

ALPHA-1 NEWS

The Official Newsletter of the Alpha-1 Association

VOL. 14 ISSUE 2

December 2003

CMS Announces Hospital Outpatient Reimbursement Rates for 2004—Alpha-1 Medicare Recipients Guaranteed Access to Care

On October 31, 2003 the Centers for Medicare and Medicaid Services (CMS) announced the 2004 hospital outpatient reimbursement rates for the plasma derived augmentation therapy used to treat the degenerative lung disease Alpha-1 Antitrypsin Deficiency (Alpha-1). The 2004 rate sustains patient access and may extend benefits to all augmentation therapies currently available for the treatment of Alpha-1. In August CMS proposed a significant change in Alpha-1 reimbursement based on averaged hospital claims data. The Foundation and Association became alarmed due to errors in the claims data that resulted in proposed rates far below the hospital acquisition cost.

According to Alpha-1 Foundation President and CEO John W. Walsh, "Claims data isn't representative of small widely dispersed populations such as Alpha-1 and would have required hospitals to administer the drug at an



annual loss of \$25,000 or more per patient. We had to convince CMS to change their approach." CMS's final rule indicates the power of the Community, comments from Alpha-1 organizations and individuals were taken into consideration. The final decision allows hospitals to bill 88% of the Average Wholesale Price - a significant increase over the August proposal.

Alpha-1 Association Board Chair John P. Morton stated, "We knew that patients would be turned away from treatment if CMS didn't handle Alpha-1 payments differently. We are grateful for the way the Community responded when we issued an Action Alert. Hundreds of Alphas wrote to CMS and their members of Congress. We have tangible proof that this effort made a difference."

More remains to be done. "The Foundation has petitioned CMS for a new Alpha-1 diagnostic code to improve data collection and we are currently planning an education program for hospital billing

departments so that Alpha-1 therapies are appropriately reimbursed and accurately represented in the system. The goal is to be removed from annual rate review, have accurate data collected in the hospitals and permanently secure access to care," stated Walsh.

The Foundation and Association continue to partner with other like minded organizations in an effort to promote a legislative solution. Both organizations continue to encourage members of Congress to co-sponsor H.R. 2700 which will amend the Social Security Act improving reimbursement for all orphan therapies. ■

2004 National Conference

Hilton Atlanta Airport Hotel will be the site of the 2004 National Education Conference. We are thrilled to report reasonable room rates of \$82.00 a night, single or double occupancy. This hotel is conveniently located just 5 minutes from the airport and features free shuttle service to and from the airport. Make plans today to join us on:

APRIL 30—MAY 2, 2004

The 13th Annual National Education Conference
at the Hilton Atlanta Airport & Towers
1031 Virginia Avenue
Atlanta, Georgia 30354
Call 1-800-774-1500 for reservations.

SEE YOU THERE!

Special thanks to Len Geiger, Lou Glenn and Cathey Horsak for their help in site review and selection.

In This Issue

Call to Action Works!

Page 1

A Letter from the Chairman

Page 3

Membership Sign-Up Form

Page 4

Pedaling Awareness

Page 4

Report on Important Genetics Meeting and Patient Notification System

Page 5 and 6

Special Alpha-1 Education Days Section

Pages 7, 8, 9 and 10

An Executive Summary On Alpha-1 Foundation Research

Page 10

A Report from the Field

Page 11

An Alpha Memory

Page 12

Public Policy Update

Page 13

Raising Awareness

Page 14 and 15

Joining Forces to Benefit Alpha-1 Community

Page 15

Alpha-1 Support Groups

Page 16

Content for the Alpha-1 News is gathered from a wide variety of sources, including professionals, staff and members. All ideas are welcomed. The editor reserves the right to screen and edit all submitted materials. Every effort is made to assure the accuracy of the information in this newsletter. Material contained here is for informational purposes only.

What is Alpha-1 Antitrypsin Deficiency?

Alpha-1 Antitrypsin Deficiency, also referred to as Alpha-1, is a relatively common genetic disorder characterized by low or no levels of the protein Alpha-1 Antitrypsin (AAT) in the blood. AAT is primarily made in the liver. The function of AAT is to protect the tissues of the body, primarily the lungs. When there is a deficiency of AAT, severe lung damage can occur. Less commonly, the liver itself is damaged, and rarely Alpha-1 causes a skin condition called panniculitis. There is no cure for Alpha-1. There are treatments available to lessen symptoms, but organ transplantation is often needed when the disease advances. It is estimated that up to 100,000 adults and children in the U.S. have severe Alpha-1, and potentially 23 million people nationwide are carriers. Given this high prevalence, it is surprising that many medical professionals perceive Alpha-1 to be rare, and it is often misdiagnosed. An alarming 95% of people estimated to have Alpha-1 have not been identified.

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Who is the Alpha-1 Association?

The Alpha-1 Association is a member-based nonprofit organization founded to identify those affected by Alpha-1 Antitrypsin Deficiency and to improve the quality of their lives through support, education, advocacy and research. We believe in treating the whole patient and all the issues facing individuals and families affected by this disorder. The Alpha-1 Association receives funding from a variety of places. We receive about 10% of our overall funding from our membership. An additional 25% comes from individual gifts, memorials, and Association-sponsored events and products. The remainder comes from gifts or grants from foundations and corporations.

How To Contact Us

Alpha-1 Association
1225 Eye St., N.W.
Suite 1225
Washington, DC 20005-5918
800-521-3025

To Contact the Editor

To contact the editor with ideas and suggestions (or even complaints!) for the newsletter, write to:

Ann Dassing
Front-Page Promotions, Inc.
14 Hatfield Street Caldwell, NJ 07006

Phone: 973-403-0032

Fax: 973-403-9174

E-mail: anndassing@comcast.net

Visit our web site at www.alpha1.org

Association Website Updated

The Association website has added a new portal, which links all Alpha-1 organizations providing service to the Alpha-1 community: Alpha-1 Association, Alpha-1 Foundation and AlphaNet. *Check us out at www.alpha1.org and see how we're collaborating to serve you better!*

This issue of Alpha-1 News was published by the Alpha-1 Association with support for writing, design, printing and distribution provided by a grant from Bayer, Baxter and Aventis Behring. No part of the promotional materials inserted in this newsletter infers that we support or endorse any of these products or services.

Membership Drive Is Going Strong, Please Join Us!

“Even if you’re on the right track, you’ll get run over if you just sit there.” —Will Rogers

December 2003

Dear Friend,

Welcome to the start of a new year! A new membership year that is. So here’s what we suggest: join or renew your Alpha-1 Association Membership. Why? Not just for the benefits listed below, although they are good.

- Join because our small numbers are made more powerful with one strong voice.
- Join because this is where it all started for Alphas, and that power needs to be maintained.
- Join because you will get the support, understanding and education you need through our support group network, regional Alpha-1 Education Days, National Education Conference, Action Alerts and the Alpha-1 News.
- Join because now—more then ever—Alphas need to unite on important Advocacy issues.

It’s no secret we’ve had a difficult year. What deserves more attention is the fact that we emerged from that challenged time intact, stronger, better organized and perhaps with a closer personal bond amongst all of us. Our May 2003 National Education Conference in Chicago was the largest, most well-attended we’ve ever had. And we know that 2004 will be a resounding success.

Why is that? This community has an uncommon drive. That drive has taken what was an unknown and ignored disease and forced it into the attention of the medical and scientific communities. That drive has resulted in some of the most effective lobbying and public policy efforts seen in Washington.

