MilestonesUpdate

News from the Cystic Fibrosis Foundation's Major Giving Campaign

FALL 2012

Milestones II
Campaign Chair
Joe O'Donnell

Dear Friends,

Reflecting on the monumental progress made this year toward curing and controlling CF, we have a lot of reasons to be grateful. In January, we made history with the FDA approval of Kalydeco™ to treat the underlying cause of CF in a small group of people with the disease. CBS Evening News anchor and longtime friend of the CF Foundation Scott Pelley reported this groundbreaking CF treatment as a "game-changer." Kalydeco proves we are on the right path toward finding a cure for CF. This milestone drug approval was followed closely by the positive results from a Phase 2 combination clinical trial of Kalydeco and VX-809 in people with two copies of the most common mutation of CF, and news that Vertex plans to start a pivotal trial in early 2013.

These great gains could only have been achieved with the generosity, commitment and dedication of our cherished *Milestones* donors. Because of you, the entire CF community is filled with a renewed sense of hope and optimism.

While we have much to celebrate and be proud of, we are very aware that our work is not yet done.

By raising $75 million through the *Milestones II* campaign, we can sustain the critical momentum needed for research and drug development. With your generosity, we will have the funds needed to accelerate development of promising new treatments, such as Kalydeco in combination with other potential drugs, and continue to strenuously expand our collaborations with new biopharmaceutical companies to advance the next generation of therapies targeting the root cause of CF.

Milestones II has the potential to be the deciding factor in the fight against cystic fibrosis. Your commitment to and leadership in this effort are deeply appreciated, and I look forward to working with you as we continue to change the face of this disease.

As always, thank you for all you do.

Sincerely,

Joe O'Donnell
Chair, *Milestones II*

Milestones II

"This year's exciting advances in CF research and care would not be possible without the generosity of *Milestones II* donors. With your ongoing support, we will continue **adding tomorrows** for all those with CF."

Robert J. Beall, Ph.D.
President and CEO
Cystic Fibrosis Foundation

CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

Accelerating the Search for a Cure

Steadfast Friends Continue Generous Support of *Milestones* Campaign

Boomer, Cheryl, Gunnar and Sydney Esiason

When their son Gunnar was diagnosed with cystic fibrosis, **Boomer** and **Cheryl Esiason** dedicated their hearts and their energies to help find a cure for Gunnar and all those with CF. Twenty years later, their commitment is just as strong, and the partnership between the CF Foundation and the Boomer Esiason Foundation remains unwavering.

Longtime friends of the CF Foundation, the Esiasons are committed to doing whatever they can to accelerate the development of promising therapies that will extend and improve the lives of people with the disease. Over the past decade, they have offered their steadfast support to the *Milestones I* campaign and have expanded that support this year to become top donors to the *Milestones II* campaign.

"There is no doubt the CF Foundation has dramatically improved Gunnar's health," says Boomer. "Cheryl and I know that our investment in the *Milestones II* campaign will help accelerate the life-saving research needed to find a cure and control for CF."

Stephen, Jill, Douglass and Jana Karp

The Cystic Fibrosis Foundation's mission to cure CF is one that **Stephen Karp** has held very close to his heart for decades. Longtime friends of *Milestones II* campaign chair Joe O'Donnell and his wife Kathy, whose son Joey lived courageously with CF until age 12, Karp and his wife Jill have helped to honor Joey through their steadfast generosity.

In 2007, the **Karp Family Foundation** expanded its support of the CF Foundation with a gift to the *Milestones to a Cure* campaign. This year, with a sense of renewed commitment and excitement about the momentum of current CF research, the Karps decided to invest in the *Milestones II* campaign.

"My wife and I, along with our children Douglass and Jana, were so inspired by the development of Kalydeco that we felt it was our duty to expand our commitment to *Milestones II*," says Stephen. "We are proud to be able to play a small part in the Foundation's efforts to accelerate the search for a cure."

New Drug Approval Inspires Two Families to Help Accelerate Research

Two years ago when **Deborah Proctor** learned her grandson Ryder had cystic fibrosis, she immediately thought of her days in nursing school.

"When you met a child who had CF, the picture that came to mind was one of ultimate loss," says Deborah. "Today, people with CF are living to adulthood, and that is because of the work of the CF Foundation."

Deborah and her family first became involved with the CF Foundation through its Southern California Chapter – Orange County Office during Great Strides season. This year, their team, Ryder's Stryders, fearlessly led by Ryder's parents Britt and Raven Pugh, raised more than $40,000. As Ryder grew, so did Deborah's involvement with fundraising events.

"I know there are several tried and true ways to fundraise," says Deborah, who is the president and CEO of St. Joseph Health System in California. "The CF Foundation has gone beyond those regular channels with its successful venture philanthropy model to accelerate its search for a cure."

Inspired by the groundbreaking approval of the new CF drug Kalydeco, Deborah decided to make a generous contribution to the *Milestones II* campaign.

"I want to support a charity that I think can change lives, and with the success of Kalydeco, it just hit me," says Deborah. "If even one child's life can be saved by what the Foundation is doing, that is absolutely worth any investment I can make."

