



Dedicated to improving diagnosis, treatment, and quality of life for people with von Hippel-Lindau disease.

171 Clinton Road, Brookline, MA 02445-5815 USA – Tel: +1-617-277-5667 – Fax: +1-858-712-8712
<http://www.vhl.org> – info@vhl.org – 1-800-767-4VHL

Annual report, 2002-2003

This eleventh year of the VHL Family Alliance has been a year of reflection and realignment to take the next big step forward. Our volunteer services are the strength and heart of this organization. As we grow and serve an ever-increasing number of people in an ever-wider geography, we realize that the core team needed to support this expanded organization and keep it growing, is quite different than what was needed ten years ago. We have done a remarkable amount in ten years with all volunteer staffing. At this point, in order to grow, we know that we need to strengthen the home office: to expand our funding base, to obtain corporate and foundation grants, and to provide our many wonderful volunteers with the support they need to continue doing their good work.

We have therefore embarked on a three-year transition plan to strengthen our core services and administration, and expand our capacity to grow with strength, maintaining the excellence of our information and support for patients and physicians throughout the United States and worldwide.

Guiding Research

We identified the VHL gene in 1993. It's all well and good to say that you have the code in your hands, but can you read it? Not just the letters, but the words and the meaning of the sentence? Little by little scientists are unraveling the function of the VHL protein in the cell -- what it does, and what goes wrong when it's not there. It has taken the same patience, persistence, trial and error that all great scientific discovery takes, and the VHL Family Alliance has helped to encourage a number of outstanding young scholars to focus on the VHL protein.

Now it is time to take the next big step -- taking that basic understanding of what's happening in the cell and translating it into real therapies. Can we design a drug to intervene in the chain of events going on in the cell, correct the problem created by the absence of the VHL protein, and fix the outcome?

We decided to shift our research funding focus from basic science to "translational research" -- the conversion of this basic science knowledge into effective treatments as expeditiously as possible.

New Drugs and Clinical Trials{tc "New Drugs and Clinical Trials"}

Fortunately for us, the VHL protein plays a key role in cell function and interacts with at least 20 other proteins, making it essential to good outcomes in many conditions, not just VHL disease. This means that the pharmaceutical companies are working on a number of drugs that are potentially beneficial with VHL. Two key pathways we know of at this time are VEGF and PDGF. If there is too little VHL, there is too much of VEGF and PDGF. There are drugs to block VEGF production, or to block the receptors that would normally respond to signals from VEGF,

making the cell behave as if its VHL and VEGF function were normal. Some of these drugs have been well tested with other conditions and may be approved and on the market as early as 2004-05.

This means that we know a good bit about their safety and side-effects, and that they work with colorectal or prostate or kidney cancer. What we don't yet know is whether they are really effective with VHL tumors. And the only way we can find that out is to try them in a systematic way with at least 50 people with VHL. The VHL Family Alliance intends to play a key role in distributing information to our membership to assist them in identifying trials that may be helpful to them, and in making it easier for researchers to study VHL, find the "modifier genes" that may make VHL better or worse, and to find a cure.

By-Law Revisions

The By-Laws were revised for the first time since 1993, to achieve two primary purposes: the grouping of state chapters into Regions, and the redefinition of the role of Chairman of the Alliance to be an Executive Director as a staff position, reporting to the Board. A copy of the revised By-Laws is available on request, and a copy is available that shows all the edits made to the original document. See <http://www.vhl.org/newsletter/vhl2003/03cjbyla.htm>

Executive Director

At its meeting in Nashville in June 2003, the Board approved the engagement of Joyce Graff as its first Executive Director under the new By-Laws. This is part of a three-year transition plan under which Joyce works full-time for \$1 in 2003-04, \$25,000 in 2004-05, and if we meet our fund-raising goals, we grow to a competitive salary in 2005-06. Following her retirement from her "day job," Joyce has in fact been serving full-time as a volunteer since January 2003. Having full-time attention on the organization has already borne fruit, tightening procedures and keeping the Board more fully informed.

State Registrations

We engaged Copilevitz & Canter, attorneys in Kansas City, Kansas, as our agents in registering the VHL Family Alliance as a charitable organization in all 50 states. Since September 11, 2001, and the abuses of charities that emerged following that event, state regulations have become more stringent and demanding, each with its own set of deadlines and requirements, and our ability to keep up with them has been sorely strained. C&C handles charity registrations for many hundreds of charities like ours, and handles them for us in a professional manner for a modest fee. We are now properly registered in all 50 states, and C&C is handling the annual renewals as required.

