

Executive Talk:

Notes from Executive Director, Kay James

Welcome to the Carolinas Chapter's first e-newsletter covering the events, activities and program updates in the chapter and information links to the national office. Our chapter covers a very large geographic area from the western Triad region to the Eastern Coastal area and from the Virginia to South Carolina borders. Our CFF events are as diverse as the communities they reflect. But we are unified with the common goal of funding the research to find a cure for cystic fibrosis. We are driven by this urgency for our patients their families and friends.

Over the past months great progress is being made toward finding that cure. You will read more about that in this edition about the pharmaceutical pipeline including the successes with VX-770 and Denufosol. We are greatly encouraged by those successes but cannot rest until the cure is found. As you read this document and link to our website I hope that you will find a place for yourself to become engaged or more deeply engaged. We are going to find a cure someday. You can be a part of that life saving success that changes the course of medical history. Start today. Volunteer.

One Step Closer to a Cure: 2008 GREAT STRIDES Walk Sites Totals

Fayetteville, NC – Saturday, April 12th, 2008 – Total Raised: \$ 17,600

Chapel Hill, NC – Saturday, April 19th, 2008 – Total Raised: \$ 93,300

Durham, NC – Saturday, April 19th, 2008 – Total Raised: \$ 64,500

Mayodan, NC – Saturday, April 26th, 2008 – Total Raised: \$ 27,800

Wilmington, NC – Saturday, April 26th, 2008 – Total Raised: \$ 45,700

Winston-Salem, NC- Saturday, April 26th, 2008 – Total Raised: \$ 156,000

Greensboro, NC – Thursday, May 1st, 2008 – Total Raised: \$ 29,600

Greensboro, NC – Saturday, May 3rd, 2008 – Total Raised: \$ 56,000

Cary, NC – Saturday, May 3rd, 2008 – Total Raised: \$ 99,500

Raleigh, NC – Saturday, May 17th, 2008 – Total Raised: \$ 106,500

Elizabeth City – Saturday, September 13th, 2008 – Total Raised: \$ 34,200

Outer Banks – Saturday, September 13th, 2008 – Total Raised: \$ 50,000

2008 GREAT STRIDES

Total to Date: \$ 780,700!!!

Thank you to our teams, walkers, sponsors, and committees! Without your hard work and dedication to the fight against CF this kind of success would never be possible.

We appreciate all that you do.

We cannot wait to see what we can achieve together in 2009!

From the Front Lines: National CFF Headlines

Foundation Announces Positive Results for VX-770 – An Investigational Drug that Could Treat Basic Defect

October 20, 2008

The CF Foundation announced today that VX-770, an investigational oral drug aimed at treating the basic defect in cystic fibrosis, showed positive results in a 28-day, Phase 2 clinical trial. These data bolster the successful interim results reported from the first part of the trial in March of this year. Both studies were done with patients who carry the G551D mutation of CF.

“Data from the Phase 2 trial of VX-770 provide evidence that a small molecule can address the basic defect in cystic fibrosis, and suggest that modulation of CFTR may play an important role in CF therapy,” said Robert J. Beall, Ph.D., president and CEO of the CF Foundation.

VX-770 is being developed and tested by Vertex Pharmaceuticals Incorporated, with support from the CF Foundation. The potential drug aims to open the chloride channels that do not function correctly in people with CF.

Going Forward

Based on the positive results from this trial, Vertex plans to work with global regulatory authorities, including the U.S. Food and Drug Administration, to move VX-770 forward in the approval process. The company is targeting the first half of 2009 to initiate a pivotal trial that could eventually lead to drug approval.

The Foundation will work with Vertex to identify which CF patients this drug may work for going forward. [Read more about about participating in CF clinical trials.](#)

Detailed data from the VX-770 clinical trial will be presented this week at the 2008 North American Cystic Fibrosis Conference. [Read the full Vertex press release here.](#)

Researchers Produce First Pig with Cystic Fibrosis

September 26, 2008

Foundation-supported researchers announced today they have developed the first promising animal model for CF research. Scientists at the University of Iowa and the University of Missouri have successfully produced pigs born with cystic fibrosis that mimic the exact symptoms of a newborn with CF.

This new model will help improve understanding of the disease and may also speed discovery of new treatments.

North Carolina and Nevada Step up Efforts to Screen Newborns for Cystic Fibrosis

August 26, 2008

North Carolina approved legislation this July to require mandatory screening of all newborns for cystic fibrosis, effective January 2009. Additionally, Nevada began conducting screening after approving the process earlier this year.

The two states join 43 others and the District of Columbia in requiring that all newborns be screened for cystic fibrosis at birth. In addition, Utah will also begin screening in January.

