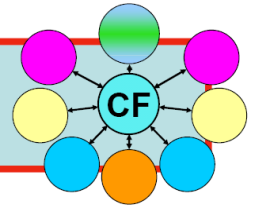


Center for Cystic Fibrosis Research

Advancing Wellness in CF Patients Through Research



June 2010
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Welcome to the Inaugural Newsletter for the New CF Research Center!

Children's Healthcare of Atlanta (Children's), in collaboration with the Department of Pediatrics at Emory University, announced on December 18, 2009, the establishment of the new Center for Cystic Fibrosis Research.

Cystic Fibrosis (CF) is the most common life-shortening, autosomal recessive disease among Caucasians in the United States. Although substantial improvements in quality and longevity of life for CF patients have been achieved over the seven decades since the disease was first described, the median predicted age at death remains less than half of that for the non-CF population; children and young adults are still dying from this disease. What will it take to solve this problem?

We need to find new ways to think about CF, new ways to focus on wellness, to turn this "disease" into a "condition". We need new ways to control this disease for better outcomes, which will result from research into the complex nature of the CF lung.

Why a CF Research Center?

Nael McCarty, PhD

Emory University, Children's, and the CF Foundation have made large investments in CF care and research at the Emory-Children's CF care center over the past two decades, including support for clinical operations, the hiring of new clinician-scientists and basic scientists dedicated to CF research, and the recent establishment of an independent adult CF program.

Important Fact:

The Emory-Children's CF center, combined with the affiliate CF program at Children's-Scottish Rite, serves more pediatric and adult CF patients than any other CF care center in the country.

With support from Children's, we have established the Center for CF Research: a collaborative research team centered around translational and basic research in CF that takes advantage of the availability of CF patients at our CF care center and other assets at Children's, Emory, Georgia Tech, and elsewhere in Atlanta. The Center will support research projects and cores that will draw together investigators with various expertise to solve critical problems associated with CF disease.

The goal of the Center for Cystic Fibrosis Research is to engage Atlanta researchers in basic and translational research that will lead to a better understanding of the pathophysiology of this disease and/or generate new devices and treatments to increase the length and quality of life for CF patients. To achieve this goal, the Center for CF Research will make available to Atlanta area investigators a set of resources to enable research that directly impacts CF.

Major Components:

- Pilot project program
- Specialized CF research cores:
 - CF Clinical Research Lab
 - CF Discovery Center including the CF Biospecimen Registry
 - CF Mouse Models Core
 - Computational Core (GaTech)
- Faculty recruitment program
- Seminars and workshops
- Administration

Building a Program

Building a sustainable program in CF research that is successful in garnering extramural grant support requires a two-pronged approach.

1) Recruitment Plan:

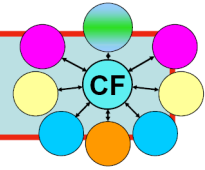
- to attract new faculty, committed to CF, into the Department of Pediatrics as well as into basic science and clinical departments

2) Investment Program:

- to draw together Atlanta researchers (at Emory, Georgia Tech, Morehouse School of Medicine, Children’s Healthcare of Atlanta, etc.) via pilot projects, cores, appropriate resources

Center for Cystic Fibrosis Research

Advancing Wellness in CF Patients Through Research



Who is Involved?

Active Center Members:

