

# Alpha-1 Nor'Easter a Support Group Newsletter (July 2010)

Serving the North Eastern US for the most part!

## UPCOMING ALPHA EVENTS

(see "SUPPORT GROUP NEWS " for more info -Page 2)

DATE	TOPIC	PLACE	CONTACT	
Thur 6/10	?	Westgate Nursing Home, Needham, MA	SheilaFavaza	1 (978) 468-7704
Sat 7/17	social meeting	Connecticut	Jo Anne Brailey	1 (860) 739-4331
Sun 7/18	PA Regional Alpha-1 Picnic	Wummers House - Douglassville, PA	Michael Wummer	1 (610) 914-6720
Sat 8/21	New England Regional Education Day	Dartmouth Hitchcock Med Cntr, VT	Vicki Cameron	1 (888) 526-0351
Sat 9/11	A1F Fundraiser	Plymouth Harbor Cruise	SheilaFavaza	1 (978) 468-7704
Sun 9/26	Educational Brunch	Metro NY	Lori Tartell	1 (212) 523-5471
10/1-3	Autumn Escape - Team Alpha-1 bike trek	Throughout Cape Cod	SheilaFavaza	1 (978) 468-7704
Tues 12/7	Holiday Dinner	Metro NY	Lori Tartell	1 (212) 523-5471

We would hope and recommend that everyone receiving this notice is a member of the Alpha-1 Association (A1A) and has enrolled in the Alpha-1 Foundation (A1F) Registry. Likewise, if you don't receive the A1F Research Registry Update you are NOT part of the cure for Alpha-1 (shame! shame!). Even if you receive a copy of the Alpha-1 News you are NOT necessarily a member of A1A. Call them just to be sure. 1 (800) 521-3025 Another great publication is the Alpha-1 magazine; contact the Alpha-1 Foundation to get a copy. If interested contact me (Joe) as shown at the end or look at our phone list.

### IMPORTANT PHONE NUMBERS: if all these numbers confuse you give me a call 201-444-7839

Alpha-1 Association (A1A)	1 (800) 521-3025	www.alpha1.org	Support
A1A Genetic Counselor	1 (800) 785-3177	courtesy of A1A	Family Concerns
Alpha-1 Foundation	1 (888) 825-7421	www.alphaOne.org	Research
Alpha-1 Coded Testing (ACT)	1 (877) 886-2383	AlphaOne@musc.edu	Free coded (anonymous) testing
Alpha-1 Registry	1 (877) 886-2383	http://www.musc.edu/	Be part of the cure
Baxter Healthcare	1 (800) 423-2090	www.Baxter.com/ Aralast.com	Aralast
Talecris Biotherapeutics	1 (800) 243-4153	www.Prolastin.com	Prolastin
CSL Behring	1-866-936-2472	www.Zemaira.com	Zemaira
Accredo	1 (866) 625-7421	www.Accredotx.com	Aralast/Zemaira
AlphaNet	1 (800) 577-2638	www.AlphaNet.org	Prolastin/Zemaira
Prolastin Direct	1 (800) 305-7881	www.ProlastinDirect.com/	Prolastin
Coram Healthcare	1 (866) 367-2174	www.CoramHealthcare.com	Aralast/Zemaira

## WORLD WIDE WEB

Most of the following information was gathered at the ALPHA-1 MAILING LISTS. As with everything else in this meeting notice take it with a grain of salt. Remember We do edit some of these E-mail posts and We select which to print. There is much more on the lists. Some of which is very interesting. If you have any questions contact me <JoeReidy@Verizon.net >. Alpha-1 International Support Groups are email networks which Alphas use to rapidly exchange information, support, suggestions, questions, plans, activities; nearly anything that comes to our minds. Many of us have had to reduce our activities and contacts; the Alpha1 contacts are a great way to maintain contact with others. It is also a big psychological boost to know that we are not alone. Here is a list of the E-mail groups to which I (Joe) subscribe:

#	Mailing List	enrollment	owner(s)	TO JOIN: E-mail to:
1	Alpha-1 International	open to most interested in Alpha-1	Connie Storey	ALPHA-1-REQUEST@home.ease.lsoft.com
2	Alpha-1 LIVER	restricted mostly liver affected Alphas & family	Nancy Cropper	ALPHA-LIVER-REQUEST@home.ease.lsoft.com
3	Alpha-1 Lungs & Life	open to all interested in Alpha-1	Sally Turner	Alpha1_Lungs_and_Life-subscribe@yahoogroups.com

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## Support Group News Alpha Nutmeggers (CT) Support Group

**DATE:** July 17th from 1 to 4 p.m.  
**PLACE:** Gaylord Hosp., Chauncey bldg, Wallingford, CT.  
**more info:** Jo Anne Brailey 1 (860) 739-4331 iluv2sew@snet.net  
 Hi Everyone!