Distributing Information

The Newsletter (paper and electronic) remains our key vehicle for delivering information. In addition to the paper readership, more than 40,000 people have visited our website. Over the years we had added a fifth issue, the Research Report or Annual Report issue, published as part of our annual donor campaign in November. We canvassed the membership in May 2003 to determine whether it might be time to transition to electronic delivery and whether we might drop one issue. There is still overwhelming preference for the print medium, but agreement that four issues were sufficient due to the costs. In Fiscal 2004 we will be transitioning back to four issues a year: March, August, October, and December. We will also do some experimentation with electronic delivery, while still maintaining full distribution of the print newsletter.

Spanish language services took a new leap this year with the addition of Spanish language hotline services provided by volunteers Alexandra and José Morais of New Jersey. We are still working to promote this service in the Spanish-speaking community. New clinical care centers in New York and Houston are both well prepared to serve people with VHL in Spanish language, and an increasing number of people from Central and South America are contacting us via the internet.

Conferences

Due to rising expenses of holding large annual 3-day meetings in a hotel, we decided to try this year holding regional meetings. Much time was spent reorganizing the state chapters into regional groupings, and a first Regional Meeting was held in Nashville in June 2003. This one-day format is less expensive and more attractive to local people. Dr. Gladys Glenn and Dr. Peter Choyke of the VHL Clinical Program at the U.S. National Cancer Institute met with 40 attendees to discuss health management in VHL. The program was very well received, and additional meetings are planned in Fiscal 2004 in Boston, Indianapolis, Los Angeles, Tampa, and New York.

Research

The Board approved commitment of another \$90,000 in research grants to three of the nine applicants for research funding received this year. We continued funding for Dr. Georges Mer for his 3-dimensional model of the VHL protein. We awarded a grant to Dr. Michael Zimmer to study HIF-1-alpha as a possible therapeutic target for controlling VHL tumor growth, and we renewed funding for Dr. Daniel George for his clinical trial of Novartis PTK-787 with VHL patients, studying in particular the impact of this drug on tumors of the central nervous system (brain, eye, and spinal cord). One patient has had 50% shrinkage of his brain tumors in three months, another has had significant shrinkage of kidney tumors, and even with the small number of enrolled patients the early results are very hopeful. We are working with this team to identify additional candidates for this study. We cannot get meaningful data that would lead to FDC approval of this drug for VHL without at least 15 patients, preferably 50.

By providing seed money grants to young researchers, we are able to help them assemble the data they need to validate the concept of their line of research, and to help them obtain even larger grants from major funders. Dr. Tien Hsu of South Carolina and Dr. William Rigby of New Hampshire both won major grants from NIH.

Clinical Care

Two new clinical care centers were added, M.D. Anderson Cancer Center in Houston and Columbia-Presbyterian Hospital in New York. We are working to strengthen this program and encourage a more even level of care among the centers. We also have the challenge of handling situations where the level of care at a participating institution is seen to have dropped below the level of compliance with our program. We have taken two centers off the list this year, and are developing a policy for dealing with this situation as constructively as possible. We are not paying them for their participation, but our primary goal is to provide a helpful service to members, and we cannot direct new patients to a situation where the communications path is broken or they will not be treated appropriately.

Public Relations

Andy Lax had to resign from the board due to pressures of the economy on his business. We do not yet have a good replacement for him. We have issued some press releases, with little results. Clearly we need professional assistance here. Maria Shipton and Joyce Graff are evaluating options, including a pay-for-success PR firm in California. Several members have had articles published in their local newspapers, and local efforts at PR and fundraising continue to be the most fruitful of our PR endeavors.

Membership

The spring membership drive with election of officers increased our paid membership another 40% this year. With more paying members, we can more easily meet our funding goals to support on-going programs, and to fund additional research.

Communications Committee

VHLFA provides online discussion groups in four languages on the internet, based at Yahoo Groups. The English group has grown 30% this year to 318 members. This group has been an excellent source of support for those going through new procedures, as well as those newly diagnosed. The teen group joined in with the adult group, as they found they got more response from others there. The German group has 34 members, 32 in the French group, and the Spanish group has doubled in size to 60 members. The membership of all these groups spans country boundaries and brings together by language group people with common concerns on multiple continents. The Spanish group, for example, includes people in the United States, Mexico, Spain, and ten countries of Central and South America.