Grey indicates investigators outside of Emory Department of Pediatrics

- Gang Bao, Ph.D.*
- Dana Barr, Ph.D.*
- Charlene Bayer, Ph.D.*
- Karen Bernard, Ph.D.**
- Barbara Boyan, Ph.D.*
- Kenneth Brigham, Ph.D.*
- LouAnn Brown, Ph.D.**
- Robert Butera, Ph.D.*
- Daniel Caplan, M.D.**
- Rachel Chen, Ph.D.*
- Richard Compans, Ph.D.*
- Guiying Cui, M.D./Ph.D.**
- Richard Cummings, Ph.D.*
- Lyndsey Darrow, Ph.D.*
- Meghan Duffy, Ph.D.*
- Doug Eaton, Ph.D.*
- Brian Evavold, Ph.D.*
- Facundo Fernandez, Ph.D.*
- Anne Fitzpatrick, Ph.D.**
- Otto Froehlich, Ph.D.*
- Greg Gibson, Ph.D.*
- Roby Greenwald, Ph.D.*
- Ruth Grossman*
- Jeannette Guarner, M.D.*
- David Guidot, M.D.*
- Robert Guldberg, Ph.D.*
- Jason Hansen, Ph.D.**
- Criss Hartzell, Ph.D.*
- Steve Harvey, Ph.D.*
- My Helms, Ph.D.*
- David Hertling, Ph.D.*
- Peter Hesketh, Ph.D.*
- William Hunt, Ph.D.*
- Eric Hunter**
- Daniel Infield**
- Robert Jerris, Ph.D.*
- Dean Jones, Ph.D.*
- King Jordan, Ph.D.*
- Melissa Kemp, Ph.D.*
- Steve Kenney, Ph.D.*
- Jochen Kessler, Ph.D.*
- Irena Kizer**
- Taylor Knotts**
- Kostas Konstantinidis, Ph.D.*
- Michael Koval, Ph.D.*
- Namrita Kumar*

- Joseph LeDoux, Ph.D.*
- Allan Levey, M.D.*
- Laura Mazer*
- Nael McCarty, Ph.D.**
- Al Merrill, Ph.D.*
- Mindy Millard-Stafford, Ph.D.*
- Martin Moore, Ph.D.**
- Andrew Muir, M.D.*
- Sankar Nair, Ph.D.*
- Robert Nerem, Ph.D.*
- Wylie Nichols, Ph.D.*
- Youngja Park, Ph.D.*
- Christine Payne, Ph.D.*
- Jeannie Peabody, R.N.**
- Junmin Peng, Ph.D.*
- Lawrence Phillips, M.D*
- Jan Pohl, Ph.D.*
- Samuel Quaynor**
- Phil Rather, Ph.D.*
- Timothy Read, Ph.D.*
- Mauricio Rojas, Ph.D.*
- Rene Romero, M.D.**
- Jeff Sands, M.D.*
- Jeremy Sarnat, Ph.D.*
- Michael Schechter, M.D.**
- Peter Scott, M.D.*
- William Shafer, Ph.D.*
- Dawn Simon, M.D.**
- Ioanna Skountzou, M.D.*
- David Smith, Ph.D.*
- Trent Spencer, Ph.D.**
- Arlene Stecenko, M.D.**
- Matthew Strickland, Ph.D.*
- Viranuj Sueblinvong, M.D.*
- Vin Tangpricha, M.D.*
- Peter Thule, M.D.*
- Paige Tolbert, Ph.D.*
- David Tong, M.D.*
- Natalia Toporikova, Ph.D.*
- Brani Vidakovic, Ph.D.*
- Eberhard Voit, Ph.D.*
- Joshua Weitz, Ph.D.*
- Lindy Wolfenden, M.D.*
- Tom Ziegler, M.D.*
- Susu Zughaier, Ph.D.*
- Michael Zwick, Ph.D.*



Meet the Director



Nael McCarty, PhD

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The Children's Center for CF Research is led by Dr. Nael McCarty as its Director, along with Drs. Arlene Stecenko and Michael Schechter as Co-Directors. In each of the first three editions of this newsletter, we will highlight one of these leaders.

Nael A. McCarty, Ph.D., is Senior CF Scientist, Associate Professor, and Associate Chief for Research in the Division of Pulmonology, Allergy/Immunology, CF, and Sleep, within the Department of Pediatrics at Emory. He holds a Bachelor of Science degree in Systematics and Ecology from the University of Kansas, and a Master of Science degree in Marine Physiological Ecology from the University of North Carolina at Wilmington. He earned the PhD in Cell Physiology from the University of Texas Health Science Center at Houston, before receiving postdoctoral training in molecular physiology in the lab of Dr. Henry Lester at the California Institute of Technology. It was during these years at Caltech that Dr. McCarty began his work on the study of CFTR, the protein defective in CF, where he made early contributions to our understanding of the function of this fascinating molecular machine.

