

December 1, 2006

Dear Supporters of the PCD Foundation:

It is that time of year when we reflect on what we are thankful for and focus on productive goals for the future. At the PCD Foundation, we are very grateful for the PCD community, the families, healthcare providers and researchers who are working every day to support one another and to find the cure for PCD. It has been a very busy year. The Foundation has experienced organizational growth and we have seen the long-awaited establishment of a clinical research network to study PCD. We now have a genetic test and are able to diagnose PCD through blood work for a large percentage of patients. There is new information about the role of cilia in disease being published almost daily, an exciting development that highlights how complex ciliary disorders really are and reminds us at the PCD Foundation of how much work remains to be done.

To maintain our momentum, the PCD Foundation needs the financial assistance of interested individuals. This letter explains a little bit about PCD and about the goals of the PCD Foundation.

What is PCD?

PCD stands for “primary ciliary dyskinesia,” an inherited disorder of the structure and/or function of cilia. Cilia are microscopic projections that resemble hair, although they have no relationship to actual hair. Cilia are found in nearly every cell of the body. There are several different types of cilia and they perform many important functions, including providing “cleaning” services for organs susceptible to outside contamination and sensing and adjusting chemical levels in cells. PCD is a disorder affecting a specific type of cilia that rely on coordinated motion to clean organ systems in the respiratory tract (lungs and sinuses), ears, reproductive organs and ventricles of the brain. When ciliary motion in these areas is impaired, these organ systems are prone to repeated infections and inflammation. Because any organ system that relies on ciliary motion can be affected, PCD is a multi-system disorder. However, for most people with PCD, chronic infections of the lungs and sinuses are the most prominent features of the disorder. Rarely, retinitis pigmentosa (a specific type of blindness), hydrocephalus and disorders of some abdominal organs may be present in individuals with PCD.

People with PCD experience symptoms from their disease every day of their lives. They are born sick and face a lifetime of infections and daily therapy to prevent infections. Many people with PCD live full and productive lives, but the reality of living with a chronic, progressive, life-altering condition cannot be dismissed. For some people with PCD, the disease may progress rapidly and they may succumb to early disability or death.

Why is PCD Important?

Rare diseases (disorders affecting fewer than 200,000 people) generally do not inspire much interest on the part of researchers and corporate research funders. This is because research is expensive and requires the dedication of significant resources. The perception is that the results of research into rare disorders will potentially benefit only a small number of people, therefore it is not cost-effective to do research in rare diseases. This is not the case with PCD, however. PCD causes inherited problems with a system called “mucociliary clearance.” Adequate mucociliary

clearance is an important feature of lung health for all people. Disorders of mucociliary clearance affect millions of people. COPD (chronic obstructive pulmonary disease) and asthma are two examples of disorders of mucociliary clearance. PCD research provides invaluable insights into mucociliary clearance, insights that can be adapted to help millions of individuals with respiratory disease.

Additionally, there is increasing evidence of overlap in disorders that affect moving cilia and those that affect sensing cilia. Sensing or non-motile ciliary function has now been implicated in a large and diverse set of human diseases, including polycystic kidney disease, pancreatic function and a number of very rare syndromes. Together, these diseases are called ciliopathies and research into PCD and other ciliopathies could potentially provide important information for the treatment of a wide variety of human diseases.

Why We Need Your Help

As a tax-exempt organization, we rely on individual donations to fund our programs (detailed program descriptions are available from the PCD Foundation). Statistically, non-profits receive 80% of their funding from private donors. This can be a challenge for groups like the PCD Foundation that serve a small population. Additionally, we represent a group of people who, because of chronic illness in the family, may already have strained resources. Board members of the PCD Foundation are acutely aware of this and make every effort to keep donation requests reasonable. However, to accomplish the goals of the Foundation we need the assistance of every PCD family and of their circle of friends.

As people who care about individuals with PCD, we are a small group in a unique position to provide answers through research that could potentially help many people, in addition to improving the quality of life for people with PCD. We have made significant progress in getting this message out to researchers and funders, resulting in the development of a North American network to study PCD sponsored by the National Institutes of Health. The biggest threat to our continued progress is lack of funding to operate our important programs.

The PCD Foundation is a 501(c)(3) tax-exempt organizations. Donations made to the PCD Foundation are deductible on your federal tax return. Please consider including the PCD Foundation in your end of year giving plans. It will be money well spent.

Sincerely,

A handwritten signature in black ink that reads "Michele Manion". The signature is written in a cursive, flowing style.

Michele Manion, President
PCD Foundation