Dear Friends,

There is more hope in the CF community than ever before. Recent successes in drug development have led to exciting new opportunities in our quest to find a cure. As I reflect on where we were before the Milestones campaigns began and where we are today, I am especially grateful for the many friends and supporters who believe in our vision and are committed to finding a cure for all people with cystic fibrosis.

We have seen many new developments in the last year, and I believe many more are on the horizon. Most exciting are recent announcements from Vertex Pharmaceuticals that it is advancing clinical trials of Kalydeco in combination with two other potential drugs in people with two copies of the most common CF mutation, and news that the Foundation has funded new CF projects with Pfizer and Genzyme.

These great gains would not be possible without the unwavering support and generosity of Milestones donors. But our work is not done. Right now, we have commitments of more than $39 million toward Milestones II’s $75 million goal. This is great news, but we’re not at the finish line, and I need your help. We must make sure we are in the best position to advance the search for a cure for all people with CF.

We got the year off to a great start when many of us came together in Las Vegas to support the CF cause with world-renowned performer Celine Dion. The Adding Tomorrows with Celine Dion concert raised more than $1.5 million. I am deeply appreciative of the enormous role Gary Loveman, chairman, president and CEO of Caesar’s Entertainment, played to make this special evening a success, and I send my thanks to Celine Dion, who lost her niece to cystic fibrosis and is committed to continuing the fight against this terrible disease.

To sustain the critical momentum we have gained, we need to keep the Milestones campaign going strong. With your continued dedication, I am confident that we will have more to celebrate in the future. Thank you for all that you do.

Sincerely,

Joe O’Donnell
Chair, Milestones II

“We have been able to accelerate the pace of research to treat the basic defect for all people with CF because of the generosity and commitment of Milestones donors.”

Robert J. Beall, Ph.D.
President and CEO
Cystic Fibrosis Foundation
Inspired by Research: Two Families Support Milestones Campaign to Help Other People with CF

When Karen and Farzad Sani’s toddler son, Benjamin, developed lung and digestive tract health problems, a specialist encouraged the family to have Ben tested for cystic fibrosis.

That’s when their research to learn more began.

“My husband and I read everything we could about cystic fibrosis,” Karen said. “We learned about breathing treatments, enzymes, clinical trials and the daily time commitment of treating CF.”

The Sanis also learned about the Cystic Fibrosis Foundation, the work of the Northeastern New York Chapter and the Foundation’s impressive drive to find a cure for the disease. Although they were relieved to find out their son does not have CF, Karen and Farzad knew they wanted to do whatever they could to help.

The Sanis have pledged to donate $20,000 a year, for the next five years, to the Foundation. As a way to encourage others to give, their gift will match annual fund donations made from other Northeastern New York Chapter volunteers. But that’s not all. The Sanis are encouraging other families in their neighborhood to raise or donate funds through the use of social media — an effort that has brought in additional contributions.

“We hope our passion for this cause will encourage others to follow our lead and become involved as well,” Farzad said. “If we can make a tiny dent to help even one person, we know we’re making a difference.”

Sarah Platt was still pregnant when, through genetic testing, she learned that her unborn child had CF. Feeling scared and overwhelmed, Sarah began researching the disease and looking for support groups.

That research led Sarah to the Cystic Fibrosis Foundation and, ultimately, another discovery: Eleanor, who is now almost two years old, has the G551D gene mutation, which affects 4 percent of the CF population.

Kalydeco, a breakthrough treatment that addresses the underlying cause of CF, was approved in 2012 for patients ages 6 and older who have this mutation.

“Knowing that my daughter may benefit from the new drug makes me want to fight even harder for the other 96 percent of people with CF who don’t have a medication to rely on,” Platt said.

Realizing how urgent the need is to develop new therapies, Sarah encouraged her family to become members of the Milestones II campaign through the Jurrens Family Foundation, established by her mother for the purpose of giving.

Each year, Sarah and her three older brothers, Grady, Marty and Damion, meet to discuss their interests and how they would like to give back to the community.

She hopes her family’s gift will help make a difference not only in Eleanor’s life, but in the lives of all people with CF.

“The Foundation has made tremendous progress, but it’s just the beginning. We are on the cusp of something huge and I think my family really understands the urgency to develop new therapies,” Sarah said. “We really want to see a drug that can help a larger percentage of people who have CF. The way to do that is to fund research and science.”
Steve and Lisa Orlando became involved with the Cystic Fibrosis Foundation when their granddaughter, Bella, was diagnosed with the disease nine years ago. They formed a Great Strides team, but always felt they could do more to help find a cure.

In 2011, the Louisiana-based couple hosted the inaugural Allison Companies Layfette Wine Opener. The event raised $63,000 in its first year, but for the Orlandos, that still wasn’t enough. Bella’s courageous spirit and the hope found within the CF community drove them to seek out more ways to support the Foundation.

