Registry Alphas volunteered for 75 studies

WELCOME to this edition of the Alpha-1 Foundation Research Registry newsletter. For new members, we plan on two editions of the newsletter each year to let you know about research happening through the Registry. In this edition, we will tell you about the Alpha-1 Foundation's continuing review of the Registry. I will use the data that we generated for that effort to let you know how important your participation has been for advances in Alpha-1.

The Alpha-1 Foundation Research Registry began in 1996, at the end of a registry sponsored by the National Institutes of Health. Registry enrollment has continued to grow slowly and steadily each year since. The Registry spent much of the last year making sure that we have current addresses for everyone and accurate information on vital status from public records. We were able to generate the graph below to show the continued growth of the Registry that likely corresponds to the rate of diagnosis of new Alpha-1 individuals in the United States.

We also generated a category of "discontinued members" for those who have moved beyond where we can find you. If you are not getting this newsletter from the mail and think that you are a Registry member, please contact us at (1-877-886-2383) or re-enroll on our website (www.alphaoeregistry.org) with electronic consent.

The most amazing statistic that we were able to generate is that we have assisted in 75 research studies over the 14 years of our existence. Some of these are limited to small geographic invitations to those of you who live close to an interested medical center. Others have been limited to specific genotypes of Alpha-1.

While not all of these have resulted in new findings or publications, every effort that we take is important in its own way to better understand Alpha-1.

In this version of the newsletter, you will find some of the recommendations made during continuing evaluation of the Registry. We are working to change gradually to a fully electronic interface, so anyone comfortable with computers can give us information entirely online — while not leaving behind those members who are not fully computer savvy. Most importantly, we will continue to stress the importance of confidentiality of the information that you have placed in our care since the formation of the Registry. The national debate on Registry usefulness continues with a report from a National Institutes of Health conference earlier this year. We also share with you an interesting article on muscle function in COPD, a topic that needs a better understanding. Lastly, gene transfer studies into muscle are continuing in Massachusetts, Cincinnati, and Denver. Please read about these research opportunities. Thank you for your continued participation in the Registry.

Please call us at 1-877-886-2383 or email us at alpahone@musc.edu with your questions or concerns.

Charlie Strange, MD
Director, Alpha-1 Foundation
Research Registry
Professor of Pulmonary and Critical Care Medicine
Medical University of South Carolina

Cumulative Enrollment

[Graph showing cumulative enrollment from 1996 to 2010]
Thank you, all of the Registry members who contacted me after my article in the Fall 2009 newsletter about incorporating more electronic communication into the Registry.

At this point, we feel comfortable in moving forward to incorporate more electronic communication when it will provide a benefit to Registry members.

One of our most requested features has been improved and available since the beginning of the year – the online Registry application. Now, Alphas interested in joining the Registry can do so online through the Registry's website at www.alphaoneregistry.org.

Simply follow the link for "Enroll now" on our Home page. The Institutional Review Board at the Medical University of South Carolina (MUSC) now allows us to accept electronic signatures, so it is no longer necessary to mail us a hard copy of your signature when you enroll online. Of course, you may still request a paper application from us, or download a PDF copy to print out.

All of the responses I have received so far are overwhelmingly positive:

...I am absolutely OK with your emails... the risk of electronic communication getting "out there" is a risk I take seriously, and I was glad to read in your recent newsletter that your research staff takes it seriously, too...

...I would prefer to get updates by email and receive links to online surveys that way as well. I am much quicker to respond to items sitting in my email inbox than I am to the stack of letters and bills that collect near my door. I also feel that I would be significantly more likely to participate in surveys if they could be accessed through an interactive web page...

...I just finished reading your article in the Fall 2009 Research Registry Update regarding receiving email from the Registry. I feel very comfortable with it. I also like the fact that saving on postage would leave more money for research...

...It is more convenient and faster/cheaper for all concerned. I thank and commend you all for the security measures you're now taking to protect our info...
We reworked our online Registry application to take advantage of a new computer program called REDCap, short for Research Electronic Data Capture.