Research studies show that newborn screening for CF will likely improve and extend the lives of those born with the disease. Early intervention has been shown to improve height, weight, nutrition, cognitive and lung functioning, and to reduce hospitalizations. The Foundation strongly urges all states to routinely screen newborns for the disease.

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Cystic Fibrosis Foundation Announces Positive Early Results for Phase 2 Clinical Trial of VX-770 – an Oral Compound to Treat CF

--Emerging Data Shows Encouraging Response in CF Patients

--Results Validate Investment in Venture Philanthropy Business Model

March 27, 2008

(Bethesda, MD)—The Cystic Fibrosis Foundation announced today that VX-770, an oral drug in development that targets a basic defect in CF, showed promising results in an ongoing Phase 2a clinical trial for patients who carry the G551D mutation of CF. The drug is being developed by Vertex Pharmaceuticals Incorporated.

Patients who took the drug for 14 days showed significant improvements in several key indicators of cystic fibrosis, including lung function, nasal potential difference measurements and sweat chloride levels. The findings suggest that VX-770 improves function of what is known as the faulty CFTR protein. This early data is promising and could have important implications for studies of other drugs in development.

This is the first time that any potential therapy has improved the abnormal sweat chloride (salt) levels in a person with CF. Excessive sweat chloride is a key clinical indicator of cystic fibrosis. The “sweat test” is the traditional diagnostic test for CF.

“These early results are an extraordinary endorsement of our hypothesis—that small molecules can correct the basic defect and affect the clinical indicators of cystic fibrosis,” said Robert J. Beall, Ph.D., president and CEO of the Foundation. “The emerging data for VX-770 represents the most exciting results we’ve seen from a Phase 2 trial and increase our confidence that we’re on the right track.”

The compound VX-770 resulted from a collaboration between the Foundation and Vertex. In 1998, the Foundation approached Aurora Biosciences (acquired by Vertex in 2001) and made an initial investment to use cutting-edge technology—known as high throughput screening—to find compounds to attack the core defect in CF. Since that time, the Foundation has invested \$79 million in the project, and the two organizations have closely collaborated to advance VX-770 and a second CF compound, known as VX-809, through research and into development.

“At a time when the model of venture philanthropy was wholly untested, the Cystic Fibrosis Foundation was willing to take a chance that Vertex’s CF program would yield meaningful results for people with cystic fibrosis,” said Joshua Boger, Ph.D., president and CEO of Vertex Pharmaceuticals. “The project carried significant risk that would have dissuaded many companies and investors from moving forward. Today, however, with the Foundation’s financial, scientific and clinical network support, we have been able to bring two novel CF compounds into development and are encouraged by these early results for VX-770.”

Overall, the Foundation has invested nearly \$300 million in CF drug research with dozens of biotech collaborators nationwide. These collaborations with for-profit companies are part of the Foundation’s unique venture philanthropy business model. The model, which is being emulated by other orphan disease organizations, has spawned a drug pipeline of more than 30 therapies in development.

Part One of the Phase 2a trial of VX-770 studied 20 cystic fibrosis patients over a 14-day period. Part Two of the study is expected to begin in the second quarter of 2008. Additional studies will evaluate the longer-term safety and efficacy of the compound.

Cystic fibrosis is a life-threatening genetic disease that affects about 30,000 people in the United States and 70,000 worldwide. CF is caused by a genetic mutation that results in a malfunctioning (or missing) protein that results in an imbalance of salt and water. This imbalance causes a cascade of mucus plugging, infection and inflammation in the lungs and other organs. The primary cause of death in CF is lung disease.

The predicted median age of survival for people with CF is age 37 – more than double what it was 25 years ago.

About the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation is the leading organization devoted to curing and controlling cystic fibrosis. Headquartered in Bethesda, Md., the Foundation funds CF research, has 80 chapter and branch offices throughout the country, and accredits a nationwide network of 115 CF care centers, which provide vital treatments and other CF resources to patients and families.

To advance the search for a cure, CFF has invested nearly \$300 million in promising scientific research in the biotechnology industry since 1998. As a result, the Foundation has more than 30 potential therapies in its drug discovery and development

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pipeline. Virtually every approved CF drug available today was made possible because of Cystic Fibrosis Foundation support. For more information visit www.cff.org.

Senate Declares May National CF Awareness Month

April 29, 2008

The Cystic Fibrosis Foundation praised the U.S. Senate for officially recognizing May as National Cystic Fibrosis Awareness Month and encouraging public awareness and understanding of CF.

Introduced by Senators Patty Murray (D-Wash.) and Jim Inhofe (R-Okla.), the legislation calls attention to cystic fibrosis, a fatal, genetic disease, and supports research to find a cure.