Our next support group meeting will be July 17th from 1 to 4 p.m. at the Gaylord Hospital, in the Chauncey building, Wallingford, CT. Directions attached. Lunch will be provided. This will be a social meeting. I will have some handouts from the 19th Annual Alpha-1 Association Conference we attended in June. Ed and I will be moving to Southport, NC in September. If we do not find a new

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support group leader, this will be the last meeting until someone volunteers. Ed and I joined this support group in 1989 when he was diagnosed and it had been meeting long before that. I hate to see this support group ending. Please consider becoming the support group leader. Its not a very demanding job and you get lots of help from the Alpha-1 Association. I will always be an email away to advise you. The Association gives you \$500 a year to pay for expenses such as ink, paper, postage, etc.

I will give you websites where I get information which I think might be of interest to you. Jo Anne

## Joint PA Support Groups.

**DATE:** Sunday - July 18, 2010 **Time:** 2:00 PM

print date: Wednesday, July 7, 2010

**PLACE:** The Home of Michael and Rene' Wummer  
381 Oley Line Road  
Douglassville, PA, 19518  
(Everything is wheelchair accessible)  
**SUBJECT:** Alpha 1 Picnic Invitation  
**RSVP:** Michael Wummer  
mrwummer@aol.com - 610-914-6720 or  
Laura Austin  
AustinLaura51@yahoo.com - 717-352-4588  
with the name and # attending By July 4th

Note: **There is no charge for this event.**

Vickie Cameron  
11 Spooner Road  
West Topsham, VT 05086

## Sad News

On July 3, 2010 Sheila Favazza wrote:

Once again I find myself the bearer of sad news. Maryann Jackson passed away earlier today at U Mass Medical Center from complication of her transplant. Maryann was transplanted just over 1 1/2 yrs ago & has a difficult time ever since.

Please remember Maryann, her family & friends in your prayers tonight.

Maryann has been receiving this newsletter since 9/06. She had a double lung transplant at the Cleveland Clinic on 11/9/08.

### Alpha's.....Summertime is for Picnics!

Please join our 2nd combined meeting of the Alpha Opportunities and Central PA Alphas Support Groups. This is a great opportunity to continue to meet other Alpha's and to pick up helpful hints on improving your daily life. Hear updates from Marlene Buchanan and Jean McCathern on their recent attendance to the Alpha One National Convention. Also our sponsors, Baxter Pharmaceuticals and Coram Specialty Infusion Services will be on hand to lead a discussion on infusion techniques. There will also be an optional open member meeting for Alpha Opportunities members starting at 2:00 PM

## Massachusetts Support Group

**DATE:** August 12, June 10, 2010 6 pm  
**PLACE:** Wingate Nursing Home  
589 Highland Ave.  
Needham, MA  
**more info:** Sheila (978)468-7704 slfavazza@yahoo.com  
Also **SAVE THE DATE BE PART OF THE CURE!**  
**Plymouth Harbor Cruise** to benefit The Alpha-1 Foundation  
**September 11, 2010 2pm to 5pm** Capt John Boats  
10 Town Wharf  
Plymouth, MA

**music** hors d'oeuvres **cash bar**  
For tickets or more information please contact: Sheila Favazza

## **7th New England Alpha-1 Regional Support Group Meeting / Education Event**

**SATURDAY, AUGUST 21 / 8:00AM - 4:30PM**  
**DARTMOUTH HITCHCOCK MEDICAL CENTER**  
One Medical Center Drive,  
Lebanon, NH 03756 Main Entrance — Auditorium F