All of these positive attributes are best channeled through membership in one organization that speaks for the entire community. Look at what the American Association of Retired Persons (AARP) has been able to do, speaking with one voice for millions, all of whom have differing primary issues.

Please keep your voice where it is best heard. Join the Alpha-1 Association today. Whatever you do, don’t just sit there! Will Rogers would be very disappointed.

Wishing you a happy and healthy holiday season.

Sincerely,

John P. Morton
Chairman, Board of Directors
Alpha-1 Association

Announcing The Pete Smith Scholarship Winners:

\$1,000—Kristi Tinnes, New Jersey

Kristi knows first-hand what it takes to manage and care for an Alpha, as her mother was diagnosed in 1996. Now, as she enters college, she has chosen education, in particular, Special Education, and hopes to use the skills she acquired while caring for her Mom, and the personal attributes of patience and responsibility, in the classroom.

\$500—J. Mica, Oregon

Says J. Mica, “The impact Alpha-1 has had on my life is intense and highly motivating. She will contribute to the Alpha community by spreading the word about Alpha-1 in film. She studied film in college and holds a B.A. in sociology, and will record Alpha-1 from many perspectives: doctors, Alphas, and relatives of Alphas. ■



1225 Eye Street • NW Suite 1225 • Washington, DC 20005-5918 • Phone: (877) 346-3212

MEMBERSHIP OPTIONS

Patient and Family.....\$25

- Receive \$50 off registration fee for the 2004 National Education Conference in Atlanta, Georgia.
- Please list the names of family members residing in your household who wish to join.

Professionals and Non-Patients.....\$50

- Receive \$25 off registration fee for the 2004 National Education Conference in Atlanta, Georgia.

The Alpha-1 Association membership year runs October 1, 2003 through September 30, 2004.

MEMBERSHIP APPLICATION

New Member Renewal

Please Print

Name _____

Address _____

City _____ State _____ Zip _____

Telephone/Home _____ Telephone/Work _____

E-mail Address _____

We want to provide accurate and appropriate information to you. To do that, please check all appropriate boxes that apply to the primary member.

- | | |
|--|--|
| <input type="checkbox"/> Lung-Affected Alpha | <input type="checkbox"/> Liver-Affected Alpha |
| <input type="checkbox"/> Panniculitis-Affected Alpha | <input type="checkbox"/> Liver-and Lung-Affected Alpha |
| <input type="checkbox"/> Caregiver | <input type="checkbox"/> Carrier |
| <input type="checkbox"/> Spouse | <input type="checkbox"/> Other _____ |

Those Alpha-1 patients who wish to receive Association literature and are financially unable to pay membership dues, please write "Waive" below.

Pedaling Awareness



All 8 Alpha Wings riders placed in the top 5 at the Illinois State Championship. From left to right: Alex Cotton (11), Jay Eymers (15), Mat Eymers (11), Kim Cotton, Samantha Brungart (10), Roger Brungart (13), Andrew Cotton (14) and Jon Eymers (9).

Early detection could add years to an Alpha's life. Raising awareness is the key reason the Eymers sons formed the BMX Bicycle Racing Team "Alpha Wings" in Spring 2001. Team Managers Michelle and Ed Eymers help these amazing kids compete in local and state competitions. It is truly wonderful to see young people involved in community outreach and helping others. The further around the country they travel the more exposure Alpha-1 receives in local communities. The Alpha Wings will spend Thanksgiving racing with the best in the U.S. at the Race of Champions and the Grand Nationals in Tulsa, Oklahoma. The Association salutes the recent Alpha Wings victory at the Illinois State Championship. ■

The Impact of Genetic Testing: Ethical, Legal and Social Issues Alpha-1 Foundation: Gordon L. Snider

Critical Issue Workshop 8

By: Symma Finn, Alpha-1 Foundation

The Alpha-1 Foundation organized its eighth critical issue workshop in conjunction with the University of Florida Foundation. Held in Coral Gables, FL on October 10, 2003, its purpose was to explore the ethical, legal and social issues (ELSI) relating to predictive genetic testing, including privacy, confidentiality, the potential for social, familial or economic discrimination and the legislative response to these emerging issues. Participation of experts in a variety of disciplines explored the implications and challenges of increased genomic information.

The Alpha-1 Foundation critical issues workshops are intended to stimulate the patient and research communities to work together to address the most important issues—in this case the social, legal, ethical and economic impacts of genetic testing. This effort will develop protective measures against genetic discrimination, and will generate interest in conducting research among a wider field of social and medical scientists.



Meeting participants, Sally Everett, Ed Schuck and Gordon L. Snider, MD (seated)



Elaine Alfonzo represented the Puerto Rico Alpha-1 Foundation

BACKGROUND AND REASON FOR A WORKSHOP

Physicians, whether or not they have expertise in the issues relating to genetic conditions, are becoming more aware of the need to recommend genetic diagnostic tests and are doing so on an increasing basis. Physicians need to be aware of the ELSI issues involved to know when to make the recommendation for testing. They should also be able to provide clear and effective informed consent to adequately protect the privacy of patients, and should be aware of the impact on families and caregivers of information discovered about an individual's genetic condition.

Social scientists, lawyers and government officials need to be informed of these issues as well in order to provide appropriate counseling, sufficient legal protections and advice, or to formulate appropriate public policies that address the issues discussed at the workshop.

WORKSHOP GOALS AND OUTCOMES

The workshop's goals included:

- 1) The identification of the major ELSI impacts of predictive genetic testing.
- 2) The identification of issues related to informed consent.
- 3) The identification of confidentiality issues related to genetic testing.
- 4) Greater understanding of the clinical aspects of Alpha-1, a genetic condition.

INFORMATION PRESENTED—THE EMERGING ISSUES

Presentations at the workshop included a background on Alpha-1, genetic testing and patient registries, the role of race in genetic research, and the law's treatment of genetic discrimination in health insurance. The keynote presentation was given by Barbara Fuller, Branch Chief for Policy & Program Analysis at the National Human Genome Research Institute, National Institutes of Health. Her talk focused on how the mapping of

the human genome opened up new areas of information and the way this genetic information is understood (or misunderstood) in society. It also touched upon the early legislative responses to the ability to identify a variety of genetic conditions. Ms. Fuller noted that although there are already 42 states with some form of state law prohibiting genetic discrimination in health insurance, in the workplace or to protect the privacy of an individual's genetic information, these protections are defined differently state by state.

And while the states have taken action there remains a strong need for legislative efforts to define and control genetic research and the upcoming vote in Washington, D.C. on the Genetic



Meeting Moderator Chip Withers, Vice-Mayor, City of Coral Gables

The issues surrounding genetic testing represent an important part of the broader societal debate about ELSI issues in genetic research and the development of therapies to treat genetic diseases. A review of disease specific literature on Alpha-1 Antitrypsin Deficiency and COPD provides some information on these issues, but as the field of genomics is advancing rapidly, new issues are constantly emerging. This workshop was held precisely because there is not a lot of current data on the social impacts of genetic testing. The workshop provided an opportunity for the participants to have numerous interactive discussions about these broader issues.

Continued on page 6 . . .

Continued from page 5 . . .

Information Nondiscrimination Act. *Editors Note: This bill was passed unanimously in the Senate and is now being considered in the House.* The presentation included other emerging topics such as liability concerns and standards of care (is there a duty to provide genetic tests, warn patients of liability to their relatives or to keep this information confidential); will genetic tests become standard clinical practice; will the advances in genetics only be available to a privileged few and will the knowledge of human variation reduce prejudice or increase it?