"We are thrilled to have the Senate's support for our mission to find a cure for cystic fibrosis. We are making dramatic progress in the development of new therapies for the disease and in extending life expectancy," said Robert J. Beall, Ph.D., president and CEO of the Foundation. "On behalf of all those affected by CF, we thank Senators Murray and Inhofe for their leadership and commitment to the cystic fibrosis community."

Inspire Announces Positive Results from Phase 3 Clinical Trial of Denufosol

June 6, 2008

Inspire Pharmaceuticals, Inc. announced today positive results from a Phase 3 clinical trial of denufosol, an inhaled therapy for the treatment of CF. Denufosol is designed to improve chloride transport in the airways and enhance mucus hydration and clearance.

The study, called TIGER-1, involved 352 patients over a 24-week period. It showed significantly improved lung function in patients taking the drug. The data from this trial is particularly encouraging because it brings the CF community one step closer to a novel treatment that addresses the basic defect in CF.

"The results from this trial are exciting because they demonstrate that denufosol's mechanism of action, activation of an alternative chloride channel that helps to hydrate and clear mucus from the lungs, had a clinically-meaningful impact on lung function," said Frank J. Accurso, M.D., director of the Cystic Fibrosis Center and professor of pediatrics at the University of Colorado School of Medicine in Denver, and lead investigator of the TIGER-1 study.

Unlike drugs in the Foundation's pipeline that address the basic defect by correcting the faulty CF protein, denufosol treats this defect by using an alternative chloride transport channel to allow sodium and chloride to move in and out of the cell.

Denufosol is the result of work that has been supported by the Foundation for more than 17 years, beginning with a grant from the Research Development Program to the University of North Carolina at Chapel Hill. This support enabled Richard Boucher, M.D., a researcher at UNC, to understand the science of alternate chloride channels, leading to the development of denufosol.

"The Foundation has worked closely with Inspire from the beginning of this program and provided funding for a key Phase 2 clinical trial. This validates our business model to support highly innovative potential new treatments for CF, and provides even more hope and optimism for people with cystic fibrosis," said Robert J. Beall, Ph.D., president and CEO of the Foundation.

TIGER-1 is the first of two Phase 3 trials conducted by Inspire. The second Phase 3 trial, TIGER-2, is currently enrolling 350 patients for a 24-week trial. [Read more about participating in the TIGER-2 denufosol trial.](#)

"We want to extend a special thank you to the hundreds of patients who participated in the first Phase 3 trial of denufosol," said Beall. "Because of your participation, we're able to move therapies to the marketplace that truly make a difference in the lives of those with CF."

Upcoming Community Events

“John Lennon and Me”

November 15, 16, and 17th at St. Mary’s School
A play by Cherie Bennett performed
by the talented young women of St. Mary’s School.
Tickets are \$7.00 at the door.

Please come join us for this night of humor and drama that focuses on the incredible relationship between two young women in the face of severe illness. This play raises awareness about CF and also serves as the kick off for the St. Mary’s “Cents for CF”, a change drive to benefit the Cystic Fibrosis Foundation.

Friday, November 14th at 8:00 pm.
Saturday, November 15th at 8:00 pm.
Sunday, November 16th at 2:00 pm.

Carolina Hurricanes CF Night

November 12th, 2008

Hurricanes vs. Washington Capitols

7:00 pm

**See www.hurricanes.nhl.com or call
919-845-2155 for more information**

Hope to see you there!

. Don't Miss Out on CFF Carolinas Chapter Events.



Cape Fear Red Trout Classic/Redbone Fall 2009

The 7th annual fishing tournament will be held at Figure 8 Island and will kick off on Thursday evening with a Captain's Party and Auction followed by 2 days of fishing concluding on Saturday with an awards ceremony and dinner. 2008 was a great success; please don't miss your chance to join us in 2009.



An Evening with Master Chefs April 5, 2009

Join us for our annual gala at The Carolina Club in Chapel Hill, NC. The evening features the Triangle's best chefs and a multi-course culinary feast of their design, paired with fine, unique wine samplings. *An Evening with Master Chefs* also includes entertaining guests and an exciting live and silent auction. This is an event not to be missed so mark your calendars now!



TAKING STEPS TO CURE CYSTIC FIBROSIS

GREAT STRIDES 2009 is right around the corner! It is never too early to begin to work with your staff partner to get an early start to your team recruitment, corporate sponsorship recruitment, and fundraising ideas! Contact us today!