Dr. McCarty joined the Emory University Department of Physiology in 1994, and soon thereafter began working with the clinician-scientists at the Emory CF Care Center, leading to an adjunct appointment in Emory's Department of Pediatrics. He was instrumental in the early phases of developing the clinical and translational research activities of the Emory CF Center, especially via building the capabilities for specialized assays that serve as important outcome measures for testing the efficacy of CF therapeutics in our patients; our Center was (and is) one of a very small number of CF care centers capable of performing these specialized assays.

In 2001, Dr. McCarty moved his lab group to Georgia Tech, where he was Associate Professor of Biology. Late in 2007, Dr. McCarty was recruited back to Emory, into his current position in the Department of Pediatrics. Even before returning to Emory, Dr. McCarty began gathering together the interest of clinicians, scientists, and engineers at Emory, Georgia Tech, and Children's, with advisement from members of the CF Foundation and the CF community outside of Georgia, to build toward the establishment of a new CF research center.

His broad training now makes him somewhat unique in the ability to apply a variety of perspectives to our efforts to grow a multidisciplinary, multi-investigator research center that will bring innovative ideas and approaches to our understanding of CF and ability to control this disease. In his role as Director of the Children's Healthcare of Atlanta Center for CF Research, he is working to tie pulmonology researchers to both engineers and investigators with expertise in systems biology approaches.

Dr. McCarty's research has been funded by the National Institutes of Health, the CF Foundation, the

National Science Foundation, the American Heart Association, Children's Healthcare of Atlanta, and others. He directs an active laboratory that focuses primarily on basic and translational research related to CF, with currently nine lab members.

Dr. McCarty is an active member of St. Luke's Presbyterian Church in Dunwoody, GA. He and his wife, Mrs. Liz McCarty, Director for Business and Finance in the Emory Department of Pediatrics, have five children, 24-17 years old. Aside from loving his many-faceted job, Dr. McCarty's favorite activities are spending time with family, hiking, and learning new things.

Important Links:

Children's Healthcare of Atlanta Center for Cystic Fibrosis Research
www.pedsresearch.org/researchcenter_overview.htm#cysticfibrosiscenterofexcellence

Cystic Fibrosis Foundation
www.cff.org

Great Strides
www.cff.org/great_strides

Emory-Childrens Center Research Office
www.pedsresearch.org

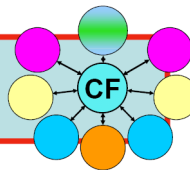
Emory University Department of Pediatrics Research Newsletters
www.pedsresearch.org/newsletter.html

Upcoming Events:

Chocolate! 2010
Thursday, June 17, 6-10 PM at Villa Christina

It's a wonderful way to support a cause, but a sinful way to support a diet! Chocolate! 2010, the tenth annual fundraiser for The Cystic Fibrosis Foundation is back for another year of Pure Indulgence. Experience Chocolate tastings from Atlanta's premier pastry chefs, martini tastings, a full dinner buffet, silent auction and much more!

For more information, contact:
Jennifer Hagen at 404-325-6973 or
jhagen@cff.org



Senior Faculty Positions Available

The Children's Center for CF Research is actively recruiting new faculty.

The **Center for CF Research**, a collaborative research team of ~70 scientists, clinician/scientists, and engineers centered around translational and basic research in CF, takes advantage of a variety of assets at Children's, Emory, the nearby Georgia Institute of Technology, and elsewhere in Atlanta. Researchers supported by the Center are using a variety of approaches to characterize the changes in activities of the cell types that make up the airway community – the epithelial cells, the macrophages, the neutrophils, *etc.* – and in the signals by which they communicate, to identify those changes that are associated with progressive lung disease. A goal of the Center is to apply systems biology approaches and computational modeling to understand how these components of the community interact. **The Center is now recruiting senior, NIH funded basic and/or translational scientists** with a research emphasis that is relevant to CF and falls into one of the following areas: **airway immunology, systems biology of chronic disease, or airway microbiology**. Faculty with expertise in other areas relevant to CF also will be considered.

