance branding
- Create and execute a comprehensive fundraising plan
- Identify and build strategic relationships/alliances

STRENGTHEN THE ORGANIZATION COMMITTEE – CHAIR, KAREN RAMSEY

- Build a strong and effective Board
- Clarify roles and responsibilities for Board, Committees, Volunteers and Staff
- Leadership Development/Succession Planning
- Revise Bylaws and policies

ENHANCE PROGRAMS COMMITTEE – CHAIR, BOBBY KRAMER

- Increase outreach efforts to both medical patient/family communities
- Augment educational efforts to both medical patient/family communities
- Enhance support efforts to both medical patient/family communities

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FINANCE COMMITTEE – CHAIR, MICHELLE CIESLAK

- Develop annual budgeting process and long-range (3 yrs) forecast
- Expand finance committee
- Evaluate programmatic efforts for effectiveness, impact and cost/benefit

NOMINATING COMMITTEE – CHAIR, JULE FOBERT

- Build volunteer base
- Review and recommend volunteers to fill leadership roles in the VHL Alliance

CLINICAL ADVISORY COUNCIL – CHAIR, SARAH NIELSEN

- Serve as experts in all areas of VHL clinical care and diagnosis
- Update standards and disseminate guidelines for all areas of VHL care and diagnosis
- Create and monitor guidelines for VHL Clinical Care Centers including approval and review of such centers
- Advance VHL Clinical Research
- Share important advancements in clinical and diagnostic care with the VHL healthcare providers and constituents
- Educate members of the given specialty about VHL diagnosis and care

RESEARCH COUNCIL – CHAIR, JIM GNARRA

- Promote VHL research
- Advise the Board of Directors on the most pertinent areas of research to be supported by VHL Alliance funds
- Review and oversee VHL research funded by the VHL Alliance
- Share important advancements in research with the VHL researchers and constituents

STAFF UNDER THE LEADERSHIP OF THE EXECUTIVE DIRECTOR

- Enhance communications to markets
- Enhance and expand volunteer involvement
- Transition knowledge base to institutional knowledge

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VHL ALLIANCE SMART GOALS: 2013-2015 STRATEGIES & TACTICS

BUILD RESOURCES

- Branding
- Communications
 - Newsletter
 - Annual Report
 - eBlasts
 - Marketing Materials
 - Media
- Fundraising
- Research Foundation
- Partnerships

STRENGTHEN THE ORGANIZATION

- Strategic Planning Process
- Accountability
- By Laws Revisions
- Website Infrastructure
- Organizational and Board Assessment

ENHANCE PROGRAMS

- Online Communities
- Outreach
- Annual Meetings
- Additional Educational Programming
- Caregiver/Family Member Program
- Chapter Expansion
- Hotline
- Medical Provider Programs

CLINICAL ADVISORY COUNCIL

- Council Infrastructure
- Clinical Care Centers
- Patient Registry
- Clinical Trials Task Force

RESEARCH COUNCIL

- Council Membership
- Data Management
- Grant-Making Process

EXISTING PROGRAMS AND SERVICES – AS OF FALL 2012

EDUCATION

ANNUAL FAMILY MEETING

The VHL Alliance holds an annual Family Meeting geared to educating patients, families, and caregivers with the goal of empowering them to become active partners in clinical care. Each year the meeting is held in a different part of the country as a way to make it accessible to different geographic areas.

The agenda is structured to provide attendees with information about diagnosis and treatment of all involved body parts as well as advances in research including clinical. Experts in VHL clinical care, diagnosis, and research are chosen to speak. Panel discussions, which include patient representatives, help to accentuate the topics. In this way, the meeting is an opportunity for attendees to learn from some of the leading VHL researchers and clinicians. The meeting also provides a chance for participants to learn from each other – a very valuable and appreciated experience.

HANDBOOKS

The ***VHL Handbook: What You Need to Know About VHL*** is a reference handbook for people with von Hippel-Lindau, their families and caregivers as well as physicians and members of their health care teams. Designed to be read by patients it explains how VHL occurs, how to monitor and test for possible medical issues, and common treatment options to consider. The book is designed to foster constructive teamwork among the patient, the family, and the various health care professionals who will be involved in their care.

