



Is it in the DNA?

Ed Tenner points out and explains the genetic component of pemphigus. *See page 5*

Same Disease, Different Treatments

Johns-Hopkins doctors shed light on the various ways pemphigus is diagnosed and treated. *See page 6*

IPF Welcomes 3 New Support Groups

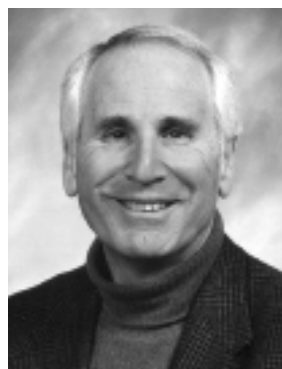
Boston, Las Vegas groups hold their first support group meetings. *See page 7*

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IPF Welcomes New Members to Board of Directors & Medical Advisory Board

The IPF is pleased to welcome Neil Kuvin to the Board of Directors, and Drs. Marcel Jonkman and David Sirois to the Medical Advisory Board.



Neil Kuvin, Louisville, Kentucky

Neil Kuvin is Managing Partner in the communications & public relations firm of Business Communication Group. BCG serves a variety of commercial, non-commercial, government and non-profit clients from offices in Louisville, Kentucky and Indianapolis, Indiana.

A graduate of Ohio University with a Bachelor of Arts degree, Neil's professional career has consisted mostly of executive management positions with TV stations in New York, Atlanta, Detroit, Boston, Pittsburgh, Raleigh and Louisville. He left TV in 1990 to form the first of three public relations and advertising agencies.

During the past 14 years, Neil has served as marketing and communications consultant to some of Kentucky's largest corporations, not-for-profits, government agencies, and their management teams, including the Kentucky Cabinet for Economic Development, Jefferson County Public Schools, BlueCross and BlueShield of Kentucky, Rally's Hamburgers, Inc., The 2001 Billy Graham Crusade and Brown & Williamson Tobacco Corp.

Neil, his wife Anita and their youngest of six children, Todd, live in a suburb of Louisville, where the family originally moved to in 1987.

Marcel Jonkman was born in 1957 in Hengelo (the Netherlands). His career has been primarily at the University of Groningen. He graduated in 1984 with an MD and finished his Ph.D. thesis on artificial skin and epidermal wound healing in 1989. From 1988-1992 he was trained in dermatology at the University Hospital (Prof. Dr. J.P. Nater). He joined the faculty as assistant professor in 1993 and was promoted to associate professor in 1997. In 1996, Dr. Jonkman was invited by Dr. J. Uitto at Jefferson Medical College in Philadelphia to become

a visiting professor. In 1999, he started the Centre for Blistering Disorders in the Dermatological Department in Groningen. In 2002, he was appointed to full Professor of Dermatology with special regard to bullous diseases. Dr. Jonkman was appointed as Chair of the Department of Dermatology in Groningen in 2003.

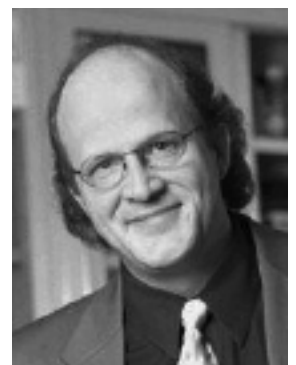
Dr. Jonkman is founder of the Dutch Society of Experimental Dermatology. At the University Hospital Groningen he runs special clinics in immunodermatology (bullous autoimmune diseases), epidermolysis bullosa, and genodermatoses. He is married to Gerrie, a family physician, and has three children.



Dr. Marcel Jonkman, the Netherlands

Dr. Sirois received the DMD degree from the University of Pennsylvania where he also completed the PhD in Neuroscience and clinical specialty in Oral Medicine. Presently he is Chairman of Oral Medicine and Head, Division of Reconstructive and Comprehensive Care at NYU College of

Dentistry. Dr. Sirois is a Diplomat of the American Board of Oral Medicine. He is nationally recognized in Oral Medicine and lectures widely on topics related to oral mucosal disease, salivary gland disorders, chronic orofacial pain. In addition to his academic and research responsibilities at NYU, Dr. Sirois also provides patient care in a private, faculty practice setting at NYU Tisch Medical Center. He has published widely in the clinical and basic sciences, focusing on oral mucosal disease, including Pemphigus Vulgaris, and trigeminal sensory physiology and pain. ☞



Dr. David Sirois, New York, New York

THE VIEW FROM HERE

Quality of Life vs. Side Effects



Janet Segall,
Executive Director, NPF

Over the years I have been asked about what drugs someone has to take, and for how long they might have to stay on them. They ask what doses are best, and if remission is possible. Sometimes these questions come from relatives of people who are living in a nursing home or assisted living situation. Many times we are told how their relative was suffering, but the doctors were reluctant to give steroids or other potent medications because they were afraid of the potential side effects.