Please help us identify superb candidates, and inform appropriate colleagues of the availability of these positions.

Current Funded Pilot Studies

As part of the initial building of the Center for CF Research, three pilot projects have been funded. These projects are as follows:

Title of Project:

Genome-wide gene expression profiling in CFRD

Award period: March 1–Feb 28, 2011
Amount of Award: \$80,000
PI s: Greg Gibson, Arlene Stecenko

Title of Project:

Colonic Bicarbonate Transport in Cystic Fibrosis

Award period: March 1–Feb 28, 2011
Amount of Award: \$75,000
PIs: Chris Hartzell, Nael McCarty

Title of Project:

Protein Biomarker Discovery in Cystic Fibrosis

Award period: March 1–Feb 28, 2011
Amount of Award: \$45,000
PIs: Junmin Peng, Nael McCarty



Three Major Funding Initiatives

The Center's three major extramural research funding initiatives targeted for the coming year:

1) NIH multidisciplinary proposal:

“Revolutionizing Healthcare for CF: The D.O.A.P.E. Study”

Overall PI: Nael McCarty
Proposal submitted: March 15
Project period: Oct 2010–Sept 2013
Budget: \$6.2M total costs

2) NIH Translational Program Project Grant proposal:

“Mechanisms and Control of CF-related Diabetes”

Overall PI: Arlene Stecenko
Proposal to be submitted: Sept. 2010
Project period: May 2011–April 2016
Budget: ~\$13M total costs

3) CF Foundation Research Development Program grant:

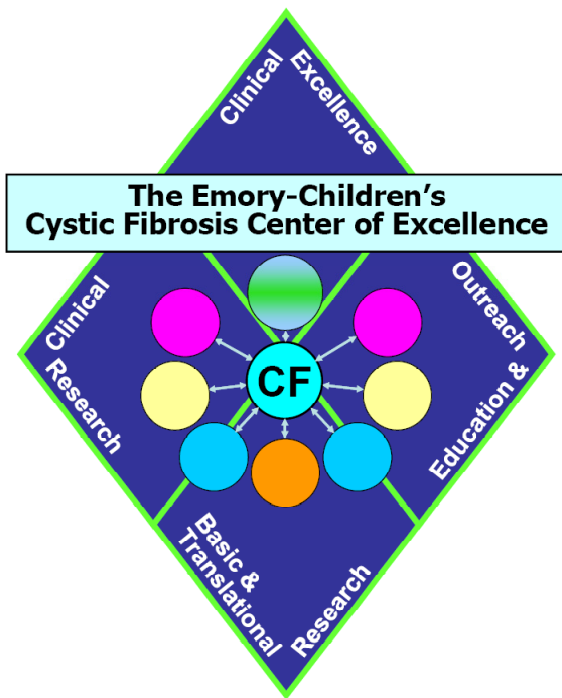
“Emory-Children's CF Center of Excellence”

Director/PI: Nael McCarty
Proposal to be submitted: Oct. 2010
1st project period: June 2011–May 2015
Budget: ~\$2.2M total costs

Look for updates on these initiatives in the coming months.

The Bigger Picture: A Comprehensive CF Center of Excellence

Over the past several years, the CF program at Emory and Children’s has grown to include excellence in **Clinical Care**, **Clinical Research**, and **Education & Outreach**. The only missing piece was a strong program in **Basic & Translational Research**; this last piece is now provided by the Children’s Center for CF Research. CF researchers, clinicians, and educators are now working together to leverage these commitments, our unique inter-institutional strengths, and our relationships with scientists and engineers at Georgia Tech to establish a Center of Excellence in Cystic Fibrosis Research, Education, and Care.



