

# 5-Minute Story

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## OVERVIEW

- We're making so much progress in the fight against cystic fibrosis. In fact, many say it's the best story in medicine today.
- CF is a rare, genetic, life-shortening disease that affects every organ in the body and makes breathing difficult. Some people with the disease say it's like breathing through a narrow straw.
- In 1955, when the Foundation was founded, children with CF rarely lived long enough to attend elementary school.
- Today, people with CF are achieving milestones like attending college, getting married, and having children — goals that used to seem impossible.
- This is due in large part to the work of the Foundation and the amazing CF community. We helped discover the gene that causes CF, created a state-of-the-art model for CF care, and have funded groundbreaking research. Nearly every drug to treat CF that is available today was made possible because of the Foundation's support.
- But we're not done. We are working every day to build on this incredible momentum, and we won't stop until there is a cure for every person with CF.

## RESEARCH ADVANCES

- Because of the work of the Foundation and our community of supporters, there are now three FDA-approved therapies that treat the basic defect in cystic fibrosis for more than half of the population.
- These therapies, Kalydeco®, Orkambi®, and Symdeko®, have transformed the lives of thousands of people.
- For the first time ever, children born today who can be treated with one of these therapies have the possibility of a future free from hospitalizations and a complex daily care regimen.
- Perhaps most exciting, more and more people will be helped by these treatments in coming years.
- Clinical trials are underway that, if successful, could mean 90 percent of our community will benefit from drugs that work as well or better than Kalydeco as soon as 2020.

## ONE-TIME CURE

- But we don't just want to treat CF. We want to end CF. For everyone, including those with rare or nonsense mutations.

- To bring that vision to life, we are focused on a very high-tech and experimental process called gene editing.
- Gene editing will remove the genetic mutation that causes CF and replace it with a normal sequence of DNA. The goal is that, by doing this, we can repair the faulty gene and cure the disease.
- Research into using gene editing to cure CF is already underway.
- Although there are many hurdles, with time and continued effort, we believe we will see a permanent, one-time cure in our lifetimes.

## BETTER TODAYS

- In addition to adding tomorrows by pursuing innovative treatments, we're also committed to helping people with CF live the best lives that they can today.
- Along with new disease-modifying therapies and a one-time cure, we're still steadfast in our efforts to develop better treatments to address the complications of CF.
- We are continuing to invest in CF care by increasing our support for the 120 centers in our innovative care network, with a focus on adult care and mental health. And we're actively focused on lung transplant initiatives for the many adults with CF who need them.
- We are facilitating new initiatives, such as virtual events and mentoring programs, to help people living with CF connect with others in their community.
- We're working diligently in the policy arena to ensure that people with CF have access to the care they need and providing support services to help navigate insurance and other issues directly through our CF Foundation *Compass* program.

## HOW YOU CAN HELP

- Despite tremendous progress, we are not yet done. We have extended life spans considerably but many people with CF still die at a young age, even as children.  
– *If appropriate or possible, cite an example from your local community.*
- Our story will not be finished until we have a cure for 100 percent of people with CF.
- We need your support now as much as ever before to reach that goal.
- There are so many ways you can help: volunteering, donating, becoming an advocate, or helping raise awareness, to name a few.



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