**8:00am REGISTRATION & CONTINENTAL BREAKFAST**  
**8:45am Welcome:** Vicki Cameron / Fred Walsh  
**9:00am Speaker:** **DR. Donald Mahler**  
Professor of Medicine, Section Medicine  
*Pulmonary & Critical Care Medicine, DHMC*  
**Topic:** *Alpha-1 Lung Disease/Importance of exercise*  
**10:00am Speaker:** **DR. Brian Berk**  
Director of End Stage Liver Disease Prgm,  
*Alpha-1 Liver Disease / recommended testing*  
**Topic:** **BREAK / BEVERAGE & SNACK**  
**11:00am Speaker:** **Dr. Andrew Wilson**  
Ass't Professor of Pulmonary Medicine  
Boston University School of Medicine /  
Gene therapy for AAT Deficiency  
**Topic:** **John Walsh**  
Alpha-1 Foundation (A1F), Pres. & CEO  
*Update on the A1F and current research*  
**1:30pm Speaker:** **Ed Brailey**  
Alpha-1 Assoc, Chairman of the Board  
*Update on the Alpha 1 Association*  
**Topic:** **Amy Chaves**  
MED,RIT,CHES, Assoc Prof of Respiratory  
*Therapy and Director of Clinical Education at*  
*the Community College Of Rhode Island/*  
*Understanding Pulmonary Function Tests*  
**2:00pm Speaker:** **Angela McBride**  
Director of Development A1F  
*Overview on the "Building Friends for a Cure"*  
**3:00pm Speaker:** **Delicious Meal**  
**3:30 Pre-Registration:** **Immediately following tprogram (on site)**

To Pre-register and reserve your seat, please call:  
**Vicki Cameron at 888-526-0351** (toll free).  
Pre-registration is not required but appreciated to help us  
plan for handouts, food and seating.  
Call if you need further information.

**From:** KENNETH BENSON <kenneth.benson@SBCGLOBAL.NET>  
**Date:** June 7, 2010 8:20:30 AM EDT  
**Subject:** [ALPHA-1] Ch-Ch-Changes

MADRID (AP) -- Spanish healthcare company Grifols SA said Monday it had acquired Talecris Biotherapeutics, based in North Carolina, for euro3.3 billion (\$4 billion) including net debt.

In a statement on its website, Grifols, a leading producer of plasma protein therapies, said the deal would create "a global leader of lifesaving and life-enhancing plasma protein therapeutics."

The company said the acquisition of Talecris is expected to generate approximately euro190 million (\$230 million) in operating synergies. The Spanish company's shares were down 5.6 percent at euro8.7 (\$10.5) in midmorning trading in Madrid that saw most companies' shares fall.

Grifols is a holding company specialized in the pharmaceutical-hospital sector and is present in more than 90 countries, the statement said.

Talecris is a worldwide biotherapeutic and biotechnology company that discovers, develops and produces critical care treatments for people with life-threatening disorders, the statement added.

## NEW IV ALPHA-1 PRODUCT

**From:** Joe Reidy <JoeReidy@Verizon.net>  
**Date:** July 2, 2010 9:48:04 PM EDT  
**Subject:** Kamada wins FDA approval for Glassia

At the National Conference I asked the Kamada spokespeople how they were going to attract customers to their product. I said that offering a low price would be an excellent strategy to win over Alphas.

They said something like "We are not allowed to do that. We are given a limited range that we can charge."

I thought to myself "Can I really believe these people?"

I found out about this on the Foundation's web site.

from: <http://www.rttnews.com/Content/BreakingNews.aspx?Id=1350151&SM=1>  
FDA Approves Kamada's Alpha-1 Antitrypsin  
7/2/2010 6:28 AM ET (RTTNews) - Israeli biopharmaceutical company Kamada Inc. (KMDA.TA: News) (KAMAF.PK) has been granted approval by the Food and Drug Administration for its intravenous lung drug Glassia, an Alpha-1 Antitrypsin, to treat the genetic disorder Alpha-1 Antitrypsin deficiency.

Alpha-1 Antitrypsin deficiency, or AATD is a shortage or absence of a protein called A-1 Antitrypsin, that blocks the destructive effects of certain enzymes. Lack of this protein can lead to the destruction of lung tissue and cause chronic lung disease such as emphysema. It can also be associated with liver disease in children.

As part of its preparations to market the drug in the United States, the company has set up a U.S. subsidiary called Kamada Inc.

According to the U.S. Alpha-1 Foundation, Alpha 1 Antitrypsin Deficiency is the most prevalent, potentially fatal, under-diagnosed disease. About 3% of COPD (chronic obstructive pulmonary disease) patients have Alpha-1 Antitrypsin deficiency.

The company is also developing an inhaler version of the Alpha-1 Antitrypsin, or AAT. The inhaler version of the Alpha-1 Antitrypsin has been designated Orphan Drug, in both Europe and the U.S. for the treatment of cystic fibrosis and Alpha-1 Antitrypsin Deficiency as well as in the U.S. for the treatment of Bronchiectasis.

Kamada's Alpha-1 Antitrypsin Glassia will now compete with

Talecris Biotherapeutics Holding Corp.'s (TLCR) Prolastin, the dominant AAT product in the market. The other AAT therapeutics on the market include , Baxter's Aralast and CSL Behring's Zemaira.

