



# Our Commitment TO A CURE

A STRATEGIC PLAN FOR MORE TOMORROWS

2014–2018



# CONTENTS

1 Letter from the President and CEO

4 Envisioning the Future

## OUR SIX STRATEGIES

19 Pipeline and Promise

27 Adherence

33 Access to Treatment and Quality Care

41 Communications

47 Fundraising and Outreach

55 Listen and Engage CF Adults

63 Appendix

72 Conclusion

# Dear Friends:

This is truly a transformational time for the Cystic Fibrosis Foundation and the CF community. We have achieved remarkable progress in the clinical development of life-changing therapies targeting the root cause of cystic fibrosis (CF). Our scientific projects to speed discovery of the next generation of disease-modifying drugs are making encouraging gains, and assure us that we are well on the way toward effective treatments for all people with CF, including those with rare mutations.

Fueling these successes is the steadfast support of an incredible community of donors, volunteers and friends, which continues to grow every day and allows us to forge new relationships and open new doors in pursuit of our mission to cure CF.

As a forward-thinking organization, tackling many challenges and achieving significant milestones throughout our journey, we have never been willing to coast on a projected trajectory. Now, at a time of unprecedented hope and optimism in our community, the importance of our mission demands that we *take even bolder steps forward* and — with courage, innovation and focus — explore every pathway that can help us reach our goal.

While some organizations take on major institutional initiatives at times of uncertainty or crisis, truly great organizations pursue close self-examination at times of success to discover ways to reach new heights. Following the FDA approval of the first drug targeting the root cause of CF in a small segment of the population in 2012, the Foundation's Board of Trustees and I concluded that it was time for the Foundation to take a deep and objective look at our mission, goals and objectives, and ask ourselves: What more *could* we do?

We asked ourselves: Is our mission still relevant? What has changed in the environment in which we operate? What new challenges or concerns have actually been generated by our success? What avenues should we explore that will propel us further and faster toward achieving our mission?

This report details the amazing journey the CF community began in November 2012 to undertake the most comprehensive strategic initiative in our Foundation's history. It describes our updated mission and identifies new and immediate priorities that will move us forward.

**Every step of our five-year strategic plan reflects our unrelenting determination to explore all opportunities that could lead to promising new treatments and enhance lifesaving care for 100 percent of the CF population.**

In building our plan, we listened to the people who are at the heart of all we do — people with cystic fibrosis. Their insights and experiences informed the goals and priorities we set forth. Most important, our strategic plan underscores our greatest promise to the CF community: We will not rest until a cure has been found for every single person with this devastating disease.

We are deeply grateful to the many individuals from all sectors of the CF community and the outside experts who generously contributed long hours in meetings, conference calls, and heartfelt discussions during this important process. The result is a testament to their hard work, creative thinking and strong commitment to the Foundation's future and the fulfillment of our shared mission.

**ROBERT J. BEALL, PH.D.**  
PRESIDENT AND CEO

**As the mother of an adult with cystic fibrosis, I measure the success of the Foundation in the most important terms I know — the opportunity my son and all people with CF have to live longer, healthier, more productive lives. As we envision a future free from the burdens of this devastating disease, this report charts the course that we need to take to make our vision a reality.**

**CATHERINE C. MCLLOUD**

CF PARENT, FLORIDA; CHAIR, CF FOUNDATION BOARD OF TRUSTEES



# Envisioning the Future

# Planning Through Consensus

The Cystic Fibrosis Foundation began its 18-month strategic initiative with a three-day meeting of the Strategic Planning Committee in November 2012. Appointed by the CF Foundation Board of Trustees, the planning committee comprised 29 individuals, representing a microcosm of the CF community—people with CF and CF family members, Foundation volunteers, care center professionals, researchers, pharmacists, dietitians and Foundation Board members, as well as chapter and National Office leaders. The goal was to build the most comprehensive strategic plan in the CF Foundation’s history. No subject would be off limits, and every voice would be welcomed.