Deanna Wilson:

Fayetteville, NC – April 25, 2009
Durham, NC – April 18, 2009
Outer Banks, NC – September 12, 2009
Elizabeth City, NC – September 26, 2009
Raleigh, NC – May 18, 2009
Cary, NC – May 9, 2009

Sophia Olson:

Wilmington, NC – May 16, 2009
Winston-Salem, NC – April 25, 2009
Chapel Hill, NC – May 2, 2009

Stephanie Davage:

Greensboro, NC – May 7 & 9, 2008
Apex, NC – May 30, 2008

Megan Cole:

Mayodan, NC – May 9, 2009



Calling ALL Students!

Want to help the Cystic Fibrosis Foundation take steps to cure CF? Join our CureFinders team! We are accepting nominations for qualified student leaders to join our *CureFinders* Student Youth Board. Students on our board will learn the fundamentals of communication, problem solving, and gain valuable leadership experience as they conduct the first *CureFinders* campaign in our area. Students will receive training on the operations and responsibilities of a working volunteer board. This is a wonderful opportunity for your students to build their college resumes or to earn community service hours.

We are looking for outgoing, energetic students to:

- **Attend four meeting per year**
- **Identify and train incoming board members**
- **Coordinate a *CureFinders* fund-raising program**
- **Identify and recruit school contacts to participate in *CureFinders***
- **Raise awareness about cystic fibrosis (CF) in their community**

The ideal candidate is an outgoing, a leader within your community, with a great sense of follow through, and a good academic record. We are limiting this honor to two students from each participating school.

If you have a student or students whom you would like to recommend for a position on the Cystic Fibrosis Foundation's *CureFinders* Student Youth Board please contact Megan Cole by phone (800) 822.9941 or at mcole@cff.org for more information.

Not old enough to join our Youth Board? Get your school involved instead. We have opportunities for all ages to be a part of finding the cure for CF.

Please contact Megan Cole by phone (800) 822.9941 or at mcole@cff.org for more information.

CFF Recent Event Round Up

The Cape Fear Red Trout Celebrity Classic was a huge success that raised \$45,000! Thank you to all of our gala guests, anglers, and guides. Also to our amazing committee, thank you for all you do to fight CF! Join us again fall 2009. This is one fishing experience you won't want to miss! Call now to sign up!

Puttin' on the Grits Again was an evening of spectacular Southern food created by Chef Pete Baxley and entertainment provided by the delightful cast of Clyde Edgerton, Celia Rivenbark, and Nan Graham. Thank you to all of our treasured guests, hugely talented speakers, and hard working committee members that made this event possible. This evening of fabulous Southern humor raised almost \$9,000!

National Spotlight on the Carolinas

Small Walks Equal Big Bucks for the Cystic Fibrosis Foundation

October 17, 2008

Some of the best things come in small packages. Take the **Carolinas Chapter-Raleigh Office's** Outer Banks **GREAT STRIDES** walk, for example—in its second year, the small community walk experienced a whopping 50 percent growth in donations to raise \$47,000. And the **Western New York Chapter's** rural Salamanca walk raised nearly double the previous year's amount!

Details That Made A Difference

Branching out was the key to success for both walk sites. Determined to grow the number of walk teams, the Salamanca GREAT STRIDES committee asked each committee member to recruit one new team or expand their own team—leading to five new walk teams and more than \$16,000 raised in 2008.

After participating in last year's first-annual Outer Banks walk, Reilly's Rebels Fighting for a Cure Team Leader Lisa Winslow and daughter Cindy split off to launch the nearby Elizabeth City walk. Held in the small North Carolina farming community, Elizabeth City managed to raise an impressive \$31,000 through mom and pop businesses and strong community support.

"We are proof that several dedicated families *can* make a difference," said Lisa Winslow, whose granddaughter Reilly has CF. "All it took was a well-developed plan, some great, caring and passionate people to work that plan and always remembering why we're working so hard—to find a cure for cystic fibrosis."

Thanks a Million

Many thanks to the following top family and corporate teams:

Elizabeth City – Family Teams:

- Ava's Angels
- Faye's Fighters
- Reilly's Rebels Fighting for a Cure
- Reilly's Rebels 2

Outer Banks – Family Teams:

- Blogger Joggers
- Church of the Outer Banks
- Embellishers
- Patty Sue
- Southern Hope

Elizabeth City – Corporate Teams:

- Long and Foster
- BB&T
- Team HREM

Outer Banks – Corporate Teams:

- Atlantic Financial Services
- Knapp and Lowman
- Nags Head Fire rescues
- Outer Banks Wedding association
- Kitty Hawk Kites



Tina Hodgson, Marie Anderson and Annette McCargo of Team Southern Hope assist with registration on the day of the Outer Banks GREAT STRIDES walk.

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