## ALPHA - ODDS AND ENDS

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** June 23, 2010 2:03:26 PM EDT  
**Subject:** [ALPHA-1] FYI: DR. EDEN'S VIEWS 6/23/2010

FYI: Our very own Dr. Eden - PeterD

|||||  
Heat, Humidity And Your Health

Reporting Dr. Holly Phillips NEW YORK (CBS) -- With summer officially underway, the heat is on in the tri-state.

But the effects of the weather on our health aren't just gauged by the thermometer. Extreme humidity can cause or worsen a number of conditions - some life threatening. Knowing the risks can help you protect yourself.

Helaine Castaldi has COPD, and humid days are hard for her.

"It gets right into your lungs, it hits you right away," she said. "It's a tightness and then it's the shortness of breath, and it can be scary."

Castaldi's illness makes hazy days especially difficult, but humidity can take a toll on everyone.

Pulmonologist Dr. Edward Eden says one of the first symptoms is fatigue.

"It's like walking through quicksand," said Dr. Eden. "The heat alone doesn't have that effect, the heat with the humidity just slows people up."

One of the reasons high humidity makes heat more dangerous is that it slows the evaporation of sweat - the body's natural cooler.

"You evaporate much less and therefore your skin doesn't cool, and you get hotter sooner," said Dr. Eden.

This can cause heat exhaustion and heat stroke, even on days when the temperature is relatively mild.

Pollution is also a problem - car exhaust and industrial pollution build up in the air faster in hot, humid conditions.

"There's no wind blowing, those particles are not shifted and accumulate and concentrate in the air," said Dr. Eden.

This makes breathing difficult even for children. A recent study found kids who participate in sports outside on days with the high pollution are more likely to develop asthma.

Our joints feel it, too. Both rheumatoid arthritis and osteoarthritis are more likely to flare up on sticky days.

Since we can't control the weather, the best bet is to play it safe.

"Do as minimal as you can outside," said Dr. Eden. "Don't do any exercise."

And always stay well hydrated. A good rule of thumb is to drink a full glass of water for every 30 minutes you're outside.

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** June 10, 2010 9:41:42 AM EDT  
**Subject:** [ALPHA-1] FYI: A1AD & DIABETES 6/10/2010

Omni Bio announces FDA IND clearance for Phase I/II diabetes trial

Symbols: OMBPE Email to friend Print Add This RSS Feed  
Font size: A A A Jun 09, 2010 (Datamonitor via COMTEX) --

Omni Bio Pharmaceutical, a biopharmaceutical company, has announced that the Barbara Davis Center for Childhood Diabetes has received investigational new drug regulatory clearance from the FDA to initiate a Phase I/II clinical trial evaluating alpha-1 antitrypsin in type 1 diabetics.

Alpha-1 antitrypsin (AAT) is an FDA-approved, off-patent drug currently indicated for the treatment of pulmonary emphysema among those with genetic deficiency of AAT. Preclinical studies demonstrate that AAT may be effective in treating a variety of medical disorders.

The Phase I/II clinical trial is being sponsored by Omni and will be conducted under the Peter Gottlieb at the Barbara Davis Center for Childhood Diabetes and other units at the anschutz medical campus of the University of Colorado Denver. Omni has licensed patent applications related to the method of use of AAT for the treatment of diabetes from the regents of the University of Colorado and a privately held corporation.

The study protocol provides for AAT administration during an eight-week treatment period in an initial group of 15 diagnosed diabetics, potentially expanding to upto 50 patients. Following the initial AAT administration, enrolled patients will be monitored for two years.

In conjunction with the receipt of the investigational new drug (IND) clearance, Omni has executed an agreement with a body of UCD and the Barbara Davis Center to conduct the clinical trial, which Omni expects to commence during the third quarter of 2010.

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** May 25, 2010 10:17:52 AM EDT  
**Subject:** [ALPHA-1] FYI: HEART DRUGS ARE SAFE - 5/25/2010

Fears about beta blockers making COPD worse are unfounded, experts say  
By Ed Edelson HealthDay Reporter

Medical tradition says that the beta blockers used to treat heart disease shouldn't be given to people who also have severe lung disease, but a new Dutch study suggests the tradition is wrong.

A study of more than 2,200 people with chronic obstructive pulmonary disease (COPD), a diagnosis that includes emphysema and chronic bronchitis, found better survival among those given beta blockers than those who did not get the drugs, claims a report in the May 24 issue of the Archives of Internal Medicine by physicians at University Medical Center Utrecht.

