WE ARE ADDING TOMORROWS FOR ALL PEOPLE WITH CYSTIC FIBROSIS.

The mission of the Cystic Fibrosis Foundation is to cure cystic fibrosis and to provide all people with the disease the opportunity to lead full, productive lives by funding research and drug development, promoting individualized treatment, and ensuring access to high-quality, specialized care.
DEAR FRIENDS,

THIS IS TRULY AN EXCITING TIME FOR THE ENTIRE CYSTIC FIBROSIS COMMUNITY.

Little more than a year after the approval of the first drug to treat the genetic defect in a small group with a specific CF mutation, we are moving forward vigorously to extend the benefits of that achievement to all people living with the disease.

Large, late-stage trials are now testing this breakthrough CF drug in combination with another potential therapy in people with two copies of the most common mutation of CF. We do not yet know the results, but we do know that whatever we learn from these important studies could lead to future trials of new, and potentially more effective, treatments for 100 percent of those living with CF.

To ensure a robust drug development pipeline, we have greatly expanded our science and medical programs and forged new agreements with leading pharmaceutical companies, many new to the CF field. Equally important, the Foundation remains committed to fostering excellence in the standard of care throughout its care center network and to helping ensure people with CF have access to the specialized care and treatments they need to maintain good health and pursue their dreams.

The progress we have made and the opportunities ahead of us are thanks to the extraordinary team of people with CF, families, volunteers, donors, caregivers, researchers and friends, whose ceaseless dedication to our mission has helped change the face of this disease. This year, the Cystic Fibrosis Foundation had the privilege of enlisting the talents and experience of many of them as we undertook a comprehensive strategic planning initiative aimed at setting the course for the Foundation over the next five years.

We came away from this rigorous exercise with renewed determination to help all people with CF lead full, productive lives, and clear steps to engage the entire CF community in that effort. As we fast approach the milestone when adults will make up more than half of the CF population, our new strategic plan places special emphasis on enriching our programs that address the unique needs of adults with CF.

Our work is not yet done. But with our community’s steadfast and passionate commitment and the support of our friends, I am confident that we will continue to forge new paths and reach our ultimate goal: a cure for all people with CF.

Sincerely,

ROBERT J. BEALL, Ph.D.
President & Chief Executive Officer
Cystic Fibrosis Foundation
ACCELERATING
CF DRUG DISCOVERY AND DEVELOPMENT

In 2013, the Cystic Fibrosis Foundation moved forward aggressively on many fronts to accelerate the discovery and development of cutting-edge therapies that address the underlying cause of cystic fibrosis in all people with the disease.

Through its nonprofit drug discovery and development affiliate, Cystic Fibrosis Foundation Therapeutics Inc. (CFFT), the Foundation is pursuing new and expanded research aimed at repairing the faulty CFTR protein caused by mutations in the CF gene. These efforts build on the tremendous momentum and knowledge gained through the development and approval of the first drug to treat the basic defect in CF and the progress of other potentially life-changing therapies now in the pipeline.

CFFT also continues to support innovative research and clinical studies to find new treatments and strategies that will help people with CF better manage all aspects of the disease and maintain and improve their health, at every stage of life.

Targeting the Underlying Cause of CF

Studies Advance to Treat Most Common CF Mutation

In February, Vertex Pharmaceuticals Inc. launched two large, international Phase 3 trials of the drug ivacaftor in combination with another potential CF therapy, VX-809, in people ages 12 and older who have two copies of the most common CF mutation, Delta F508.

Both ivacaftor and VX-809 are the result of long-term investments made by CFFT in programs using small molecule compounds to modulate the defective CFTR protein and improve key symptoms of the disease. Ivacaftor — also known as Kalydeco™ — was approved by the U.S. Food and Drug Administration (FDA) in 2012 for people ages 6 and older with the G551D mutation, and is the first drug to treat the basic defect in CF.

More than 1,000 volunteers are participating in these much-anticipated studies, which are taking place at nearly 200 clinical trial sites in North America, Europe and Australia. The trials are moving forward under an expedited timeframe, following a decision by the FDA to award this combination treatment “Breakthrough Therapy Designation,” intended to speed development of select potential therapies that treat life-threatening diseases or conditions. Results from the trials are expected in summer 2014.
About 50 percent of people with CF in the United States have two copies of the Delta F508 mutation, and nearly 90 percent have at least one copy. In people with this mutation, a series of problems prevents the CFTR protein from folding into the correct shape to move to the surface of the cell, where it is needed to help regulate the flow of salt and fluids in and out of the cells of the lungs and other organs. VX-809, which is known as a “corrector,” is designed to help move the protein to the cell surface; ivacaftor, a “potentiator,” increases the activity of CFTR once it is there.

In an earlier Phase 2 trial, participants with only one copy of Delta F508 who took the combination treatment showed improvements in lung function, but their gains were smaller than those seen in people with two copies of Delta F508. Vertex is now conducting an additional Phase 2 study to evaluate different doses of VX-809 when combined with ivacaftor, including in those with one copy of Delta F508.

Vertex is also studying ivacaftor coupled with another potential corrector, VX-661, in people with the most common mutation. In April, Vertex released promising results from a Phase 2 trial evaluating four different doses of VX-661 when combined with ivacaftor in people with two copies of Delta F508. Study volunteers who received the treatment showed a significant improvement in lung function, with those who took the highest doses of VX-661 showing the greatest improvement.

Vertex is conducting a second Phase 2 trial of VX-661 and ivacaftor in people with two copies of Delta F508, and plans additional studies that will include people with CF who have a single copy of this mutation.

In addition, N30 Pharmaceuticals Inc. has begun a Phase 1 multicenter study of the potential drug N6022 in people with two copies of the Delta F508 mutation ages 18 and older. N6022 is an injectable compound designed to increase the amount of CFTR that reaches the cell surface and to stabilize the protein once there to help decrease inflammation in the lungs. It is the first of a new class of compounds aimed at increasing levels of a molecule known as GSNO, which plays an important role in respiratory function and has been shown to be decreased in people with CF.

**FOUNDATION’S DRUG DEVELOPMENT STRATEGY MAKES HEADLINES**

A full-length feature article in the September issue of a leading science magazine, *Discover*, spotlighted the Foundation’s role in bringing a life-changing therapy to those living with CF. Sharing the experiences of two sisters with CF who are thriving on the drug, the story describes how, in 2000, CFFT took the unusual step of establishing a research arrangement with a for-profit company to help discover new approaches to tackle the disease at its root. The article notes that the science that led to the discovery of this treatment is fueling the search for more drugs to benefit greater numbers of people with CF.
Ivacaftor Expands to More Patient Groups
As this report was being prepared, the FDA approved ivacaftor as a single therapy for people ages 6 and older with eight other mutations of CF, in addition to G551D: G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P and G1349D. With the FDA’s decision, ivacaftor is now available to about 5 percent of the CF population in the United States.