“Our involvement in the CF Foundation has been life changing,” Steve said. “When you go to conventions, meet parents, see the faces of children who have this disease and hear their dreams, you really begin to see the human side of CF and you realize just how devastating this disease is.”

So when Steve and Lisa were asked to support the Milestones II campaign, they jumped at the opportunity. Their contribution was matched by an anonymous donor — an act that set a precedent. Steve and Lisa hope their gift will spur additional giving in the southeast region.

“Make a commitment to the Milestones II Campaign, payable over five years, at one of the following levels:

- **Discovery Level** – $50,000 ($10,000/year for five years)
- **Breakthrough Level** – $100,000 ($20,000/year for five years)
- **Acceleration Level** – $250,000 ($50,000/year for five years)

The work this organization does is incredible and the business model established by Bob Beall makes the CF Foundation really stand out. The organization is efficient and effective,” Steve said. “But above all, we’re doing this for our granddaughter.”

Bella, now 9 years old, is known for her compassion toward animals, her love of gymnastics and her bravery in the fight against cystic fibrosis. She’s strong-willed, determined and takes responsibility for her treatments.

“When she stays over at our house, she’ll ask me to set the alarm clock for 5:45 in the morning so she can wake up to do her vest and nebulizer treatments,” Lisa said. “She knows what she needs to do to stay healthy.”

Along with their support of Milestones II, the Orlandos will continue to chair the wine opener and look forward to starting a golf tournament in Louisiana this fall.

“We are closer than ever before to finding a cure for CF, and Lisa and I feel lucky to be involved with the Foundation at such an exciting time,” Steve said. “We’re committed to doing everything we can to help the Foundation succeed.”

C. Richard Mattingly
Executive Vice President and Chief Operating Officer
Cystic Fibrosis Foundation
Longtime friends of the Milestones major giving campaign, devoted volunteers and families affected by the disease attended a once-in-a-lifetime event with Celine Dion at Caesars Palace in Las Vegas, which raised $1.5 million for the Foundation. The concert was made possible thanks to the efforts of Gary Loveman, CEO of Caesars Entertainment Corporation, the entertainment group AEG Live, and Celine Dion, who lost her niece to CF in 1993.

Celine Dion sang “Lullaby” in honor of people with cystic fibrosis and their families.
Vertex Pharmaceuticals Inc. will soon start two international Phase 3 clinical trials of Kalydeco™ in combination with the potential drug VX-809. Both Kalydeco and VX-809 are designed to treat the underlying cause of CF. The two six-month trials will evaluate the combination regimen in people ages 12 and older who have two copies of the most common CF mutation, Delta F508. The Food and Drug Administration (FDA) has awarded Kalydeco and VX-809 “Breakthrough Therapy Designation,” intended to speed development of therapies for life-threatening diseases.

The Phase 3 studies will enroll about 1,000 volunteers at approximately 200 clinical trial sites in North America, Europe and Australia.

Vertex is also studying Kalydeco in combination with another potential small molecule therapy, VX-661. In April, the company announced promising results from a Phase 2 trial of Kalydeco and VX-661 in people with two copies of the Delta F508 mutation, ages 18 and older. Study volunteers who took the Kalydeco and VX-661 combination treatment showed a significant improvement in lung function, compared with those who received a placebo. Four different doses of VX-661 were evaluated in combination with Kalydeco. Those who received the two highest doses of VX-661 showed the greatest improvement in lung function.

Vertex plans to conduct more studies of VX-661 and Kalydeco, pending discussions with regulatory agencies.

Cystic Fibrosis Foundation Therapeutics Inc. (CFFT) has significantly expanded its research collaboration with Pfizer Inc. to discover new drugs to treat people with the Delta F508 mutation of CF. The goal of the new program is to advance multiple drugs that could be used in combination to target the basic defect in CF, with promising drug candidates moving into clinical trials by the end of the six-year collaboration.

CFFT is collaborating with Genzyme, a Sanofi company, to discover new drugs to address the most common CF mutation. This research program is building on Genzyme’s experience in rare diseases, while also taking advantage of Sanofi’s vast resources worldwide.

Both collaborations are focused on identifying therapies that help restore normal function of the defective CFTR protein, which does not fold correctly in people with the Delta F508 mutation. Nearly 90 percent of people with CF in the United States have at least one copy of this mutation.

In keeping with our venture philanthropy business model, we have contractual agreements with Vertex, Pfizer, Genzyme and certain other companies to receive royalties from the sales of any drugs that are developed as a result of CFFT funding. Any royalties we receive are reinvested in support of our mission.

CF scientists and management from Pfizer met with CFFT’s medical team and advisory panel to discuss Pfizer’s CF drug discovery and development program.
“The CF Foundation is giving our beautiful daughter the chance to lead a full and active life.”

Sue Lohsen, mother of Elena, age 9