Vanderbilt University initiated the REDCap program, which currently includes 110 medical universities, hospitals, and other medical research groups. REDCap consortium partners include Harvard, Stanford, and Yale, the Mayo and Cleveland clinics, and many others. REDCap offers secure, web-based applications designed to support data capture for research studies. Here at MUSC, the Alpha-1 Registry is one of 30 active studies using REDCap. We store all data collected through REDCap on secure servers.

Because REDCap is such an easy system to use, both for Registry members at home on their own computers and for us at the Registry office, we will expand its use to future Registry surveys.

Registry members who provide us with their email address will receive a survey invitation in their email with a link to the survey. Note that only members who are interested in completing surveys online will receive this email. We will still provide paper surveys for everyone else. We expect the electronic surveys to provide a great cost saving for the Registry.

As we make the gradual change from US mail to email, it is important that we have current email addresses from those members interested in participating in future surveys electronically.

Registry members can update this information with a quick toll-free call to us at 1-877-886-2383, or an email to alphaone@musc.edu.

We plan to incorporate REDCap into another Alpha-1 Foundation-sponsored study here at MUSC – the Alpha-1 Coded Testing study (the ACT study). This study provides free, confidential testing for Alpha-1 through an at-home test kit.

Currently, people who are interested in participating in the ACT study must call or email us to request a kit. We mail them a test kit that includes everything they need to do the finger stick test, along with a research questionnaire and consent form.

If the person forgets to include the research questionnaire or consent form when they return their test kit to us, we cannot send their sample to the lab to be tested. We hold these kits until we receive whatever information is missing, then send them to the lab to be tested.

We plan to offer participants the option of ordering a test kit online. Using REDCap for this will allow participants to complete their consent form and research questionnaire online, ensuring that we have both of these critical items before someone receives the test kit in the mail. This should reduce waste and save both time and money. We will still send test results to participants through regular postal mail, to ensure confidentiality.

We hope these initiatives will make it easier for you to use the Registry. We believe that they will save all of us some time and provide some cost savings for the Registry. As always, we welcome your comments and want to know your concerns. Please call us toll-free at 1-877-886-2383, or send us an email at alphaone@musc.edu.

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**Summer 2010 Research Registry Update Contents**

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You may contact the Alpha-1 Foundation Research Registry staff by email, at alphaone@musc.edu for additional assistance in locating resources related to Alpha-1 research, to obtain information about current research activities, to participate in the Research Network or Registry, or to receive Foundation publications.
Are your muscles wasting? One-lim

By Mark Olfert, PhD, RRT
West Virginia University School of Medicine
Center for Cardiovascular and Respiratory Sciences

Chronic obstructive pulmonary disease (COPD) describes lung disease resulting from airways narrowing, which happens most commonly with emphysema or chronic bronchitis.

Breathing difficulty can vary widely from mild to severe. Daily physical activity (such as walking up stairs or around the block) and higher levels of exercise are often difficult for patients with COPD. For some, exercise is made even harder due to other diseases (co-morbidities) often associated with COPD, such as heart disease, sleep-disordered breathing, and loss of skeletal muscle mass (known as cachexia). All of these can contribute to exercise intolerance, leading to reduced quality of life and a shorter life.

Exercise is important in maintaining health. Large studies have shown that low exercise capacity (with or without cardiopulmonary disease) is a stronger predictor of mortality than other established risk factors.

Exercise capacity is a strong predictor of patient health status in alpha-1 antitrypsin-deficient COPD patients. While it is clear that abnormal lung function and low oxygen levels are at the root of the problem limiting exercise capacity in COPD, it is interesting to note that lung function (i.e., the breathing test called FEV1) is only weakly associated with exercise capacity. This finding suggests there are other causes responsible for limiting exercise capacity in patients with COPD.