In people with G551D and the additional CF mutations, the defective CFTR protein does not function normally at the surface of the cell. Ivacaftor is also being tested in a Phase 3 trial in children ages 2 to 5 who have at least one of these mutations, with results expected by the end of 2014.

RESULTS FROM GOAL STUDY SHOW MULTIPLE IMPROVEMENTS
The ongoing, multicenter G551D Observational (GOAL) Study, supported by CFFT, is collecting clinical data and samples from more than 150 people with the G551D mutation who are now on ivacaftor. Results from the GOAL Study to date show that those taking ivacaftor have experienced improvements in multiple areas, including increased lung function and body mass index, lower sweat chloride, restoration of mucus clearance, reduced lung infections caused by the bacteria *Pseudomonas aeruginosa* and fewer gastrointestinal symptoms.

Several substudies of GOAL are underway to identify and measure health outcomes closely tied to CFTR function in different parts of the body. These studies could help researchers determine how much CFTR activity is necessary to restore the health of a person with CF and will also inform future drug development efforts targeting the defective protein. As ivacaftor becomes available as a single therapy to more patient groups, the GOAL Study will enroll those with other mutations of CF besides G551D.

In December, Vertex released results from a Phase 3 trial of ivacaftor in people ages 6 and older with the R117H mutation. Close to 3 percent of people with CF have this mutation, which also causes abnormal CFTR function at the cell’s surface. When the results of all participants were averaged, they did not show a sufficient improvement in lung function to meet the primary endpoint of the trial. However, those 18 and older who took ivacaftor did show a significant improvement in lung function, and Vertex is now in discussion with the FDA to determine the next steps for future studies in adults with the R117H mutation.

New Trial Planned for Ataluren
PTC Therapeutics Inc. plans to begin a new Phase 3 trial in 2014 to test the potential CFTR modulator ataluren in people who have nonsense mutations of CF. About 10 percent of people with CF have these rare mutations, which prematurely stop production of the CFTR protein, resulting in a shorter than normal protein that does not function properly. The new study will evaluate the drug in people with nonsense mutations who are not receiving inhaled aminoglycosides (a group of antibiotics that includes TOBI®). Results from an earlier Phase 3 trial indicated that the use of aminoglycosides may have interfered with the effect of ataluren.
CFFT Expands Support for Discovery Programs

Results from late-stage studies of ivacaftor and other potential therapies, now bolstered by findings from the GOAL Study, have provided scientists with critical knowledge that could help speed development of life-saving therapies for all people with CF, including those with rare mutations. To maximize these scientific advances, CFFT has significantly increased its research funding opportunities to identify more potential compounds that treat the basic defect in CF and could improve upon the effectiveness of the first generation of CFTR modulators.

Through its venture philanthropy model, CFFT has attracted an array of leading pharmaceutical companies to the CF effort. In 2013, CFFT expanded its program with Pfizer Inc. to discover new correctors and potentiators to be used in combination to restore CFTR function in people with the Delta F508 mutation. CFFT is also supporting research to identify second-generation correctors through programs with Vertex, Proteostasis Therapeutics Inc., Parion Sciences, Reata Pharmaceuticals Inc. and Genzyme, a Sanofi company.

Just two years after its launch, CFFT’s research laboratory in Bedford, Mass., has made significant progress in its efforts to help speed early discovery of therapies that address the root cause of CF. In addition to running its own tests to find additional correctors to treat the Delta F508 mutation, the CFFT Lab helps verify the activity of potential correctors identified by other researchers and has developed new high-throughput screening tools that it shares with academic and industry groups.

CFFT is also actively pursuing opportunities to find and develop effective treatments for people with rare CF mutations. In one program, the CFFT Lab worked with researchers at the University of Alabama at Birmingham and Southern Research Institute to screen more than 1,600 FDA-approved drugs to determine whether they can help the production of a full-length CFTR protein in people with nonsense mutations of CF. Because these drugs have already been approved as safe, any promising compounds identified could potentially move more quickly into clinical studies in those with CF nonsense mutations.
Preserving and Improving Health in People with CF

Lung Health
Despite the tremendous advances in combating CF lung infection and damage, respiratory problems remain a serious and chronic problem for those living with the disease. Through CFFT, the Foundation continues to support work to advance potential new antibiotics and anti-inflammatories, along with new uses for existing treatments, that could help prevent damage to the lungs.

A leading cause of lung damage in people with CF is the bacteria Pseudomonas aeruginosa, found in about half of all people with CF and more than 70 percent of adults with the disease. In March, the FDA approved TOBI® Podhaler™ (tobramycin inhalation solution), a dry powder form of TOBI, a nebulized antibiotic commonly prescribed to treat CF lung infections caused by P. aeruginosa. The new TOBI Podhaler is administered with a pocket-sized inhaler and does not require a nebulizer or refrigeration, significantly shortening treatment time.

Two late-stage clinical trials of antibiotics to manage chronic lung infections in people with CF were completed this year, and additional studies are underway. A multicenter Phase 3 study of an inhaled formulation of levofloxacin in children and adults with CF evaluated the drug’s effectiveness in controlling lung infections caused by P. aeruginosa and other bacteria. In an earlier Phase 2 study, participants who took the drug showed improvements in lung function and a reduction of P. aeruginosa.

Results from a completed Phase 3 trial of the inhaled antibiotic Arikace™ in people with CF who have P. aeruginosa infections also showed a reduction in the bacteria and improved lung function in those who received the drug. A separate Phase 3 study is investigating Arikace in people with lung disease caused by nontuberculous mycobacteria (NTM), a group of bacteria found in nearly 12 percent of people with CF. One NTM species, Mycobacterium abscessus, is becoming more prevalent and can be very hard to treat. Arikace is able to target the bacteria in cells of the lungs where the bacteria hide from the body’s defenses.

The bacteria methicillin-resistant Staphylococcus aureus (MRSA) is also growing more prevalent and is now found in 25 percent of people with CF. MRSA is resistant to a number of antibiotics and can cause lung infections that often become a chronic condition. CFFT is supporting clinical studies to address this serious problem, including a Phase 2 trial of AeroVanc™, a new inhaled version of the antibiotic vancomycin, in people with CF ages 12 and older. The study will evaluate whether aggressive treatment with the drug can eradicate or suppress MRSA in those with persistent infections, improving lung function and respiratory symptoms and reducing the need for IV antibiotics and hospitalizations.
The EPIC Observational Study is a large, multicenter investigation into early CF lung disease, focused on learning more about the risk factors that may lead to *P. aeruginosa* infections in young children and the impact of those infections over time. Launched in 2004, EPIC was originally a five-year study, enrolling close to 1,800 children, ages 12 and younger.