Medical Advisory Board

Our Medical Advisory Board continues to provide the firm foundation on which we stand, ensuring that the medical information we share is correct, and guiding us in serving the medical community. We are planning in the coming year to try out some additional innovative ways of delivering training to health care professionals so that it is more convenient and more available when needed.

Finance Committee

As we plan for the next decade, the Finance Committee spent 2003 tightening up financial procedures and establishing a framework for achieving our funding goals in the years ahead. The financial support of our membership is the key funding base for our programs. As we move forward we will reach out and encourage all to be paying members.

We expanded our participation in the Combined Federal Campaign, joining the Health & Medical Research Charities of America, a federation which will bring greater visibility to our cause in the coming campaign. A growing percentage of our income comes through CFC and United Way (write-in) donations.

There were many successful fundraising events this past year, and the time and effort of all those involved is greatly appreciated. Two walk-a-thons are in the planning stages, in New Jersey and California.

We are working to identify Corporate partners and Foundations to assist us in our funding goals. We welcome any input from the membership in helping us grow and succeed in this area of fundraising.

Staffing

Our staff now consists of an Executive Director, one faithful volunteer in the office, Rachael Morgenstern, who has served us 10-20 hours a week since 1993, and a large number of wonderful volunteers throughout the United States and the world. Each of our Board members and committee chairpersons contributes some time to the management of the organization. Altheada Johnson manages info@vhl.org and the hotline staff which are our primary lines of contact with the membership and the world. She is assisted by Robin Kadlecek (Texas), Audrey Clifton (North Carolina), and Lois Erickson (Minnesota).

Complementing this core staff are Copilevitz & Canter (state registrations) and Health & Medical Research Charities (CFC applications).

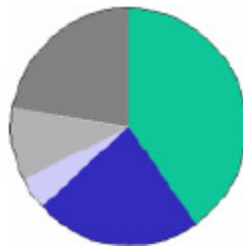
We continue to work in “virtual” space – we pay no office space but leverage telecommunications technology to collaborate via telephone and internet. As we continue to grow, however, we do see that this will change at some point. We will make every effort to grow carefully and with strength, ensuring that our expenditures remain a very modest percentage of our income, and remaining true to our charter to increase diagnosis, treatment, and quality of life for individuals and families affected by VHL.

Financial Report

INCOME:	
Contributions and membership fees	\$202,149
Income from sales and conference registrations	\$1,275
Interest and dividends	\$920
Grants received	30,000
TOTAL INCOME:	\$234,344
Research	\$90,000
Education and Support	\$50,312
Fundraising	9,955
Administration	\$22,672
TOTAL EXPENSES:	\$172,939
Excess (or deficit) for the year	\$61,405
Net assets beginning of year	-11489
Net assets end of year	\$49,916

:

Total Revenue for Fiscal 2003 (ended June 30, 2003) was \$234,344. Of this, \$90,000 was allocated to research funding.



41% Research
 23% Education & support
 4% Fundraising
 10% Administration
 22% Reserve

Special projects in need of funding:

- Update and redistribution of the handbook
- Outreach to the medically underserved
- Funding for more local meetings for members
- Smaller and more accessible Continuing Medical Education training events for physicians
- Support the Clinical Care Centers with CCC Bulletins
- Research biobank repository to facilitate research on modifier genes and therapies

Research Grants Awarded:

Dr. Daniel George, Dana Farber Cancer Research Institute, Boston, Massachusetts, and Duke University, Durham, North Carolina, for research on the VHL project “Clinical trial for patients with VHL using a novel inhibitor of VEGF and PDGF.” \$30,000

Dr. Georges Mer, Mayo Clinic Foundation, Rochester, Minnesota, for the VHL project “Probing the molecular mechanism of hypoxia-inducible factor-1 α recognition by the von Hippel-Lindau tumor suppressor protein.” \$30,000

Dr. Michael Zimmer, Massachusetts General Hospital, for the VHL project “Hypoxia-inducible factor (HIF) as a molecular therapeutic target”. \$30,000

Board of Directors:

Maria Shipton
Joyce Wilcox Graff
Kathleen Braden
Altheada Johnson, MS, RD
Mattison C. Painter
Myriam Gorospe, Ph.D.

