

ACHRI Researchers Work to Extend and Enhance the Lives of CF Patients

In the US, about 30,000 children and adults have cystic fibrosis (CF), an inherited chronic disease that affects the lungs and digestive system. The defective gene and its protein cause the body to produce unusually thick, sticky mucus. This mucus clogs the lungs and leads to life-threatening lung infections and obstructs the pancreas preventing natural digestive enzymes from helping the body to break down and absorb food.

Therapeutic approaches to address CF mainly treat its symptoms, but do not cure the disease. These treatments are mainly aimed at reducing respiratory infections and breaking up thickened mucous secretions that cause airflow obstruction and harbor bacteria. In the 1950's, few children with CF lived to attend elementary school. Today, through research and medical advances, many people with CF can expect to live into their 30s, 40s, and beyond.

At ACH, pulmonologist Dr. Dennis Schellhase is one of several clinical researchers working to extend and enhance the lives of CF patients. He is an ACH site investigator for two multi-center CF clinical trials. ACH is the only pediatric site for CF clinical trials in the state. Dr. Schellhase's efforts involve new pediatric therapies to reduce lung infections and to break up thickened mucous secretions. The drugs being studied include a dry powder and a nebulized solution that CF patients inhale.

The current clinical research involves patients of 6 to 21 years of age with CF. One of these patients is 16-year-old Ashley. "I wanted to participate to see if it would work," she says of joining the trial. "Also, I know I'm helping others."

Ashley is able to coordinate her normal clinic visits at ACH with her appointments for the clinical trial. Dr. Schellhase conducts his clinical trial at the Pediatric Clinical Research Unit at ACH. "The PCRU makes it possible to do these multi-center trials here," he says. Of the PCRU and its staff and services, he comments, "They make it simple." Ashley notes the staff is nice and fun. Ashley's mother Joanna finds the staff organized and compassionate.

Both mother and daughter know that Ashley may or may not benefit from participating in the clinical trial, but Ashley chose to participate anyway. Joanna says that Ashley has always been one to want to help others, and this teenager's brave, unselfish actions demonstrate it.

Ashley will complete her role in the trial in February. To other people with CF, Ashley says, "It will be rough, but don't give up hope. Stay positive. We're getting closer to a cure than we've ever been." The "we" isn't rhetorical, it's accurate. Because pediatric patients like Ashley are participating in clinical trials at ACH and across the country and dedicated physicians like Dr.



Ashley, a 16-year-old with cystic fibrosis, knows that her participation in a clinical trial at ACH will help others.

Schellhase are involved in clinical research, new therapies for CF are becoming available and the lives of these patients are improving.



Established in 1989, Arkansas Children's Hospital Research Institute provides an on-site research environment for faculty of the University of Arkansas for Medical Sciences working on the Arkansas Children's Hospital campus. Over 120 pediatric researchers with expertise and experience that span the breadth of medical disciplines comprise ACHRI's roster of investigators who work to fulfill its mission to improve children's health, development, and well-being through high quality research. For more information, visit <http://achri.archildrens.org>.