Muscle function is abnormal in COPD. The muscle abnormalities have been studied and described by numerous researchers. This work has resulted in a review of what is known by the American Thoracic Society and European Respiratory Society. Among the abnormalities seen are loss of peripheral muscle strength, decreased muscle endurance, changes in muscle fiber size and composition, and reduced numbers of small blood vessels that go to the muscles.

Any of these abnormalities could be expected to contribute to impaired exercise capacity. However, what still remains unclear is whether these abnormalities are due to muscle deconditioning (resulting from sitting around too much) versus abnormality of the muscle that results from some other aspect of COPD (low oxygen levels or inflammation).

Not all patients with COPD will develop skeletal muscle problems. This may be due, at least in part, to the wide spectrum in severity of lung disease among patients with COPD. But even for those who develop skeletal muscle dysfunction,
Exercise is easier, builds strength

It still remains unclear,

1) what causes this dysfunction, and
2) which patients are most susceptible and
why.

In some cases, the origin of muscle
dysfunction is easier to understand.
For example, long-term use of oral
corticosteroids (prednisone), or drugs that
suppress the immune system after lung
transplantation (cyclosporine A) contribute to
skeletal muscle weakness. But, for many
patients not on these drugs, the origin of skele-
tal muscle dysfunction remains uncertain.

Current research is focused on a number of
blood chemicals that can damage muscles in
COPD. These include inflammatory mediators,
oxidative stressors (excess production of free
radical molecules), reductions in circulating
hormones (such as testosterone or insulin
growth-like factor-1) and blood vessel growth
factors (such as vascular endothelial growth
factor).

But before medical scientists, clinicians,
and COPD patients can understand the
mechanism(s), much less the best treatment
strategies to employ, we must be able to
separate out the effects of deconditioning (lack of exercise)
versus direct injuries to skeletal muscles. While much work
has been done, future studies must be more rigorous in
comparing healthy inactive subjects against those with COPD.

Get stronger, one leg at a time: The good news for
those with COPD is that regular physical activity, even when
lung disease is advanced, can still improve skeletal muscle
function and can minimize loss of exercise capacity. While it
is true that overall exercise capacity will be reduced in COPD
(due to irreversible damage in the lungs), even low levels of
regular exercise can be beneficial.

Furthermore, there is hope that performing “small muscle
mass” exercise — that is, exercising only one leg (or arm)
at-a-time — may be particularly helpful for improving and/or
maintaining muscle function in patients limited by heart or
lung disease.

While whole-body exercise (such as running, cycling,
etc.) often results in shortness of breath; exercising a smaller
mass of muscle (just one leg) results in much lower demand
on the heart or lungs. Therefore, when only exercising one
leg at a time, a person with COPD is able to perform maximal,
or near maximal, aerobic exercise (at the level of the muscle)
and sustain muscle work to its true metabolic capacity.

Although we still need training studies using this kind of
exercise in patients with COPD to determine the best exercise
regimes to adopt, this form of exercise holds the promise to
improve some muscle problems. Unfortunately, most home
exercise machines are not well suited for single limb exercise.

Nevertheless, the message is clear: Patients with COPD
can help preserve and/or maintain their skeletal muscle
function, once they chose to adopt and engage in some level
of regular physical activity. Future studies aimed at
determining the type of exercise and how much is needed
will be useful in providing specific exercise guidelines for COPD.

Lastly, I want to thank all of the participants in our
COPD research projects on muscles. Without your
participation, we would not be making progress in this
important aspect of COPD care.

Literature cited:
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risk factors associated with myocardial infarction in 52 countries (the
2. Myers J, Prakash M, Froelicher V, Do D, Parthington S, Atwood JE.
Exercise Capacity and Mortality among Men Referred for Exercise Testing.
3. Dowson IJ, Newall C, Guest PJ, Hill SL, Stockley RA. Exercise capac-
ity predicts health status in alpha(1)-antitrypsin deficiency. Am J Respir
4. Skeletal Muscle Dysfunction in Chronic Obstructive Pulmonary
Disease. A Statement of the American Thoracic Society and European
Congratulations! We at the Alpha-1 Foundation Research Registry thank all of our members for joining the Registry and for participating in the research trials we offer from time to time.