Another very informative presentation, by Beth Labasky, used the legislative initiatives in the State of Florida as a model of how one genetic condition (Alpha-1) has dealt with funding issues

and state legislation on genetic therapies such as stem cell therapy. The Foundation, has been very successful at making the case for the importance of this type of research and to obtain funding for targeted detection of Alpha-1 to increase identification of this genetic condition in the State of Florida. Ms. Labasky noted the difficulty of a unified societal approach to these issues, when each state defines "cloning" differently and takes a different approach to protect against discrimination.

Eric Juengst, Ph.D., explored newly emerging issues resulting from an increase in our society in the amount of genetic research, numbers of individuals tested for genetic conditions and readily available information about one's genetic makeup. His talk focused on whether

increases in genetic information and research will enhance health care or be used for self-improvement, whether enhanced human achievements (through gene repair or gene therapies) are real achievements and whether these improvements would only be available to those who can afford them. He proposed social policies that could prevent the worst abuses including regulation of gene transfer technologies and providing equal access to all individuals.

The workshop identified a number of areas requiring further social research and will hopefully lead to an increase in interest in addressing these important issues within the field of Alpha-1 Antitrypsin Deficiency research. ■

PNS Provides Notification of Plasma Product Withdrawals and Recalls

This article was provided by PPTA, the trade association representing the plasma manufacturing industry.

The Patient Notification System (PNS) is a free, confidential, 24-hour communication service for plasma protein therapeutics consumers providing information on plasma-derived and recombinant therapy withdrawals and recalls, including alpha-1 products. Launched in 1998, the Plasma Protein Therapeutics Association (PPTA) administers the PNS with direct input from consumers, industry and the FDA.

The PNS is truly a one-of-a-kind tool. There is no other communication service like it in any other segment of the healthcare industry. It enhances consumer confidence in plasma protein therapy quality by providing up-to-date information on withdrawals and recalls.

Maintaining patient confidentiality is one of the cornerstones of the PNS. To ensure confidentiality, the PNS is operated by the NNC Group, an independent organization that specializes in pharmaceutical notifications. All patient information is held in strict confidence with the NNC Group and begins when an interested party registers with the PNS. General information is submitted to the system, including the preferred method of notification. Although, registrants are encouraged to be notified via e-mail, they can also be notified by telephone, fax and express mail.

If a product is withdrawn or recalled, the company involved immediately contacts the NNC Group which then notifies the enrolled participants directly. Every effort is made to notify participants within 24 hours. Each will also receive a letter by first-class mail to ensure receipt of the information.

In addition, consumers can go online to www.patientnotificationsystem.org or call a 24-hour, toll-free number (1-888-UPDATE-U) for current information on product withdrawals and recalls.

There is no fee to participate in the PNS, as the system is fully funded by the producers and distributors of plasma-derived and recombinant products. Consumers need only sign-up via the Web or the toll-free number.

The PNS is a cooperative effort between industry and consumers. Key consumer groups, including the Alpha-1 Association, worked closely with the plasma protein therapeutics industry in designing the PNS. Consumer groups remain active in the continued development of the PNS through participation in a quarterly advisory committee meeting. Recent enhancements have included the launch of a French-language version in early 2003, and process improvements to speed information delivery (participants are again encouraged to register for e-mail notification for instantaneous, traceable access anywhere in the world).

The system is only as effective as the number of registrants. Consumers, healthcare providers, etc. are also encouraged to participate to make the PNS the location for all information pertaining to withdrawals and recalls.

For more information on the Patient Notification System, or to register, visit www.patientnotificationsystem.org today. You rely on plasma therapies, now rely on the PNS. ■

Education Days Represent the Latest Medical Information and Plenty of Camaraderie For Alphas

New York COPD and Alpha-1 Educational Conference

**By: Robert A. Sandhaus
M.D., PhD., FCCP**

The Alpha-1 Education Day program has been an extremely successful joint effort of the Alpha-1 Association and the Alpha-1 Foundation. Bringing together outstanding speakers from the local community, guest speakers, patients, and healthcare professionals for a day of learning and discussion, these Education Days have provided a focal point for awareness and advocacy regarding Alpha-1 for communities around the country.

The Education Day concept recently took a giant step forward with the introduction of a unique new format at the New York COPD and Alpha-1

Educational Conference held on October 17 and 18, 2003. Combining the resources of St. Luke's-Roosevelt Hospital Center, the James P. Mara Center for Lung Disease, the COPD Resource Network (formerly NECA) with those of the Alpha-1 Association, the Alpha-1 Foundation, and AlphaNet, this two day event drew large groups of interested participants. With a format concentrating on COPD (chronic obstructive pulmonary disease) during the first day and Alpha-1 Antitrypsin Deficiency during the second, the seeds of a unique alliance were planted between the 25 million persons with COPD and the 100,000 persons with Alpha-1 in this country.

The roster of speakers read like a who's-who of experts within the Alpha-1 and

COPD communities both locally and nationally. The conference started out with COPD talks by the hosts of the event, local celebrities (in a medical sense) Dr. Gerard Turino and Dr. Edward Eden, who spoke about the causes and treatment of COPD, respectively. Talks by Dr. Mark Brantly, Dr. Alan Barker, John Leaman, Dr. Norma Braun, Barbara Rogers, Dr. Byron Thomashow,



**Ryan Dickson, Alpha-1
Research Registry**



**John Leaman, Editor,
Respiratory News
and Views**



**(l to r) Diane Walsh, Meredith and Ed Baines,
and Sharon Friedman**



**Barbara Rogers, President COPD Resource
Network, conference initiators, Dr. Edward
Eden, and Dr. Gerard M. Turino with John
Walsh**

Dr. Mary O'Sullivan, and Dr. Cliff Connery followed. Topics included screening for Alpha-1 within the COPD community, bronchiectasis, gender differences in COPD, the role of the respiratory therapist, results of the NETT study, and surgical treatments for emphysema. There were also presentations on COPD from the patient perspective as well, including a description of the growing COPD Resource Network.

The following day, the concentration was directly on Alpha-1 with several of the first day's speakers providing their

Note: This conference was sponsored by the James P. Mara Center for Lung Disease, St. Luke's-Roosevelt Hospital Center, Alpha-1 Foundation and Alpha-1 Association and COPD Resource Network. Unrestricted educational grants were provided by AlphaNet, GlaxoSmithKlein, Dey, Aventis Behring, Baxter Healthcare Corporation, Bayer HealthCare Biological Products Division and Coram Healthcare.

perspectives and additional speakers including Dr. Jamie Stoller, John Walsh, Miriam O'Day, Dr. Sandy Sandhaus, Dr. Robert Klitzman, Dr. Edwin Silverman, Dr. Joshua Sonnett, and Dr. Mark Zern. Topics ranged from descriptions of Alpha-1 liver and lung disease to a description of the newly published "Standards for the Diagnosis and Management" of Alpha-1 document. Also included were talks on lung transplantation, patient advocacy, the roles of the Alpha-1 Foundation and the Alpha-1 Association, the AlphaNet Alpha-1 Disease Management and Prevention Program, issues related to genetic privacy, and new therapies for Alpha-1. Joe Reidy even got to put in his two cents on what Alpha-1 means to patients! Panel discussions with question and answer sessions ended each day's events.

The high quality of the presentations and the identification of common themes among the COPD and Alpha-1 communities made this educational conference a stand-out. Could there



**Pam Schreck, R.N.,
University of Florida Alpha-1
Research Program**

be a combined Liver Disease and Alpha-1 Educational Conference in our future? The schedule of upcoming conferences is always included in this Newsletter. Please keep an eye out for one that you might attend.