EPIC has now been extended to 2019 to collect long-term data on lung infections, bacteria and symptoms in the children who participated. Clinical samples from EPIC’s large patient cohort are stored in CFFT’s Biorepository and linked to data in the Foundation’s patient registry. Researchers are studying information from EPIC to identify potential markers of disease progression that could be targeted in future interventional trials of promising CF treatments.

**Nutrition and Good Growth**

In CF, thick secretions build up in the pancreas and prevent the release of digestive enzymes, making it difficult for the body to absorb essential vitamins and nutrients. In recent decades, early diagnosis of CF through newborn screening and the initiation of pancreatic enzyme supplements have helped reduce the frequency of malnutrition in infants with CF. However, many babies and children continue to have poor growth, which appears to have a negative impact on pulmonary function later in life.

The ongoing, multicenter Baby Observational and Nutritional Study (BONUS) is closely following infants with CF through their first year in order to better understand the factors that interfere with good growth. The study is collecting information on the infants’ diet, respiratory and gastrointestinal symptoms, medications and hospitalizations. Findings from BONUS could potentially lay the groundwork for studies of a range of interventions that could help all children with CF get off to a healthy start in life.

The oral antioxidant multivitamin and mineral supplement AquaDEKS® is designed to increase absorption of certain fat-soluble vitamins and other micronutrients in people with CF. Even though they take multivitamins and pancreatic enzyme supplements, people with CF often have low levels of antioxidants and this deficiency may contribute to some of the damage to their lungs. A Phase 2 clinical trial is studying the effects of a reformulated version of AquaDEKS on inflammation, antioxidant levels and oxidative stress in people with CF to determine whether taking more vitamins and antioxidants helps improve lung health.

Many people with CF have insufficient levels of vitamin D, which is crucial to bone health. A Phase 2 trial supported by CFFT is investigating whether vitamin D also plays a role in the immune systems of people with CF. The study will examine whether a large, one-time dose of the vitamin can rapidly correct vitamin D deficiency in people with CF who have been admitted to the hospital for a pulmonary exacerbation.

**Cystic Fibrosis Liver Disease**

About 10 percent of people with CF have a form of cystic fibrosis liver disease (CFLD). While not a common complication of CF, CFLD can be a serious problem that can lead to liver transplantation or death. It is not fully known what factors contribute to CFLD.

Some people with CFLD develop cirrhosis of the liver, caused by thick secretions that block the bile ducts. In cooperation with the National Institutes of Health, CFFT is supporting a large observational study, called PUSH, to test whether certain abdominal ultrasound patterns can help identify those with CF at increased risk for cirrhosis. The largest CFLD study in the world, PUSH could lead to better understanding of the progression to cirrhosis, earlier diagnosis and therapeutic strategies to prevent and treat CFLD.

Claire, 3, who has CF, receives care at SUNY Upstate Medical University in Syracuse, N.Y. Her mom, Jessica, says the family couldn’t ask for a better group of nurses and advocates.
Advancing CF Clinical Research

Therapeutics Development Network
CFFT’s Therapeutics Development Network is a nationwide network of 77 research centers that conduct clinical trials of potential new CF therapies. Since its founding in 1998, the network has conducted more than 100 clinical studies across a range of therapeutic areas, including CFTR modulators, antibiotics, anti-inflammatories and nutritional therapies. A newly expanded section on the Foundation’s website, — www.cff.org/research/TDN — offers information about TDN research sites and services to researchers, industry sponsors and people with CF and their families.

To support the efforts of TDN centers to conduct studies safely and efficiently, the Foundation developed an interactive learning tool, called eQUIP-CR, that helps teams integrate quality improvement (QI) principles into their clinical research programs. While most centers use eQUIP-CR independently, eight centers have participated in a Foundation-supported program, now in its second year, which offers coaching by a principal investigator and research coordinator to help strengthen clinical research activities. The Foundation also offers a mentoring program that matches experienced research coordinators with apprentices who are new to the discipline or to CF.

“I Am the Key” Initiative
The Foundation continues to expand its portfolio of publications designed to increase awareness of and participation in CF clinical trials. As part of its “I Am the Key” education campaign, the Foundation published an illustrated book, Emma Green: Science Superstar, to creatively engage children ages 8 to 12 in the clinical research effort by describing it through the eyes of one young girl with CF. Written by a longtime clinical research coordinator at a TDN research center, the book follows the main character, Emma, as she learns about the different steps in a clinical trial and ultimately decides to volunteer for one herself.

New publications of the Foundation’s “I am the Key” education campaign include expanded Spanish-language resources and an illustrated children’s book about one young CF clinical trial volunteer.
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In keeping with the Foundation’s venture philanthropy business model, CFFT has contractual agreements with Vertex, Pfizer, Genzyme and other companies to receive royalties in the event of the approval and/or sales of certain drugs — including Kalydeco, VX-809 and ataluren — that are developed as a result of CFFT funding. Any royalties CFFT receives are reinvested in support of the Foundation’s mission.
The Foundation's nationwide network of more than 110 CF care centers are at the forefront of driving improvements in the standard of care for people with cystic fibrosis and have been essential to improving the length and quality of life for people with the disease. Key to this progress are the highly specialized, multidisciplinary teams of health care professionals at each center who collaborate directly with people with CF and their families to customize care based on each individual's unique needs.

**Improved Care Leads to Advances in Length and Quality of Life**

Each year, the Foundation collects information on the health of more than 27,000 people with CF who have agreed to share their medical data for research purposes. The data collected gives the CF community a detailed picture of the progress that has been made in CF care and the areas where more improvement is needed.

The most recent data continue to show incremental improvements in the health of people with CF of all ages. Over the years, these improvements have added up to substantial gains in key outcomes like nutritional status and pulmonary function. The median predicted age of survival continues to rise, and the day is quickly approaching when adults will account for more than half of all people with CF.

More people with CF continue to be diagnosed in their first year of life, thanks to nationwide newborn screening. Early diagnosis is critical, as research shows that people with CF who are diagnosed through newborn screening have better weight and healthier lungs later in life than those who are diagnosed only when they show symptoms of CF.

Other aspects of the Foundation's patient registry data underscore the challenges that people with CF still face, such as the high prevalence of secondary complications like cystic fibrosis-related diabetes and depression. Through Cystic Fibrosis Foundation Therapeutics Inc. (CFFT), the Foundation continues to support initiatives to address the full range of health issues related to CF and spur research.
and clinical studies that could lead to new treatments and strategies to help people with CF better manage the complications of the disease.

