Accelerating the Search for a Cure
ON THE COVER
Meaghan was diagnosed with CF at birth. Today, the sweet and courageous 3-year-old loves playing with her two brothers on their backyard swings. Every day Meaghan takes dozens of medications and uses her vest to break up the sticky mucus in her lungs. You can help ensure a long and healthy life for Meaghan and all people with cystic fibrosis by supporting the Milestones II campaign.
Dear Friends,

My wife Kathy and I first learned about cystic fibrosis when our son Joey was diagnosed with CF. That was the day our lives changed forever.

Our beautiful little boy would need to balance baseball games with breathing treatments, and days at the beach with weeks in the hospital, until the day when this devastating illness would ultimately take his life.

In 1986, he took his last breath. He was just 12 years old.

In Joey’s memory, Kathy and I have committed ourselves to doing whatever it takes so that parents won’t have to experience what we endured. To this day, I have been overwhelmed by the generous support of an army of friends, families and colleagues—some with no direct connection to CF—who have joined me in working to turn this dream into a reality.

With the recent approval of Kalydeco™, the first drug to treat the underlying cause of CF for a segment of people with the disease, I am filled with tremendous hope and optimism that we will harness this breakthrough to achieve even more progress for all people with CF.

We are so close to curing this disease, and it’s up to us to quickly raise the additional funds needed to develop more breakthrough therapies.

Thank you for joining me in Milestones II as we accelerate our journey to the finish line. We truly cannot wait to reach the day when no young life is cut short by this disease.

Sincerely,

Joe O’Donnell
Chair, Milestones II
When the Cystic Fibrosis Foundation was established in 1955, children born with CF were not expected to live long enough to attend elementary school. Little was known about the devastating genetic disease, and no effective treatments were available. In an effort to save their children and help all those with CF, a concerned group of parents banded together with the goals to advance the understanding of CF, create new treatments and find a cure.

Since then—and through the outstanding commitment of a wide community of volunteers and donors—the Foundation has achieved tremendous progress. Thanks to investments made by the CF Foundation in research and comprehensive care, people with CF are living into their 30s, 40s and beyond.

While we have achieved enormous gains in quality of life and life expectancy, living with cystic fibrosis is not easy. For those with the disease, every day is a challenge. Morning and bedtime routines are typically filled with time-consuming treatments to keep lungs healthy and help break up the sticky mucus that impairs breathing and invites life-threatening infections. Meals and snacks are usually accompanied by dozens of enzyme pills to aid in digestion. For many with CF, frequent hospitalizations mean missed school and work days and major interruptions to “normal” life.

Despite the rigorous treatment regimen needed to manage the disease, families remain optimistic and hopeful.

“I take dozens of pills and medications every day to keep my lungs healthy. But I have bigger dreams than just breathing.”

KASEY, AGE 19, COLLEGE FRESHMAN

ABOUT CYSTIC FIBROSIS

• CF is a life-threatening disease that affects the lungs and digestive systems of approximately 30,000 children and adults in the United States.

• More than 10 million Americans are symptomless carriers of the defective CF gene.

• The disease occurs in one of every 3,500 live American births, and about 1,000 new cases of CF are diagnosed each year.
Throughout the last five decades, the Foundation has achieved many milestones that have changed the course of the disease.

In 1989, a team of scientists supported by the Cystic Fibrosis Foundation discovered the cystic fibrosis gene and opened the door to understanding the disease at its most basic level. With this knowledge in hand, our next step was clear: to leverage the discovery of the CF gene and develop powerful new therapies that attack the disease at its core.

Yet throughout the 1990s, the pace of drug development for the disease remained painfully slow. Few pharmaceutical companies were willing to invest in research to develop therapies for the relatively small cystic fibrosis population.

To quicken the pace of drug discovery and development, the Foundation pioneered a highly innovative business model known as “venture philanthropy,” which applies the same results-driven approach of a for-profit company to finding new treatments for CF. The venture philanthropy concept was put into action with the establishment of the Therapeutics Development Program (TDP) in 1997, a program designed to build collaborations with pharmaceutical companies for CF research and drug development. The TDP incentivized companies by supplying research dollars during the early phases of drug development, when the risk is greatest.