Mission Statement for the Emory-Children’s Cystic Fibrosis Center of Excellence:

“to build a comprehensive program that will not only continue to provide the highest quality care but also will lead to new breakthroughs in CF research that will advance the quality and longevity of life for our patients.”

The goals of the Emory-Children’s Cystic Fibrosis Center of Excellence reflect each of the four components of the Center, as follows:

Clinical Excellence

Provide the highest quality of care for our CF patients, such that ours is recognized as the CF program with the best outcome measures in the country.

Clinical Research

Become the best center in the country for clinical research that helps bring to CF patients new therapies, drugs, and devices that improve the quality and longevity of their lives.

Education & Outreach

Train the next generation of top CF clinicians, researchers, and other healthcare workers in interdisciplinary care, and also support education of CF patients and their families.

Basic and Translational Research

Foster the growth of the premier internationally-recognized interdisciplinary CF basic and translational research program that leads to new breakthroughs which will advance a deeper understanding of the mechanism of CF disease and thus identify completely new areas for clinical intervention. In addition, bring substantial new grant dollars, attract philanthropic support, and form a focus for recruiting stellar researchers by partnering with other Atlanta institutions to cure and control this high-visibility disease.

We are seeking funding for the Center of Excellence from Children’s, Emory, the CF Foundation, and others, in order to make these goals become realities. With the status of our current comprehensive CF care programs, the substantial opportunities made possible by strengths that exist in combination only in Atlanta, and the incredible momentum evident in our team of scientists, engineers, clinicians, and clinician-scientists, **this effort is poised to revolutionize CF care to make it personalized, predictive, proactive, and preventative, enabling Prospective Healthcare for CF.**

Working Groups for the Center of Excellence

In order for the Emory-Children’s CF Center of Excellence to accomplish all of its goals, and to be something of which Atlanta can be proud, input will be needed at a variety of levels. Therefore, the following boards and committees are being formed.

Members include:

Internal Advisory Board:

- Ken Brigham, MD
- Ray Dingledine, PhD
- Robert Guldberg, PhD
- Allan Levey, MD
- Barry Shur, PhD
- Barbara Stoll, MD
- William Woods, MD

(External Advisory Board: to be determined)

Program Steering Committee:

- Richard Cummings, PhD
- David Guidot, MD
- Nael McCarty, PhD
- Robert Nerem, PhD
- Michael Schechter, MD
- Peter Scott, MD
- Paul Spearman, MD
- Arlene Stecenko, MD
- Eberhard Voit, PhD
- Lindy Wolfenden, MD

Community Advisory Board:

- Ellen Bailey
- Jay Berkelhamer, MD
- Laurence Downey, MD
- David McKenney
- Sara Sanders
- Will Shearer
- Tom Stribling
- Paige Martin (ex-officio)
- Steven Wagner (ex-officio)

Outreach Activities

Emory-Children’s “Great Strides” Team Raises ~\$30,000 for the CF Foundation!

The Cystic Fibrosis care and research teams at Emory and Children’s are wrapping up their major annual efforts in fundraising on behalf of the Cystic Fibrosis Foundation. On Saturday, May 15, the Georgia Chapter of the CF Foundation held its “Great Strides” walk-a-thon on the campus of Georgia Tech.

The composite CF team (the Emory-Children’s pediatric outpatient clinic, the Emory-Children’s pediatric inpatient wing at Egleston, the lab of Dr. McCarty, and the Emory adult outpatient clinic) was represented by approximately 55 members, most of whom were there for the walk. Through the generosity of our donors, the composite team raised ~\$30,000 toward the important mission of the CF Foundation: to enable research and care for CF patients in Georgia. This effort supports the Outreach & Education component of the Emory-Children’s Cystic Fibrosis Center of Excellence, which continues to partner with the CF Foundation on behalf of our patients.