Continued on page 8 . . .

Continued from page 7 . . .

Alpha-1 Education Day—Texas

By Cathey Horsak

I am constantly amazed and inspired by the people I encounter on a daily basis in the Alpha-1 Community. The Texas Education Day, held October 17-18, was another

opportunity for me to witness Alphas in action. First and foremost, a big Texas tip of the Stetson goes to Patti Brown, our AlphaNet coordinator, and Association Board Member, Lou Glenn who organized this great event. Marcie Heitzman said "Lou Glenn and Patti Brown were so giving of their time to help schedule this event for us, and for that we are thankful.

It means the world to us to have the chance to come to such an event. I got to put faces to names in person and feel so fortunate to have the opportunity to do so." Marcie attended the Texas Education Day with David Heitzman. Earlier this year they lost their beautiful baby boy, Hunter, not yet 7 months old. Hunter had been diagnosed with Alpha-1 liver disease and received an unsuccessful transplant. Hunter died waiting for a second liver. These determined young parents have established The Hunter Lee Heitzman Endowment Fund and are doing everything possible "to educate anyone and everyone about Alpha-1". See what I mean about amazing and inspiring people?

The Texas Education Day program began on Friday evening with a reception at the Hilton Inn in Arlington, Texas. The food was delicious and artistically displayed. As is the case with all Alpha events, it lasted well beyond the two-hour time limit. Any time a group of Alphas get together, there is much camaraderie to share.

Saturday morning's program began with the Alpha-1 Association update. The Association is on track and fulfilling their



Cathey Horsak

mission statement by providing services to its membership. Our volunteers are doing a great job and responding to our Advocacy Alerts to the community.

Dr. James Stocks discussed the critical importance

of participation in clinical trials. Most of the 75+ attendees knew Dr. Stocks very well. Of the 38 Alphas present, most of them were currently and/or had been his patients. Nancy Creech, RN MSN CCRC and Kelly Pierson from Dr. Stocks' office were on hand to sign up Alphas for the Research Registry, Clinical Trials at UTHC at



Shelley & Dennis Pollock and Lou Glenn

Torres, a pulmonologist at Southwest Pulmonary Associates in Dallas, Texas. He also serves as Director, Pulmonary Hypertension Clinic at St. Paul University Hospital/UTSWMC. Dr. Torres is very familiar with Alpha-1. His presentation covered both pre and post lung transplant issues. Dr. Torres' parents, both of which are doctors in Puerto Rico, were visiting in Dallas and attended our program. They both commented after their son's presentation how they will begin looking for Alpha-1 patients in Puerto Rico.

Fellow Alpha Dave Courtney discussed Presumed Consent and how it could affect organ donation and our Alpha community. If you read the guest column written by Dave in the last newsletter you know that his work is directed at presuming that everyone is an organ donor, and that you would have to "opt out" instead of the

current system of "opting in". Dave and his wife, Jo, coordinate the West Texas Alpha-1 Self Support Group and are both Alpha-1 Association peer guides. Dave, currently on the lung transplant list himself, shared with us that 17 people die

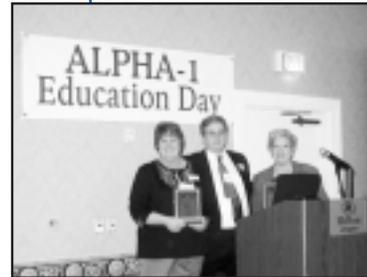
every day waiting for a transplant because there are not enough organs available. Presumed Consent is currently in

effect in Spain,

Italy, Belgium, France, Austria, Finland, Luxembourg, Norway, Denmark, and Singapore with an opt out rate of approximately 2%. It could work in the United States, too. Dave's message "Call your legislators and let them know you support Presumed Consent."

Bob Callahan, Vice President for Development for the Alpha-1 Foundation, shared with us the update on the Foundation. Foundation funding has reached over \$15 million in research, with AlphaNet contributing \$10 million of that total. This further emphasizes the importance of our Alpha organizations working together for the good of the Community. It was interesting for us to hear Bob's presentation on a patient centered research model. Bob summarized his presentation by encouraging everyone to join the Alpha-1 Foundation Research Registry, enroll in the DNA & Tissue Bank, participate in clinical trials and research, get involved in advocacy, and help raise funds for research. Patient participation is the power behind the Alpha Community successes.

Our last speaker for the day was Dr. Natalie Murray. She is a hepatologist at Baylor University Medical Center in Dallas. She shared with us some very interesting statistics. She said you could "round-up" all those on any skid row in any major city in the United States. You could test them for a battery of liver disorders and only 10% of them would



Patti Brown, Bob Callahan and Lou Glenn

Tyler, and the DNA & Tissue Bank. The Community response kept them very busy all day.

Our next speaker was Dr. Fernando

Continued on page 9 . . .

Continued from page 8 . . .

have cirrhosis of the liver from alcohol alone. All the rest of them would have other contributing causes, i.e. Alpha-1, hemochromatosis, Wilson's Disease, etc. This seems to "shoot down" the theory of alcoholic cirrhosis that so many of our Alpha liver patients are misdiagnosed with. She shared her concern about the

affected heterozygote Alpha population. She also said blood levels were deceptive and phenotyping is a much more reliable test.

To quote another of our attendees, Ray Reynolds said "This was the first Alpha-1 Education Day I have attended and I was really impressed with the way

everything went together. The program was great, the speakers were excellent, and the hotel accommodations just super. . . I am really looking forward to attending another one. . ."

I couldn't agree more and I hope to see you at an Education Day in your area soon! ■

EDUCATION DAY—SALEM, VIRGINIA

By Jeanne Ruff

Being an Alpha can be a lonely experience, especially in the beginning, together with the "orphan" drug that makes the patient feel like an orphan, to boot. Attending an Education Day takes care of that fast, as my husband learned, after recently attending the Salem, Virginia Education Day on October 4th. We decided to sign-up, based on the detailed agenda, which we received well ahead of time, and on convenience: The site of the event was extremely easy to get to, located just off the interstate, with the motel right next to the Lewis Gale Foundation conference center. Furthermore, our AlphaNet coordinator, Gayle Allison, had rounded up the crème de la crème of presenters. How could we resist?

With ample time to register and receive a generous bag of conference materials, we had an

opportunity to walk around to the exhibits and talk to the very well informed representatives. That was a good beginning. Then the program began promptly as scheduled (how many conferences can claim that?) at 9 a.m. with a welcome from Gayle and Linda Richardson, co-organizers. Miriam O'Day, who gave a spirited update on federal advocacy, followed this. It was astounding

to learn the kind of work, which is required to do a good job of this,

disease management, which is of course is a primary concern for all of us. Later in the morning, Dr. Mark Brantly, who came from Florida for this event, spoke about

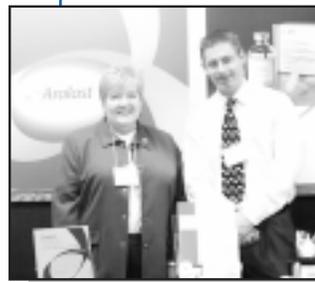
novel treatment therapies, complete with slides of DNA and other relevant microscopic things. He also spoke about what's involved in research: how research ideas are generated, how those ideas finally come together to form an acceptable project, the difficulty of attracting funding, and the length of time usually involved for a project to come to completion. As with all research, some projects bear fruit, and others have to be scrapped even after great time and effort have been spent on them. He then urged us all to consider contributing to the DNA and tissue bank, which had been set up in an adjoining room. I don't know how many people there that day actually contributed, but from where we stood, it looked like a sizeable percentage. I certainly hope so.