Drug side effects can be serious. However, often they can be managed with additional drugs. So, the question becomes - does the fear of the side effects then become more important than managing the disease? Leaving many open sores can be very serious because the skin is our first line of defense against infection. If someone has had healthy skin all their lives, it is easy to take it for granted. I don't think people think about the skin as an organ or something that protects us. But in fact, the skin remaining intact is extremely important.

There are many questions that could be asked, but the reality of it comes down to the individual's need. One person might do very well living with a few lesions. Others want completely clear skin with no lesions. So this should be a question that a patient has to discuss with the doctor so that the doctor knows what the goal is. Of course, the only way to get lesion free skin (at least in the short term) is with higher doses of drugs.

One of the most difficult questions to answer is - What do you do if a patient is elderly and in a nursing home or assisted living situation? What if the patient is not cognizant of the world around them. The patient develops pemphigus or pemphigoid. Then, does one worry about the patient's comfort if that means drug doses have to be high, but his/her age might be a negative factor to high drug doses? Or should the quality of life be better even if it may shorten the patient's life? Often a family member will know what would be best for this type of patient.

The individual person living with pemphigus or pemphigoid knows best what he or she can tolerate in terms of drug side effects and pain or discomfort from lesions. In many cases patients need time to understand and figure out what is best for them. I want to encourage the doctors involved to give the patient the time they need to adjust to their situation and discuss all the possible scenarios including the ramifications of short and long term drug use. For the best possible outcome it could be to the patient's advantage to have someone to advocate for them - to go with them to the doctor. Have the questions prepared before hand. Follow our patient checklist. And I ask the doctor's to remember that this is a new experience. The Foundation exists to help both the patient and the doctor find the best possible solutions.

I'd like to apologize to both Peptimmune and BioFusion for not thanking them in my message in the last newsletter regarding our 2003 Patient/Doctor Meeting in Florida. We are very grateful for all your help and contributions to our Foundation. ☺

Save the Date - 7th Annual Patient/Doctor Meeting

The 2004 Meeting will be held in Las Vegas, October 9th, 2004. We will be changing the format this year and will focus the meeting more toward the needs, concerns, and issues of importance to patients. Doctors will be there as well. This will be an exciting meeting! See you there! ☺



International Pemphigus Foundation

"A common hope, an uncommon bond"

International Pemphigus Foundation is a 501(c)(3) nonprofit organization. Our goals are to increase awareness of pemphigus and pemphigoid among the public and the medical community; to provide information and emotional support to pemphigus or pemphigoid patients and caregivers; to provide referrals to specialists; and to support research into advanced treatments and a cure. Founded in 1994.

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PRESIDENT'S MESSAGE

The Role of the IPF Board of Directors



Jean Barish,
President

On behalf of the board and staff of the IPF I would like to thank everyone for their support during our Holiday Fund-raiser. Your generosity is greatly appreciated. Thanks to your contributions the Foundation is able to continue

providing valuable services to everyone in the pemphigus and pemphigoid community.

The work of the Foundation is important to so many, and our staff and volunteers work tirelessly to help you. One especially hard-working group of volunteers serves on our Board of Directors. From time to time I have been asked what the board does and what role it plays in the Foundation. I would like to briefly answer that question.

According to the IPF bylaws: "...the activities and affairs of the IPF shall be managed, and all ... powers shall be exercised, by or under the direction of the Board."

Under these by-laws, the Board of Directors has several important obligations, including the following: determining the organization's mission and purpose; providing proper financial oversight; ensuring adequate resources; ensuring legal and ethical integrity and maintaining accountability; ensuring effective organizational planning; selecting the chief executive; enhancing the organization's public standing; determining, monitoring, and strengthening the organization's programs and services; supporting the chief executive and assessing his or her performance; and, recruiting and orienting new board members and assessing board performance.

In sum, the board is the Foundation's management team. It determines the Foundation's by-laws and operating policies, and establishes its strategic plan and major programs. Like the captain of a ship it sets the course of the Foundation and maintains watch to make sure it is moving toward its destination. This destination can be summed up in the IPF Mission Statement: To increase awareness of pemphigus/pemphigoid, both among the general public and the medical community; to provide emotional support to patients living with these diseases, their families and friends; and to advocate research

into causes, diagnoses, treatments and cure of pemphigus and pemphigoid.

