

CYSTIC FIBROSIS PROGRAM

Children's Hospitals and Clinics of Minnesota has a large pediatric cystic fibrosis (CF) program with more than 150 patients. Children's provides a comprehensive, coordinated, team approach to care, which supports families from time of diagnosis through young adulthood. Children's care for patients with CF ranks among the top programs nationally in key outcomes measured by the Cystic Fibrosis Foundation's (CFF) national registry.

Designated by the CFF as a Therapeutic Development Network Center in 2009, Children's has also grown significantly in CF research. Currently, Children's ranks #1 out of 77 centers in percentage of CF patients enrolled for interventional studies.

CYSTIC FIBROSIS CLINICAL RESEARCH

IRB Approved Studies Open for Enrollment

- (Twin/Sibling) Genetic modifiers system of cystic fibrosis study
- Genetic modifiers system of cystic fibrosis liver disease

IRB Approved Studies Closed for Enrollment

- (ISIS-002) Infant Study of Inhaled Saline in Cystic Fibrosis
- Evaluating the effects of yoga on children with cystic fibrosis: Pain, sleep, anxiety and depression
- (CSREP) Physical Therapy study cystic fibrosis core strengthening and respiratory exercise program
- The EPIC observational study: Longitudinal assessment of risk factors for and impact of *Pseudomonas aeruginosa* acquisition and early anti-pseudomonal treatment in children with cystic fibrosis (Protocol #EPIC-002). DNA extension actively enrolling
- The incidence of pain, other physical symptoms, and depression/anxiety in pediatric patients with cystic fibrosis: Impact on overall quality of life, and eating disorders-body image



IRB Approved Retrospective Research

- Evaluating the Effects that *Bordetella bronchiseptica* has on the CF Patient Population
- A report on the Minnesota state newborn screen for cystic fibrosis: 3 years of experience
- Incidence of Cystic Fibrosis and CFTR Mutation Distribution in the Minnesota Newborn Screen Population
- MRSA Characteristics in a Cystic Fibrosis Pediatric Population

University of Minnesota Site Referral Studies

- Biomarkers of lung injury and remodeling in cystic fibrosis. Referral to the University of Minnesota
- Prediction by Ultrasound of the Risk of Hepatic Cirrhosis in Cystic Fibrosis (PUSH)

To learn more about the Cystic Fibrosis Research Program, contact:

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CYSTIC FIBROSIS CLINICAL RESEARCH

Completed Studies

- (Tiger-II Expansion) Study 08-114: Open-Label Extension of Study 08-110—A Multi-Center Study of Denufosal Tetrasodium Inhalation Solution in Patients with Cystic Fibrosis Lung Disease. Completed 2011
- (Tiger II) A Phase 3, International, Multi-Center, Randomized, Double-Blind, Placebo-Controlled, Parallel- Group Efficacy and Safety Study of Denufosal Tetrasodium Inhalation Solution in Patients with Cystic Fibrosis Lung Disease and FEV1 \geq 75 percent but \leq 110 percent Predicted (Inspire 08-110). Completed 2011
- High-Frequency Chest Wall Oscillation in CF: An Investigation into Adherence and the Reliability of Patient Report. Completed 2010
- The EPIC clinical study: Early Pseudomonas Infection Control (Protocol #EPIC-001). Completed 2010
- Expanded access program for aztreonam lysine for inhalation in patients with cystic fibrosis and
- Pseudomonas aeruginosa airway infection who have limited treatment options and are at risk for disease progression. Completed 2010
- Prevalence and impact of depression and anxiety in CF patients and their caregivers. Completed 2010
- (Solvay 128) An open label, multi-center study to assess the safety and tolerability of Pancrelipase Delayed Release Capsules in infants and children less than seven years of age with pancreatic exocrine insufficiency due to cystic fibrosis. Completed 2009
- (Solvay 127) Pancrelipase delayed release 12,000 unit capsule in subjects with pancreatic exocrine insufficiency due to cystic fibrosis. Abstract presented at the 2009 NACFC. Completed 2009.
- Barriers to implementation of infection control guidelines for cystic fibrosis (Phase I, II and III). Completed 2009.

Publications and Abstracts

- Gavin R. Graff, MD; Karen Maguiness, MS, RD, CSP; John McNamara, MD; Ronald Morton, MD; David Boyd, PharmD; Katrin Beckmann, MSc; and Djenane Bennett, BSN: Efficacy and Tolerability of a New Formulation of Pancrelipase Delayed-Release Capsules in Children Aged 7 to 11 Years With Exocrine Pancreatic Insufficiency and Cystic Fibrosis: A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Two-Period Crossover, Superiority Study. *Clinical Therapeutics*, Vol. 32, No. 1, January 2010.
- Graff GR, McNamara J, Royall J, Caras S, Forssmann K: Safety and tolerability of a new formulation of pancrelipase delayed-release capsules (CREON) in children under seven years of age with exocrine pancreatic insufficiency due to cystic fibrosis: an open-label, multicentre, single-treatment-arm study. *Clin Drug Investig*. 2010; 30(6): 351-64.
- Miriam M. Treggiari, Margaret Rosenfeld, Nicole Mayer-Hamblett, George Retsch-Bogart, Ronald L. Gibson, Judy Williams, Julia Emerson, Richard A. Kronmal, Bonnie W. Ramsey, EPIC Study Group Early anti-pseudomonal acquisition in young patients with cystic fibrosis: Rationale and design of the EPIC clinical trial and observational study. *Elsevier, Contemp Clin Trials* (2009).
- Read L, McNamara J, Johnson M, Liu M.: MRSA Characteristics in a Cystic Fibrosis Pediatric Population. Abstract accepted for presentation at the American Thoracic Society Conference, May 2011.
- Temme R.; McNamara J.; Johnson M.; Read L.; Liu M.: Incidence of Cystic Fibrosis and CFTR Mutation distribution in Minnesota Newborns. *Pediatric Pulmonology*; supplement 33, 2010: 187.
- Christiansen J.; Thompson L.; McNamara J.; Johnson M.; Fenlon K.: Cystic Fibrosis Core Strengthening and Respiratory Exercise Program; *Pediatric Pulmonology*; supplement 33, 2010: 465.
- Laguna T.; Lin N.; Wang Q.; Powers A.; McNamara J.; Regelmann W.: A Comparison of Two Quantitative Methods of Sweat Chloride Measurement in Infants with a Positive Newborn Screen for Cystic Fibrosis. *Pediatric Pulmonology*; supplement 33, 2010: 485.
- S. O'Conner-Von; L. Johnson; E. Leighton; J. McNamara; M. Finkelstein; L. Read; M. Johnson: Symptom Profile of Pediatric patients with Cystic Fibrosis: Body Image, Pain, Sleep, and Depression. *Pediatric Pulmonology*; supplement 32, 2009: 594.