**Expanding International Collaborations**
The Foundation continues to work closely with CF advocacy groups internationally, and in 2013 expanded its collaboration with Cystic Fibrosis Canada to compare health outcomes of people with CF in both countries, focusing on survival analyses. Similar analyses were conducted using patient registry data from the CF Foundation and Cystic Fibrosis Trust in the U.K., which showed that pulmonary function of children and adults, up to age 25, is significantly better in the United States compared with the U.K. Some therapies, such as hypertonic saline and Pulmozyme®, are used more frequently in the United States than in the U.K., which may in part explain this difference in lung function.

**Foundation Remains Committed to Improving Quality of Care**
As part of its ongoing quality improvement (QI) initiative, the Foundation launched its eighth yearlong Learning and Leadership QI Collaborative aimed at improving care within CF centers. The latest project includes both adult and pediatric care teams and focuses on ensuring a smooth, seamless transfer of care from pediatric to adult programs. To address transitions to sub-specialty care, the collaborative also includes care providers from gastroenterology, endocrinology and other disciplines.

The Foundation completed two Learning and Leadership QI Collaboratives this year, including one focused on addressing issues specific to adult care programs, such as the challenges facing emerging adults (those ages 18 to 25). Nine adult care programs participated in the yearlong series, which called for a partnership between adults with CF, their families and health care providers to improve nutrition, pulmonary care and advanced care; promote disease self-management and shared decision-making; and enhance referral processes to specialty care. Another nine teams participated in a second collaboratively focused solely on improving nutrition and pulmonary outcomes across the continuum of pediatric and adult care.

In partnership with the Dartmouth Microsystems Academy, the CF Foundation convened a yearlong leadership development pilot program to assess individual and team leadership skills and further develop participants’ abilities to spearhead quality improvement at pediatric and adult centers.

**SURVEY GAUGES EXPERIENCE OF CARE AT INDIVIDUAL CENTERS**
The Foundation expanded the reach of its national patient and family experience of care survey — moving it out of the pilot phase to be implemented as part of its care center accreditation process. The survey, carried out in 47 centers in 2013, provides people with CF and their families the opportunity to share their personal experiences of and insights on important care issues, such as ease of communication with care center staff and infection control practices.

The survey results are shared with each center through center-specific reports — giving care teams an invaluable perspective on how care is experienced at their centers. The centers can then apply the feedback to guide their improvement activities.

The Foundation also launched a palliative care initiative to address quality-of-life issues for people with advanced cystic fibrosis. Because people with CF typically receive more aggressive treatment as their disease worsens and, in some cases, as they await lung transplantation, they may benefit from palliative care approaches that could help relieve their pain and other symptoms and complications, including depression.

To take a deeper look at advanced care planning and end-of-life care for individuals with CF, the Foundation has formed a palliative care working group, inviting centers to document their current practices in an effort to establish recommendations. Results from 72 centers revealed that advanced care planning was receiving more attention than had been previously reported, but inconsistent practices and other barriers remain to planning for advanced care and advance directives, such as a reluctance to discuss illness progression and differing priorities among patients, families and medical providers.

Following a request for applications and rigorous peer review, CFFT funded two palliative care projects.
The first is focused on training CF care teams in palliative care and a second is evaluating a model of care for adults with CF that integrates palliative care with routine CF care throughout the course of the disease.
Guidelines Promote Best Practices in CF Care

The Foundation continued to support the development of a robust set of CF care guidelines, which offer a framework that helps all Foundation-accredited care center staff apply the latest knowledge and best practices in CF care.

Following a review of the latest medical data, a Foundation-sponsored committee of experts in CF care and infection prevention and representatives of the CF community updated the care guidelines on infection prevention and control to identify ways to reduce the risk of people with CF spreading or acquiring destructive germs that can lead to life-threatening lung infections. Final recommendations were endorsed by the Society for Healthcare Epidemiology of America and the Association for Professionals in Infection Control and Epidemiology, and will be published in June 2014.

In March, the Foundation released a webcast, featuring Foundation medical leaders as well as a national expert in cystic fibrosis infection prevention and control. Focusing on the latest research, the webcast explained why it is particularly important for people with CF to protect themselves against germs and described ways to minimize the spread of CF-specific pathogens.

The Foundation and the European CF Society partnered to develop guidelines to ensure consistent standards of care for people with CF who are infected with nontuberculous mycobacteria (NTM) — an emerging, harmful pathogen. Recommendations will be published in 2014.

The Foundation undertook a review with the Johns Hopkins Evidence Based Practice Center of existing recommendations and medical literature to inform the development of guidelines for the prevention and eradication of Pseudomonas aeruginosa infections in individuals with CF. The guidelines — the first to address eradication of P. aeruginosa infections — will be published in 2014.

Mentoring Programs Promote Professional Development

Through the Foundation’s care center network, care teams have ongoing opportunities to take part in CF-specific programs that foster quality improvement and professional development. Among them are the highly successful, discipline-specific mentoring programs, which first launched in 2007. During the six-month program, experienced CF clinicians work closely with those new to CF care to improve their skills and knowledge of the disease.

In 2013, a total of 62 apprentices participated in targeted mentoring programs for clinic coordinators, dietitians, social workers, respiratory therapists, physical therapists and research coordinators.

The Foundation’s mentoring programs have continuously received positive feedback, with center directors most commonly noting that mentorships increased team communication, enhanced apprentices’ knowledge and boosted caregivers’ confidence in their work.

AN INTRODUCTION TO CYSTIC FIBROSIS FOR PATIENTS AND FAMILIES

The Foundation released the sixth edition of An Introduction to Cystic Fibrosis for Patients and Families. Authored by James C. Cunningham, M.D., and Lynn M. Taussig, M.D., the comprehensive guide covers all aspects of life with CF, from diagnosis through adulthood, to help patients and families better understand life with the disease. The book is available in English and Spanish.
At the core of the Foundation’s mission is its commitment to help ensure that all people with cystic fibrosis have access to the high-quality, specialized CF care and treatments they need to live longer, healthier lives. As new CF therapies emerge from CFFT’s drug development pipeline, coverage for treatments and access to accredited CF care centers are vital.

In 2013, the Foundation made significant progress expanding and focusing its initiatives aimed at facilitating access to care for people with CF.

**Programs Connect People with CF to Resources and Support**

The Foundation has an array of programs to help people with CF get the health care coverage they need to stay healthy and keep their CF treatment on track. Through the Patient Assistance Resource Center (PARC), people with CF and their families can receive help as they decide on coverage that’s right for them, make the most of their existing health care coverage and find programs to help them pay for treatments.

This year, the PARC received nearly 15,000 calls from members of the CF community, with experts from several PARC programs answering their questions.