The IPF is fortunate to have an outstanding Board of Directors. Dr. Grant Anhalt, Justice Janice Taylor, Lee Heins, Robyn Christo, Neil Kuvin and Sonia Tramel have all been touched by pemphigus, either as patients, medical practitioners, or as a parent of a pemphigus patient. Each of these board members has special skills and talents that provide the Foundation with exceptional knowledge and support. And each dedicates countless hours working hard to support the Foundation's mission and their obligation as a board member. It's hard work, it's not especially glamorous, and it can be very frustrating. But it's also stimulating, creative, and very fulfilling.

In addition to participating in regular board meetings, every board member serves on one or more committees focused on issues including fund raising, government affairs, medical affairs and public relations. We are in regular contact with each other... discussing, debating, and resolving issues of importance to the Foundation.

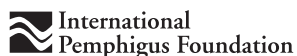
Board members have been actively involved in all Foundation activities. We are involved with fundraising by helping to plan our annual fundraisers, soliciting contributions from family and friends, and strategizing other fundraising efforts. We have created and helped implement several support

and educational programs, including our on-line list serve, local support groups, and medical and dental education programs. We work with several organizations including the American Academy of Dermatology, the National Health Council, American Autoimmune Related Diseases Association (AARDA), and the Coalition of Skin Diseases to educate Congress, government institutions such as Medicare and NIH, and the general public about issues of importance to our community. We work with our Medical Advisory Board to help organize research programs. And, perhaps most important, we are always available to support the staff and other volunteers by sharing ideas, exchanging information, and providing guidance.

Everyone on the board is dedicated to helping you live better and more successfully. We welcome and encourage your feedback. Hearing from you helps us serve you better. If you have questions, comments, or suggestions please feel free to contact us by sending a note to the IPF office in Albany, CA, by sending an e-mail to president@pemphigus.org, or by calling the IPF office at 510-527-4970.

We are all part of a very special, worldwide community of people who care about each other and who want to help. We are here for you and welcome the opportunity to provide assistance to you and the Foundation. ☺

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Pempuls Clinical Trial

A prospective, multi-centre, double-blind, placebo-controlled parallel-group, randomised clinical trial.

Following is a description of a clinical study currently being conducted in Europe:

Pemphigus is a severe chronic skin disease which appears incurable. Oral glucocorticoids (prednisone, prednisolone, dexamethasone) are the cornerstone of treatment. From previous uncontrolled studies it has been suggested that the use of high dose intravenous glucocorticoid pulse therapy may lead to complete remission without further therapy (cure). Prospective placebo-controlled studies of adjuvant pulse therapy for pemphigus are lacking.

This prospective, multi-centre, randomised, double-blind, placebo-controlled trial assesses the efficacy and steroid-sparing effect of oral high-dose glucocorticoid pulse therapy as adjuvant added to the standard combination therapy of prednisone and azathioprine in the treatment of pemphigus vulgaris, in terms of complete remission rate (primary endpoint), initial control, disease control, and adverse events.

In the experimental group, glucocorticoid (dexamethasone) pulse therapy is given, comprising monthly pulses with 300 mg oral dexamethasone on three consecutive days. In addition the patients receive daily prednisone and azathioprine treatment, the latter dose adjusted to the thiopurin methyltransferase level.

In the control group, the glucocorticoid pulse therapy is replaced double-blind by monthly oral placebo capsules on three consecutive days. In addition, the patients receive the same daily combination schedule with prednisone and azathioprine as in the experimental group. This study design requires 60 patients to be included, 30 in each group, with one year follow-up. *Only new patients with pemphigus vulgaris and/or pemphigus oris are included.*

Should the pulse therapy prove effective, it could become a useful strategy for treating this chronic disease. Furthermore, the study also addresses the applicability and safety of oral glucocorticoid pulses, which is considerably more cost-effective than intravenous administration.

Pulse therapy regimen: Dexamethasone/placebo: 300 mg (6 tablets of 50 mg). Dexamethasone is a fluoridated glucocorticoid. The glucocorticoid effect is per mg about 6.7 stronger than prednisolone. Since the pulse is given by oral route, which has a bioavailability of approximately 63%, 300 mg of oral dexa-

methasone is comparable to 1000 mg methylprednisolone by intravenous route. Monthly, an oral intermittent high dose dexamethasone is administered on three consecutive days. The capsules are swallowed with water in the presence of the trial doctor or nurse.

Criteria for Enrollment in Study Groups

All patients diagnosed as pemphigus vulgaris de novo during the inclusion period may join the study. *Patients that have been treated with long-term glucocorticoids are excluded since accumulated side effects may have occurred.* Patients with pemphigus foliaceus are excluded since this disease follows another pattern.