"To our knowledge, this is the first observational study that shows that long-term treatment with beta blockers may improve survival and reduce the risk of exacerbation of COPD in the broad spectrum of patients with a diagnosis of COPD," the researchers wrote.

"This is strikingly different from what our medical students are taught today," said Dr. Don D. Sin, a professor of medicine at the University of British Columbia in Vancouver, Canada, and co-author of an accompanying editorial. "Our traditional teachings are wrong."

The rap against beta blockers has been that while they improve heart function, they can cause airways to contract, a problem for people with COPD, Sin explained. "They demonstrate in this article that even people with COPD who use beta blockers did very well, better than people who didn't use beta blockers," he said.

Fears about beta blockers and COPD date back to the 1980s, when there were reports of "some nasty effects in patients with asthma, especially with high doses," said study author Dr. Frans H. Rutten, an assistant professor of medicine at Utrecht. The study demonstrates that the drugs can be handled safely for people with COPD, he noted.

"I know of no real problems now, especially when you start with a low dose so that the bronchial airways can adjust to the drug," Rutten said.

COPD was diagnosed in 560 patients at the start of the study in 1996, and 1,670 developed the condition by the end, in 2006. Of these, 665 were prescribed beta blockers for heart conditions, while 1,565 were not.

During an average follow-up time of 7.2 years, 27.2 percent of the people who took beta blockers died, compared to 32.3 percent of those not given the drug. The incidence of exacerbation -- severe flare-up -- of COPD was 42.7 percent among beta blocker users and 49.3 percent among nonusers.

The study isn't the final word on beta blockers and COPD, Sin said. That would have to come from a randomized, controlled study, which almost certainly will never be done, he said.

"If I had a heart attack, I wouldn't want to be in a clinical trial where there was a 50 percent chance I would get a sugar pill," Sin said. "So, this study may be the best evidence we get."

And the incentive of profit from increased use of beta blockers isn't there to have a drug company fund such a trial, since beta blockers are widely available in inexpensive, generic form, he added.

Sin acknowledged that he has been "an outlier" on the issue, already prescribing beta blockers for people with COPD. "But with this paper, I am much more confident that COPD patients can tolerate beta blockers," he said.

There are some exceptions, Sin noted. "For people with very bad asthma who have very reactive airways, I am much more cautious," he said. "I would start with the lowest dose possible, and then titrate upwards."

More information

To learn more about COPD, visit the U.S. National Heart, Lung, and Blood Institute. SOURCES: Don D. Sin, M.D., professor, medicine, University of British Columbia, Vancouver, Canada; Frans H. Rutten, M.D., Ph.D., assistant professor, medicine, University Medical Center Utrecht, the Netherlands; May 24, 2010, Archives of Internal Medicine

# LIVER ISSUES

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** June 3, 2010 2:58:49 PM EDT  
**Subject:** [ALPHA-1] FYI: A POSSIBILITY 6/3/2010

Pitt team finds commonly used seizure drug could treat severe genetic liver disease

PITTSBURGH, June 3 – The liver scarring of A1AD, the most common genetic cause for which children undergo liver transplantation, might be reversed or prevented with a medication that has long been used to treat seizures, according to findings from Children's Hospital of Pittsburgh of UPMC and the University of Pittsburgh School of Medicine that will be published in Science and are available online today through the Science Express website.

Because the anti-seizure drug is familiar to doctors and has a well-understood safety profile, clinical trials could begin immediately to see whether it can help patients with AT deficiency, said senior author David H. Perlmutter, M.D., physician-in-chief and scientific director, Children's Hospital, and Vira I. Heinz Professor and Chair of the Department of Pediatrics, Pitt School of Medicine.

In the classic form of the disease, which affects 1 in 3,000 live births, a gene mutation leads to an abnormal protein, dubbed ATZ, that unlike its normal counterpart is prone to aggregation.

"These aggregates of ATZ accumulate in the liver cells and eventually lead to scarring, or fibrosis, of the organ and set the stage for tumor development," Dr. Perlmutter said. "The disease sometimes doesn't show itself until adulthood, when the liver starts to fail due to cirrhosis or cancer."

For the study, he and his colleagues treated an ATZ cell line with carbamazepine, or Tegretol. Although this drug has been used primarily to treat seizure disorders, some recent work has suggested that it could enhance a natural cellular pathway called autophagy, or self-digestion, and so the Pitt researchers reasoned that it might be able to rid the cells of the toxic aggregated ATZ.