As many of you know, the Registry is housed at the Medical University of South Carolina (MUSC). MUSC has recently been awarded a large National Institutes of Health research grant to improve research infrastructure. This grant, called the Clinical and Translational Science Award, has also been given to 40 other large academic medical centers to enhance research.

At MUSC, the award has allowed us to create the SUCCESS center. This center provides support to make sure that research is done well and that young researchers will be trained to be the best at what they do.

One of the important parts of good research is good communication with research participants. With this thought in mind, everyone who has recently participated in research through MUSC has been awarded the official title of "MUSC Hero." This slogan is all around Charleston on billboards and in advertisements for the medical center. Since most of you do not live in Charleston, we want you to know you are being celebrated by the university.

Everyone who has recently participated in research through MUSC has been awarded the official title of "MUSC Hero".

There are other parts of the Hero initiative.

For those of you who live in South Carolina or the surrounding states, you may be interested in looking at a partial list of research trials available at MUSC.

You can find this extensive list by going to the MUSC Hero website, www.muschero.org, and clicking on Find a Study. There you can navigate to just about any disease (The Alpha-1 Foundation Registry and Alpha-1 Coded Testing study are under "Lung"). These pages will provide the name of the principal investigator, a description of the disease, the study qualifications, and a contact person for the study.

www.researchmatch.org – created to assist people who would like to participate in research studies.

National research volunteer registry – Another valuable resource on the MUSC Hero website is a new national volunteer registry called ResearchMatch. This registry was created to assist those who would like to participate in research studies.

A common research problem is that many studies are forced to end because they do not have an adequate number of participants enrolled. This wastes time and resources and can delay new health treatments from becoming available. As a solution to these problems, universities and other research institutions across the country created a free and easy-to-use tool – a registry for all diseases.

Many studies require people with a specific health condition as well as those who are healthy.

On ResearchMatch, you can create a profile by filling in your contact and basic health information. Then, when a researcher thinks your profile matches their study's needs, you receive a message through ResearchMatch that tells you more about the study. You decide whether to respond “Yes” or “No.” A “Yes” response releases your contact information, so the researcher can contact you directly with more information. A “No” response keeps your contact information hidden.

ResearchMatch uses a secure, central database to store your information. All ResearchMatch data that is sent between the web server and browsers is coded...
Genetic counselors help with Alpha-1 research

By Dawn McGee, MS, CGC
Program Director
Alpha-1 Association Genetic Counseling Program
Medical University of South Carolina

Genetic counselors are graduate degree-trained professionals who focus on understanding human genetics and giving explanations and advice to patients about genetic test results. Genetic counselors need an array of skills that include counseling techniques and an understanding of medicine. These skills allow genetic counselors to work in many different career activities.

In the early days of the profession, genetic counselors mainly provided clinical counseling in prenatal and pediatric settings to predict the outcome of pregnancies in which genetic diseases ran in the family. Then genetic counselors began to expand the field. Counselors began working in cancer centers, genetic testing laboratories, and educational institutions. Only recently have genetic counselors applied their skills to research.

A master's degree in genetic counseling requires training in communication, critical thinking, psychosocial assessment, professional ethics and values. Because genetic counselors are trained in both medical genetics and counseling, they can help with research involving both social sciences and genetics.

Quantitative genetic research involves statistical analyses and objective data. The counseling and interpersonal skills of genetic counselors equip them to conduct qualitative research as well. This type of research focuses on subjective data and involves interviews to study behaviors and emotions. Most researchers believe that the two types of research are complementary.