The **Cystic Fibrosis Patient Assistance Foundation (CFPAF)** offers the only national patient assistance program designed specifically for the CF community. This year, CFPAF helped more than 1,200 people with co-pay assistance, Social Security application guidance and other coverage issues. Since its inception in 2008, CFPAF has provided financial assistance for deductibles and co-pays to more than 1,600 people with cystic fibrosis, and has helped nearly 500 people with CF apply for Social Security benefits. A subsidiary of the Foundation, CFPAF is funded by contributions from the pharmaceutical industry.

Funded by the Foundation and operated by an outside law firm, the **CF Legal Information Hotline** provides free information about the laws that protect the rights of individuals with cystic fibrosis. The Hotline saw a 10 percent increase in the number of calls received this year — fielding over 5,800 calls from the CF community.
CF AND THE AFFORDABLE CARE ACT

In 2013, the Foundation created comprehensive online resources on health insurance and the Affordable Care Act, providing information on key questions for choosing a health plan and other topics important to the CF community.

The Foundation continued to expand its Patient Assistance Resource Library (PARL), an online database for people with CF, their families, caregivers and insurers. The PARL offers extensive resources to help people with CF get and maintain health care coverage, including information on assistance programs and how to navigate common insurance obstacles. The library also connects health professionals and insurers to such resources as CF care guidelines, evidence-based journal articles and white papers.

The Mutation Analysis Program (MAP) provides free and confidential genetic testing to patients with a confirmed diagnosis of cystic fibrosis to help them and their families make informed decisions about treatment options. Launched in 2012, the MAP has completed nearly 1,500 genetic tests for people who have not yet had testing to determine their CF mutations, or those who have been tested previously but still have one or more unknown mutations. As more drugs targeting the basic defect move through the pipeline, it will become increasingly important for people with CF to know their mutations.

Thanks to the help of the Foundation’s Patient Assistance Resource Center, Scott, 30, did not have to choose between paying for his education or his CF treatments and is now pursuing his dream of earning a doctorate in wildlife ecology.

Case Managers Provide Expert Advice to CF Community

The Foundation expanded its case management team to better serve people with CF, their families and care center staff. Throughout the year, the team helped the CF community with insurance benefits, network exceptions and prior authorizations, and helped individuals gain access to care by assisting with filing appeals and identifying local sources of financial aid. Case managers also helped people with CF navigate the new insurance landscape following the implementation of the Affordable Care Act.

New Project Helps Reduce Administrative Burden on Care Center Staff

The PARC distributed a survey to Foundation-accredited care centers to measure how much time care teams spend on administrative tasks related to access. Results revealed that centers are spending up to 50 percent of their time on reimbursement and coverage activities for their patients.

One particularly taxing responsibility for center staff, and a common barrier to coverage, is the process known as prior authorization, which requires physicians to get approval from a patient’s health insurance plan when prescribing certain medications. The Foundation launched a project with CoverMyMeds, a health care technology company, to help reduce the amount of time care center staff spends on administrative tasks.

Through this service, physicians and pharmacists can complete prior authorization and other insurance forms for almost all drug plans — allowing care center staff to spend less time on paperwork and more time with patients.

Outreach Prompts Action

Federal and state governments play a vital role in CF research, drug development and the ability of people with CF to access care centers and therapies. Through its advocacy efforts, the Foundation continued to work with local chapters to engage more than 140,000 volunteers to educate others about CF, help raise awareness of the disease and build support at the federal and state levels.

Cystic fibrosis advocates held more than 400 meetings with elected officials and sent almost 55,000 messages to legislators. This outreach spurred a number of federal and state accomplishments, prompting, in one instance, 37 members of Congress to send letters to the Social Security Administration (SSA) expressing their concern about a proposed rule that could make it more difficult for people with CF to receive disability benefits.
National Advocacy Efforts Advance CF Research and Drug Development

The Foundation worked with Congress and federal agencies to support critical research and the swift development and review of vital new CF treatments. Foundation staff and advocates held hundreds of meetings with members of Congress and their staff, urging them to provide greater funding for biomedical research at the National Institutes of Health and for drug review and approval at the U.S. Food and Drug Administration (FDA) to help move therapies more quickly to the people who need them. Following these efforts, both agencies received increased funding for fiscal year 2014, which could help people with CF and other rare diseases.

Local Advocacy Efforts Boost Foundation Outreach to State Officials

Through letter-writing campaigns and extensive grassroots advocacy, the Foundation continued building state efforts to ensure all people with CF have access to high-quality, specialized care. In partnership with Foundation chapters, care centers and volunteer advocates, the Foundation conveyed the importance of quality CF care and treatments in all 50 states.

State advocacy chairs and care center staff partnered with their local chapters to hold meetings in 18 governors’ offices across the country to explain how vital Medicaid and other coverage programs are to the CF community. In addition, the Foundation submitted testimony to 22 state committees that were reviewing coverage for key CF therapies, including inhaled antibiotics and enzymes. Care center physicians supported the Foundation’s message on behalf of their patients in several of these committee reviews.

Foundation Encourages the Development of Patient-Centered Care Delivery System

As challenges and questions continue to emerge on the rising cost of health care and therapies, the Foundation is at the forefront of discussions supporting a patient-centered care delivery system. This care model promotes quality improvement by involving patients and families in the development of customized care plans. Serving as a leading voice on the issue, the Foundation identified opportunities to develop this delivery system for CF care and to recruit policy and health care experts to help in the effort. Foundation executive leadership and staff co-hosted a multi-stakeholder forum, entitled “Patient Value and Innovation,” and participated in other prominent forums, including congressional briefings and meetings to advance the effort.

Volunteers conducted more than 400 meetings with elected officials to raise awareness of cystic fibrosis, advance CF research and drug development, and promote access to care.
RAISING FUNDS TO FUEL
THE SEARCH FOR A CURE

Some walked. Some biked. Others danced, climbed or golfed. Together, thousands of dedicated volunteers, donors, corporate sponsors and friends from across the country came together in 2013 so that people with CF can breathe easier and live longer, healthier lives.

Determined to help find a cure for cystic fibrosis, thousands from the CF community and beyond participated in and contributed to hundreds of unique events throughout the United States, raising tens of millions of dollars to support the Foundation’s mission.

For the second year in a row, sporting-based events — including Xtreme Hike and CF Cycle for Life — grew in popularity, capturing the attention of amateur and professional athletes across the country and inspiring new friends to join the search for a cure.

Thousands of long-time friends of the Foundation answered the call to become members of Partners in Progress, the Foundation’s Annual Fund, now in its third year.

To accelerate vital CF drug discovery and development efforts, major donors continued to power the Foundation’s mission, many reaffirming their commitment to the Foundation’s major giving campaign, Milestones II: Accelerating the Search for a Cure, and many others joining for the first time. More than 100 first-time donors generously contributed to the Milestones campaign in 2013.

Across all chapters, Foundation staff continued to provide crucial education for the public about the disease and keep the CF community informed about resources and services offered by the Foundation, including information on CF care centers and clinical trial opportunities.