The handbook has been translated into Spanish, French, Greek, Dutch, Chinese, Japanese, Hindi, and Arabic. The handbook was last updated 2012 as a 4th edition.

A PDF file of the handbook is downloadable from the VHL Alliance's website:

www.vhl.org/handbook. It is available for purchase from the VHLA office, through the VHL.org website, and through Amazon.com. The handbook is also available in an e-format. In addition, the content of the handbook is incorporated into the VHL Alliance's website: www.vhl.org.

The ***VHL Handbook Kids' Edition*** was written by a team of parents and professionals. Illustrations were created by children with VHL. The book is upbeat and hopeful, helping children understand VHL and how to manage their health. The Kids' Edition is also available in Spanish.

The handbook, published in 2009, is available for purchase from the VHLA office, through the VHL.org website, and through Amazon.com. It is also available in an e-format.

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NEWSLETTER

A VHL Alliance newsletter is published quarterly. The newsletter is designed to educate and empower patients and others impacted by VHL. It is a critical piece of VHLA's educational and support efforts. Educational articles form the basis for future editions of the VHL Handbook.

Primarily written by VHLA staff, the newsletter provides an update on important clinically-related information including diagnosis and treatment. Updates on critical research findings related to the quest for a cure are incorporated into the newsletter, as appropriate. Patient stories are also a valuable part of the newsletters. Stories are chosen that emphasize critical facts related to living with VHL.

Also included in the newsletter is information about the VHL Alliance, including, but not limited to: information on Clinical Care Centers, announcements of VHLA activities and events, VHL awareness, and fundraising-related material. Fundraising information includes patient-driven efforts as well as a list of donations made during the prior quarter.

Constituents are provided access to the newsletter using multiple formats: mail printed copies, online pdf's housed on www.vhl.org, and an audio version for the visually impaired. In addition, articles are incorporated into the VHL Alliance's website, as appropriate. Concurrent to mailing of the newsletter, an e-Blast is sent to all constituents announcing the new newsletter. This helps decrease the number of newsletters printed cutting down on costs and helping the environment.

WEBSITE

The VHL Alliance's website, www.vhl.org, is the organization's primary source of education and updates. The website is designed to provide up-to-date information on VHL clinical care, diagnosis, and research. In addition, details about upcoming VHL or VHLA-related events are posted on the website.

In 2012, members of the Board of Directors re-evaluated the website the existing site which had been launched in 2008. A committee was created to evaluate content, usability, navigation, appearance, and ease of editing. Research included exploration of websites belonging to similar organizations, particularly those with multiple stakeholders, and an in-depth analysis of information on VHLA's current site.

A challenge to organization of VHLA's website is the multi-faceted use. It serves patients, families, the medical community (including those very familiar with VHL, the new diagnosed patients, and those in search of knowledge), researchers, and the general public. At the same time, it is a source of VHL education, patient/family support, sources for clinical care, VHLA updates, research updates, and VHLA fundraising. The diverse use and content creates a challenge in terms of presenting information in a readable fashion and in an organizational structure that eases navigation and minimizes overlap.

Six main units were identified from which information could easily flow (see Appendix A for Website Sitemap):

- Patients/Caregivers
- VHL Research
- Health Professionals
- About VHLA
- Ways to Help
- Contact Us

RESEARCH

MEDICAL SYMPOSIUM

The biennial International VHL Medical Symposia bring together the leaders in VHL basic, translational, and clinical research, as well as the leading clinicians in VHL clinical diagnosis and treatment. The conference creates a stimulating environment while helping to make connections among these professionals spurring the pace of progress in understanding and treating VHL – and toward finding a cure.

VHLA invites a leading institutions to sponsor the 2 ½-3 day Biennial Symposium. The sponsor is responsible for all logistics including creation of the meeting’s agenda and obtaining funding. Sponsors are encouraged to create a well-rounded agenda which includes a full-day of research updates, a full-day of clinical or treatment updates, and at least a half-day designed specifically for patients and other lay people.

It is also strongly recommended that a presentation by a patient be included in the agenda of the research/clinical-focused part of the meeting. It has been found that hearing from a patient has an inspiring and positive impact particularly for lab-based researchers. It takes the syndrome from something abstract to “reality”.

The Symposium travels from continent to continent allowing participation of researchers, clinicians, and patients from around the world.