Inclusion

- Patients with pemphigus vulgaris with recent disease flare-up.
- Age of at least 18 years.

Exclusion

- Diagnosis of pemphigus different from the vulgaris type; i.e. diagnosis of: paraneoplastic pemphigus, pemphigus herpetiformis, pemphigus foliaceus, IgA-pemphigus, drug-induced pemphigus, if the drug has been withdrawn shorter than one month previous.
- Current use of adjuvant treatment other than azathioprine. A wash-out period of at least 2 weeks is required before inclusion.
- Presence of contra-indications for the use of high dose steroids: severe infections (herpes simplex oculi, tuberculosis), hypertension (diastolic >110mm Hg, systolic >180mm Hg; treated or not-treated), manifest decompensatio cordis, cardiac arrhythmias with the exception of atrial fibrilla-

tion, status after myocardial infarction (not within 12 months), low serum potassium, psychiatric history, cave psychosis, Cushing syndrome, pregnancy, infections.

- No availability for follow-up.
- Concomitant diseases treated with oral steroids.
- Glucocorticoids administered for pemphigus vulgaris longer than two months.
- Known presence of contra-indications for the use of azathioprine: homozygote patients for the low activity allele of TPMT, current use of allopurinol.

Our trial is already running in several countries in Europe:

The Netherlands

Groningen Prof. Dr. M.F. Jonkman
Leiden Dr. J.N. Bouwes Bavinck
Utrecht Dr. C.J.G. Sanders

Other Countries

Italy (Rome) Dr. G. Zambruno
Hungary (Szeged) Prof. Dr. Sándor Husz
Spain (Barcelona) Dr. José M. Mascaró, Jr.
Serbia Dr. Pilar Iranzo
Belgrade Milo D. Pavlovi, MD
Belgium (Gent) Prof. Dr. H. Beele

We hope to include 60 new pemphigus vulgaris patients by the end of 2005. We are still looking for more centers in the world to join our trial. For more information please visit our homepage: www.pempuls.nl and contact our trial coordinator.

www.pempuls.nl

Contact: Centre for Blistering Skin Diseases, Department of Dermatology, Groningen University Hospital, Hanzeplein 1, NL 9700 RB Groningen, The Netherlands ☎

Peptimmune's Phase I Trial of PI-0824 Reaches Critical Milestone

We are very excited to report that the first of three planned stages of Peptimmune's Phase I trial of PI-0824 for the treatment of PV is near completion! A total of up to 21 people will be enrolled in the trial, and currently there are seven participants, or 1/3 of the goal. Five of those seven participants have completed treatment with the investigational study drug and are in process of completing the follow-up safety visits.

Peptimmune is actively recruiting participants to complete the last 2 stages of the trial! In an effort to make the trial more accessible to interested participants, Peptimmune plans to open up to four additional investigative sites (NY, MA, FL) in the upcoming weeks. When they receive approval to begin the study at each of these locations their address and contact information will be added to Peptimmune's current participating center list posted on www.clinicaltrials.gov. If you do not have internet access please contact Sherry Reynolds, Study Manager @ 617-715-8013. ☎

FROM OUR READERS

The Genetics of Pemphigus; It's Not Simple

by Edward Tenner M.D.

When doctors looked into the causes of pemphigus, one area of interest was if there was any genetic or familial basis for these diseases. In the medical literature the number of reports of pemphigus occurring in the same family was very few. However, a genetic basis of pemphigus has been found and is felt to relate to predisposing genes involved with the human leukocyte antigen (HLA) system, the human version of the major histocompatibility complex (MHC). You may have heard of this system because it is involved in tissue typing which is used when doing organ transplantation. It is also involved in the body's adaptive immune system's response to foreign bacteria and viruses. In some instances when things go awry it can be associated with certain autoimmune diseases.

The HLA complex is on chromosome 6 and contains many genes. We all inherit some of them from each of our parents and there is a 50/50 chance we will pass a specific HLA gene to our children.

In different ethnic groups and nationalities these HLA genes are present at different rates. It has been discovered that certain HLA genes are present in people with pemphigus at rates much higher than the general population. For instance, in Jewish and non-Jewish pemphigus vulgaris (PV) patients HLA DRB1*0402, HLA DRB1*0401 and HLA DQB1*0503 (these designations represent a specific gene) are highly prevalent. In patients with pemphigus foliaceus (PF), HLA DR4 genes including DRB1*04 and DRB1*14 are often present.