They found that carbamazepine did, indeed, cause a marked decrease in ATZ because the abnormal proteins were degraded more quickly via autophagy, and so they did another experiment in a mouse model of AT deficiency.

"The amount of ATZ decreased in the livers of the mice treated with carbamazepine," Dr. Perlmutter said. "The most amazing finding was that the drug reversed the fibrosis in the livers of the mice and, after two weeks of treatment, the liver tissue resembled that of a healthy mouse."

The ability of carbamazepine and drugs like it to "soup up" the cell's autophagy machinery might have value in other disorders such as Alzheimer's disease, Huntington's disease and Parkinsonism that are thought to be caused by toxic effects of protein clumping in the brain. Dr. Perlmutter and his colleagues are now exploring these possibilities in preclinical studies.

**From:** Joe Reidy <JoeReidy@VERIZON.NET>  
**Date:** June 6, 2010 5:23:24 PM EDT  
**Subject:** Re: [ALPHA-1] FYI: A POSSIBILITY 6/3/2010

As a follow up to Peter D's post I thought it prudent to post what was on the Alpha-1 Foundation web site.

News Anti-seizure drug improves liver disease in Alpha-1 mice  
A note from Robert A. Sandhaus, MD, PhD, clinical director of the Alpha-1 Foundation and medical director of AlphaNet:

In the interest of presenting the latest information to our Alpha-1 community, the following press release has been posted here. We would like to point out some issues in considering the information presented in this release.

First, this is not a scientific article, although it refers to one, but rather a "story" written by a communications person at the Pittsburgh medical center. With respect to the research described, this is very preliminary research. The article discusses work done on cells in a culture dish and a mouse model of Alpha-1 Antitrypsin Deficiency.

When they mention that the drug used in this study has a "well-understood safety profile," it is important to note that Tegretol's well understood safety-profile includes a "black box" warning from the FDA that it can cause aplastic anemia (loss of the ability of the body to make red blood cells in the bone marrow), agranulocytosis (loss of the ability of the body to make white blood cells in the bone marrow), and skin reactions so severe that they have

been fatal.

This is in addition to the many additional side effects that have been described with this drug. Having pointed out these issues, this remains a very promising initial set of experiments published by one of the major experts in the field of Alpha-1 liver disease. We certainly look forward to seeing additional information on this line of research.

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** June 5, 2010 2:37:16 PM EDT  
**Subject:** [ALPHA-1] FYI: THE NEW FRONTIER 6/5/2010

A cutting-edge procedure is offering new hope to liver transplant patients. Doctors say the new approach is much less invasive and allows donors to recover in half the time.

When 13 year old Aiden Ward needed a liver transplant, his father Michael didn't hesitate.

"Desire was so strong to help my son that the worry was oh no what if they find something and I'm not able to donate," he said.

Michael was a perfect match. But surgery would mean months of recovery at a time when he needed to care for his son.

Dr. Ben Samstein offered a cutting-edge alternative.

"The entire operation is done in small ports just the size of the tip of my finger," Dr. Samstein said.

Instead of a large incision, the new technique is done laproscopically with 5 small incisions. Recovery time is typically cut in half with patients out of the hospital 3 days sooner. They're also able to stop taking medications within a week. Doctors say most patients feel normal within 2 weeks.

The procedure has only been done 5 times in the U-S and only between adult donors and pediatric patients. But it's been so successful, doctors are hoping to offer it as a better option.

Michael Ward said, "It was far less painful and debilitating than I had expected."

Each year nearly two thousand people die waiting for a liver transplant. Doctors hope this new technique will encourage more live donors and significantly shorten the waiting list."

"If half of all liver transplants performed in the United States were from a living donor, there would be no waiting list," said Dr. Samstein.

Aiden and Michael are both on the road to recovery.

Aiden says he has more energy now. They hope they'll inspire others to save lives.

# LUNG ISSUES

**From:** Dutt's Logger <pdlogger@WINDSTREAM.NET>  
**Date:** June 25, 2010 9:28:28 AM EDT  
**Subject:** [ALPHA-1] FYI: TWO FRONTS COMING IN 6/25/2010

N.E. researchers create functioning lung tissue A vital step in the quest to build organs

By Carolyn Y. Johnson, Globe Staff  
Two teams of researchers from New England have built living, breathing lung tissue in the laboratory — feats of engineering that could speed up the development of new drugs and bring researchers a step closer to the tantalizing dream of growing replacement lungs for patients.

Both achievements, described in reports published yesterday by Harvard and Yale scientists, are part of broader efforts among researchers to build a range of organs, from the heart to the liver. Such research could provide powerful tools to test drugs and identify toxins, and eventually grow new tissue to repair damaged organs.