Later in the day, we heard from Dr. Charlie Strange about the Research Registry, another vital effort in the search for a cure, and we learned about the on-going activities of the Foundation and AlphaNet.

The transplant panel was most interesting and encouraging, but interestingly, Dr. Sandhaus added the necessary cautionary note to that information. Finally, after time for naps, we gathered



The Acredo Crew



The Baxter Crew



The Aventis Crew



Dr. Sandhaus and Harwell



Richardson, Sandhaus and Allison



Delegate Reid

and we certainly admired the passion she brings to this effort. Delegate Jack Reid spoke about state advocacy, bringing his own personal commitment to the effort. Then, traveling from Colorado to be with us, Dr. Sandy Sandhaus spoke about a



O'Day

Continued on page 10 . . .

Continued from page 9 . . .

for "Dinner with the Docs", where we really experienced a feeling of community with other Alphas and their families, and got an extra dose of appreciation for the commitment of "the docs."



Richardson and Allison

During our breaks throughout the day and here at the dinner, we had some wonderful conversations.

I have to say that what amazed us the most was not the expertise of these medical professionals (they never talked down to us), but their accessibility and their candor. We learned things we could not have in any other way, and we came away with a feeling of gratitude, not just for their having made the effort to come

to a small town in Virginia, but for their complete openness to talk with all of us individually, at any moment during the conference.

Lest you think that the entire day was bathed in high seriousness, I



The Coram Crew

should mention that the wonderful dinner was punctuated by abundant laughter and

good cheer. It was fun and reassuring to me to meet other Alphas for the first time in a relaxed setting. Finally, I must say



DNA and Tissue Bank Staff

that as a result of this Education Day, I no longer feel isolated, and the word "orphan" no longer applies to the personal situation of any of us who attended. The Alpha-1 organizations have adopted us and we are now part of this special family. Many thanks go to all who worked hard at organizing this successful and meaningful event. ■

Alpha-1 Education Day Series

In the last issue of *Alpha-1 News* we included a report on the Sioux Falls, SD Education Day. This special report highlights the New York, Virginia and Texas regional Alpha-1 Education Days in the voice of a doctor, Education Day participant and staff member. Alpha-1 Education Days are a special collaboration of the Alpha-1 Association and Alpha-1 Foundation to raise awareness and bring information to

Alphas, family members and the healthcare community. Since the last edition of the *Alpha-1 News* Education Days were also held in Portland, OR; Denver, CO; Dartmouth College, NH; and Cleveland, OH. We extend our appreciation to those who organized, traveled to participate on the agenda and attended these events.

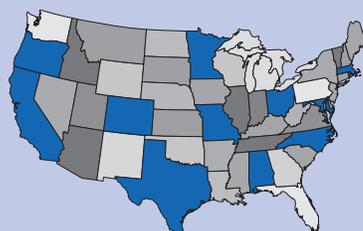
We are grateful to the companies that have assisted in underwriting the cost

associated with the Alpha-1 Education Days. Education Day 2003 Series sponsors include: AlphaNet; Bayer; Baxter; Aventis. In addition, numerous local institutions and companies have made contributions that have helped us bring these programs to you. ■

Alpha-1 Education Days by States for 2004

Information below is as of press time. Dates and locations will follow as they become available.

Alabama	Birmingham—University of AL	Minnesota	Minneapolis—MN Veteran's Res. Institute
California	Los Angeles, Harbor—UCLA	Missouri	St. Louis
California	San Francisco—University of CA	North Carolina	Chapel Hill
Colorado	Denver—Denver Health & Hospital	Ohio	Cincinnati
Maryland	Baltimore—University of Maryland	Oregon	To be determined
Massachusetts	Boston—Brigham & Women's Hospital	Texas	Tyler



■ Indicates states where Education Days will be held.

ALPHA-1 FOUNDATION RESEARCH PROGRAM UPDATE

By: Bruce C. Trapnell, M.D.

As Scientific Director of the Alpha-1 Foundation, an important responsibility is oversight of the Grants Award Program, the Foundation's mechanism to support a broad range of basic and clinical research aimed at improving the health and quality of life of people with Alpha-1 Antitrypsin Deficiency. To date, fifty-six research grants, totaling more than \$12 million dollars, have been awarded to investigators at 34 different research centers worldwide. The program has grown so rapidly that the number of grant applications received this year alone is nearly equal to the number of grants funded since inception of the program four years ago. Such strong enthusiasm from the scientific and medical community is a measure of their interest in helping to develop new therapies and a cure for Alpha-1. Currently, the data obtained from these funded research projects are undergoing an impact analysis to determine the program's effectiveness. Results of this analysis will be presented in the first annual Scientific Director's report, which is intended to describe how this research funding has improved our understanding of Alpha-1 and to review our progress towards development of effective therapies.

REASSESSING THE RESEARCH AGENDA

The Foundation's research agenda is also under concurrent review. The intention is to sharpen the focus towards funding research projects aimed at developing new therapeutic approaches. This will mean emphasizing support for research projects aimed at identifying molecular targets of disease, i.e., specific molecular abnormalities that cause some clinical

abnormality or disease process, as well as projects directed at evaluating new therapeutic approaches in both pre-clinical and clinical studies.

ESTABLISHMENT OF THE RARE LUNG DISEASE CLINICAL RESEARCH CONSORTIUM

An important objective of the Foundation has been to obtain external funding to support research. To this end, the National Institutes of Health recently awarded \$5.5 million to establish a Rare Lung Disease Clinical Research Consortium consisting of a coordinating center (Children's Hospital, Cincinnati, Ohio) and clinical sites in Ohio, Florida, Maryland, Massachusetts, Oregon, South Carolina, Texas, Japan, and Australia. The Alpha-1 Foundation will serve as the administrative center for patient recruitment and enrollment and these clinical centers will interface with other Foundation research centers across the United States to provide an integrated network that will accelerate development of diagnostics and treatments for Alphas. This will be accomplished by promoting collaboration between outstanding clinical research centers, the Alpha-1 Foundation and the community of Alphas. The NIH funds will also be used to recruit and train bright young clinicians in the care of Alphas. Clinical data from Alphas living in various regions around the world will be collected, striped of patient-specific identification information and the data will be collated and placed into a centralized database. These data will then be made available, via a special NIH-sponsored internet site, to people who are—or who may be affected by a rare lung disease, their physicians, other clinicians and researchers and the general public. ■



By Cathey Horsak,
Director,
Field Services

"Coming together is a beginning; keeping together is progress; working together is success."—Henry Ford

Successful leadership

When your doctor says "you have Alpha-1" and you have read the literature and surfed the internet—where do you turn? The Association's staff and voluntary leadership are there to offer you hope. We know from our recent National Survey that many Alphas live into their 60's and 70's and yet the literature still gives the average life expectancy as 54. Our local groups offer an opportunity to meet other Alphas and family members who are walking in your shoes.

Improving the day-to-day lives of individuals living with Alpha-1 is core to the Association's mission and we are

striving to find ways to improve those services. In September, the Board of Directors met and approved the establishment of a special leadership group to strengthen our support group network. The Board also approved the introduction of a grassroots grants program to provide support group funding for administrative costs such as mailings and telephone charges. Our goal is to use the experience of successful leaders to enhance our community outreach.

Individuals were invited to join the Support Group Leadership Advisory Council based on excellence in service to their local Alpha Community and geographic diversity. Bobbie Stafford, CA; Dennis Pollock, OK; Gayle Allison, WV; Marvin Sineath, SC; Rosemary Bybee, KY and Fred Walsh, MA have accepted our invitation to serve a one-year term on the Council.

