

What Alpha-1 Research Studies Are Currently Enrolling?

By: Robert A. Sandhaus, MD, PhD, FCCP, AlphaNet Medical Director

People with Alpha-1 Antitrypsin Deficiency, or Alpha-1 for short, often ask about research studies that they can volunteer to join. There are a number of studies currently looking for participants and several on the near horizon that might be of interest. Remember that each study has a list of criteria that a prospective participant needs to meet (called inclusion and exclusion criteria) and these differ from study to study. In addition, many of these studies provide reimbursement for your time and effort, and some will cover your travel expenses as well. The best way to learn about studies that you might qualify to participate in is to join the Alpha-1 Foundation's Research Registry. They will notify you of trials that are currently enrolling individuals with Alpha-1. To enroll, simply go to the website www.alphaoneregistry.org. Another great resource is the website www.clinicaltrials.gov.

Please remember that participation in any research study is voluntary and you can choose not to participate at any time – even after you've agreed to join a study. In addition, the descriptions here are only for introductory purposes. To learn about any particular study, you'll need to contact the study site, which can give you the latest information and answer any questions.

Liver Studies

A major research study sponsored by the Alpha-1 Foundation is called the "Alpha-1 Antitrypsin Deficiency Adult Clinical and Genetic Linkage Study." It's designed to learn about the natural history of liver disease in Alpha-1 and it's open to individuals with ZZ Alpha-1. Family members with ZZ Alpha-1 are encouraged to enroll so that the family influences on liver risks can be determined. This is a long-term observational study (no new therapies are being tested) to help researchers learn more about liver disease. Much information will be collected over the course of the study and you'll be asked to undergo two liver biopsies – one at the start of the study and one five years later. You must be an adult to join this study and, interestingly, you can't have cirrhosis. The leader of this study is Dr. Jeff Teckman at St. Louis University. In addition to this site, enrollment is also taking place at the University of California San Diego and Boston University. To learn more about this study, please contact Rosemary Nagy, the Study Coordinator at St. Louis University at (314) 977-9350 or rnagy@SLU.edu.

A similar study is being performed at the University of Florida by Drs. Mark Brantly and Virginia Clark. This study follows adults with and without evidence of liver disease for about 3 years. One liver biopsy is included in this trial. Dr. Brantly is hoping to enroll people who can't make it to one of the centers in Dr. Teckman's study described above. Please contact Tracie Kurtz at (866)229-6313 or tkurtz@UFL.edu for more information.

Dr. David Perlmutter at the University of Pittsburgh Children's Hospital of Pittsburgh is evaluating a new treatment for severe liver disease in Alpha-1. This study is looking to enroll patients who are ZZ and between 14 and 75 years of age who have significant liver disease with portal hypertension. The drug carbamazepine is being evaluated to see if it can prevent or reverse liver scarring in Alpha-1. For more information you can visit the website www.chp.edu/at_study or contact the study at (855) 428-8881 or liverstudy@chp.edu.

Lung Studies

An interesting study that not many Alphas know about is the PELICAN trial. PELICAN stands for "Peer-led O2 inhome for patients and caregivers" and it's aimed at improving the health and wellbeing of people with lung disease due to Alpha-1 and other causes. It is sponsored by PCORI, the Patient Centered Outcomes Research Institute, which is funded through the Affordable Care Act. Any adult Alpha-1 patient who has been prescribed oxygen 24 hours a day can participate. No travel is necessary; you'll just participate by telephone using written materials you can read at home. If you're interested in learning more, call the PELICAN study staff at (844) 627-5587.

The SPARTA study, sponsored by Grifols, is enrolling subjects with Alpha-1 and emphysema in order to learn whether a higher dose of intravenous augmentation therapy might work better than the usual dose. People with Alpha-1 who join this study will be randomly assigned to receive either the usual dose of augmentation therapy every week, a double dose of augmentation therapy every week or a placebo every week by intravenous infusion. Participants will stay on their assigned drug for 3 years. There are currently 44 centers around the world that are enrolling subjects in this study, including 10 in the U.S. To learn more about this study or to consider enrolling, contact Emberly Dumayas at emberly.dumayas@grifols.com or visit clinicaltrials.gov.

Dr. Michael Campos at the University of Miami is also studying the effects of a double dose of augmentation therapy on the lungs. In his study, participants will receive augmentation therapy at a standard dose for one month, then receive one month of double the standard dose, and then an additional month of a standard dose again. A bronchoscopy will be performed at the end of each of the three dosing months. Blood testing will also be performed. If you'd like to learn more, contact Dr. Campos at (305) 243-3045 or mcampos@med.miami.edu.