A disease-specific genetic counselor is a specialized position that has only recently become popular. These genetic counselors are funded by communities and usually also perform research. An important part of their job is to promote testing and diagnosis while at the same time collaborating with other healthcare professionals. The Alpha-1 Association Genetic Counseling Program receives calls from nurses, pulmonologists, hepatologists, primary care physicians and other genetic counselors. If the information they receive leads to increased testing and discussion with other healthcare providers, one can see the ripple effect of Alpha-1 awareness and diagnosis.

The Genetic Counseling Program at MUSC has been involved in various research studies in Alpha-1 over the past two years. There has also been an increase in Alpha-1 research elsewhere within the genetic counseling community. Two genetic counseling graduate students at Arcadia University will be conducting Alpha-1 research for their graduate thesis projects. Another genetic counseling student from the University of Arkansas is interested in conducting her thesis research on Alpha-1. Each student is interested in different aspects of Alpha-1. Collectively, all three will help to provide further insight into this condition.

Above all, we believe that study of Alpha-1 will advance the cause of the community by improving professional knowledge about this condition we all care so much about.
NIH seeks to help research registries for rare diseases and health conditions

By Charlie Strange, MD

The Office of Rare Diseases at the National Institutes of Health (NIH) held a conference in January called "Advancing Rare Disease Research: The Intersection of Patient Registries, Biospecimen Repositories and Clinical Data."

The NIH estimates that 6,000 rare genetic diseases exist. Our goal at the conference was to define what Alphas share with the other relatively rare conditions. The NIH does little to support individual registries unless the agency is involved in research with those individuals. As a result, there is little uniformity to the support services offered to the different patient registries around the world.

**Registries come in all sizes:** This variability was highlighted in my visit to the conference. I had the opportunity to meet with people who know of as few as 15 individuals in the world with a given disease. These registries are sometimes run from the kitchen table of a dedicated parent who talks to the other parents with regularity. On their own, these families try to find dedicated researchers or care providers that know anything at all about their diseases.

Other rare diseases such as Cystic Fibrosis (CF) have a robust registry that likely captures almost everyone with the disease in the United States. Children and adults affected by CF are seen yearly by a CF Foundation-supported physician, and their data is entered by the physician into the registry. An extensive network of research centers invites these same patients to participate in research at the CF center.

As you can imagine, the difference in cost between these two models is dramatic. Registries are easy to start but costly to maintain. Costs are proportional to the number of participants, frequency of data collection, and number of telephone calls received. As a result, the more studies that come out of a registry, the more costly the infrastructure.

**Rare genetic diseases sometimes allow researchers to understand diseases that are more common.** Research on Alpha-1-related COPD or liver disease has led to progress in the study of chronic obstructive pulmonary disease (COPD) and cirrhosis, both common in the general population. Furthermore, pharmaceutical companies get an improved pathway to drug licensing when studying drugs through the Orphan Drugs Act, a 1983 law that has been responsible for many therapies for rare diseases.

The NIH is exploring ways to provide a platform that would allow all rare disease registries in the US to get some degree of support. By current policy, the NIH does not keep any patient-specific data in any of their extramural programs. All data in every study is coded. Therefore, there is no current mechanism for the NIH to host a registry and keep contact information that participants can give to their local physicians or a research study.

**National NIH site for volunteers?** One option proposed was to enroll – in a national program – every person willing to consider a research study. There are a few programs already established that help to fulfill this role. The NIH is sponsoring a consortium of research universities to perform translational research through "Clinical and Translational Science Awards" grants. This program attempts to bring researchers and participants together even though they live in different cities. Some rare diseases have been studied by collaborative universities in these programs; however, most involve a single research study.

Although we are biased, we believe that the current Alpha-1 Foundation Registry model still provides the optimal balance between cost and success. Our mission remains to find enough willing participants for the research studies that are planned for the coming years. Please encourage Alphas you know to join the Registry. The studies planned for the future will require many more Alpha-1 patients than we know are diagnosed in the United States. The race toward a cure must involve us all.
Gene transfer trials need volunteers

Alpha-1 Antitrypsin (AAT) Deficiency Adeno-Associated Viral (AAV) Gene Transfer Trials have now entered Phase II. Volunteers are being recruited for the safety and efficacy study of the study drug, rAAV1-CB-hAAT, for Alpha-1 Antitrypsin Deficiency.