Harvard scientists re-created a critical area of lung tissue on a silicon rubber chip the size of a quarter, and found that it responded to bacteria and tiny particles carried in the air just like a living lung. Using a different approach, Yale University researchers regenerated lungs and transplanted them into rats, where they functioned successfully for up to two hours.

This work is not the first successful effort to build functional tissue. In the late 1980s, researchers first began to apply engineering approaches to human tissue, and advances have begun to work their way into the clinic — most notably in advances in artificial skin.

But the two new studies are significant milestones in the quest to build a functional organ in the lab — although it will still be many years yet before doctors reach the science fiction dream of regenerating lungs to help patients.

More immediately, the benefits of the new work could be seen in allowing pharmaceutical companies to test drugs on a tiny, cheap chip that closely resembles the complexity of the human body.

The "lung is pretty tough [to replicate]. It's also pretty important — people get lung cancer, and lots of people get lung diseases that are pretty serious," said Robert Langer, a professor at MIT who was not involved in either group's research. Langer said the new techniques also could provide a substitute for animal testing.

For years, scientists have tested drugs using cells in a dish and lab animals. But many organs have layers of complexity that simply can not be replicated with current models, and drugs that show promise in mice often do not pan out as cures in humans.

The lung contains an intricate branching architecture of airways, blood vessels, and hundreds of millions of tiny sacs, called alveoli, where gases are exchanged. And the cells move and are stretched with each breath.

In an organ, multiple tissues come together at an interface, and synergize. Higher-order structure and physical movements, like the effect of [breathing] movement on a lung, or a heartbeat on the heart" need to be considered, said Dr. Donald Ingber, director of the Wyss Institute for Biologically Inspired Engineering at Harvard University and senior author of one of the papers, published in the journal Science.

Ingber and colleagues created tiny, microscopic channels in a clear, silicon rubber chip. Sandwiched in the middle of the channel was a thin, flexible membrane seeded with lung and blood vessel cells that mimicked the wall of the alveoli — the tiny sacs where oxygen enters the bloodstream.

In a living lung, the alveoli stretch and swell as they fill with air, and oxygen enters blood while carbon dioxide exits. Researchers used a vacuum to cause the membrane to stretch.

Then, they tested whether the tiny lung-on-a-chip acted like a lung in the body, seeing how it reacted to bacteria and small particles, such as those found in pollution.

It has the dynamics of a real lung, with stretching," said Shuichi Takayama, associate professor of biomedical engineering at the University of Michigan, who was not involved in the research. "Testing results seem to really nicely mimic what happens in the body.

In a second study published online in the journal Science, Yale researchers took a promising step toward the long-term goal of growing functioning lung tissue for transplant.

First, they removed an adult rat lung and carefully removed all of its living cells. Removing the cells was like taking eggs from an egg carton; it left behind a delicate matrix structure of the lung.

Then, using cells from baby rat lungs, they were able to use that matrix as a scaffold to build a functioning lung that worked in adult rats for up to two hours. Such techniques are at least two decades from being used in people.

The long-term goal is developing a platform for lung replacement," said Dr. Laura Niklason, a professor of anesthesiology and biomedical engineering at Yale, who led the work. "I've seen a lot of lung disease — and lungs don't get better very well. They don't heal themselves very well.

Boston-area researchers have long played a leading role in the attempt to engineer tissues and organs.

Pioneering efforts by local researchers have included a "biorubber" material that could be used to help craft blood vessels, cartilage, and heart tissue, to making a liver-on-a-chip, which could be used to test drugs for toxicity.

At the Wyss Institute, researchers are also crafting a gut-on-a-chip and cancer models. Last year, Harvard researchers announced they had created a pulsing strip of heart muscle from mouse embryonic stem cells.

Carolyn Y. Johnson can be reached at [cjohnson@globe.com](mailto:cjohnson@globe.com).

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**From:** Dutt's Logger <[pdlogger@WINDSTREAM.NET](mailto:pdlogger@WINDSTREAM.NET)>

**Date:** June 27, 2010 8:43:59 AM EDT

**Subject:** [ALPHA-1] FYI: CHIPPING AWAY 6/27/2010

Now, living, breathing human lung-on-a-chip  
Washington: American scientists have used a microchip to develop a device that mimics a living, breathing human lung.

Researchers from the Wyss Institute for Biologically Inspired Engineering at Harvard University, Harvard Medical School and Children's Hospital Boston collaborated to come up with the device.