Great Strides Walkers Step Up Their Support for a Cure
The Foundation’s largest national fundraising event, Great Strides, continued to gain momentum, generating a remarkable $41.3 million in 2013 — an increase of more than 5 percent over the previous year. Inspired by the difference the CF Foundation is making in the lives of those touched by the disease, more than 125,000 walkers laced up their shoes to participate in approximately 600 walks across the country.

For the third year in a row, families affected by the disease, corporate supporters and friends of the Georgia Chapter rose to the occasion to

Families from across the D.C. metro region came together to help support the mission of the Foundation at the Metropolitan Washington, D.C. Chapter’s Great Strides event in Bethesda, Md.
generate more than $1 million at the Atlanta Great Strides Walk. Leading the way was the Baker family of Atlanta, whose team, the Baker Boys’ Battalion, raised $331,213 to secure the position as the top overall fundraising Great Strides team in 2013. Team Sean’s Pals, led by the Squires family of the Greater New Jersey Chapter, became the second-highest overall fundraising team after inspiring allies in the fight against CF to give an incredible $226,136.

**Unique, Diverse Special Events Break Fundraising Records**

Young professionals, community leaders, supporters new to the cause and cherished friends of the Foundation generously gave their time and talents to raise more than $95 million at hundreds of galas and special events, hosted by the CF Foundation’s 70 chapter and branch offices.

The Metropolitan Washington, D.C. Chapter wowed the CF and philanthropic communities by generating an astonishing $3.8 million through its *Breath of Life Gala*. The event, hosted by Scott Pelley, CF Foundation ambassador and anchor and managing editor of *CBS Evening News*, marked the single largest night of fundraising in the Foundation’s history.

**NEW WEBSITE FOR GREAT STRIDES**

In October, the Foundation launched a new Great Strides website that provides visitors with more powerful tools they can use to fundraise effectively. The new site encourages healthy competition and enhances overall social media sharing via Facebook, Twitter and LinkedIn. A new Great Strides mobile application allows walkers to easily solicit donations via email, text and social media while on the go.

Guests at the Greater New York Chapter’s *Breath of Life Gala* celebrated the event’s 10th anniversary by raising nearly $2 million to support the Foundation’s mission. The night featured an elegant dinner, a musical performance by the New York Pops Band, a heartfelt *Bid for a Cure* speech and touching remarks from current and past honorees who have dedicated themselves to adding tomorrows to the lives of all people with CF.

Treated to unique culinary experiences by some of Baltimore’s top chefs, guests at the Maryland Chapter’s *Passion for Food and Wine* event supported the search for a cure by raising $180,000, while more than $365,000 was raised by the energy industry community at the Texas Gulf Coast Chapter’s *Energy Giving Back* celebration.

Young entrepreneurs across the country showed their commitment to the CF cause at 46 different “Finest” events, collectively raising more than $2.7 million for the Foundation. The Western Pennsylvania Chapter’s *50 Finest* celebrated 25 men and 25 women in the local business community who are committed friends of the Foundation, while 25 young professionals in Little Rock were recognized at the Arkansas Chapter’s *Taste of the Finest* event. In addition to raising critical funds to support the mission, honorees participated in socials and group meetings where they had the chance to network and learn more about cystic fibrosis and the many Foundation programs and activities that benefit people with CF and their families.
Cycle and Hike Volunteers Rise to New Heights

The Foundation’s fastest-growing fundraising event, CF Cycle for Life, saw a remarkable 30 percent increase over the previous year, raising $3.4 million to help support the search for a cure. Approximately 5,000 cyclists pedaled their way through miles of rolling hills and city streets in 42 events across the country.

The Massachusetts/Rhode Island Chapter’s ambitious cycle program raised more than $200,000 — up from $92,000 the previous year — while the Western Pennsylvania Chapter’s inaugural cycle event netted $60,000.

The number of Xtreme Hike events more than tripled in 2013, generating an outstanding $1.4 million. Missouri’s Gateway Chapter raised $92,000 at its inaugural hiking event, making it the most successful first-time Xtreme Hike event to date.

Partners in Progress

Inspired by the touching stories of families affected by CF, nearly 10,000 members of Partners in Progress, the Foundation’s Annual Fund, donated $2.3 million to help the Foundation carry out its mission. Gifts to the Annual Fund, now in its third year, help provide the vital resources the Foundation needs to reach its goal of finding a cure and improving the quality of life for all people with CF.

Foundation’s “Top Agents” Gather to Share Successes at Leadership Conference

The Foundation’s 2013 Volunteer Leadership Conference, held in Reston, Va., opened with a single question: Do you accept the mission to cure CF? The question was met with a resounding “Yes!” from 500 of the Foundation’s most committed volunteers, corporate sponsors and staff, who came together to meet new friends and colleagues, share fundraising success stories and learn more about the state of CF science and how Foundation programs can help people with CF and their families address the challenges of living with this disease.

Led by co-chairs Mary and Lou DeFalco, the conference’s theme “Mission: Possible” reflected the unyielding determination of the CF Foundation and its volunteers to find a cure for all people with CF.

The conference also provided a venue to recognize and honor many of the Foundation’s most inspirational volunteer leaders, whose work has made a lasting impact on the lives of those touched by cystic fibrosis.

Stacy Motenko, a lifelong volunteer for the Foundation, world traveler and CF advocate, was honored with the Alex Award for her bravery and resolve to help the CF community. Named in memory of the daughter of Frank Deford, chair emeritus of the Foundation’s Board of Trustees, the award recognizes someone with CF who is a role model for those with CF.

Amos Beason and John Barlow received the Jena Award for their role as “founding fathers” of the CF Foundation’s Xtreme Hike event, which raised $1.4 million nationally in 2013 and has challenged more than 300 hikers since 2009. The award recognizes outstanding volunteers with no direct personal connection to CF and is presented by longtime volunteer leaders Marc and Margarete Cassalina, whose daughter, Jena, lost her battle with CF at the age of 13.
Corporate supporters and friends continued to play an instrumental role in helping the Foundation achieve its mission to find a cure for CF.

For 29 years, American Airlines has been a loyal friend whose steadfast commitment to help people with CF live longer, healthier lives is an inspiration. The company has led the way in corporate giving by generously hosting unique national events, including its signature Celebrity Ski event and the premiere Ultimate Golf Experience, and by offering flight packages to be used as auction items at Foundation special events. American Airlines again proudly served as the Foundation’s Outstanding Corporate Partner, providing invaluable support of the Foundation’s mission to cure cystic fibrosis.

From grassroots efforts by employees to national corporate giving campaigns, BB&T Bank has supported the Foundation’s mission for more than five years. In 2013 alone, chapter events in six states were powered by BB&T’s corporate and employee engagement.