Terence Flotte, MD, dean and executive deputy chancellor of the University of Massachusetts Medical School, is the principal investigator.

This is a multicenter trial, with sites at Cincinnati Children’s Hospital Medical Center in Ohio; National Jewish Health, Denver; University of Massachusetts Medical Center, Worcester, MA; and Beaumont Hospital, Dublin, Ireland.

Gene transfer is the delivery of normal or healthy genes to replace, manipulate or supplement genes that are not functioning properly.

The clinical gene transfer trials for Alpha-1 use a gene carrier or vector, called adeno-associated virus (AAV) to carry the normal gene with the correct information (DNA) to produce normal alpha-1 protein in muscle cells, rather than in the liver.

Researchers inject the AAV virus directly into a volunteer’s muscle, stimulating the cells to make alpha-1 protein and secrete it into the bloodstream.

AAV is a common virus that infects people but does not make them sick. It inserts its genes into cells in a way that lasts for a long time. These characteristics make it very useful in gene transfer. The AAV vector does not appear to cause immediate side effects, but this vector system has had limited use in human studies.

Previous studies in mice showed that a single injection of the vector into the muscle appeared to bring prolonged production of the normal alpha-1 protein.

The study is sponsored by Applied Genetic Technologies Corp (AGTC), along with funding by the National Heart, Lung, and Blood Institute (NHLBI).

Two previous trials directed by Flotte and Mark Brantly, MD, at the University of Florida, have been completed.

The first trial in humans, using AAV serotype 2, showed favorable safety characteristics, but expression of the normal M-specific alpha-1 protein was barely seen, and in just one of the 12 subjects participating. Research in mice showed an advantage for vectors packaged in AAV serotype 1.

For that reason, the second trial used the same gene cassette but packaged it in an AAV serotype 1 capsid/shell. Three groups of three subjects, with an increase in dose in each group, were studied and again the AAV serotype 1 was well tolerated in the nine subjects studied.

This time, normal alpha-1 protein was expressed above the baseline in all subjects in cohorts 2 and 3 and was sustained for at least a year at the highest dosage level. The second trial was sponsored by AGTC and both trials received funding from the NHLBI and monitoring by their Gene Therapy Data Safety Monitoring Board. The Alpha-1 Foundation, University of Florida, and Shands Hospital at the University of Florida contributed to the manufacturing of the vector. With this background, the clinical trials have now moved to Phase II.

The goal of the Phase II trial is to see if the amount of alpha-1 protein in the blood can be increased, along with testing safety of the study drug.

Each participant will receive the study drug on a single occasion. Three groups of three subjects each will receive the study drug by intramuscular injection.

Those in group 1 will receive 10 injections distributed across a single muscle site; group 2 will receive 32 injections distributed across three muscle sites; and group 3 will receive 100 injections distributed across 10 muscle sites.

The volume of each injection will be about 1/4 teaspoon. The injection density at each site will be the same as the injection density that was well tolerated in the previous Phase 1 clinical trial. Safety and efficiency will be monitored.

Participants will include both male and female adult alpha-1-deficient individuals with at least one PI*M allele. Pregnant and breastfeeding women will be excluded, as will anyone who has previously participated in AAV Gene Transfer Clinical trials.

Enrollees cannot have used augmentation therapy (Alpha-1 infusions) in the three months before the trial and must be willing and able to remain off augmentation therapy for one year following the study injections.

For more information, see www.clinicaltrials.gov. Put this code in the search box: NCT01054339.
By Angela McBride  
Director of Development,  
Alpha-1 Foundation

Building Friends for a Cure (BFC) is a grassroots initiative to raise funds through community fundraisers, including our Team Alpha-1 Program, and to foster an even more involved community.