RESEARCH GRANTS AND RESEARCH EFFORTS

Finding a cure for VHL is at the core of the VHL Alliance’s vision. Towards this end, VHLA has awarded more than \$1.3 million in research money since 1998. Grants are awarded through a competitive research grant application process supervised by the organization’s Research Council. (Awardess are listed in See Appendix B.)

Research supported by the VHL Alliance has led to a better understanding of the underlying mechanism responsible for VHL tumor development, improved diagnosis and treatment of VHL and an increase of more than 16 years to the average life expectancy of someone with VHL. In addition, VHL Alliance sponsored research, has contributed to the understanding that the VHL gene controls the major feeding pipeline of every tumor type including those found in breast, kidney, adrenal, lung, colon, pancreatic and liver cancers. To date, there have been 8 targeted

therapies approved by the FDA for different forms of cancer, including kidney cancer and breast cancer. Unfortunately, none of these agents found as a result of VHL research have been approved for VHL.

In January 2012, the Research Council strongly recommended that the VHL Alliance focus its research efforts on an International Patient Registry. While many of the major medical centers possess database of patient clinical data at their individual institutions, these data are clinician driven and not easily shared between institutions. The vision of VHLA's registry is that the data will be patient driven allowing for quality of life information to be obtained. In addition, the registry will be open to a larger population providing greater statistical analysis. Finally, VHLA will be the defined owner of the data easing collaboration between researchers.

An International Task Force was assembled with some of the leading experts in VHL to develop a comprehensive questionnaire to collect data that will answer a multitude of questions including:

- *Incidence, prevalence, and natural history of VHL lesions*—allowing us to better characterize the disease and to potentially uncover undocumented clinical consequences of VHL mutations.
- *Studies of clinical and environmental cofactors that may influence the natural history of VHL*—providing us with a better understanding of how occupational history, smoking history, concomitant medical history, dietary factors and co-morbidities influence VHL lesions as well as an understanding on why and when pregnancy changes the outcome of VHL related lesions.
- *Studies to predict the development of specific lesion patterns within families*—allowing us to better understand why within families with VHL there are differences in the types of lesions seen within even siblings with the disease.

Experts in HLRCC, and BHD have also been included with the decision to expand the registry to include these syndromes (see Collaborations, page 24).

SOURCE OF CLINICAL INFORMATION

OFFICE-BASED ADVOCACY AND SUPPORT

The VHL Alliance office is the immediate support and advocate. Thanks to Joyce Graff, who has lived with VHL in her family for 40 years, the office has developed closed relationships with leaders in the field of clinical care (diagnosis and treatment) and research. With immediate access to these experts, many of whom sit on VHLA's Committees, Councils, and Task Forces, the office is a central source of information.

The newly created position of Director of Wellness was created to maximize and expand this resource. The responsibilities of the Director of Wellness include:

- Responding to constituent calls, emails, and social networking messages questions and concerns, as appropriate
- Appropriately, guiding constituents to suitable medical professionals, Chapter Leaders or other volunteers or professionals
- Directing medical questions to Clinical Advisory Council, as needed

Office, or volunteer driven guidance, is geared to empowering and supporting the constituent. Key to this effort is implementing communication practices that consider issues of liability.

VHL CLINICAL CARE CENTERS

Because VHL is a rare syndrome, few clinicians have ever seen, diagnosed, or treated a patient with VHL. The consequences of this make it difficult for patients to receive knowledgeable clinical care. The VHL Alliance has worked to create Clinical Care Centers, or hubs of VHL care, throughout the U.S. and worldwide. The goals of the program are:

- To improve diagnosis and treatment of VHL
- To provide coordination of care across medical specialties
- To provide resource centers for patients and physicians who are new to VHL
- To provide a ready channel for communicating advances to these centers of expertise
- To provide a model that can be replicated elsewhere

In order to be a participant in the Clinical Care Center program, cooperating institutions agree that they will designate a single point-of-entry into the institution. This person will know how to assist and direct patients through the institution's system so that they can be seen in a timely fashion. The institutions take responsibility to help find all needed specialists and check all the appropriate areas of the body which require proper screening. The center ensures communication among the specialists involved in a patient's care and, wherever possible, they do their best to coordinate appointments.