Detailed studies have revealed that these specific HLA genes in humans do seem to have a role in the etiology of pemphigus. In Ashkenazi Jews who develop PV, over 90% are found to have the gene HLA DRB1*0402. However, according to certain Israeli studies noted by Dr. Grant Anhalt only about one in 10,000 with this gene go on to develop pemphigus vulgaris. While many have looked into what may cause or trigger PV to develop in those who have a susceptible HLA gene, except for certain medications that are known to trigger pemphigus like penicillamine and ACE inhibitors, little specifically is known about why some people go on to develop pemphigus.

The reason why these HLA genes increase the possibility that someone will develop the autoimmune disease pemphigus is very interesting. The scenario goes like this:

- In autoimmune diseases, antibodies are made against antigens or proteins that are part of the person, so called self-antigens. In pemphigus vulgaris the autoantibodies are against desmoglein 3 which is a protein that makes up structures that hold skin cells and mucous membrane cells together. In pemphigus foliaceus the autoantibodies are against desmoglein 1 which also helps hold skin cells together though in a different layer of the skin than desmoglein 3. Desmoglein 1 and 3 are proteins made of amino acids which are linked together in a chain which in real life fold together into complex shapes. When the desmoglein molecules are attacked by autoantibodies they are not able to function and skin cells pull apart and form blisters.

HLA genes control special proteins on the surface of cells that act as receptors which present antigen to white blood cells as part of the immune response of our bodies. Within these receptors are groves or pockets (think of a lock) into which will fit bits or parts of a protein (think of a key). These small pieces of protein are also called epitopes. When the "lock" is filled with an appropriate "key" this cell-antigen or cell-epitope complex can then link up with a T cell and aided by some other signaling chemicals start the immune response that leads to antibodies formation against the epitope or the "key" that fits this unique "lock".

Researchers have been able to look at the HLA grooves relevant to pemphigus and have been able to figure out that parts of the desmoglein protein molecule fit into them. Different HLA markers have different "locks" on them and thus react to different pieces of the protein. In other words, the shape of the groove on certain HLA receptors fit pieces of the desmoglein molecule which sometimes leads to the formation of antibodies against desmoglein causing pemphigus in some people.

In the case of the Peptimmune clinical trial only people with the HLA DRB1*0402 gene are being involved in the study. This is because the treatment is based on this gene and its corresponding receptor which would not necessarily work for others with pemphi-

gus and a different gene. The treatment involves using a piece of the desmoglein 3 protein i.e. the immunodominant epitope that fits into these groves in an effort to alter the immune response. This very specific treatment it is hoped will turn off autoantibody production which in turn would help control pemphigus without affecting the rest of the immune system like all of the present treatments for the disease do.

So in summary we all inherit HLA genes from our parents and some of them have been found to be associated with pemphigus, an autoimmune disease. However not everyone one with these genes goes on to have disease. Something has to trigger the body into making autoantibodies. Therefore even if we pass these genes to our children, it is very unlikely they will also develop the same disease. Also by studying these predisposing HLA genes doctors may be able to devise medications which will stop the production of autoantibodies that cause pemphigus. ☞



Pemphigus & Pemphigoid Chat Room

We are pleased to announce that Loretta Weatherill has organized an on-line chat room through MSN groups for people with pemphigus and pemphigoid and their caregivers.

The first chat event was held on Thursday, February 12 and the participants enjoyed it immensely. This is a wonderful chance for you to get support and share ideas and information.

If you would like to learn about future chats please contact Loretta Weatherill at dachshundstwo1@juno.com. ☞

Differences and Similarities Among Expert Opinions on the Diagnosis and Treatment of Pemphigus Vulgaris

From the Department of Dermatology, Johns Hopkins University, School of Medicine, Baltimore, Maryland

Daniel Mimouni, MD,* Carlos H. Nousari, MD, Deborah L. Cummins, BS, David J. Kouba, MD, PhD, Michael David, MD,** and Grant J. Anhalt, MD

*Currently affiliated with the Department of Dermatology, Rabin Medical Center, Petah-Tiqva. **Currently affiliated with Sackler Faculty of Medicine, Tel-Aviv University, Tel-Aviv, Israel

Introduction

The lack of well-designed studies of the treatment of pemphigus has left both physicians and patients with no clear standard of care. This study is the first to assess expert opinions regarding the diagnosis and treatment of PV.

We conducted a telephone-based survey of 24 physicians from academic, tertiary care centers. Eighteen clinicians were from the United States and 6 from Canada, France, Israel, and Spain. The physicians surveyed have treated a total of 2050 patients, and have had an average of 20 years' experience treating pemphigus. Currently, these physicians treat 796 patients, with 456 new patients per year. The survey included questions about referral patterns, diagnostic techniques, and therapeutic regimens. The sample of experts was drawn from a list of referral physicians provided by the International Pemphigus Foundation.