A key part of the Alpha-1 Foundation’s reason for being is research for better health and ultimately a cure for Alpha-1. Every year, these fundraisers play a greater part in funding our research programs.

That’s why we believe in training our volunteer fundraisers.

This past spring, Alphas, their family members and friends learned the details of raising both awareness of Alpha-1 and funds for Alpha-1 research at our Building Friends for Cure training in Needham, MA.

The trainees learned the best and easiest ways to get involved. At the end of the program, they walked away with the tools they need to raise funds for Alpha-1 research in their communities.

We outlined event organizing, including how to choose an event (make sure it’s something you’re comfortable with), recruiting friends, family and others to help, how to get donations and find sponsors, budgeting, paperwork, and getting help from the Foundation staff.

Two experienced volunteers talked to the group about their experiences. Sheila Favazza discussed the Celtic Connection fundraising dinner and organizing its silent auction; Fred Walsh talked about the annual Escape to the Cape bike ride on Cape Cod.

Foundation President & CEO John Walsh presented the new BFC trainees with their graduation certificates and spoke to the group about the importance of their work in both getting out the Alpha-1 message and helping to support Alpha-1 research programs.

Then the new BFC training graduates teamed up with committee members for one of our most successful events for the past two years — the Celtic Connection.

They watched the organizers in action, assisted with preparations and the event itself, and enjoyed an evening of Irish music, dancing and a traditional St. Patrick’s Day dinner.

All the graduates plan to organize their own community event within the next year, using their newly acquired knowledge.

The training graduates came from all over the country: Julia Torres Barden from Ohio; Sarah Bradley from Iowa; Mindy and Neal Catron from South Carolina; Tom Corron from Iowa; Judi Kaplan Elkin from Massachusetts; Peg and Peter Iverson from Iowa; Ken Richmond from Virginia;
THE GRADUATES — These are the graduates of the Alpha-1 Foundation's Building Friends for a Cure training in Needham, MA, last spring. The next training will be in Las Vegas, NV, in early December.

Sandy Ringgard from Massachusetts; Lucinda Shore from Tennessee; Troy Sutherland from Iowa; Timothy and Desiree Truett from Arizona; Pam Vanssoy from Virginia; and Jay Whitmore from Georgia.

Would you like to take advantage of our next BFC training? We’ll hold it in Las Vegas, NV, in early December. Contact Angela McBride at (888) 825-7421, Ext. 233, or amcbride@alpha-1foundation.org.

Several of these BFC training graduates are already involved in events to raise Alpha-1 awareness and funds in their communities. We’re sure the others will all come up with ideas for events involving something they like to do!

They have many examples to follow that are being done already, including organized walks, bike rides, an ice cream "social," a wine tasting, a harbor cruise, golf, even a croquet tournament.

See our schedule of Building Friends for a Cure and Team Alpha-1 events for the rest of the year on the next page — and see if you like any of these ideas for your own event!

TAKING A WHACK AT ALPHA-1 — Darrell Kotton, MD, lead author of a recently-published study on gene therapy for Alpha-1 for which he received a Foundation research grant, receives a shillelagh (the club or cudgel used for centuries in Ireland) from Foundation President & CEO John Walsh at the Celtic Connection dinner.
Calendar

For the most up-to-date listings, check our website at www.alpha-1foundation.org.

