The Children's Hospital of Philadelphia® | RESEARCH INSTITUTE

CHOP Research Institute Summer Scholars Program

Ronald Rubenstein, M.D., Ph.D.
Pulmonary Medicine and Cystic Fibrosis Center

Basic Research: Research most often conducted in a laboratory setting that is designed to enhance our scientific knowledge base (does not involve human subjects)

Protein Trafficking in Cystic Fibrosis

My laboratory is interested in the mechanism of action of novel small molecule drug therapies for Cystic Fibrosis (CF). These studies have led us to investigate how novel drugs may regulate the biogenesis, intracellular trafficking and function of proteins and/or epithelial ion channels that are important in CF.

- **Potential summer research project**:
  The lab has several projects focused on understanding how molecular chaperones that are regulated by sodium 4-phenylbutyrate, the prototype small molecule, mechanism based therapy for CF, may regulate the biogenesis and function of CF-relevant ion channels (CFTR, ENaC) in epithelial cells.

- **Students will learn one or more of the following techniques**:
  (1) Manipulation of specific protein expression in cultured cells by overexpression or siRNA-mediated depletion; **(2)** Immunodetection of proteins using fluorescence microscopy, immunoblotting and/or immunoprecipitation; and **(3)** Assessment of ion channel function in epithelia using Ussing chambers.

Please click here to learn more about Dr. Rubenstein