Grifols is sponsoring a study to evaluate a liquid formulation of their augmentation therapy product. People with Alpha-1 who join this study will receive 8 weeks of the usual powdered formulation of augmentation therapy and 8 weeks of the liquid formulation of this same augmentation therapy. Which order these two versions of augmentation therapy are given is randomly assigned at the start of the study. There are five study sites around the U.S. To learn more, contact Renu Jain at renu.jain@grifols.com or go to clinicaltrials.gov.

The EMPROVE study, sponsored by Spiration, is intended to evaluate bronchoscopically-placed valves in the airways of patients with Alpha-1 lung disease. This is intended to be a non-surgical lung volume reduction procedure. All Alpha-1 patients who participate in the study will receive the valve treatment (in other populations being studied some subjects are randomized to receive a "sham" bronchoscopy with no valves being placed). For more information, visit www.emphysematrial.com. Please note that some of their literature still says that Alpha-1 patients are excluded, but this is not true.

Studies in the Near Future

There are a number of trials that are expected to start enrollment in the near future in the U.S. Aplylam is moving toward the start of a study designed to turn off the production of the Z protein in the liver of individuals with Alpha-1. Baxalta (formerly Baxter) is expected to look at some formulation questions in their liquid augmentation therapy product. To find out what new trials are starting enrollment, please visit the Alpha-1 Foundation website frequently (www.alpha1.org), stop by clinicaltrials.gov or ask your AlphaNet Coordinator.

Some brochures for studies that are discussed here can be found on the AlphaNet website, www.alphanet.org.

Community Calendars

Alpha-1 Foundation Education Days

July 24-26 – National Education Conference
Garden Grove, CA

September 19
Cleveland, OH

November 7
Orlando, FL

To find out more about conferences and support groups, visit the Alpha-1 Foundation website at www.alpha1.org or call toll-free: (888) 825-7421, ext. 331.

Alpha-1 Foundation Events Calendar

August 9 – Step Forward for Alpha-1 Walk
Littleton, CO

September 25 – Escape to the Cape Bike Trek
Cape Cod, MA

To find out more about these or other events in your area, log on to www.alpha1.org or call toll-free: (888) 825-7421, ext. 233.

 3300 Ponce de Leon Boulevard
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For Your Information...

Your AlphaNet Coordinator is a great resource, whether you are a newly diagnosed Alpha or a long-time AlphaNet subscriber. Take advantage of his/her expertise.

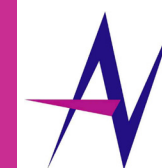
Check out the "Questions to Ask Your Coordinator" pamphlet and other FAQ's at www.alphanet.org.

Story Ideas?

Do you have an interesting story that you think would make a great article for an upcoming issue of *The AlphaNetter*?

If so, please submit any ideas, comments and/or suggestions to Christine Lanser at clanser@alphanet.org.

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The AlphaNetter

AlphaNet Celebrates 20 Years of "Alphas Serving Alphas"

AlphaNet has reached a major milestone: Twenty Years of *Alphas Serving Alphas*. Since its founding in 1995, AlphaNet has been at the forefront of providing health management services to the Alpha-1 community. Over the past twenty years, AlphaNet has grown from an organization with just a handful of AlphaNet Coordinators to now, in 2015, an organization with 45 Coordinators, who are located in 32 states, and who serve over 5,000 Alphas in the United States and Canada. For the Coordinators who have worked for AlphaNet since its inception, this 20th anniversary bears special significance, as working for AlphaNet has provided them with the opportunity to serve their fellow Alphas while still being employed. Fred Walsh, who was among the first AlphaNet Coordinators, states that, "Being an AlphaNet Coordinator these past 20 years has allowed me to stay employed and feel empowered."

One of the most significant achievements that AlphaNet has accomplished in the past year is that this organization now has partnerships with all three pharmaceutical companies that manufacture Alpha-1-related therapies. This means that AlphaNet can now work with any Alpha, regardless of their augmentation therapy, and even if they are not infusing therapy.

As part of our mission, AlphaNet is committed to supporting research for a cure, as well as providing funding for educational and clinical research programs for the Alpha-1 community. Since its inception, AlphaNet's cumulative contributions to the Alpha-1 Foundation have totaled \$45 million. "We continually strive to find new ways to increase AlphaNet's contributions to the Alpha-1 Foundation," CEO Robert C. Barrett said.

Ultimately, we look forward to fulfilling our motto of *Alphas Serving Alphas* for the next twenty years to come.



AlphaNet's headquarters at 3300 Ponce de Leon Boulevard, in Coral Gables, Florida

Did You Know...