RESULTS

Referral Patterns

All experts reported that they receive pemphigus referrals from general dermatologists. Other referral sources were general and family practitioners and internists, dentists and oral medicine specialists, and direct referrals from the emergency department. Additionally 4 physicians have been contacted by self-referred patients who had researched their symptoms on the Internet. Of those surveyed, 50% receive referrals within 6 months after onset of symptoms, 17% within 1 year, and 8% within 3 years.

Diagnostic Process

Diagnosis is secured by 96% using skin biopsy specimen with direct immunofluorescence, and by indirect immunofluorescence alone for 4%. None of the participating physi-

cians make the diagnosis of PV solely on clinical and histologic evidence.

Treatment

Of the physicians, 75% initially treat with prednisone, used either alone or in conjunction with adjuvant steroid-sparing agents. The remaining 25% initially use nonsteroidal agents, e.g. such as gold, tetracycline, or attempt to eliminate potential triggers such as drugs, concurrent infection, and emotional stress. The physicians who initially used noncorticosteroid drugs did so with no relation to the nature or extent of the disease.

Corticosteroid Treatment

Initial steroid dose ranged from 1mg/kg/d 3mg/kg/d. Long-term dosing approaches varied as follows: 37% of physicians polled stated that their goal is to eliminate corticosteroids completely; 11% had a goal of an alternate daily prednisone dose of 5 mg; 26% were comfortable with a dose of 5 mg/d; and 26% were satisfied with 10 mg/d. All participating physicians stated that their therapeutic approach was influenced by the following parameters: disease extent; rate of disease progression; and clinical response.

Immunosuppressants

Azathioprine (Imuran®) was the most frequently used adjuvant immunosuppressant (44% of physicians). Mycophenolate mofetil (CellCept®), cyclophosphamide, and cyclosporine are used by 20%, 16%, and 4% of physicians, respectively. Forty-six percent reported they maintain steroid-sparing immunosuppressants in their patients for 6 months to 1 year, whereas 36% continue the immunosuppressants for 1 to 2 years, and 18% maintain this regimen indefinitely.

Definition of disease control

Of the physicians polled, 53% defined "control" as an absence of new blisters, whereas simply a reduction in the number of blisters was adequate for 32%, and 15% said "control" implied blister-free without any active lesions. Only 5 physicians (21%) use serologic antibody titers routinely to monitor their patients.

Discussion

Despite the high morbidity and potential mortality in PV, we have found distinct differences in the approach to both diagnosis and treatment of PV. As expected, most referrals came from general dermatologists. However,

the long delay from onset of symptoms to expert evaluation is quite striking. In PV, similarly to other autoimmune diseases, if the process is not promptly and appropriately controlled, the disease hardens. This generates the opportunity for epitope spreading, making the pemphigus more difficult to treat.

While the vast majority of the experts surveyed confirm their clinical diagnosis by tissue biopsy specimen for histopathologic and direct immunofluorescence examination, a wide variety of therapeutic approaches, from initiation to end point, were observed. While the most common therapy was prednisone, 26% add adjuvant immunosuppressants immediately. The most frequently used adjuvant is azathioprine. However, mycophenolate mofetil, which has only recently been introduced, is also frequently prescribed. The vast majority (82%) of experts use adjuvant immunosuppressants for 2 years or less. Eighteen percent use these agents indefinitely.

An additional, unresolved issue is the final goal for prednisone maintenance and the possibility of complete cure. There is a lack of standardized criteria for discontinuation of prednisone. This poses a major obstacle in the objective comparison of different studies. This survey also demonstrated that the grading of disease activity in pemphigus is also quite variable. Fifty-three percent of the experts define "disease control" as no newly forming blisters, whereas 15% of the experts defined "control" as blister-free, and nearly one third were satisfied with simply a reduction in the number of blisters.

In summary, there is wide variation in the assessment, diagnostic techniques, and treatment regimens of PV, even among the most experienced authorities in the field. The lag time from symptom onset to referral emphasizes the need for heightened awareness of this potentially fatal disease among primary care physicians, oral specialists, and general dermatologists. Although tremendous strides have been made in the care of patients with pemphigus, there is clearly a need for improved standardization. Large-scale, randomized, controlled trials and consensus standards with regard to patient stratification would facilitate the objective assessment of drug efficacies and help to establish an optimal standard of care for all patients with pemphigus. ☞

SUPPORT GROUP NEWS

New Support Groups in Boston, Las Vegas, Sacramento

Las Vegas

by Cherie Jacobson, Las Vegas, PV

Bev Siegel graciously offered her home for the first meeting of the Las Vegas Pemphigus Support Group on Wednesday, November 19, 2003. We were a total of five women and an instant friendship was formed between us. Of course, we talked non-stop for a good two hours with no one taking a breath.



