Cystic Fibrosis Foundation & Jake's Ladder

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CYSTIC FIBROSIS FOUNDATION®

About the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation is the world's leader in the search for a cure for cystic fibrosis. The Foundation funds more CF research than any other organization, and nearly every CF drug available today was made possible because of Foundation support. Based in Bethesda, Md., the Foundation also supports and accredits a national care center network that has been recognized by the National Institutes of Health as a model of care for a chronic disease. The CF Foundation is a donor-supported nonprofit organization.

What is CF?

- Cystic fibrosis (CF) is a life-threatening genetic disease that affects the lungs and digestive system.
- There are 30,000 children and adults suffering from this horrible disease.
- Just two generations ago, most children with CF didn't live long enough to attend elementary school.
- Today, many people with CF are living into their 30s, 40s, and beyond. Much progress has been made toward finding a cure for CF, but the Cystic Fibrosis Foundation's work is far from over, as we continue to lose precious lives to this disease far too often.

FDA Approves Triple Combination

- Last fall, the FDA announced its approval of ***Trikafta***, a new, highly effective CFTR modulator therapy that treats the underlying cause of CF and could eventually benefit around 90 percent of people with the disease.

- This represents the most transformative therapeutic advance in the history of CF.

- As a result of decades of work and tireless commitment from the CF community, dedicated clinicians and researchers, and the incredible staff at the CF Foundation, more people with CF will have effective treatments for their disease, which may result in better health and longer lives.

Nonsense and Rare Mutations and Path to a Cure

- Ten percent of people with CF in the U.S. have a nonsense, splicing or other rare mutation for which there is currently no therapy that targets the disease at its root.
- The Cystic Fibrosis Foundation is funding many scientific approaches to develop new treatments for this population through ***Path to a Cure***, an ambitious research agenda to accelerate treatments and drug development for the underlying cause of the disease and ultimately to deliver a cure.
- The Foundation's ***Path to a Cure*** centers around three core strategies to address the underlying cause of CF: repairing broken CFTR protein, restoring CFTR protein when none exists, and fixing or replacing the underlying genetic mutation to address the root cause of CF.

Jake’s Ladder

- For 16 years, the Cystic Fibrosis Foundation has annually held “Jake’s Ladder”, celebrating Jacob Dyne, the young son of Shellee and Larry Dyne, who was diagnosed with cystic fibrosis (CF) at the age of 14 months.
- To date, the event has raised over $2 million and all proceeds from the event benefit the CF Foundation. Revenue received in support of the annual Jake's Ladder walk is used to fund the CFF Nonsense and Rare Mutations Research and Therapeutics Initiative.