In addition to the specified point of contact, each center must have an identified sponsoring clinician along with identified clinicians in at least the following specialties:

- Clinical Genetics
- Ophthalmology
- Neurosurgery
- Urology
- Endocrinology

The Clinical Care Centers also serve as a source of second-opinions or referrals from Health Maintenance Organizations and physicians less familiar with VHL.

Standards of care, including guidelines for annual patient screening, have been developed by VHLA's Clinical Advisory Council in cooperation with the participating institutions.

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SUPPORT

HOTLINE

The VHL Alliance's Hotline is often the first point of contact to the VHLA. Frequently callers will have been recently diagnosed with VHL, a syndrome of which they had never heard. Callers may be curious about VHL along with being frightened by the diagnosis and what the future may hold. The role of the Hotline is to be a friendly listening ear as while providing support and education to callers.

The Hotline is staffed by volunteers who, through their own personal experience, have learned much about VHL and about living with VHL. At the same time, volunteers are encouraged to use the VHLA office as an additional resource.

The VHLA office supports the Hotline volunteers in multiple ways including a telephone conference held every other month. The VHLA office is always available as a listening ear, particularly when a volunteer is faced with an emotionally draining conversation.

LOCAL CHAPTERS

VHLA Chapters have been created to provide support at a more local level. Originally, Chapters were defined by State boundaries because of the differences between health insurance requirements and benefits provided at the state level. A recent shift to geographic regions is being encouraged in order to make face-to-face events more accessible.

Chapter Leaders are encouraged to:

- Welcome new constituents in their region
- Proactively reach out to constituents including patients, family, friends, and donors
- Support regional constituents, as needed
- Answer questions and provide support, including informing constituents about the closest Clinical Care Center
- Hold regional get-togethers, social gathering or educational programs
- Initiate regional fundraising efforts
- Raise regional awareness through local news outlets and the medical community
- Represent VHLA at CFC (Combined Federal Campaign, workplace charity campaign for federal civilian, postal and military donors) events in their area
- Provide the VHLA office with updated contact information and constituent status

The VHLA office supports the Chapter Leaders in multiple ways including telephone conference held every other month. The VHLA office maintains a database of US Chapters and International Affiliates which have submitted contact information to VHLA.

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ONLINE SUPPORT NETWORKS

The Internet provides an extraordinary opportunity to people to communicate across geographic boundaries. The VHL Alliance has created online venues to access this international venue for support.

VHL Alliance is involved with a number of social media outlets for online communications by which to get updates and support:



www.facebook.com/VHLA (Facebook Fan page)



www.facebook.com/groups/VHLAwareness (Facebook discussion page)



A safe, completely secure Facebook group for teens dealing with VHL. Permission to join, including approval from parents, required through the VHLA office.



www.inspire.com



www.rareconnect.org/en/community/von-hippel-lindau



Group: VHL Alliance



<https://twitter.com/VHLA>

OTHER

COLLABORATION

The VHL Alliance strongly believes in collaboration. Such collaborations should never be at the sacrifice of the work, efforts, or expense of VHLA and should be mutually beneficial. The collaborative efforts with the International VHL Affiliates exemplify the nature of working together that VHLA seeks.

HLRCC (Hereditary leiomyomatosis and renal cell cancer) and BHD (Birt-Hogg-Dubé) are two genetic syndromes each which can result in kidney cancer. The VHL Alliance considers collaboration with HLRCC and BHD highly valuable, particularly because of overlapping research. Understanding the commonalities as well as the differences will shed light on ways to improve quality of life, developing effective treatments, and, ultimately, finding a cure.

The VHL Alliance is involved in collaboration with a number of different organizations. Examples of existing collaborations include, but are not limited to:

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- Angiogenesis Foundation
- Cancer Care
- Coalition for Urologic Research and Education
- Family Voices
- Genetic Alliance
- HLRCC Family Alliance
- National Organization of Rare Disease (NORD)
- National Coalition for Cancer Survivorship
- SPORE at Beth Israel Hospital, Kidney Cancer Research at Dana Farber Cancer Institute

An evaluation to determine cost/benefit (where cost include staff time) would be of benefit.

FUNDRAISING

Over 95% of the funds supporting the VHL Alliance are through individual donations. Appeals include self-address envelopes in the quarterly newsletter and an Annual Appeal, at the end of the calendar year, is implemented through the Annual Report and in eBlast format. A Donate Now button is included on VHLA's website for online giving (Network for Good, approximately 3% overhead).

