Title: Longitudinal Assessment of Risk Factors For and Impact of Pseudomonas Aeruginosa Acquisition and Early Anti-Pseudomonal Treatment in Children With CF

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Sponsor: CF Therapeutics Development Network Coordinating Center

Purpose of Research

The EPIC Observational Study is a longitudinal, prospective, observational study that was originally conducted at 59 sites. The current five-year extension study is being conducted at 54 sites.

The EPIC Observational Study will serve as a freestanding epidemiologic study of the risk factors for and clinical impact of initial Pa acquisition and anti-pseudomonal therapy. Defining the risk factors for Pa acquisition can potentially allow for preventive measures and identification of high-risk populations requiring closer monitoring. Despite rigorous data collection, previous studies have been limited by small sample sizes and by conduct at one or two centers. This study will include a much larger sample size from many more centers than previous studies. It will thus provide for more generalizable results and more precise risk estimates for previously identified risk factors for Pa acquisition, and it will allow for exploration of novel risk factors not included in earlier studies. Better understanding of the clinical outcomes associated with Pa acquisition and the outcomes associated with different types of anti-pseudomonal therapies will inform the development of rational early intervention treatment regimens. Better knowledge about temporal relationships between respiratory signs and symptoms, Pa serology, and CF airway microbiology may lead to improved strategies for early detection of Pa and could have important implications for the timing of interventions aimed at preventing or treating early Pa acquisition. Finally, this study will serve as an important source of Pa and S. aureus isolates, serum samples, and DNA samples that will be used and banked for studies designed to enhance the understanding of the pathogenesis of CF, e.g., microarray investigations of early Pa isolates, investigations to identify proteomic biomarkers of airway inflammation, and investigations to identify genetic factors related to CF disease progression, including early lung disease, and clinical outcomes

Inclusion Criteria

1. Male or female ages less than or equal to 12 years.
2. Diagnosis of CF based upon the criteria established by the 1997 CF Consensus Conference: (i) sweat chloride > 60 mEq/L by quantitative pilocarpine iontophoresis; or (ii) genotype with two identifiable mutations consistent with CF; or (iii) an abnormal nasal transepithelial potential difference, and (iv) one or more clinical features consistent with CF.
3. No prior isolation of Pa from respiratory cultures (1 or more cultures in 24 months prior to enrollment), or, if prior isolation of Pa from respiratory cultures, at least a two-year history of Pa negative cultures (1 or more cultures/year), or concurrently enrolled in the EPIC Clinical Trial.
4. Signed informed consent to participate in data submission to the CFF National Patient Registry.

5. Signed informed consent by parent or legal guardian.