Over the next several years, through the generosity and hard work of its donors and volunteers, the Foundation laid the groundwork for a number of breakthrough new therapies and built the first ever drug development pipeline for cystic fibrosis.

Crucial new therapies became part of the daily treatment regimen of thousands of CF patients, including Pulmozyme® and TOBI®, and life expectancy began to steadily climb. Nearly every CF drug available today was made possible because of the Foundation’s support and its ongoing work with researchers and the pharmaceutical industry to find a cure.

“Both my children were born with cystic fibrosis. Jena lost her battle at age 13. Eric is still fighting. I know that with the strong support of generous people around the country, we will find a cure soon.”

MARGARETE CASSALINA, CF MOM
When the *Milestones to a Cure* campaign began in 2004, promising opportunities in CF drug discovery and development outpaced the Foundation’s ability to fund them. Refusing to accept this reality, an ever-growing number of major donors across the country joined together to do what no one thought was possible.

The idea of a $175 million multiyear major giving campaign, conducted by a voluntary health agency, was viewed as a herculean—if not impossible—task. Yet from day one of the campaign’s launch, the support from the CF community and beyond was astounding. Gifts ranging from $10,000, to multiple commitments in excess of $10 million each, bolstered confidence and positioned the Foundation through its non-profit drug discovery and development affiliate Cystic Fibrosis Foundation Therapeutics (CFFT) to leverage unprecedented opportunities.

By raising $175 million to help close the gap between promising drug development opportunities and the funds available to pursue them, the *Milestones* campaign enabled the Foundation to intensify its efforts and establish a new, heightened pace for progress in drug development that matches the urgency of our mission.

Most important, thanks to the contributions of a remarkable group of people across the country who support the CF Foundation mission, we were able to advance development of new therapies and help achieve the momentous approval of Kalydeco, the first drug to address the underlying cause of cystic fibrosis.

Because of this progress, today children and adults with CF are living longer, healthier lives.

**MILESTONES TO A CURE MAJOR ACHIEVEMENTS**

- In 2006, hypertonic saline, a simple and effective CF therapy, was made available to people with CF after successful CFFT-supported clinical trials showed improvement in lung function and mucus clearance.

- In 2008, scientists achieved the most important “proof of concept” in our history, demonstrating that we can treat the basic defect of CF and improve pulmonary outcomes through small molecule therapies.

- Cayston®, a much-needed antibiotic alternative for CF patients who battle recurrent lung infections, was approved by the U.S. Food and Drug Administration and made available to patients in March 2010.

- In 2012, the Food and Drug Administration announced approval of Kalydeco for patients ages 6 and older with the G551D mutation of CF. Patients who took the drug in clinical trials showed marked improvements in a number of key indicators of the disease, including lung function, weight gain and sweat chloride levels.

- The Foundation’s achievements in CF care and research have attracted the attention of major media outlets and scientific journals, including the *New England Journal of Medicine*, *The New York Times*, *The Boston Globe*, *Forbes* and *The New Yorker*.

- Two Harvard Business School case studies have showcased the Foundation's innovative leadership and business model. The case study featuring our successful venture philanthropy approach to drug development and leadership decisions is now part of the required curriculum for first-year MBA candidates.

“*The credit for our progress in CF drug development goes to the many caring and generous people – especially our *Milestones* donors – who were willing to listen to our story, embrace our vision and invest in an innovative ‘pipeline’ of potential therapies. Because of their extraordinary dedication, we are transforming this disease.*”

*C. RICHARD MATTINGLY, EXECUTIVE VICE PRESIDENT AND CHIEF OPERATING OFFICER, CYSTIC FIBROSIS FOUNDATION*
MILESTONES SUPPORT ACCELERATES DEVELOPMENT OF KALYDECO AND OPENS DOORS TO NEW RESEARCH

Through its outstanding support, the Milestones campaign has fueled the acceleration of several new cystic fibrosis treatments, including Kalydeco, the first drug approved to address the underlying cause of the disease.