Among the Council's ambitious goals is to improve the consistency of services and resources across the country so that if you are diagnosed in Portland, Maine you are

assured the same level of support as someone in Portland, Oregon. The Council will immediately set minimum national guidelines for Association affiliation and create standards of excellence. They will establish criteria for the grassroots Grants Program; review local grant applications; and make funding recommendations to the Board of Directors. The Council will also monitor the Peer Guide Program.

It is my pleasure to work with this dynamic group of volunteers as we set out to share our experience and help other Alphas deal with life's challenges.

Four New Support Groups Forming

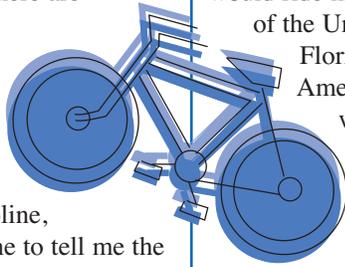
Through our grassroots network we have seen many exciting and rewarding results. Following the last issue of the *Alpha-1 News* volunteers have come forward to offer leadership to new support groups giving many more Alphas the information they need. If you are interested in forming a new group in your area please contact me at 877-346-3212 or Horsak@alpha1.org. ■

In Memory of . . . Paul Mansdoerfer (1947-2003)

With so many new Alphas joining our community each month, there are probably many who have never heard of Paul Mansdoerfer, or, as he liked to sign his emails and writings, The Alpha-1 Crusader. Paul died on September 11, 2003. Caroline, his devoted wife, called me to tell me the news. She started by saying, "Another hero died on 9/11."

Paul was a hero in many ways. For those who didn't know him, here's a brief description of some of his exploits. I may not have all the dates exactly right but most of the facts are correct. When Paul was diagnosed with Alpha-1 in 1990 he already had end-stage lung disease and was started on oxygen that same year. His doctor suggested at that time that he consider a motorized chair to improve his mobility. Paul headed down to the medical supply store to pick one out. While there, a stationary bike caught his eye. He decided the motorized chair wasn't for him and came home with the exercise bike instead. After working out on the exercise bike for several weeks, he decided to get a real bike. He began riding his bike around town. By early 1991 he was entering some local bicycle races. He found he was a pretty good competitor . . . he was always a pretty competitive sort of guy.

Paul was amazed at how few people, including docs, knew anything about



Alpha-1. He came up with the idea that he would ride his bike around the perimeter of the United States, from Florida to Florida. He called it Trek America. I think the total mileage was over 14,000 miles. At each town he rode through, he stopped and talked to anyone who would listen about Alpha-1. He claimed (and I have no reason to doubt him) that his FEV1 at this time was 17%. He sometimes used oxygen, he always had his augmentation therapy while on the road, and there were people accompanying him along the way, both by car and bike. He started Trek America in March of 1991 and finished it in October of the same year!

Almost immediately after completing Trek America he competed in and completed the Ironman Triathlon in Kona, Hawaii. You know what a triathlon is; that's where you run 26.2 miles, bike 112 miles, and swim 2.4 miles your way to total exhaustion. The Ironman is rarely completed by perfectly healthy, well-trained athletes! In 1992, Paul competed in a triathlon and biathlon in Florida and then decided to run the Boston Marathon. On April 20, 1992, Paul competed in and completed the Boston Marathon. As I recall, that was one of several years that Dr. Jamie Stoller ran in the Boston Marathon as well.

Through all of this, Paul preached the gospel of exercise and self-improvement.

He was an irascible guy. He often berated people who were considering lung transplantation because he felt they were wimps who weren't exercising enough and eating right. He was a loud presence at the early Alpha-1 Association annual meetings, often finding himself in verbal battles with people with whom he didn't agree.

Still, he lived by his words. He never would accept a lung transplant in spite of pleadings from his physicians. We convinced him to come to the Alpha-1 Association meeting in Charlotte in 2000 to receive an award. I was supposed to introduce him and let a whole new generation of Alphas know the Paul Mansdoerfer story. I regret to say that I was called out of the meeting to help an ill patient just before I was supposed to do Paul's introduction, so I never did get to pass on the Story of the Alpha-1 Crusader.

I was fortunate to be able to keep in touch with Paul over the past several years and even visited his home near Sarasota, Florida a year or so ago. As ever, his beautiful wife, Caroline, was by his side, as she was until the end. Even though many Alphas may not have known him or known of him, he affected the lives of many, many Alphas in ways they probably don't realize.

Goodbye, Paul—Dr. Sandy ■

In Memorium

In Memory of Carolyn Woods Davis
Kaye Teffeteller-Henry

In Memory of James Fickes
Don MacDonald

In Memory of Imogene Foster
Martha Lutz

In Memory of Steve Gilbert
Shirley Bail
Judith Bordonaro
Eleanor Kaye Burrows
Dee L. Cain
Chelmsford Senior Center
Barry Friedman
Lynn Friedman
Jacqueline Sue Gilbert
Frank Haran
Helen E. Hufendick
Dennis Kerley
Susan T. Kinzie
Kathryn R. Melville
Robert Olson

Patricia Brady Soderquist
Anne T. Van Allen
Dr. Charles Wilkinson
Cynthia E. Wilson

In Memory of Warren J. Gordon
Betty C. Darnell

In Memory of Thomas Leahy
Emmaine Adelman
Lorraine Bolling
Daphne S. Broderick
Helen Burke
Annette Chapin
Lesley Chick
Lorraine Dzurenda
Jacqueline Glover
Harold Hebb
Stella Konopka
Donna Lange
Timothy E. Lynch
Cynthia Mahan
Krystyna P. Nyerick

Alina Pope
Sharon Staffieri
Suzie Southard

In Memory of R. Paul Mansdoerfer
Leslie Driggs

In Memory of William Nargan
Susanne T. Hanely
Mildred Hanson

In Memory of John Shirrell
Jessen Funeral Home
Sue E. Shirrell

In Memory of Janet Willey
Sandra Carpenter
Ginger Kamps
Brenda Lancaster
Katherine Neeley
Leota Nihart
Robert Schwalm
June Ware
Linda White

Public Policy Update

By Miriam O'Day

At press time we continue to wait for Congress to move forward on a Medicare prescription drug benefit and funding for medical research. There is however, some good news to share with you.

Medicare Hospital Outpatient Reimbursement

On the cover of this issue of *Alpha-1 News* the spectacular Medicare victory is announced. I want to extend my personal thanks to all who responded to the Association Action Alert by communicating your concerns to CMS Administrator Scully and members of Congress. You make a difference.

Chronic Obstructive Pulmonary Disease (COPD) and Alpha-1 Advocacy Day and Congressional Briefing

The first national COPD conference is being held in Arlington, VA November 14-15, 2003. This groundbreaking conference presented by the US COPD Coalition will bring together leaders in COPD management, education, research and patient advocacy to develop strategies to shape the future agenda for effective action against COPD in the US. As the leading genetic risk factor for COPD, Alpha-1 representatives will play an important role in this conference. On the

Thursday prior to the start of the Conference Alpha-1 will partner with COPD advocates to hold a Congressional Briefing. Our meeting space in the US Capitol is sponsored by Congressman Cliff Stearns (R-FL) co-sponsor of the National COPD Awareness Resolution. The community will have the opportunity to bring attention to the health policy needs of individuals living with chronic lung disease with congressional health policy staffers. I want to remind you that you don't have to be in Washington, DC to help us raise awareness about Alpha-1 and express your views to elected officials. Congressional recesses are a good time to meet with members while they are at home in the district.