About the size of a rubber eraser, it acts much like a lung in a human body and is made using human lung and blood vessel cells.

Because the lung device is translucent, it provides a window into the inner-workings of the human lung without having to invade a living body.

It has the potential to be a valuable tool for testing the effects of environmental toxins, absorption of aerosolised therapeutics and

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the safety and efficacy of new drugs.

Senior author Donald Ingber, founding director of Harvard's Wyss Institute, said: "The ability of the lung-on-a-chip device to predict absorption of airborne nanoparticles and mimic the inflammatory response triggered by microbial pathogens, provides proof-of-principle for the concept that organs-on-chips could replace many animal studies in the future."

He added: "We really can't understand how biology works unless we put it in the physical context of real living cells, tissues and organs."

The lung-on-a-chip microdevice takes a new approach to tissue engineering by placing two layers of living tissues—the lining of the lung's air sacs and the blood vessels that surround them—across a porous, flexible boundary.

Air is delivered to the lung lining cells, a rich culture medium flows in the capillary channel to mimic blood and cyclic mechanical stretching mimics breathing.

The device was created using a novel microfabrication strategy that uses clear rubbery materials. The strategy was pioneered by another Wyss core faculty member, George Whitesides, the Woodford L. & Ann A. Flowers University Professor at Harvard Univ.

First author Dan Huh, a Wyss technology development fellow at the Institute, said: "We were inspired by how breathing works in the human lung through the creation of a vacuum that is created when our chest expands, which sucks air into the lung and causes the air sac walls to stretch. Our use of a vacuum to mimic this in our microengineered system was based on design principles from nature."

To determine how well the device replicates the natural responses of living lungs to stimuli, the researchers tested its response to inhaled living *E. coli* bacteria.

They introduced bacteria into the air channel on the lung side of the device and at the same time flowed white blood cells through the channel on the blood vessel side. The lung cells detected the bacteria and, through the porous membrane, activated the blood vessel cells, which in turn triggered an immune response that ultimately caused the white blood cells to move to the air chamber and destroy the bacteria.

Rustem Ismagilov, professor of chemistry at the University of Chicago, who specializes in biochemical microfluidic systems, said: "The ability to recreate realistically both the mechanical and biological sides of the in vivo coin is an exciting innovation."

Huh said: "The team followed this experiment with a "real-world application of the device."

They introduced a variety of nano-scaled particles (a nanometer is one-billionth of a meter) into the air sac channel. Some of these particles exist in commercial products; others are found in air and water pollution.

Several types of these nanoparticles entered the lung cells and caused the cells to overproduce free radicals and to induce inflammation. Many of the particles passed through the model lung into the blood channel, and the investigators discovered that mechanical breathing greatly enhanced nanoparticle absorption. Benjamin Matthews, Harvard Medical School assistant professor in the Vascular Biology Program at Children's Hospital Boston, verified these new findings in mice.

Huh said: "Most importantly, we learned from this model that the act of breathing increases nanoparticle absorption and that it also plays an important role in inducing the toxicity of these nanoparticles."

Robert Langer, MIT Institute professor, said: "This lung-on-a-chip is neat and merges a number of technologies in an innovative way. I think it should be useful in testing the safety of different substances on the lung and I can also imagine other related applications, such as in research into how the lung functions."

According to Ismagilov, it's too early to predict how successful this field of research will be. Still, "the potential to use human cells while recapitulating the complex mechanical features and chemical microenvironments of an organ could provide a truly revolutionary paradigm shift in drug discovery," he said.

The research appears in the June 25 issue of Science.

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Thank you,

Joe Reidy

66 Moore Ave  
Waldwick, NJ 07463

1 (201) 444-7839

E-mail

[JoeReidy@Verizon.net](mailto:JoeReidy@Verizon.net)

print date: Wednesday, July 7, 2010

Joe Reidy

66 Moore Ave.

Waldwick, NJ 07463

1 (201) 444-7839

E-mail JoeReidy@Verizon.net



# Alpha-1 Nor'Easter (July 2010)

## Regional Events

more info in "Support Group News" on page 1

Sunday 7/18

Pennsylvania Regional Alpha-1 Picnic  
Wummers House - Douglassville, PA

Saturday 8/21

New England Regional Education Day  
Dartmouth Hitchcock Med Cntr, VT

Saturday 9/11

A1F Fundraiser  
Plymouth Harbor Cruise, Plymouth, MA

The NY/NJ Alpha-1 Support Group (not this newsletter) is endorsed by the:

# Alpha-1 Association



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