



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

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Annual report, 2003-2004

2004 was a year of strengthening the VHL Family Alliance, increasing our capacity for growth, and broadening our reach. We now serve people across a greater expanse of the world, and are communicating and learning with a larger community of patients and physicians world-wide. In order to be able to reach out, we have taken the time to create a stronger base. We are pleased with the progress we have made, and look forward to building an ever stronger future.

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. The following new Medical Advisors joined us in fiscal 2004:

- Dr. Richard Lewis, geneticist and ophthalmologist, from Baylor College of Medicine, Houston
- Dr. Joseph Locala, Medical Psychiatrist from the Cleveland Clinic Foundation, to assist with issues of depression
- Dr. Armand Rodriguez, an Internist from Florida, providing the perspective of the family physician

Distributing Information

The Newsletter (paper and electronic) remains our key vehicle for delivering information. In addition to the paper readership, more than 16,000 people per month visited our website, coming from about 90 countries, reading an average of 4588 pages per day. With the help of a network of volunteer translators, information is available on the web in English, Spanish, German, French, Japanese, Danish, Italian, Flemish, and Dutch. In addition to pointers on www.vhl.org, an index of European country support groups is maintained by our German affiliate at www.vhl-europa.org

With the continuing help of Dr. Myriam Gorospe as translator, we enhanced our package of information about VHL in Spanish language. We are now well prepared to respond to a request for information in Spanish, and the number of requests for Spanish has increased, though modestly. Six Spanish language packets were sent out in Fiscal 2004. With sufficient funding, we are hoping to expand services to speakers of Spanish in FY05.

Online Meetings

VHLFA provides online discussion groups in four languages on the internet, based at Yahoo Groups. These internet-based conversations provide support 24 hours a day, worldwide, in communities based on common language. The English language group has grown another 14% this year to 361 members. This group has been an excellent source of support for those going through new procedures, as well as those newly diagnosed. The German group has 46 members; there are 34 in the French group, and 61 in the Spanish group. 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.

Meetings

In an effort to make it possible for more people to attend face-to-face meetings, we created a series of Regional Meetings, in a one-day format (10-4), allowing people to commute to the meeting. A modest donation is requested to cover the cost of lunch and facilities. Local doctors provide most of the "talent", with the occasional addition of an expert from outside the area, and a representative of the VHLFA Board.

Meetings were held in FY04 in

- Indianapolis, Indiana, 25 October 2003
- Boston, Massachusetts, 1 November 2003
- Tampa, Florida, 31 January 2004
- Los Angeles, California, 21 February 2004
- Bellevue, Washington, 20 March 2004
- Charlotte, North Carolina, 17 April 2004
- Dallas, Texas, 24 April 2004
- Elmhurst, Queens, New York, 19 June 2004

The Annual Meeting was held in Queens, New York, near LaGuardia Airport. Chairman Altheada Johnson assembled an excellent agenda, the speakers were wonderful, and the attendees felt very well taken care of.

Medical Education

Our Biennial Medical Symposium was held in May 2004 in Kochi, Japan, hosted by Kochi Medical School and chaired by Dr. Taro Shuin, bringing together clinicians, researchers, and families from many countries of the world. Dr. Shuin has headed a large research project on kidney cancer and VHL in Kochi for many years. He and his colleagues have always attended the worldwide meetings. We were delighted to accept their invitation to Japan in 2004. Many physicians from all over Japan were in attendance, as well as about 40 VHL patients and family members.

The program included speakers from China and Korea. Joyce Graff traveled on to China to speak in Guangzhou as the guest of the First Military Medical University there. She was also able to meet in Beijing with the Urology Department of the First Hospital of Peking University.

The first Canadian Patient/Provider meeting was held in Whistler, British Columbia, June 24-27. Co-Chairs Dr. Steven Pautler and Jill Shields created a rich experience, complete with Continuing Medical Education Credits for physicians, several of whom were also in Whistler for the Canadian national Urology meeting.