Small Luxury Hotels of the World joined the search for a cure in 2013 by offering hotel and vacation packages at CF Foundation events and galas. Stays at the independently owned boutique luxury hotels were offered as auction items at some of the Foundation’s most prestigious chapter events and raised $154,000.

Corporate support also played a critical role on Great Strides walk days and at CF Cycle for Life events, with thousands of employees from AbbVie, Walgreens, GEICO, Chubb, Aptalis, Vertex and many other corporate friends coming together to help find a cure for CF and improve the quality of life for those with the disease.
Major Donors Reaffirm Their Commitment to a Cure

Thanks to advances in CF research and care supported by the Foundation, people with CF are living longer, healthier lives and are meeting life’s milestones in ways they never before thought possible. They are graduating from high school and college, pursuing careers, getting married and starting families of their own. In 2012, the first drug to treat the underlying cause of the disease — discovered and developed with essential Foundation support — was approved by the U.S. Food and Drug Administration for a small segment of the CF population, providing an important roadmap for the development of more new treatments that target all mutations of cystic fibrosis.

These significant achievements would not be possible without the exceptional generosity and steadfast commitment of major donors, who boldly stand as champions of the Foundation’s mission. This extraordinary group of dedicated individuals continues to play an integral role in accelerating the development of therapies that could lead to a cure for all people with CF.

Eager to keep the momentum for drug development efforts going strong and not lose a single opportunity to accelerate progress, the leader of the Milestones to a Cure Campaign, Joe O’Donnell — entrepreneur, philanthropist and father of a beloved son Joey, who lost his life to CF — agreed to stay at the helm to spearhead a second phase of the campaign. The first phase of the Milestones campaign raised an incredible $175 million between 2004 and 2010, and many of the original donors joined O’Donnell in renewing their commitments.

Milestones II: Accelerating the Search for a Cure set an aggressive new goal of raising $75 million by the end of 2015. Inspired by the tenacity of those who came before them, Milestones II donors and Milestones Club members have put the campaign ahead of schedule. In just three years,
"We are closer than ever before to finding a cure for CF, and my wife Lisa and I feel lucky to be involved with the Foundation at this exciting time. **We’re committed to doing everything we can to help the Foundation succeed.**"

– Steve Orlando, CF grandfather

*Milestones* members enjoyed a once-in-a-lifetime benefit concert featuring Celine Dion, the best-selling female music artist of all time.

this outstanding group has contributed $56 million toward the campaign’s goal, with $19 million donated in 2013.

Bolstered by the accomplishments their determination and foresight have helped create, *Milestones* members are committed to embracing all opportunities that will speed the pace of CF drug discovery and development and bring the CF community yet closer to its ultimate goal: a cure for all people with CF.

**The Paul Di Sant’Agnese Planned Giving Society**

Named in memory of one of the pioneering medical leaders in cystic fibrosis, the Paul di Sant’Agnese Planned Giving Society was established to recognize those generous individuals who have included the Cystic Fibrosis Foundation in their long-term plans, through a bequest, life-insurance gift or other estate planning vehicles. As one of the CF Foundation’s most valued groups of supporters, the Paul di Sant’Agnese Planned Giving Society honors the tradition of excellence that Dr. di Sant’Agnese pursued in developing the sweat test and throughout his distinguished medical career. Members of this supportive group contributed more than $2.48 million in bequests to the CF Foundation in 2013.

**The Doris F. Tulcin Major Giving Society**

A pillar of the CF community and founding parent of the CF Foundation, Doris F. Tulcin continues to inspire others to understand the important role of major giving in the advancement of CF research and drug development. Cultivating the support of major donors, she helped lead the effort to fund the establishment of the research development network. Nearly 60 years later, the Doris F. Tulcin Major Giving Society continues to honor her leadership and vision by recognizing the outstanding generosity of its 330 members who have made total commitments of $100,000 or more to the Foundation’s major giving initiatives since 1998.
CYSTIC FIBROSIS FOUNDATION
CONSENSOLED FINANCIAL INFORMATION*
CONSOLIDATED STATEMENT OF FINANCIAL POSITION
as of December 31, 2013 and 2012

Assets
Cash and cash equivalents $57,811,689 $89,335,582
Investments 611,130,801 315,250,322
Prepaid expenses and other assets 1,880,156 1,716,452
Receivables, net 16,294,391 22,927,904
Membership interest in specialty pharmacy 6,979,043 7,286,043
Fixed assets, net 4,367,186 3,204,597
Total assets $698,463,266 $439,720,900

Liabilities and Net Assets
Accounts payable and accrued expenses $22,624,610 $25,136,530
Awards payable 78,072,810 63,954,487
Total liabilities 100,697,420 89,091,017
Unrestricted net assets:
Undesignated net assets 335,947,148 177,610,330
Board-designated net assets 239,000,000 153,000,000
Total unrestricted net assets 574,947,148 330,610,330
Temporarily restricted net assets 18,850,884 16,291,346
Permanently restricted net assets 3,967,814 3,728,207
Total net assets 597,765,846 350,629,883
Total liabilities and net assets $698,463,266 $439,720,900

Investments
Investments as of December 31, 2013 included primarily corporate bond mutual funds, short duration bond mutual funds, equity mutual funds, inflation hedge mutual funds and alternative investment funds of funds. Authoritative guidance on fair value measurements requires an entity to maximize the use of observable inputs when measuring fair value. The guidance describes three levels of inputs that may be used to measure fair value: Level 1 - Quoted prices in active markets for identical assets or liabilities. Level 2 - Observable inputs other than Level 1 prices, such as quoted prices for similar assets. Level 3 - Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets. The Foundation carries its cash and cash equivalents, all investment balances and certain other assets at fair value. Financial instruments measured at fair value on a recurring basis were $484,322,357 in Level 1 assets, $92,709,644 in Level 2 assets and $44,112,265 in Level 3 assets.

Operating lease commitments
The Foundation is obligated under various operating leases for office space as of December 31, 2013. The approximate future minimum rental commitments, subject to escalation, are $19,762,422.

Awards payable and commitments
The Foundation and Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) generally award medical/scientific grants and contracts for periods of three years or less. Grants are awarded contingent upon the availability of funds at the beginning of each award period. As of December 31, 2013, the Foundation and CFFT have medical scientific grant commitments of approximately $25,628,000, which extend through December 31, 2020. These subsequent year awards are contingent upon renewal criteria, and therefore the costs and liabilities are not reflected in the consolidated financial statements. Certain CFFT agreements provide for future contracted drug discovery and development research payments amounting to approximately $74,538,000. These costs will be expensed when the services are provided.

Unrestricted – Board-designated net assets
The Foundation’s Board of Trustees has designated $239,000,000 of the Foundation’s net assets as of December 31, 2013 to be used for drug discovery and development programs.