Thomas Lusk
Andrew Lax
Peggy J. Marshall
Patricia Rasmussen
Thomas D. Rodenberg, Esq.
Sheila Tepper

Medical Advisory Board:

Lloyd M. Aiello, M.D., *Beetham Eye Inst
Joslin Diabetes Center, Boston, Mass.*

Debra L. Collins, M.S., *Division of Genetics
Univ. of Kansas Med Center, Kansas City, KS*

Yujen Edward Hsia, M.D.,
Prof. Genetics, Retired, Honolulu, HI

Col. James M. Lamiell, M.D., *Clinical Invest. Reg
U.A. AMEDD Cntr & School, Fort Sam Houston, TX*

John A. Libertino, M.D.,
*Chief, Institute of Urology; CEO, Lahey Clinic Medical Center,
Burlington, MA*

Eamonn R. Maher, M.D.
*University of Birmingham,
Edgbaston, Birmingham, England*

Virginia V. Michels, M.D.,
*Chair, Medical Genetics,
Mayo Clinic, Rochester, Minnesota*

Haring J. W. Nauta, M.D., *Chief of Neurosurgery
Univ. Texas Medical Branch, Galveston, Texas*

Hartmut P. H. Neumann, M.D., *Nephrology
Albert-Ludwigs University, Freiburg, Germany*

Andrew C. Novick, M.D., *Chair, Urology
The Cleveland Clinic, Cleveland, Ohio*

Edward H. Oldfield, M.D., *Chief, Surgical Neurology
Nat'l Inst of Neurological Disorders, Bethesda, Md*

Stéphane Richard, M.D., *Neuro-Oncologie
Hôpital Kremlin-Bicêtre, Paris, France*

R. Neil Schimke, M.D., *Dir. of Genetics
University of Kansas Med Center,
Kansas City, Kansas*

Robert B. Welch, M.D., *Chair Emer,
Ophthalmology, Greater Baltimore Medical Center, Baltimore,
Maryland*

International Affiliates:

AUSTRALIA:

Jennifer Kingston
#35 The Esplanade
Pallarenda
Townsville, QLD
4810 Australia

BELGIUM:

Ms. Chris Hendrickx
Dorpstraat 127A
Emblem
2520 Belgium

BRAZIL:

J. C. Casali da Rocha, Oncology
Ludwig Inst for Cancer Research
Rua Prof. A. Prudente 109-4 andar
São Paulo - SP
01509-000 Brazil

CANADA:

Jill Shields
4227 Hamilton Rd
Dorchester ON
N0L 1G3 Canada

CHILE:

Pierre Jacomet Ollivet
95 Los Cormoranes
Renaca
Vina del Mar Chile

DENMARK:

Richard & Vibeke Harbud
Fiskervejen 10
Veddelev
Roskilde, 4000 Denmark

ENGLAND, U.K.:

Mary Weetman
2 Cherry Grove
Rochdale
OL11 5YT England, U.K

FRANCE:

Guy Allegre, Genet-Oncologie
EPHE FacMed Paris-Sud
63 rue Gabriel PERI
LE KREMLIN-BICETRE
94276 France

GERMANY:

Gerhard Alsmeier
Kleehof 11
Meppen 49716 Germany

HUNGARY:

Dr. Helga Süli-Vargha
H-1518, P.O. Box 32,
Budapest
112 Hungary

IRELAND:
Ms. Gloria Proby
Riverfield Farmhouse
Inch Gorey
County Wexford, Ireland

ITALY:
M. Luisa Guerra
Loc. Malvicina, 19
Gavi (AL),
15066 Italy

JAPAN:
Dr. Taro Shuin, Urology
Kochi Medical School
Kohasu Okoh-cho
Nan-koku Kochi, 783-8505 Japan

THE NETHERLANDS:
Joke Jansen-Schillhorn van Veen
Schoolstraat 33,
Zevenhoven
NL-2435 AA The Netherlands

NEW ZEALAND:
Jon & Valerie Johnson
3B Luana Way
Howick, Auckland New Zealand

POLAND:
Dr. Karol Krzystolik, Dept Genetics
Pomeranian Academy of Medicine
ul. Powstancow Wlkp. 72
Szczecin
70-111 Poland

SPAIN:
Dr. Karina Villar
Plaza Platanos N°1 Galeria 2 Puerta 8
08207 Sabadell
Barcelona Spain

171 Clinton Road, Brookline, MA 02445-5815 USA – Tel: +1-617-277-5667 – Fax: +1-858-712-8712
<http://www.vhl.org> – info@vhl.org – 1-800-767-4VHL