Improving Clinical Care

Two new clinical care centers were added: University of Pennsylvania Medical Center in Philadelphia, and Vanderbilt University Medical Center in Nashville, Tennessee. We are working to strengthen this program and encourage a more even level of care among the centers. Chairman Patricia Rasmussen continues to work with the Centers to strengthen their focus on internal communications and coordinating care for each patient. This year there has been increased communication and collaboration among the sites.

Guiding Research

Increased focus was placed this year on monitoring the progress of research and drug development for the primary issues of VHL. A research committee of the Board met with Research Director Joe Verdi April 7 to debrief from the Renal Cancer Symposium and understand where we might put our money to best move the research forward. The quandary we face is the one outlined in an article in the March issue of *Fortune* magazine: in order to maximize the leverage of the relatively small pot of money at our disposal, we decided to focus mostly on facilitating the development of consortia and to organize information about our membership in more useful ways. By making information and tissue available for research, and making available some development grants, we hope attract more young researchers into VHL research.

We have been shifting the focus of our research funding efforts to projects that have a higher chance of "translating" into real patient therapies in the next several years. As part of that effort we have been developing a strong working relationship with the Renal SPORE, an NIH-funded focal effort on translational research for kidney cancer, based at the Dana Farber/Harvard Cancer Center in Boston. Joyce Graff serves as a consumer advocate on its governance board. We are in conversation with them about moving our tissue bank to their facility, so that we can store more information than our current bank is prepared to store, and gather more raw material to facilitate research on VHL.

We are also working with several scientists on developing some projects to use the newly-available DNA chip technology for genetic profiling (e.g. Affymetrix chip), to find connections between mutations and outcomes -- not simply mutations in the VHL gene, but accompanying factors in the genome that might influence the organs affected or the severity of the disease. By identifying which other genes are turned on or off, we might be able to identify some additional targets for therapy.

Research Grants

The Board approved the recommendation of the Research Advisory Board to fund one grant for a two-year commitment to Dr. Judith Frydman of Stanford University for her study "Cellular Pathways leading to degradation of tumor-causing variants of VHL." As part of the body's self-repair mechanism, there are checks that ensure that each step in the process of cell division and replication is going right. If not, a process of "degradation" gets rid of the incorrect cells so that the process will succeed. Seen from this perspective, a tumor is caused by a failure of this quality-control process. Dr. Frydman theorizes that instead of using a drug to work specifically on the VHL gene, we might use a drug to bolster this quality-control process and let the body take care of the problem normally -- eliminating the cells that lose that second copy of the VHL-gene.

We have put the remainder of our FY02 grant to Dr. Daniel George on hold, pending approval of the proposed clinical trial by his new Internal Review Board at Duke and the Novartis pharmaceutical company, and pending demonstration of patient accrual. Issues have arisen with the design of the trial. He is working to reduce the amount of time patients must spend in the center. The drug being studied, PTK-787, inhibits a number of protein kinases, notably PDGF a and b, EGF-R, and FGF-1 and 2. The patients who have had the best long-term responses are those who began the trial with relatively low VEGF levels in the blood. During the study, the levels of VEGF actually rise, which is potentially problematic. He is therefore considering combining PTK with a VEGF inhibitor like Avastin or Rapamycin to reduce the amount of circulating VEGF.

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.

Clinical Trials for VHL

There have been several clinical trials this year, but most are still focused on metastatic kidney cancer, which we try hard to avoid. We are learning from these early trials, and the researchers are learning about trial design factors that are important to our population. They are accustomed to designing trials for people with metastatic disease and serious imminent threat to life. For our group, however, we are looking for a health maintenance drug, to keep people working and earning a living. Our group is worried about time away from the job, what the costs will do toward maxing out the insurance coverage -- issues that are not usually of great importance to most people with metastatic disease.

Administrative Configuration and Control

We spent a great deal of time this year consolidating operations, documenting our Procedures, and identifying areas where we could gain greater efficiencies by reorganizing the staffing or flow of the work.

Building Annuity Income

The Finance Committee spent 2004 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.