Advocacy enhances Federal Investment in Rare Disease Research

Alpha-1 Advocacy Day in 2002 focused on gaining support for the Rare Diseases Act - HR 4013. This legislation was signed into law, in large part due to the hard work of our membership organization the National Organization for Rare Disorders (NORD). This bill codified the National Institutes of Health (NIH) Office of Rare Diseases, increased their annual budget and requested the establishment of rare disease clinical centers. NIH recently announced \$51 million in grant funding for seven Rare

Disease Clinical Research Centers. Following the NIH grant announcement Diane Dorman, NORD's Vice President for Public Policy stated "A dream has been realized for the rare disease community." How does the NIH announcement have an effect on Alpha-1? By increasing the federal investment in Alpha-1 research! One of the seven Clinical Research Centers announced by NIH will focus on Rare Lung Disease such as Alpha-1. The Association applauds the Alpha-1 Foundation and Dr. Bruce Trapnell who will head up the coordinating center (Children's Hospital, Cincinnati, Ohio) on obtaining this significant grant! Please see Dr. Trapnell's report on page (11).

Genetic Non-discrimination

The Senate recently passed a genetic non-discrimination bill leaving responsibility for moving this legislation forward in the hands of the House of Representatives. It is being reported that the House is not eager to move this bill to the floor for a vote and will instead conduct hearings to discuss the bill's provisions. It is important that federal protective legislation be enacted to ensure that genetic information is not used to discriminate against individuals in employment and health insurance.

Call your Representatives and let them know that you want action on S 1053 in the House. ■

What's in a Number?

Reprinted with permission from Alpha-1-To-One a quarterly magazine of the Alpha-1 Foundation

The Alpha community has petitioned the government to assign Alpha-1 a specific diagnostic code.

Does Alpha-1 deserve classification as a unique disorder? Or should medical statistics lump it together with other, vaguely related diseases?

Those questions underlie a proposal by the Alpha-1 Association and the Alpha-1 Foundation to assign a specific coding number for Alpha-1 under the International Classification of Diseases, 9th Edition, Clinical Modification (ICD-9-CM) system. Doctors, hospitals, and insurance companies use ICD-9-CM codes in patient files, charts, reimbursement forms, and other databases. For example, the National Center for Health Statistics—a data-collection division of the Centers for Disease Control—relies on ICD-9-CM codes when it crunches numbers to spot health trends and support public policy.

Currently, Alpha-1 shares a number with hereditary angioedema under the vague category, "Other and unspecified disorders of metabolism." In a joint effort, the Association and the Foundation want to change all that. They have petitioned the committee that oversees the official ICD-9-CM codes in the United States to give Alpha-1 its own number, under the category "Disorders of plasma protein metabolism."

There's more than scientific precision at stake. Basically, a unique number would give Alpha-1 an identity all its own in the broad health care community. It would make Alpha-1 "count" whenever medical statistics are compiled. It would improve the accuracy of Medicare, Medicaid, and private insurance forms. "We also see it as an opportunity to educate physicians," says Miriam O'Day, senior director of public policy for the Alpha-1 Association. "A successful petition would help raise the awareness of Alpha-1 and really impact the larger collection of data."

The Foundation and Association expect a ruling on their petition in early 2004. ■

Alpha-1 Timely Exhibit Raises Awareness at the American Academy of Family Physicians Scientific Assembly

By Cathey Horsak

Five-thousand plus doctors at The American Academy of Family Physicians Scientific Assembly gained a greater understanding of Alpha-1 due to efforts by both the Alpha-1 Association and the Alpha-1 Foundation, and their exhibit, which featured well-timed information. The conference, held from Oct 2-4 in New Orleans, closely followed The American Journal of Respiratory and Critical Care Medicine's publishing of the "Standards for Diagnosis and Management of Individuals with Alpha-1 Antitrypsin Deficiency." The condensed version, of 300-plus pages recommends Alpha-1 testing for patients presenting with asthma, COPD, chronic bronchitis, emphysema, or cryptogenic liver disease and was commissioned and reviewed by The American Thoracic Society (ATS) and the European Thoracic Society (ETS).

Carol Motsinger of the Alpha-1 Foundation created a poster announcing the ATS-ETS standards for our booth. It was a

showstopper. We had countless doctors stop to ask us about the new evidence based publication which was featured on the poster. Many doctors were aware of Alpha-1 Antitrypsin Deficiency, but said they had never diagnosed a patient with the disorder. We challenged them to find Alphas in their community by testing those patients presenting fixed airflow obstruction or patients of any age with unexplained liver disease. Almost every doctor allowed us to capture their contact information and/or they added our literature to their exhibit collection.

It is always gratifying to have the opportunity to create awareness about Alpha-1 in the medical community. Both Carol and I left this conference with a renewed sense of satisfaction and accomplishment.

To obtain a condensed version of the ATS/ERS Consensus document to share with your healthcare provider, please contact Carol Motsinger at 1-888-825-7421 ext 246. ■

Looking for Answers

Reprinted with permission from Alpha-1-To-One a Quarterly magazine of the Alpha-1 Foundation

In probing Alpha-1's effects on the lung, Foundation researchers ask questions relevant to all COPD patients.

They have difficulty breathing even on short walks. They wheeze or cough during the mildest exertion. And they may number as many as 32 million in the U.S. They are people with chronic obstructive pulmonary disease (COPD)—a broad term that includes emphysema, chronic bronchitis, and other obstructions in the lungs. Not all COPD sufferers are Alphas. But in adults, Alpha-1 often manifests itself first as COPD.

The Alpha-1 community is an excellent study group for COPD because Alpha-1 is easily diagnosed, sufferers are often younger and have less co-morbidities than other pulmonary diseases, and Alpha-1 has a rapid progression.

That's why the connection between Alpha-1 research and COPD research seems natural. In learning more about precisely how alpha-1 antitrypsin (AAT) deficiency attacks the lungs, scientists also learn more about COPD in its various forms. Of the 56 research grants funded by the Alpha-1 Foundation since 1998, 25 relate to COPD.

What are these researchers looking for? The questions fall into five general categories.

What role does AAT play in lung inflammation?

"Inflammation is a hot topic in Alpha-1 research, if you'll pardon that choice of words," says Dr. Bruce Trapnell of the University of Cincinnati School of Medicine, the Foundation's scientific director. For decades, scientists have blamed a protease-antiprotease imbalance for Alpha-1-related lung

inflammation. Too little protective antiprotease (AAT) allows the body's infection-fighting proteases to attack lung tissue itself. But some researchers wonder, is it that simple? Is something else at work—something that might inactivate AAT? "For example, in cystic fibrosis, which is the most inflammatory lung disease known, there is no detectable AAT activity," says Trapnell. "AAT is present. There's just no activity." Alpha-1 researchers investigating the anti-inflammatory role of AAT may also shed light on other forms of COPD.

What role do growth factors play?

Certain types of proteins known as "growth factors" attach themselves to cells and trigger the formation of blood vessels, which in turn supply the nutrients for healthy tissues. Without the growth factors, there are no blood vessels; without blood vessels, tissue dies. In the lung, researchers have focused on a particular growth factor, Veg F. Does this process of vascular cell death affect—or is it affected by—the protease-antiprotease imbalance? Or is it completely independent?

What role does abnormal AAT play?

Maybe this protease-antiprotease imbalance has nothing to do with inflammation at all. People with the Z allele not only produce low levels of AAT; the AAT that they do produce is abnormal. According to conventional medical wisdom, expression of this abnormal AAT primarily causes liver disease; lung disease is a secondary effect. But recent research indicates that abnormal AAT itself causes inflammation in the lung. Scientists would like to know why and how.