Chapter Leaders and are asked to initiate fundraisers within their State. VHL constituents are encouraged to engage in individual fundraising efforts such as those suggested within on the website. One popular approach is a personal fundraising page using FirstGiving.com.

The VHL Alliance is a member of the Federated CancerCure of America and Independent Charities of America, providing access to the Combined Federatal Campaign (CFC), state and corporate workplace campaigns, and state charitable registrations nationwide. Fundraising through these efforts utilize the DBA of Alliance for Cancer Research and Support –VHL.

Charitable state registration services are an exclusive optional service for which VHLA pays a flat fee of about \$6,000. Federated CancerCure of America recoups its costs and deducts its fees for CFC and work place registration through the distribution of funds to VHLA. Participation in the CFC is currently at no cost to member charities and state and corporate campaigns currently cost 7.5% of donated funds from these campaigns. Federation membership is renewed annually, each fall, for the subsequent year's fund drive. All services are exclusive to membership in Independent charities of America and Cancer Cure.

Additional fundraising efforts include donations through online shopping (igive.com); recycling of ink cartridges, some computer equipment, and mobile phones (Cartridges for Kids); donations of used car or other vehicles (CancerCure of America). The VHL Store on the VHLA website sells a number of items that help raise VHL awareness or have been created by constituents of VHLA. Proceeds from these sales minimally contribute to the VHLA income (less than 1%).

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The VHLA's office has the capacity of accepting donations of cash, checks, credit charges (processed through Authorize.net, approximately 3% overhead), and stock.

INTERNATIONAL AFFILIATES

The VHL Alliance is also the home for the International VHL Alliance an international network of VHL Affiliates. Email communications and biennial meetings are used as a means of communications and sharing of information and materials. Through this network of Affiliates, VHLA is connected to approximately 90% of all diagnosed VHL patients, worldwide.

International Affiliates are considered as independent organizations and shall work in accordance with applicable local laws. However, they shall be asked to conduct activities in an ethical manner as mandated in the VHL Alliance Volunteer Manual. Donations received from supporters do not cross national boundaries and will be used within the country in which they were collected. International donations may also be made directly to VHLA to support the home office. These donations will be pooled with all other VHLA U.S. donations

VHL Alliances currently exist in the following countries:

- Belgium
- Brazil
- Canada
- China
- Croatia
- Denmark
- France
- Germany
- Great Britain
- Greece
- Hungary
- Ireland
- Italy
- Japan
- Lithuania
- Netherland
- Norway
- Poland
- Portugal
- Spain
- Sweden
- Switzerland
- Turkey

VON HIPPEL-LINDAU (VHL)

THE SYNDROME AND THE GENE

Von Hippel-Lindau (VHL) is a genetic disease affecting approximately 1 in 35,000 people² (approximately 10,000 in the United States and approximately 200,000 worldwide). The syndrome is caused by a dominant mutation with a deletion in the VHL gene located on the short arm of chromosome 3³. The VHL gene is the key regulator of cellular hypoxia (low oxygen) signaling via its product, the VHL protein (pVHL). pVHL is indirectly responsible for enhanced levels of growth factors including vascular endothelial factor, platelet derived growth factor, and transforming growth factor alpha. Increased levels of these growth factors result in growth of new blood vessels or angiogenesis⁴.

The role of the angiogenesis pathway has been shown to play a crucial role in tumor promotion in other cancers including breast, brain, colon, gastric, lung, skin, ovarian, prostate, renal, and pancreatic⁵. As such the VHL gene is the key to understanding how tumors grow and how potential therapies can inhibit angiogenesis.

CLINICAL PRESENTATION

The clinical presentation of VHL is diverse and includes various cysts and tumors, including renal cysts, clear cell carcinoma, pancreatic cysts and cystadenomas, pancreatic neuroendocrine tumors, pheochromocytomas, and retinal and central nervous system hemangioblastomas, and endolymphatic sac tumors⁶. Tumors in the central nervous system can develop in the brain, the retina of the eyes, and cerebellum, brain stem, and spinal cord.