In addition, we need effective ways to reach beyond our core constituents and develop a base of income from others. This year 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. As a member of a Federation, we are eligible this year for the first time to participate in the state-level CFC campaigns. We expect the Fall 2004 campaign to go well, and are hoping to at least triple our income through the CFC in the coming season. Monies pledged in fall 2004 will be received by the end of calendar 2005. 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. Most recently, a family new to VHL sent letters to their friends and relations, asking them to help, and raised \$3600 for VHLFA.

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, Joyce Graff; one faithful volunteer in the office, Rachael Morgenstern, who has served us 10-20 hours a week since 1993; a new volunteer Project Assistant, Robin Cochrane, three days a week; a part-time bookkeeper, 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 has been assisted this year by Robin Kadlec (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 use donated office space in Brookline, and leverage telecommunications technology to collaborate via telephone and internet. As we continue to grow, however, we anticipate that we will grow out of our existing space within the next 2-3 years. 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 improve diagnosis, treatment, and quality of life for individuals and families affected by VHL.

Financial record-keeping is now done by staff in Brookline, with records shared with our Treasurer in Michigan through a service called QuickBooks Online, a service of Intuit on the internet, that allows people in multiple locations to log in and look at the same set of books simultaneously. Intuit donated this service to us for one year. We are very satisfied with the service and are continuing with it.

Volunteer Robin Cochrane has taken over the sending of packets for new members as well as a number of other office projects to assist the Executive Director as necessary. We are recruiting for a volunteer to assist with meeting planning.

Public Awareness

We engaged a pay-for-success public relations firm, Going Global Communications, to assist us with our May 2004 VHL Awareness Month campaign. We prepared press kits, held two teleconference training sessions for chapters and other volunteers nation-wide, and sent out press releases nationally. Local volunteers added their own touches to the national effort. Going Global Communications coached them and sent out some local press releases to reinforce their efforts. 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.

Research Grants Awarded:

Dr. Judith Frydman, Stanford University, "Cellular Pathways leading to degradation of tumor-causing variants of VHL." \$80,000 over two years.

Research Grants Suspended:

Dr. Daniel George, Duke University, \$20,000 is on hold until satisfactory progress is shown. This is second-year funding originally awarded in FY02, and is carried in the Balance Sheet as Accounts Payable.

In-Kind Donations

In addition to the support of so many wonderful donors and volunteers, we have been gifted with valuable in-kind donations from the following vendors:

	<u>Value</u>
Allegiance Telecom, web hosting service July thru December 2003	\$750
MailWise Spam and Virus Protection Services for all vhl.org accounts	\$1500
Dr. Backup Offsite Backup Service for Brookline office	\$1500
Intuit Quickbooks Online Service	\$270

Special projects in need of funding:

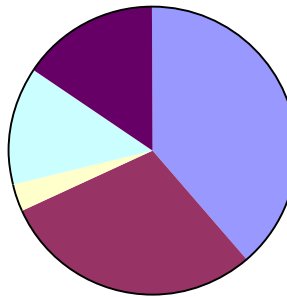
These projects could bear the name of the donor:

- Update and redistribution of the handbook (coming out early 2005)
- Outreach to the medically underserved
- Funding for more local meetings for members (\$1000 each)
- Smaller and more accessible Continuing Medical Education training events for physicians (\$1000 each)
- Support the Clinical Care Centers with CCC Bulletins (\$2000 per year)
- Research biobank repository to facilitate research on modifier genes and therapies (25,000 per year)

Financial Report

INCOME:	
Contributions and membership fees	\$190,684
Donated Services	\$4,020
Income from sales and conference registrations	\$6,292
Interest and dividends	\$468
Grants received	5,000
TOTAL INCOME:	\$206,464
Research	\$80,000
Education and Support	\$61,385
Fundraising	\$6,151
Management & General	\$26,739
TOTAL EXPENSES:	\$174,275
Excess (or deficit) for the year	\$32,189
Net assets beginning of year	49,916
Net assets end of year	\$82,105

Total Revenue for Fiscal 2004 (ended June 30, 2004) was \$206,464. Of this, \$80,000 was allocated to research funding.



39% Research
30% Education & support
3% Fundraising
13% Administration
15% Reserve

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