A Promising New Treatment for CF

OSF HealthCare Newsroom
Shelli Dankoff – Media Relations Supervisor

In November, those who care for and those affected by cystic fibrosis (CF) were excited by the announcement from the Food and Drug Administration (FDA) that it had approved Trikafta, a new therapy to treat the underlying cause of the most common form of CF in people 12 and older. It's estimated more than 90% of people with CF could benefit from this new therapy.

Approximately 30,000 people in the United States have cystic fibrosis with some 1,000 new cases of CF diagnosed each year.

CF is a defect in a chloride channel in the body. The flow of salt and water across a cell is important to how the cell functions. With CF, there's a problem with that protein, so salt and water can't flow properly, which causes a build-up of sticky mucus. In the lungs, the mucus build-up can cause damage, infections, and difficulty breathing, and often requires multiple daily treatments to loosen the mucus so it can be coughed out; in the pancreas, it's not able to secrete digestive enzymes.

According to Heather McLauchlan, MD, a pediatrician with OSF HealthCare Children's Hospital of Illinois and Associate Professor of Clinical Pediatrics at the University of Illinois College of Medicine at Peoria who specializes in caring for CF patients, Trikafta helps that abnormal CF protein get to the surface of the cell so it can open and close properly and not have that build-up of mucus. It's expected Trikafta will improve lung function by 10%.

It took some time for the newly approved drug to make its way to clinics. The Cystic Fibrosis Center at OSF Children's Hospital of Illinois cares for 135 patients, so far 41 patients that have been able to obtain Trikafta.

"Ten-percent is a huge difference. But even more important than the 10% is the amount that it is going to slow the progression of the disease. So you'll see an increase in your lung function at first, but even more important than that, it won't decline as fast as it would without the medication. So on the end, as patients are getting older and older with CF, it really does prolong life," explained Dr. McLauchlan.

Dr. McLauchlan says while they're excited about the 90% who will benefit from Trikafta, the search goes on for treatments to help everyone else.

To that end, the national Cystic Fibrosis Foundation is allocating $500,000 to its Path to a Cure initiative, with a goal of accelerating treatments for the underlying cause of the disease for all people with CF and, ultimately, deliver a cure.

Dr. McLauchlan applauds that goal, not only for her patients, but for the medical community overall.

"I think these types of breakthroughs are applicable to people with Cystic Fibrosis but also in the bigger picture, are important discoveries for all different types of genetic diseases and rare diseases. We're making a lot of progress."

Learn more about cystic fibrosis and the services offered through the CF Center at OSF Children's Hospital of Illinois.