What role do macrophages play?

"For many years, everyone considered neutrophil elastase to be the bad actor in the lung," explains Trapnell. Neutrophils—or

Continued on page 15 . . .

Continued from page 14 . . .

white blood cells—patrol the body and fight infection by producing elastase. In Alphas, this elastase becomes what Trapnell calls “a double-edged sword.” It attacks not only invading cells, but also lung tissue lacking a protective AAT screen. The neutrophils on their seek-and-destroy mission aren’t the only cells that produce elastase, however. Macrophages—resident defenders in the lungs—do, too. Does their elastase also affect inflammation?

How significant is lung inflammation in the absence of infection?

“When you get pneumonia, for example, you get a ton of neutrophils in the lung to kill bacteria,” says Trapnell. “There so many, the lung looks like cheese.” In non-Alphas with

pneumonia, AAT protects the lung tissue from these massive armies of neutrophils. But in Alphas (who have limited AAT protection at best), the neutrophil armies may exacerbate a pre-existing, low-level inflammation. In other words, Alphas may suffer a spectrum of lung damage, from a low-level inflammation in the absence of infection to severe inflammation during a bout of pneumonia. Research is exploring this spectrum further and gauging its significance.

Science is still looking for answers about the exact mechanism of COPD in general and Alpha-1-related lung disease in particular. With its resources and expertise, however, the Foundation has contributed to taking the first steps—asking the right questions. ■

Aventis Behring Joins Forces With AlphaNet to Benefit Alpha-1 Community

Aventis Behring L.L.C. announced an agreement with AlphaNet Inc., to provide health management and support services to patients who rely on Zemaira™

(alpha1-proteinase inhibitor [human]). In July, Aventis Behring received approval for Zemaira™ from the U.S. Food and Drug Administration (FDA) for use as chronic augmentation and maintenance therapy in adults with alpha1-

proteinase inhibitor deficiency (Alpha-1.) Under the agreement, health management services provided by AlphaNet will include patient education, monthly telephone contact and support, insurance counseling and financial hardship referrals, access to a 24-hour toll-free “Help Line”, caregiver and family support programs. Zemaira™ patients will also be eligible to participate in AlphaNet’s Alpha-1 Disease Management and Prevention Program (ADMAPP), as well as AlphaNet’s IRB-approved clinical trials.

“As with any chronic illness, the management of Alpha-1 is complex and requires a multi-faceted approach. AlphaNet works with the Alpha-1

Community to help patients work to improve their general health and gain greater independence in the management of their disorder. Through our services, we aim to empower patients to take a more active role in their healthcare,”

says Robert

C. Barrett, Chief Executive Officer, AlphaNet Inc. “We are pleased that Aventis Behring recognizes the value of our services and look forward to working with them in an effort to benefit patients who rely on Zemaira™.”

“While Aventis Behring has invested significant time and resources into making Zemaira™ available as a treatment option to people with Alpha-1, we also believe in the importance of

providing support beyond safe and effective drug therapies,” comments Joseph Pugliese, Vice President and General Manager, Aventis Behring North America. “By working together, I am confident that AlphaNet and Aventis Behring will make a considerable impact in the quality of life for patients with Alpha-1 and their families.” ■



Alpha-1 representatives tour the Aventis A1PI Kankakee, IL manufacturing facility.



Aventis A1PI Kankakee staff Tim Moore, Wally Casey, Craig Shelaskey and Dominic Randazzo receive recognition from our group. The plaque reads: “With the deepest appreciation and respect we recognize your dedication and commitment to production of new therapies for the treatment of Alpha-1”

Alpha-1 Association Support Groups

NAME	STATE	PHONE	MEETING FREQUENCY	NAME	STATE	PHONE	FREQUENCY
NELL REDMILL	AL	205.822-6339	QUARTERLY	DEBORAH HANSON	NH	603.532-4940	3RD SAT, E-O-MONTH
BARBEE BENNINGTON	AZ	480.946-5257	2ND WED. OCT-APR.	JOE REIDY	NJ	201.444-7839	QUARTERLY, 1ST SAT
BOBBIE STAFFORD	CA	352.466-3947	NEED TIME	STACY MILLER	NC	919.544-7532	3RD SAT, E-O- MONTH
GENE JOHNSON	CA	510.581-6196	3RDSAT, MONTHLY	MARTHA GIROLAMI	NC	919.362-5759	SAME
KARALEE KARP	CA	559.673-3948	QUARTERLY	DENNIS POLLACK	OK	580.510-9286	4TH SAT. MONTHLY
BILL DAWKINS	CO	818.362-5018	QUARTERLY	FRANK MORRIS	OK	405.366-7542	PANNICULITIS-NATL.
KRIS VAYAN	CT	720.870-4204	MONTHLY	DR. BARKER	OR	503.494-7680	2ND TUES. MONTHLY
DEBORAH DELOACH	FL	352.466-3947	EVERY OTHER MONTH	MARLENE BUCHANAN	PA	610.754-6302	WED,E-O MONTH
BOB CAMPBELL	FL	888.399-0443	EVERY OTHER MONTH	CATHY GOULD	PA	724.464-0487	EVERY OTHER MONTH
SUE LANDERS	FL	727.588-0320	3RD SAT., E-O-MONTH	MARVIN SINEATH	SC	843.762-0967	SEMI-ANNUALLY
LEN GEIGER	GA	770.503-1292	SEMI-ANNUALLY	JIM HOSLEY	NY	607.734-1148	QUARTERLY, SUNDAY
FRED HUMPHREYS	IL	309.444-5423	QUARTERLY	STEVE WOLBRINK	SD	605.996-8801	1ST SAT, MONTHLY
DIANE ANGELL	ID	208.345-6958	5 X'S PER YEAR	WILLA LONG	TN	865.995-2986	ANNUALLY
LIN NEAL	KS	316.755-1215	E-O-MONTH, 1ST SAT.	JIM GOERING	TX	972.241-4487	3RD SAT, MONTHLY
ROSEMARY BYBEE	KY	502.366-6181	3RD WED. QUARTERLY	DAVE COURTNEY	TX	806.291-0146	QUARTERLY
DIANA PATTERSON	LA	866.556-6622	SEMI-ANNUALLY	CATHEY HORSACK	TX	877.346-3212	QUARTERLY
FRED WALSH	MA	800.791-3194	EVERY OTHER MONTH	LAMAR JOHNSTON	TX	512.261-9914	QUARTERLY
JOYCE FINCH	MI	888.225-9012	SEMI-ANNUALLY	JACK FOSTER	TX	281.980-4286	3RD SAT, MONTHLY
STEWART WINDHAM	MS	662.680-3293	QUARTERLY	VICKI CAMERON	VT	888.526-9077	NEW-2003
BRETT HOSKINS	ME	207.512-3132	QUARTERLY	PAM VANSOY	VA	540.439-0686	QUARTERLY
DICK BUEKER	MO	816.942-6277	QUARTERLY	LINDA RICHARDSON	VA	540.380-3022	SAT., QUARTERLY
JERRY MILLER	MO	417.725-1790	QUARTERLY	GAYLE ALLISON	WV	877.982-5742	QUARTERLY
JERRY TREICHEL	NV	702.645-3035	EVERY OTHER MONTH	KATHIE SIVESIND	WA	866.244-1026	QUARTERLY
				NOREEN JAMES	WI	920.208-6408	2ND THURS, MONTHLY



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