Imagine our surprise when Sonia Tramel of the Foundation emailed us and said she was coming to Las Vegas and would like have a meeting. So again, we met at Bev's home for a lovely, impromptu luncheon on Saturday, November 22nd.

After meeting Sonia, we could not stop talking! The warmth of friendship was felt by all. We spoke of our disease, our lives since the onset of Pemphigus and of course, our families and their support. We vowed to keep in touch and to meet again after the holidays.

Thank you, Sonia, for coming. I know I speak for all of us when I say that we now feel as if we are part of a group - and no longer alone. ☺

Sacramento

Stay in touch for details on the newly formed Sacramento support group. ☺

Boston

by Alan Papert

A new support group is being started in Boston, to serve the surrounding areas. The aim is to hold two formal meetings a year, and as many informal meetings as members desire.

The first meeting has a target date of Saturday, May 22nd, at the premises of Peptimmune Inc. in Cambridge, where they will provide a speaker who will update us on their progress in developing and testing a revolutionary treatment for pemphigus.

Anybody interested in attending should contact Alan Papert at (978)463-0965 or at alppy@comcast.net.

More information including confirmation of the date will be provided through the IFP Discussion Group, and personally to those expressing interest. ☺

New York

by Matt Koenig, Las Vegas, PV

At the December meeting of the New York Support group, we were treated to a presentation by one of our own. Diane Hirsch, whose son Steve has Pemphigus Vulgaris, led a presentation and discussion on Diabetes. Diane works at St. Lukes-Roosevelt Hospital in Manhattan as a nurse / Diabetes Educator. Her expertise in the subject helped all of us to gain a better understanding of the causes, symptoms and treatments of Diabetes. This is a special concern for people who take Prednisone, because it has such a strong effect on our body's metabolism. Many of us have family histories that include diabetes, as well as age and weight factors that can only encourage its development! Afterwards, in the true spirit of a support group, we postponed our concerns for pasta, and went out for a delicious early dinner at the Notaro Italian restaurant! Happy and Healthy New Year to all! ☺

Toronto

by Dan Goodwill

The Toronto Pemphigus Support Group held its most recent meeting on a cold winter's evening in early February. Now in its ninth year, the group consists of a number of "old timers" who are in remission, and some newcomers, who are coping with the trials and tribulations of the disease. The interaction between the two groups is the most beneficial aspect of these meetings.

One fairly new patient is trying to treat the disease with homeopathic and naturopathic remedies. Since her bone density test produced a below normal reading, she believed this was a prudent approach to dealing with PV. While these remedies have helped treat the symptoms, the patient is gaining weight and is not making the kind of progress she would like to see. This individual was encouraged to consult with a knowledgeable dermatologist and begin her drug regimen.

There was a debate on the role of stress in contributing to the onset of the disease. While this does not seem to be endorsed by the medical community, the feeling among the attendees was that stress plays some role

in triggering an outbreak of Pemphigus.

There was also a discussion on the possibility of high blood pressure as a long term side effect of the drug treatments. Two individuals currently in remission have both been dealing with elevated blood pressure readings. This begged the question as to whether this is a result of age and heredity, or have the years of drug treatments (e.g. Prednisone, Imuran) been a contributing factor.

Yoga and weight training were encouraged during the drug treatment period to strengthen bones and provide increased peace of mind. The former should be considered in addition to one of the established calcium supplements and drugs (e.g. Fosomax). Vitamin supplements were also recommended to replace the loss of potassium.

One patient talked about his very positive experience with a product that worked as a "second skin". A plaster worn on top of blisters and medicine, allowed the individual to enjoy a Caribbean vacation. He was able to swim without any problems. Some patients in remission mentioned that there they had no trace of the blisters and gum problems they experienced when the disease was active.

IVIg treatments, possibly in conjunction with CellCept, were discussed for patients who are not responding well to their Prednisone treatments. Some members of the Toronto Support Group have shown very dramatic improvements after undergoing IVIg treatments.

There was also much discussion on Prednisone drug regimens. The patients discussed the importance of starting their treatment at a sufficiently high level (60 – 80 mg, every two days) to quickly bring the disease under control. For those individuals who started at higher levels (e.g. 100 – 125 mg/day), and remained at these high levels for extended periods, this resulted in calcium depletion and serious problems (e.g. avascular necrosis for a couple of patients and fractured bones for another).