A single cigarette destroys all the alpha-1 antitrypsin in the lungs. Cigarette smokers inhale over 400 toxins and 43 known carcinogens every time they puff. As smoke enters the respiratory tree and lungs, it causes irritation and triggers inflammation. This inflammation causes the body's defenses to send white blood cells to the area. While performing their normal function, the white blood cells release a powerful enzyme, known as neutrophil elastase. Neutrophil elastase is destructive to unprotected lung tissue.

You probably know, alpha-1 antitrypsin (AAT) is the protein in our bodies that provides protection to the lungs by neutralizing this powerful enzyme. We know the chemicals created from burning tobacco destroy alpha-1 antitrypsin. In fact, a single cigarette destroys all the alpha-1 antitrypsin in the lungs. For Alphas, the exposure to cigarette smoke accelerates damage to the lungs, and symptoms may develop 30 years ahead of someone with "normal" lungs. For information on smoking cessation, please contact your AlphaNet Coordinator.



Clinical Corner

By: Teresa Kitchen, BSN, RN, AlphaNet Clinical Nurse Manager

Essential Dietary Guidelines for Alpha-1 Patients

This excerpt was taken from the AlphaNet Big Fat Reference Guide (BFRG).

The list of nutrients needed by our bodies is long. However, we will discuss only a few that are especially significant to those with lung disease. Also, it is important to note that some medicines for lung disease react with some nutrients. You should consult with your doctor or dietitian about possible interactions with the specific medicines you are taking.

Protein

Protein is important because it plays an essential role in protecting the body. It produces antibodies to fight infection.

*Add skim milk powder to oatmeal, cereal, eggs, soups and ground meat dishes. This will add extra protein and calcium to your diet.

*Blend finely chopped hardboiled eggs or egg substitute into a sauce, gravy or soup.

Fluids

Drinking enough fluids is essential for the thinning and clearance of your pulmonary secretions. Also, supplemental oxygen therapy may dry your mucus membranes and cause irritation. Fluids keep you hydrated.

Sodium

An excess amount of sodium chloride, a common table salt, can cause fluid retention (also called edema), increased blood pressure and shortness of breath. If your doctor asks you to reduce your sodium, follow these guidelines:

*Do not add salt while preparing food or to food served at the table.

*Read labels and ingredients on all foods. Avoid those that contain high sodium levels.

Calcium

Calcium works with magnesium to regulate lung function, muscle contraction and blood clotting. It also plays an important role in the structural strength of bones, the transport of nerve impulses and the work of the immune system.

*Osteoporosis, or "porous bones," affects more than 25 million women and six million men in the U.S. It is very common in people with COPD and even worse if you are taking corticosteroids. You should talk with your doctor about your risks.

*Osteoporosis can be prevented by the intake of daily dairy products or food high in calcium, as well as weight bearing exercise (walking, running or weight lifting).



Alpha Cycles His Way to Improved Health

Alpha Ben Miles from Winfield, Alabama has found that his passion for cycling has led him to better overall health.

Miles, who has worked in an underground coal mine for 31 years, began experiencing difficulties with his lungs a few years ago.

"I had been diagnosed with bronchitis and pneumonia on multiple occasions within a short time frame. This led to my doctor deciding he would run more tests." As a result of this additional testing, Miles was diagnosed with Alpha-1 in October 2012.

Before being diagnosed, Miles picked up the hobby of occasionally riding his bicycle for exercise, and he would ride up to ten miles each time, give or take. Once he received his Alpha-1 diagnosis, Miles' doctor emphasized that he needed to find some form of exercise that he enjoyed and could do on a regular basis. Since he had already taken up cycling on occasion and had an interest in it, he began to pursue it more seriously.

Miles started his new workout schedule with a low mileage ride every morning after he returned home from working the night shift at the coal mine. From there, he increased his riding distance frequently and explains that his competitive drive helped him accomplish this goal relatively quickly. "I am a competitive person and want to accomplish all that I possibly can. With that being said, I added distance to my ride every time I reached the point where I felt content."

Miles was clear in what he wanted to achieve. He notes, "My goal was to ride my bike to my lake house, which is 51.5 miles away. I accomplished this in early November 2014. Now, I ride around 38 miles a day."

Cycling has helped Miles in more ways than he can explain. "Some of the benefits I have experienced include increased energy levels, improved lung strength and weight loss. I have lost 55 pounds since my Alpha-1 diagnosis." Furthermore, Miles states that the asthma symptoms he had experienced since birth became "nonexistent" when he started cycling regularly. He also increased his lung capacity as a result of his cycling. He points out that, "Anyone with Alpha-1 knows that it is difficult to increase your lung capacity but, with exercise, it can happen."