Kalydeco has so far been shown to be effective for about 4 percent of patients who have a specific CF mutation, but the science behind the drug has opened exciting new doors to research and development that may one day lead to a cure for all people living with the disease.

The Foundation’s top priority now is accelerating the development of an effective treatment for people with the most common CF mutation.

The Foundation has significantly expanded its collaborations with leading pharmaceutical companies, including Genzyme and Pfizer, to speed the discovery and development of new drugs that will help more people with CF.

“The unique and mutually beneficial partnership [between the Cystic Fibrosis Foundation and Vertex Pharmaceuticals] that led to the approval of Kalydeco serves as a great model for what companies and patient groups can achieve if they collaborate on drug development.”

MARGARET A. HAMBURG, M.D.
U.S. FOOD AND DRUG ADMINISTRATION COMMISSIONER

“We have a unique opportunity with the cystic fibrosis story to figure out how we can help other diseases.”

FRANCIS S. COLLINS, M.D., PH.D.
DIRECTOR OF THE NATIONAL INSTITUTES OF HEALTH
LEADER OF THE RESEARCH TEAM THAT DISCOVERED THE CYSTIC FIBROSIS GENE
As we embark on the next leg of our journey and accelerate to the finish line, we have truly entered a new playing field of drug discovery and development: **We have never before been able to progress so quickly and efficiently toward a cure, and we cannot turn back now.**

Thanks to the remarkable generosity of Milestones donors, the hope and promise of a cure for all people with CF is within reach—yet we still lose precious young lives to cystic fibrosis every day. Those with CF deserve every chance for success in finding new tools to battle this disease and live a full, healthy life.

To build on recent advances and accelerate the vital pace of progress, the CF Foundation has identified the need for $75 million in major gifts by the end of 2015. **We need your help to achieve this goal.**

You can help us end this disease once and for all.

“I love the efficiency of the CF Foundation. In giving to the Milestones campaign, we are confident that every dollar will be invested wisely so that real progress can be made in our efforts to cure cystic fibrosis.”

**Kate Niehaus, Milestones Executive Committee Member**
You can participate in the *Milestones II* campaign by making a major gift*. With your help, we can raise the $75 million needed to ensure the Foundation is in the best position to:

- Accelerate the development of drugs currently being tested that are viewed most promising to treat the underlying cause of CF in more people with the disease;
- Discover and develop new drugs that will give us the best chance of developing lifesaving therapies for all those with cystic fibrosis;
- Attract more biopharmaceutical companies to the CF drug development effort;
- Advance the development of treatments that attack the disease from every angle, from symptom and nutrition management to infection and inflammation therapies; and
- Continue to pursue every promising avenue toward a cure until we reach our goal.

* a gift of $10,000 or more made in a calendar year or payable over two to five years.

If you would like more information on ways you can participate in the *Milestones II* campaign, please contact:

**Regina R. Schewe**  
Vice President, Major and Planned Giving  
301-907-2506  
rschewe@cff.org
Why Give to the Cystic Fibrosis Foundation?

INNOVATION
The CF Foundation’s innovative approach to research has yielded a robust pipeline of potential therapies that target the disease from every angle.

EFFICIENCY
The Foundation is an accredited charity of the Better Business Bureau’s Wise Giving Alliance. Approximately 90 cents of every dollar of Foundation revenue goes to vital CF research, medical and education programs.

RESULTS
Thanks to wise investments by the Foundation in CF research and care, people with CF are living longer, healthier lives.

WHAT TO EXPECT FROM YOUR INVESTMENT

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<td>Develop therapies that work for all people with cystic fibrosis.</td>
<td>Advance new trials to study potential therapies that can be used in combination to treat a larger segment of the CF population.</td>
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<td>Discover and develop more therapies to treat the range of symptoms suffered by people with CF, including those who have undergone transplants.</td>
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“Because of the selfless generosity of our Milestones donors, people with CF and their friends and families throughout the world have real hope for a better, brighter future. Please join us in our quest. We will not rest until we find a cure for all people with CF.”

ROBERT J. BEALL, PH.D., PRESIDENT AND CHIEF EXECUTIVE OFFICER, CYSTIC FIBROSIS FOUNDATION