Pemphigus patients were encouraged to use a steroid sparing drug (e.g. Imuran) to allow for a more rapid reduction in Prednisone levels. There was also a dialogue on the need to taper more slowly at the lower Prednisone levels (below 20 mg every two days). Since the percentage drop is large as you scale down, pemphigus patients were encouraged to reduce by 2.5 and 1 mg increments, to more effectively bring the disease under control. ☺

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MEETING PROGRAM**Morning****What We Can Learn from the Use Of IVIg in Auto-Antibody Mediated Diseases**

Autoimmunity and Mechanism of Action of IVIg
 Mechanism Of Action In Autoantibody Mediated Diseases
 IVIg Properties and Adverse Events
 IVIg in Non-Dermatologic Diseases
 Economic and Practical Issues of Administration

Afternoon**IVIg in the Treatment of Pemphigus**

Pathogenesis of Pemphigus: implications for improved therapies
 Current Therapies of Pemphigus: benefits and problems
 IVIg for the Acute Control of Active Disease
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 Mechanism of action of IVIg in Pemphigus: implications for improved use

Feel free to send this announcement to colleagues.

For more information contact:

The International Pemphigus Foundation, Atrium Plaza, 828 San Pablo Avenue, Suite 210, Albany CA 94706
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Foundation Wish List

The IPF is a nonprofit organization with the goals of increasing awareness of pemphigus and pemphigoid among the public and the medical community, providing information and emotional support to pemphigus and pemphigoid patients and caregivers, providing referrals to specialists and supporting research into advanced treatments and a cure. It relies on the support of each and every one of us. Remember when you first learned about the IPF? Remember the relief you experienced when you learned that (although

your experience is unique) there are others working through their own pemphigus/pemphigoid experience? We each have something to contribute to help the foundation continue to thrive. Were a small group of people with a rare disease and even something small can be a big help. Below are a few suggestions for how you can help:

- donate airline mileage (to be used for outreach and necessary travel),
- write articles for the newsletter (personal

experiences not necessarily your pemphigus story — we want to get to know each other) (we also want to hear from support people),

- write a letter to the editor (stimulate discussion or just get a question answered),
- volunteer (to support people in crisis who live near you, run a local support group, represent the IPF at a meeting),
- when making donations inquire/remember employer matching programs, and
- a photocopy machine. ☺

Heart Heart

If you need to talk to someone about pemphigus, contact one of our volunteers.

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MEDICAL NEWS

Study at NYU Compares Two Treatment Regimens for Pemphigus

by Dr. Jean Claude Bystryn

A new clinical trial has begun to study the effect of **Intravenous Immunoglobulin** (IVIg) to treat progressive or treatment-resistant pemphigus at the New York University School of Medicine.

IVIg is a new and apparently effective way to treat pemphigus. IVIg is a formulation of immunoglobulin, a component of blood serum, derived from healthy screened blood donors. It is thought to work by decreasing the amount of pemphigus antibodies in the blood. While IVIg appears to be effective for treating pemphigus, the optimal way to use IVIg has not been determined.



Currently, IVIg is given in two ways, either by itself or together with cyclophosphamide, an immunosuppressant drug also used to treat pemphigus. The current trial should determine if IVIg works better if given alone or together with cyclophosphamide. The answer is important because of the need for better therapies for pemphigus.

In the trial one-half of the participants will be randomly assigned to receive IVIg alone and one-half will receive the IVIg together with cyclophosphamide at a standard dose. IVIg will be given by intravenous infusion over several hours on four consecutive days. The treatment will be repeated every two weeks for a total of three cycles, and will be given in the outpatient infusion center at the NYU Medical Center in New York City.

To be eligible for the trial, patients must meet the following entry criteria:

- A. Age 18 to 75
- B. Diagnosis of pemphigus by skin biopsy and blood test.
- C. Require at least 20 mg of prednisone daily to treat pemphigus.
- D. Have failed conventional therapy with prednisone, methotrexate, CellCept, or other immunosuppressive therapy; or have a relative contraindication to conventional therapy because of co-morbid conditions such as infection, peptic ulcers, osteoporosis, hypertension, diabetes, or others.

The Principal Investigator for this clinical trial is Dr. Jean-Claude Bystryn. If you are interested in participating, please contact Dr. Kendra Bergstrom at (212) 263-5246 or kendra.bergstrom@med.nyu.edu for more information about the study. ☞

Support the International Pemphigus Foundation with a generous donation.
Call, mail, or donate on-line at www.pemphigus.org. Thank you.

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