"My story is definitely a work in progress and could not be possible without the Lord and my family being there for me every step of the way. My AlphaNet Coordinator, Elizabeth Eich, discovered during one of our conversations how riding has helped me and it inspired her to begin riding. It also encouraged her to help spread the word about the benefits of exercise."

"I am proud of the milestones that I have accomplished. I do hope that it inspires at least one more person to join me in a movement to improve our health and not let this condition define who we are and what we can do," Miles said.

Miles' perseverance and devotion to cycling is certainly something that we can all aspire to!



AlphaNet Welcomes New Coordinators



Michael Ray joins AlphaNet as a Baxter Coordinator after a 30 year career working at an international, multi-faceted energy corporation and after working as a consultant for an engineering group. He and his wife Terry reside in Kansas City, Missouri.

Ray has always led a healthy lifestyle with little or no effects from Alpha-1 despite being a ZZ. His brother, on the other hand, was not as fortunate. Being a heavy smoker, he began having pulmonary issues at the age of 50.

While preparing for a double lung transplant, Ray's brother was tested and diagnosed with Alpha-1. Even though Ray had no issues, it was recommended that he be tested. His Alpha-1 test kit revealed that he was a ZZ (same as his brother).

In July 2014, Ray became eligible for Medicare. With a little help from AlphaNet and the Alpha-1 Foundation, he was able to find an Alpha-1 specialist and began augmentation therapy in September 2014. He then joined a local Alpha-1 support group in Kansas City.



Mike Hixon is excited to join AlphaNet as a Zemaira Coordinator. Hixon lives in Terre Haute, Indiana with his wife, Vicky, and their cat Ms. Kitty. He has two adult children: a daughter who lives in Greenville, Michigan, a son in Terre Haute, Indiana and three grandchildren.

Hixon was diagnosed with Alpha-1 Antitrypsin Deficiency through family testing. In 1995, his younger brother was diagnosed at the age of 40 as a ZZ Alpha. Hixon was promptly tested and also found to be a ZZ, but had no symptoms with a normal pulmonary function test. Unfortunately, his brother's condition worsened and he passed away in 2002.

In 2008, Hixon's wife was diagnosed as an MZ Alpha, their daughter was found to be a ZZ and their son was diagnosed as an MZ. Hixon knows firsthand the true necessity of Alpha-1 family testing and living a healthy lifestyle, which he emphasizes involves not smoking and keeping as active as possible.

For Your Information...

Don't forget to stop by the AlphaNet and REACH booths at the National Education Conference in Garden Grove, California.

We look forward to celebrating 20 years of *Alphas Serving Alphas*, as we reflect on all that we have accomplished.



AlphaNet Welcomes You to AlphaVille!

Get the most comprehensive Alpha-1 information available by accessing our *Big Fat Reference Guide* and *Skinny Little Reference Guide* collection.

Visit www.alphanet.org and click on the BFRG link to register.

Kamada Inhaled Study

Alphas seem very interested in whether an inhaled version of augmentation therapy might be available soon. One way to help evaluate inhaled therapy is to participate in a study being performed at two sites in the U.S. This study is being sponsored by Kamada and is a double-blind, placebo controlled trial. Patients already receiving intravenous augmentation therapy will have to stop this treatment for 8 weeks prior to the study and during the entire time of participation in the study. If you decide to join this study, you will receive a bronchoscopy at the beginning and again after 12 weeks on either placebo inhalations, or one of two different doses of inhaled augmentation therapy. At the end of the blinded portion of the study, participants may elect to continue into a 12-week open label portion during which everyone will receive the highest dose inhaled alpha-1 antitrypsin. To learn more about this study, contact Joanna Nolte in Gainesville, FL at (352)273-7225 or noltej@ufl.edu; or contact Dr. James Stocks in Tyler, TX at james.stocks@uthct.edu.

The PELICAN Study



Frequently asked questions

What is PELICAN?

Peer-led O₂ inhome for patients and caregivers (PELICAN) is a research program that aims to improve the health and wellbeing of people with COPD and their caregivers.

Who can participate?

PELICAN may be for you if...

- You are an adult (over 18 years old)
- You have COPD and are using oxygen 24hrs/day

We hope you join PELICAN!

What procedures are involved in PELICAN?

The study will take place at your home:

- You will be given written material, which you can read at home, at your own pace
- Phone calls will be made to your home
- You will be compensated for your participation.

Interested?

- From your home, call toll-free a PELICAN study staff member at **(844) 627-5587**.
Hours: M-F, 7:30 AM – 6:30 PM (Central Time)



Developed by  **COPD FOUNDATION** and other partners.

PELICAN Flyer, v. 2.3, 3/3/15
UIC IRB # 2014-0385

