Cynthia George, MSN, FNP
Senior Director, Partnerships for Sustaining Daily Care
Cystic Fibrosis Foundation
2019 Junior Investigator Awards
2019 Junior Investigator Best Abstracts

Basic Science
Melanie Spero, PhD
Caltech

Clinical Research
Vincent Giacalone, BSc, PhD Candidate
Emory University
Mary M. Kontos Care Champion Award

In recognition of a passion for excellence, commitment to care, and advocacy for individuals with cystic fibrosis, and leadership in the CF community
Mary M. Kontos Care Champion Award

Barbara Jansma, CRNP, PNP-BC
Nurse Practitioner and CF Center
Co-Coordinator, Children's Hospital of Philadelphia

#NACFC
Mary M. Kontos Care Champion Award

Kathleen Richards, RRT
Respiratory Therapist and Pulmonary Function Lab Supervisor
Intermountain Cystic Fibrosis Center
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Barbara Jansma, CRNP, PNP-BC
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Children's Hospital of Philadelphia

Kathleen Richards, RRT
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Intermountain Cystic Fibrosis Center
The Carolyn and C. Richard Mattingly Leadership in Mental Health Care Award

In recognition of leadership and a commitment to the mental health and well-being of individuals with cystic fibrosis.
The Carolyn and C. Richard Mattingly Leadership in Mental Health Care Award

Janice Abbott, BA, PhD, PGCert, CPsychol
Professor of Health Psychology
School of Psychology
University of Central Lancashire, UK
Peter J. Mogayzel, Jr., MD, PhD, MBA
Johns Hopkins Hospital
Professor of Pediatrics,
Director, Eudowood Division of Pediatric Sciences
Director, Cystic Fibrosis Center
This isn’t the story of a medical miracle
Evolution of CF Care: Innovation and Impact
Presenter Disclosure

Peter J. Mogayzel, Jr., MD, PhD, MBA

Eudowood Division of Pediatric Respiratory Sciences
The Johns Hopkins School of Medicine

There are no relationships to disclose related to this presentation.
Jan van Grevenbroeck (1731-1807)
Venetian doctor during the time of the plague. Museo Correr, Venice
EFFECT OF PHORBOL ESTER ON CFTR mRNA LEVELS IN TET-OFF CELL LINES THAT CONTAIN A FUNCTIONAL FULL-LENGTH CFTR GENE.

Peter J. Moggezel, Jr. and Melissa A. Rosenfield
National Human Genome Research Institute, NIH, Bethesda, Maryland 20892 and
The Johns Hopkins Medical Institutions, Baltimore, Maryland, 21207.
PATH TO A CURE
MANY ROUTES, ONE MISSION
CHALLENGE

A FATAL DISEASE

5 months old

Doershuk et al, J Pediatr. 1964; 65:677-693
A therapeutic regimen for patients with cystic fibrosis

A comprehensive therapeutic regimen for patients with cystic fibrosis has been evolved, in part empirically, based on our understanding of the pathogenesis of the pulmonary lesion and in large part supported by the results of clinical and pulmonary function evaluations. The aims of this regimen are the maintenance of adequate pulmonary hygiene both prophylactically and therapeutically, effective control of the pulmonary infection, and comprehensive care of the patient. Prophylactic pulmonary therapy is instituted as soon as the diagnosis is made regardless of whether or not active pulmonary involvement is present. Pulmonary infection when present is treated specifically and intensively and is kept at a minimum by monthly clinical and laboratory evaluation of the patient.

LeRoy W. Matthews, M.D.,* Carl F. Doershuk, M.D.,** Melvin Wise, M.D., George Eddy, M.D., Harry Nudelman, M.D., and Samuel Spector, M.D.

Cleveland, Ohio
Doershuk et al, J Pediatr. 1964; 65:677-693
CF Foundation Mentoring Programs

>800 mentees
Care Guidelines
Clinical Care Guidelines

The Cystic Fibrosis Foundation provides several clinical care guidelines related to diagnosing CF.
A NEED FOR DATA

A 5 year clinical evaluation of a therapeutic program for patients with cystic fibrosis

A clinical evaluation of a comprehensive prophylactic therapeutic program for patients with cystic fibrosis is presented. Ninety-six consecutive patients were followed for 18 to 60 months (average 37 months) and evaluated with the use of a modification of the Shwachman scoring system. Eighty-two per cent of these patients showed improvement, 11% remained the same, 4% showed progression beyond their initial status, and only 3% died. None of the deaths occurred before 5 years of age. Evidence is presented supporting the desirability of early diagnosis and the early institution of an intensive prophylactic and therapeutic regimen.

Carl F. Doershuk, M.D.,* LeRoy W. Matthews, M.D.,**
Arthur S. Tucker, M.D., Harry Nudelman, M.D., George Eddy, M.D.,
Melvin Wise, M.D., and Samuel Spector, M.D.

CLEVELAND, OHIO
88.7 percent of infants diagnosed in 2018 have their first clinic encounter, genotype, or sweat test within 30 days of birth.
Bridging the Gap

CHALLENGE

VARIATION IN OUTCOMES

Optimal, Individualized Care
Historical and Predicted Survival

Adapted from Ramsey & Welsh. *Am J Respir Crit Care Med* 2017;195(9):1092-1099
Number of Children and Adults with CF, 1988–2017

- **Adults 18 Years and Older**: 53.5%
- **Children Under 18 Years**: 46.5%

**Year 2000**: CF Foundation mandates creation of adult care programs.
EnVision CF: Emerging Leaders in CF Endocrinology II Program

This award program is intended to fund training for physicians interested in developing expertise in the endocrinologic care of patients with cystic fibrosis.

Awards Overview

The Cystic Fibrosis Foundation will award up to $60,000 per year for three years for salary support, including fringe benefits, and travel for the awardee. Of the $60,000, up to $2,000 for travel may be released on a year-by-year basis.

Travel funds also cover the annual Health Professional CF Conference (HAPCF) week fall, the first HAPCF, opened the awards will be October 25-29, 2019 in Nashville, and the second HAPCF, scheduled for the Envision CF Program in October. The annual travel funding also includes support for a small group meeting each spring.

In addition, $3,000 for research costs may be requested. This award is for approximately 20 months per year, for three years.

General Guidelines and Eligibility

- Applicants must be physicians in active training in internal medicine or pediatrics with a subspecialty emphasis in endocrinology or they must be physicians trained in reproductive medicine.
- Applicants must be an assistant professor at an academic institution and, if affiliated with a Foundation-accredited program, must be affiliated with the Foundation-accredited endocrine program.
- Applicants must be in the second or third year of endocrinology fellowship training at the time of application.
- Training and evaluation plan, one of the key components of the program, must include a structured educational program.
- Applications – one from a primary care physician and one from a subspecialist physician.
Most disease foundations are passive grant-givers. The Cystic Fibrosis Foundation is a very different beast—more like a biotech financier.
Vision of the Future Population

Note: Healthy defined as FEV₁ >90%, moderate as FEV₁ 40%-90%, and advanced disease as FEV₁ <40%
Source: 2018 Revised Model for July 2018 CF Foundation Medical Strategy Retreat
Vision of the Future Population

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**Children**

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<th>~5 year vision</th>
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Vision of the Future Population

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