While manifestation of clinical symptoms of VHL is generally in late childhood/early adulthood, clinical presentation can occur at any point in a patient's life, including early childhood. Retinal involvement occurs in 60% of people with VHL, and has been seen in children as young as 3 years of age. These lesions can cause blindness.

² Lonser RR, Glenn GM, Walther M et al. von Hippel-Lindau disease. *Lancet* 2003; 361: 2059-2067.

³ Latif F, Tory K, Gnarra J et al. Identification of the von Hippel-Lindau disease tumor-suppressor gene. *Science* 1993; 260: 1317-1320.

⁴ Maher ER, Neuman NHPH, Richards S. von Hippel-Lindau disease: A clinical and scientific review. *Eur J Hum Genet* 2011; 1-7.

⁵ Guan Y, Ramasamy R, et al. G-rich oligonucleotides inhibit HIF-1 α and HIF-2 α and block tumor growth. *Nature* 2009; 18(1): 188-197.

⁶ Jonasch E, McCutcheon IE, et al. Pilot trial of sunitinib therapy in patients with van Hippel-Lindau disease. *Ann Oncol* 2011; 22: 6261-2666.

VHL patients are also at a higher risk for certain types of cancer, especially **kidney cancer**; 25% of VHL patients are diagnosed with clear cell renal cell carcinoma (ccRCCs)⁷.

CHALLENGES OF LIVING WITH VHL

VHL is a complicated disease with the potential to affect 8 different body parts. Tumors in the kidney or pancreas can lead to metastatic cancer; hemangioblastomas in the retina can result in blindness; and adrenal tumors (pheochromocytomas) cause elevated catecholamines and metanephrines presenting as cardiac disease or broad mood fluctuations. The pressure caused by brain or spinal cord hemangioblastomas can lead to extreme and constant pain and, even, paralysis. Endolymphatic Sac Tumors can result in deafness and cysts in the reproductive tract can be the source of infertility.

Early detection through annual screening is strongly recommended in order to determine when surgery to remove a tumor is necessary as a means of avoiding further life threatening conditions such as RCC. The questions that arise are: What are the best methods of tumor detection in any given body part? What is the best treatment procedure for any given tumors? How can kidney function be maintained or blindness avoided? What hormones are needed when the pancreas or the adrenal glands no longer function?

In addition to the physical implications of VHL, there is the emotional impact on the patient as well as their loved ones. How does one live with the fear of the unknown? When will the next tumor develop? Where will it be and how will it impact one's quality of life? How does one speak to one's children about VHL - children with a 50% chance of inheriting the disease?

⁷ Maddock IR, Moran A, Maher ER, et al. A genetic register for von Hippel-Lindau disease. J med Genet 1996; 33: 120-127.

Strategic Plan



APPENDIX A

SITE MAP FOR WWW.VHL.ORG

- Home
- Patients/Caregivers
 - Annual Meetings
 - Basic Facts about VHL
 - VHL is Not So Rare
 - Commonly Occurring VHL Manifestations
 - VHL and Cancer
 - How Do People Get VHL?
 - Diagnosis
 - The History of VHL
 - Progress Toward a Cure
 - Investigating Metabolism for Cancer Therapy with Dr. Marston Linehan
 - Clinical Trials Currently in Progress
 - Genetic Research and VHL
 - Living with VHL
 - The Emotional Side of VHL
 - Caring for Your Health
 - Diagnosis and Treatment (Screening and Testing Protocols; Suggested Screening Guidelines; Side Effect of MRI Contrast Agents)
 - Preventing Complications After Surgery
 - Questions to Ask the Doctor about VHL
 - Clinical Care Centers
 - Talking with Children about VHL
 - Help Uncover a Cure (Current Clinical Trials, VHL Tissue Bank, VHL Research)
 - Stories of Hope
 - Additional Resources
 - Getting Support
 - Toll-Free Hotline
 - Support Groups: United States
 - Support Groups, International
 - Inspire Online Discussion Group
 - Standards for our Online Communities
 - Get Involved
 - Calendar of Events and Meetings
 - Fundraise
 - Sign up for E-News
 - Send Us Your Ideas
 - Resources
 - VHL Handbooks
 - Newsletters
 - U.S. Chapters
 - International Affiliates
 - DNA Testing
 - Suggested Reading
 - Medical Terms

