# Children's Hospital Colorado breakthrough therapy approved for people with cystic fibrosis

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Researchers at the Children's Hospital Colorado Breathing Institute, one of the largest cystic fibrosis (CF) clinical care centers in the U.S., are part of a Therapeutics Development Network that oversaw clinical trials leading to FDA approval of TRIKAFTA, a new, highly-effective CF treatment for people with CF 12 years of age and older. The team, led by Drs. Scott Sagel and Edith Zemanick and in partnership with the University of Colorado School of Medicine, are currently involved in trials of TRIKAFTA in children ages 6-11 years. Eventually researchers hope the treatment will be approved for use beginning in early infancy, which could prevent the devastating lung damage and disease progression that commonly occurs in CF. TRIKAFTA will help approximately 90% of people living with CF.

TRIKAFTA, a combination of three medicines, targets the underlying causes of the disease—a defective protein called the cystic fibrosis transmembrane conductance regulator (CFTR) protein. Most people with CF have little to no CFTR protein function. This treatment can improve the CFTR protein function to over 50%, which may lessen many of the symptoms and complications of cystic fibrosis.

In clinical trials, TRIKAFTA treatment led to remarkable improvements in several key measures of disease including:

- Significantly improved lung function

- Reduced frequency of pulmonary exacerbations (respiratory illnesses which require hospitalizations and antibiotic therapy)
- Improved weight and growth
- Improved quality of life

"This is a tremendous milestone and breakthrough for people with CF," said Scott Sagel, MD, Ph.D., a pediatric pulmonologist at Children's Hospital Colorado and director of The Mike McMorris Cystic Fibrosis Research and Care Center. "This transformative therapy will modify the course of CF, improving health and survival in the majority of our patients."

Despite this recent breakthrough, researchers emphasize the importance of learning more about the biology and long-term clinical effectiveness of this therapy. The cystic fibrosis treatment center research team at Children's Hospital Colorado helping to lead a nationwide CF Foundation-sponsored study called PROMISE. This study will examine the long-term effects of TRIKAFTA therapy on airway infection and inflammation, digestion and pancreatic disease, cystic fibrosis-related diabetes and liver disease.

"While approval of TRIKAFTA is certainly cause for celebration, we are now working to identify treatments for people of CF who don't qualify for TRIKAFTA based on their underlying genetic mutations," said Edith Zemanick, MD, pediatric pulmonologist at Children's Hospital Colorado and director of the CF Therapeutics Development Center at Children's Hospital Colorado. "We need 100% of our patients to benefit from a highly-effective, disease-modifying therapy such as TRIKAFTA. We are leading local efforts in the RARE study to collect cells and biospecimens from the almost 10% of the cystic fibrosis population with two rare mutations."

Drs. Sagel and Zemanick highlight that significant ongoing efforts are

targeting the underlying CF gene abnormality. "Once we figure out how to repair or replace the defective gene, this will get us closer to a genetic cure for all people with CF."

Provided by Children's Hospital Colorado

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