The day Kalydeco was approved was one of celebration for Deborah, as well as for her son Britt and daughter-in-law Raven. Two-year-old Ryder has the G551D mutation, and will benefit from this breakthrough treatment. Deborah finds herself asking, "How can we help create another Kalydeco for all those with CF?"

"There are a lot of diseases that make me think, 'Boy, we might find a cure for this someday,' but with CF, I think it will actually happen in my lifetime," says Deborah.

Nick Stephens and his wife, **Lisa Kunstadter**, know a good investment when they see one.

"We know from experience that the CF Foundation makes very efficient use of its funds, with very little overhead and dollars raised going straight to research to find a cure," says Nick, who is 58 years old and has CF.

When Nick was born, he was not expected to live past his twenties. He never met his older brother Thomas, who died of CF at just three months old, and his younger brother John lived with CF until 2007. "We support the CF Foundation so that children born with CF today can live a long and productive life, just as Nick is doing," says Lisa.

Nick is a partner at Edgewood Management LLC, an investment firm in New York, and serves on the Greater New York Chapter's board of directors and major gifts committee. Nick was motivated to get involved with the CF Foundation when he heard about the CF gene discovery in 1989. Since then, Nick and Lisa have supported Great Strides and regularly attend other chapter events.

In the years following the gene discovery, Nick hoped that a CF cure would speedily be discovered.

"I think Kalydeco has really broken the log jam in drug development," says Nick. "Ten years ago, a drug like Kalydeco would have seemed like science fiction."

Nick and Lisa's excitement led them to make a generous contribution to the *Milestones II* campaign, in support of research that will help the next generation of people with CF.

Nick often meets new CF parents, still in shock and coping with the diagnosis. "I try not to sound overly corny when I explain to them that yes, CF is a burden, but thanks to the CF Foundation and all that is going on in drug development, their child should live a great and normal life."

Milestones Donors Gather in New York and Texas

New York:

Key volunteers and campaign leaders gathered in New York this September to discuss new ways to rally support for the *Milestones II* campaign.

Paul Whetsell, Gina Schewe, Bob and Kate Niehaus, Mike Beatty, Bob and Cyndi Troop, Joe O'Donnell, Amy Weinberg, Jeff Joyce, Cynthia Kempner, Rich Gray, Martine Denis, Rich Mattingly

Kate and Bob Niehaus, Cynthia Kempner, Joe O'Donnell, Amy Weinberg

Mike Beatty, Jeff Joyce

Houston:

This summer, Ralph and Becky O'Connor generously hosted a dinner in Houston to thank donors for their steadfast support of the *Milestones* campaign, and to celebrate the FDA approval of Kalydeco.

Michael and Barbara Caswell

Mary Jayne McGinnis and Sissy Boyd

Bryan and Karen Dudman, Joe O'Donnell

Jason and Sharon DeLorenzo, Joe O'Donnell, Carolyn and Michael Asher

Milestones Investments Yield Major Progress in CF Drug Development

Following the swift FDA approval of Kalydeco™ — the first drug to treat the underlying cause of CF in a small segment of those with the disease — Vertex Pharmaceuticals Inc. announced positive results from a Phase 2 study of Kalydeco combined with the potential therapy VX-809. People with two copies of the most common CF mutation, Delta F508, who took the combination treatment showed a significant improvement in lung function. Vertex plans to begin a pivotal trial of Kalydeco and VX-809 in early 2013.

Genzyme, a Sanofi company, and Cystic Fibrosis Foundation Therapeutics Inc. (CFFT) have entered into a research collaboration to discover potential new drugs to treat the basic defect of CF in people with the Delta F508 mutation. Researchers will take advantage of the vast compound libraries at Genzyme and Sanofi R&D facilities around the world to identify small compounds that may help the key protein in CF — CFTR — function correctly.

To help speed the development of therapies targeting the basic defect in CF, CFFT has entered into a collaboration with Pfizer Inc., one of the world's largest pharmaceutical companies. The new agreement grows out of an earlier collaboration between CFFT and the biotech company FoldRx Pharmaceuticals Inc., acquired by Pfizer in 2010. Pfizer will also be able to draw on its massive library of about 5 million chemical compounds to screen for potential CF therapies.

A collaboration between Proteostasis Therapeutics Inc. (PTI) and CFFT will also focus on finding small molecule compounds that could be used to treat people with the Delta F508 mutation. PTI scientists will use a unique screening platform to identify potential new compounds to help the defective CFTR protein fold correctly and move to the surface of the cell.

CFFT has opened its own nonprofit research laboratory, representing another important step in the effort to discover new potential compounds that target the basic genetic defect in CF. Based in Boston, the CFFT Lab will work in collaboration with biotech firms and academic researchers to swiftly move promising drug candidates into development.

In 2012, the CF Foundation's significant achievements in advancing CF research and care were featured by several major news outlets and medical journals, including *The Boston Globe, Forbes, The Economist* and *Nature Medicine.*

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CYSTIC FIBROSIS FOUNDATION

"Thanks to the CF Foundation, I am more confident than ever before that CF will soon stand for 'Cure Found.'"

Mike Sanchez, father of Maya, age 1