*The independently audited financial statements of the Cystic Fibrosis Foundation are available online at the Foundation’s website, cff.org, or by contacting Cystic Fibrosis Foundation, 6931 Arlington Road, Suite 200, Bethesda, MD 20814.
Revenue

Support received from the public
  Special event revenue $111,517,097 $104,375,857
  Direct benefit expenses (13,429,403) (12,866,218)
  Net special event revenue $98,087,694 $91,509,639
  General contributions 39,225,217 42,580,399
  Total support received from the public 137,312,911 134,090,038
  Investment income 79,578 1,372,381
  Royalty revenue 257,340,860 156,593,238
  Other 10,797,035 5,624,365
  Total revenue $405,530,384 $297,680,022

Expenses

Program services
  Medical programs 134,684,528 114,438,974
  Public and professional information and education 17,842,159 17,241,391
  Community services 8,135,278 7,801,153
  Total program services 160,661,965 139,481,518
Supporting services
  Management and general 13,755,362 10,588,156
  Fundraising 15,912,998 16,092,899
  Total supporting services 29,668,360 26,681,055
  Total expenses 190,330,325 166,162,573
  Increase in net assets from operations 215,200,059 131,517,449

Discontinued operations

Gain from discontinued pharmacy operations - 29,203,832

Other changes in net assets

Net nonoperating investment income 31,935,904 14,459,687
Increase in net assets $247,135,963 $175,180,968

Measure of operations

The Foundation includes in its measure of operations all support received from the public, income on investments designated for operations including interest and dividends and realized and unrealized gains and losses, royalty revenue, other revenue and all costs of program and supporting services. The measure of operations excludes gains or losses on discontinued operations and nonoperating investments. Nonoperating investments are amounts identified by the Investment Committee of the Board of Trustees for investment over an intermediate term.

Revenue recognition

Support received directly or indirectly from the public is recorded as revenue when received or when the donor has made an unconditional promise to give. Conditional promises to give are not recognized until the conditions on which they depend are substantially met. Contributions of assets other than cash, including gifts-in-kind, are recorded at their estimated fair value at the date of the gift. Contributions received are recorded as unrestricted, temporarily restricted, or permanently restricted support, depending on the existence or nature of any donor restrictions. All donor-restricted support, including related investment income and realized and unrealized gains and losses, is reported as an increase in temporarily or permanently restricted net assets, depending on the nature of the restriction. When a restriction expires (that is, when a stipulated time restriction ends or purpose restriction is accomplished), temporarily restricted net assets are reclassified to unrestricted net assets and reported in the consolidated statement of activities as net assets released from restrictions.

At times CFFT may sell its rights under certain agreements in exchange for a lump sum. Amounts received under these agreements are recorded as royalty revenue when rights are forfeited and proceeds are receivable.

In May 2012 and in May 2013, CFFT entered into agreements to sell a portion of its future royalty revenue under a drug discovery agreement. Net royalty revenue in 2013 includes $247,900,946 relating to the May 2013 transaction, which consists of gross proceeds of $250,000,000 net of $2,099,054 of transaction costs. Net royalty revenue in 2012 includes $146,120,334 relating to the May 2012 transaction, which consists of gross proceeds of $150,000,000 net of $3,879,666 of transaction costs.

Discontinued operations

Established in 1988, Cystic Fibrosis Services, Inc. (CFS) is a full-service specialty pharmacy specializing in cystic fibrosis medications, patient advocacy and reimbursement support. CFS is a fully owned subsidiary of Cystic Fibrosis Foundation Pharmacy, LLC (CFFP). On December 6, 2012, the Foundation completed its sale of pharmacy operations and recorded a gain on sale totalling $27,022,449. Under the terms of the sale agreement, the purchaser, an unrelated third party, acquired 80% of the outstanding membership interests of Cystic Fibrosis Foundation Pharmacy, LLC (CFFP). The Foundation retained a 20% interest in CFFP totalling $7,286,043 which is reported on the December 31, 2012 statement of financial position as membership interest in specialty pharmacy.

There was no income from discontinued operations in 2013. In November 2013, the Foundation sold a portion of its investment back to the purchaser. The Foundation received $307,000 in connection with this transaction and accordingly reduced its membership interest in specialty pharmacy. The membership interest in specialty pharmacy at December 31, 2013 is $6,979,043.
LOOKING FORWARD: AN EXPANDED MISSION

In 2013, the Foundation engaged in the most comprehensive strategic planning process in its history with a goal of charting the Foundation’s path for the next five years and ensuring that we are well positioned to achieve our mission.

The 18-month effort engaged the insights and expertise of nearly 200 individuals from every part of the CF community, from every part of the country — including adults with CF, parents, caregivers, donors, researchers and Foundation and CFFT staff — who served on the Strategic Planning Committee and on discipline-specific task forces dedicated to creating measurable steps for going forward. Informing the process further were hours of research and hundreds of discussions with others in the CF community who generously shared their personal experiences to broaden the perspective of all who participated in this endeavor.

The result was a comprehensive, milestone-driven plan that includes multiyear steps to implement key strategies across every division of the Foundation. The Plan was unanimously approved by the Board of Trustees, along with a revised mission statement that reflects a clearer vision of the Foundation’s role within the CF community and its commitment to people with CF and their families. In addition to curing cystic fibrosis, the Foundation will intensify its efforts to provide all people with CF the opportunity to lead full productive lives by funding research and drug development, promoting individualized treatment and ensuring access to high-quality, specialized care.

Strategic planning members devised the following five key strategies to be carried out over the next five years. A sixth strategy was added when another important concern came to light: the need to better understand, listen to and address the concerns and priorities of the growing adult CF population.

1. Support and grow the pipeline of innovative therapies that modulate the defective CFTR protein in CF and treat various manifestations of the disease.

2. Develop a multipronged approach to help people with CF overcome barriers to adhering to their CF treatments.

3. Develop and implement plans that will help ensure all people with CF have access to high-quality, specialized care, Foundation-accredited CF care centers and prescribed therapies.

4. Expand communications to, among other goals, more fully engage the entire CF community.

5. Increase fundraising to achieve the Foundation’s mission and objectives.

6. Increase engagement of the growing adult CF population and establish an adult advisory council to give adults with CF a greater voice in Foundation initiatives.

With the solid support of the Board behind this effort, implementation and execution of the Strategic Plan began immediately at the end of 2013 and is a significant part of the Foundation’s work for 2014. An important feature of the Strategic Plan is its ability to be adjusted and modified as we move forward in order to accelerate our progress toward our shared goal: longer, healthier lives for all people with cystic fibrosis and a cure for 100 percent of those living with the disease.
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AND CORPORATE OFFICERS
AS OF DECEMBER 31, 2013

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