Strategic Plan



- VHL Research
 - VHL Research: Leading to a Cure
 - Basic Science of VHL
 - VHLA Sponsored Research
 - VHLA Patient Registry Task Force
 - VHLA Clinical Trials Task Force
 - VHLA Competitive Grants Program
 - Research Grants Awarded
 - Meet the VHLA Research Council
 - Clinical Trials: The Hope for a Treatment
 - Clinical Trials Currently in Progress
 - Tools for Research / How to Support Research
 - International VHL Research Meetings
 - Tissue Bank
 - VHL Mutation Database
- Health Professionals
 - VHL Handbooks
 - VHL Research Updates
 - Medical Information about VHL
 - When to Suspect VHL
 - Screening Protocols
 - Basic Science of VHL
 - Clinical Trials in Progress
 - International VHL Medical Symposia
 - Meet the VHLA Clinical Advisory Council
 - Events
- About VHLA
 - VHLA Vision and Mission
 - VHLA Update
 - VHLA Organizational Structure
 - Staff
 - Board of Directors
 - Research Council
 - Clinical Advisory Council
 - Calendar of Events
 - Get Involved
 - Sign up for E-News
 - VHL Store
 - Press
 - VHL Patients in the News
 - VHL in the Media
 - YouTube Videos about VHL and VHLA
 - Privacy Policy
 - Disclaimer
 - 2012 Fiscal Year Report
 - Articles of Incorporation

Strategic Plan



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- Ways to Help
 - Donate Now
 - Ways to Give to the VHL Alliance
 - Fundraising Ideas
 - Why Support the VHL Alliance
 - Who Supports the VHL Alliance
 - Get Involved with the VHLA
 - Contact Us

Strategic Plan

APPENDIX B

GRANT RECIPIENTS

Eamonn R. Maher, MD, FRCP (2011) for Eleanor Rattenberry <i>University of Birmingham</i>	Haifeng Yang, PhD (2011) <i>Cleveland Clinic</i>
Brenda Petrella, PhD (2010) <i>Dartmouth Medical School</i>	Rachel Giles, PhD (2009) <i>University Medical Center Utrecht</i>
Thera P. Links, MD, PhD (2008, 2009) <i>University Medical Centre Groningen</i>	James Handa, MD (2008) <i>Johns Hopkins University School of Medicine</i>
Rupal Bhatt, MD, PhD (2008) <i>Beth Israel Deaconess Medical Center</i>	Armin Pause, PhD (2007) <i>McGill University</i>
Bin Teh, MD, PhD (2007) <i>Van Andel Institute</i>	Donald Bellgrau, PhD (2006,2007) <i>University of Colorado, Denver</i>
George V. Thomas, MD (2006) <i>Oregon Health and Science University's Knight Cancer Institute</i>	Robert Weiss, MD (2005) <i>University of California</i>
Susanne Schlisio, PhD (2005) <i>Ludwig Institute for Cancer</i>	Kimryn Rathmell, MD, PhD (2005) <i>University of North Carolina</i>
Shufen Chen, MD, PhD (2005) <i>University of North Carolina</i>	Ian Frew, PhD (2005) <i>University of Zurich</i>
Andrea Ruxandra-Schmitzer, PhD (2005) <i>University of Montreal</i>	Judith Frydman, PhD (2004, 2005) <i>Stanford University</i>
Michael Zimmer, PhD (2003) <i>Massachusetts General Hospital</i>	Daniel J. George, MD (2002, 2003) <i>Duke University Medical Center</i>
Tien Hsu, PhD (2002) <i>Boston University Medical Center</i>	Pierre Jacomet, MD <i>Catholic University Medical Center</i>
Maria Czyzyk-Krzeska, MD, PhD (2000, 2001, 2002) <i>University of Cincinnati</i>	Robert J. Duronio, PhD (2001, 2002) <i>University of North Carolina</i>
Ehud Gazit, PhD (2001) <i>Tel Aviv University</i>	Shahriar Koochekpour, MD, PhD (2000, 2001) <i>Louisiana State University Health Sciences Center</i>
James R. Gnarra, PhD (1998, 1999) <i>University of Pittsburgh Medical Center</i>	Robert D. Burk, MD (1998) <i>Albert Einstein College of Medicine of Yeshiva University</i>



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