**Building Friends for a Cure 2010/2011 Calendar of Events**

<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
<th>Location</th>
<th>Contact Information</th>
</tr>
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<tbody>
<tr>
<td>August 9</td>
<td>2nd Annual Memorial Paul (PJ) Healy Golf Tournament</td>
<td>Halifax, MA</td>
<td>Bob Healy: <a href="mailto:Bobhealy125@msn.com">Bobhealy125@msn.com</a></td>
</tr>
<tr>
<td>September 11</td>
<td>Harbor Cruise</td>
<td>Plymouth, MA</td>
<td>Sheila Favazza: <a href="mailto:sfavazza@yahoo.com">sfavazza@yahoo.com</a></td>
</tr>
<tr>
<td>September 16</td>
<td>Alpha-1 Golf Tournament</td>
<td>Greenwich, CT</td>
<td>Ken Irvine: <a href="mailto:airvine3@optonline.net">airvine3@optonline.net</a></td>
</tr>
<tr>
<td>October 1-3</td>
<td>Team Alpha-1 Escape to the Cape</td>
<td>Cape Cod, MA</td>
<td>Sue Binnall: <a href="mailto:sbinnall@comcast.net">sbinnall@comcast.net</a></td>
</tr>
<tr>
<td>October 9</td>
<td>Brookside Croquet Championship in Honor of Todd Zinni</td>
<td>S. Nyack, NY</td>
<td>Brent Him: <a href="mailto:croquet@optonline.net">croquet@optonline.net</a></td>
</tr>
<tr>
<td>November 20</td>
<td>2nd Annual Alpha-1 5k Walk Miami</td>
<td>Miami, FL</td>
<td>Angela McBride: <a href="mailto:amcbride@alpha-1foundation.org">amcbride@alpha-1foundation.org</a></td>
</tr>
<tr>
<td>December</td>
<td>Building Friends for a Cure Training</td>
<td>Las Vegas, NV</td>
<td>Angela McBride: <a href="mailto:amcbride@alpha-1foundation.org">amcbride@alpha-1foundation.org</a></td>
</tr>
<tr>
<td>March 12</td>
<td>Celtic Connection</td>
<td>Needham, MA</td>
<td>Angela McBride: <a href="mailto:amcbride@alpha-1foundation.org">amcbride@alpha-1foundation.org</a></td>
</tr>
<tr>
<td>April 2</td>
<td>Alpha-1 5K “Hero” Walk</td>
<td>Richmond, VA</td>
<td>Pam Vanscoy: <a href="mailto:pamvs2000@yahoo.com">pamvs2000@yahoo.com</a></td>
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**2010 National Education Programs**

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<thead>
<tr>
<th>Date</th>
<th>Event</th>
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<tr>
<td>August 28</td>
<td>Alpha-1 Education Day</td>
<td>Des Moines, IA</td>
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<tr>
<td>October 23</td>
<td>Alpha-1 Education Day</td>
<td>Seattle, WA</td>
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The Alpha-1 National Education Series is co-sponsored by the Alpha-1 Foundation and the Alpha-1 Association and is made possible by unrestricted education grants from AlphaNet, Baxter BioTherapeutics, Centric Health Resources, CSL Behring, and Talecris Biotherapeutics.

For information on attending or exhibiting at an education program, contact Marlene Erven at 1-800-521-3025 or email mserven@alpha1.org. For information on Building Friends for a Cure events, contact Angela McBride at 1-888-825-7421, Ext. 233 or amcbride@alphaone.org.

*Commitments and dates are subject to change.

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**Alpha-1 Foundation**

The Alpha-1 Foundation is dedicated to providing the leadership and resources that will result in increased research, improved health, worldwide detection, and a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1). The Foundation has invested nearly $39 million to support Alpha-1 Antitrypsin (AAT) research and programs in more than 70 institutions in North America, Europe and Australia.

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**Alpha-1 Association**

The Alpha-1 Association is a member-based not-for-profit organization founded in 1991 to identify those affected by Alpha-1 Antitrypsin Deficiency and to improve the quality of their lives through support, education and advocacy. The Association has a network of more than 70 volunteer-led support groups around the U.S.

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**AlphaNet**

AlphaNet, Inc. is a unique disease management organization. Through its medical and operations staff, AlphaNet provides a wide range of integrated support services to individuals with Alpha-1 Antitrypsin Deficiency who require augmentation therapy, oversees and sponsors clinical trials involving Alpha-1 therapies, and makes available a comprehensive disease management and prevention program to improve the quality of life of those affected by Alpha-1.

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The Registry Update is funded by unrestricted educational grants from CSL Behring and Talecris Biotherapeutics.