## STRATEGIC PLANNING COMMITTEE

### PAM BAKER

CF parent, Georgia

### ROBERT J. BEALL, PH.D.

President and CEO, CF Foundation

### PIPER BEATTY, J.D.

Adult with CF, Colorado

### DRUCY S. BOROWITZ, M.D.

Director, CF Center, Women and Children's Hospital of Buffalo; Professor of Pediatrics, SUNY at Buffalo School of Medicine and Biomedical Services

### PRESTON W. CAMPBELL, III, M.D.

Executive Vice President for Medical Affairs, CF Foundation

### BEAN CORCORAN

CF parent, Connecticut

### AMY DEMARIA

Senior Vice President of Communications, CF Foundation

### DAN DEMSHER

CF parent, California

### MARY DWIGHT

Senior Vice President of Public Policy and Community Affairs, CF Foundation

### TERESA ELDER

CF parent, Colorado

### LAURA GORDON

CF parent, Maryland

### GAYLE GREENBERG

CF parent, Maryland; Senior Director of Major Gifts, CF Foundation

### MICHELLE HOWENSTINE, M.D.

Pediatric Pulmonologist, Riley Hospital for Children, Indianapolis

### FELICE KELEM

Senior Development Director, Delaware Valley Chapter, CF Foundation

### DANIEL KLEIN, M.H.S.

Senior Vice President of Patient Access Programs, CF Foundation

### C. RICHARD MATTINGLY

Executive Vice President and COO, CF Foundation

### CATHERINE C. MCLOUD

CF parent, Florida; Chair, CF Foundation Board of Trustees

### MARYBETH MCMAHON, PH.D.

Vice President of Communications, CF Foundation

### BOB NIEHAUS

CF relative, New York; CF Foundation

*Milestones to a Cure* committee member

### JOE O'DONNELL

CF parent, Massachusetts; Chair, CF Foundation

*Milestones to a Cure Campaign*

### ANN PALMER

Senior Vice President of Field Management, CF Foundation

### JOSEPH PILEWSKI, M.D.

Adult CF Program Director, University of Pittsburgh Medical Center

### BLAIR PLOTT

CF parent, Alabama

### MOLLY RILEY

Executive Director, Greater Illinois Chapter, CF Foundation

### BERYL J. ROSENSTEIN, M.D.

Professor of Pediatrics, Johns Hopkins University School of Medicine

### BILL SQUIRES

CF parent, New Jersey

### CHARLES J. THAYER

Trustee, CF Foundation Board of Trustees

### TED TORPHY, M.D.

Head of Research Strategy, CF Foundation

### VERA H. TWIGG

Executive Vice President and CFO, CF Foundation



Even before our first meeting, we recognized that an undertaking of this magnitude would be more effective if we had an independent, expert facilitator to guide us. After an extensive search, we secured the services of Howard Feddema, Ph.D., a noted leader in strategic facilitation whose philosophy of “consensus” decision-making became a cornerstone of our process. With this philosophy, no one wins and no one loses in any debate on any topic. At each stage, focused exercises and open, respectful and dynamic discussions advance the process until consensus is achieved by the committee or expert group.

Another fundamental feature of the process was our agreement that the plan we created would be a “living” or evolving plan that had the capacity to change to meet future needs. Understanding that no person or even group of people had the ability to project the future or fully anticipate changing circumstances, we unanimously agreed that the plan must help us address challenges and goals as we currently see them, and be flexible enough to allow us to change our goals and priorities as we adapt to new circumstances over the five-year period.

Through extensive and robust discussions, the planning committee analyzed volumes of data, listened to each other intently, and identified and reached consensus on the most important issues facing the Foundation. Setting rank and personal preconceptions aside, these extraordinary individuals contributed not only their invaluable expertise and experience to the committee but their enduring passion and commitment.

**Why is our strategic plan so important?  
Because if you don't build a plan, you will drift. And if you drift, you waste time. Our greatest enemy in the search for new treatments and a cure is the clock. We do not have a moment to waste. With so many lives depending on our progress, we must ensure that every step we take brings us closer to achieving our ultimate goal.**

**C. RICHARD MATTINGLY**

EXECUTIVE VICE PRESIDENT AND CHIEF OPERATING OFFICER,  
CF FOUNDATION



The planning committee began the more than yearlong process with a thorough examination of the DNA of our organization, identifying our shared beliefs and defining our strengths and weaknesses. No topic was off limits for discussion. In addition, the committee took a hard look at important external influences that impact our work now or will in the future. The committee also addressed the

shifting medical and scientific environment, and forecasted political and economic influences, the changing demographics of the CF community, and evolving technology and media landscapes.

## SHARPENING OUR MISSION

After establishing our common understanding of the Foundation's role in addressing the challenges that lie ahead, the planning committee began by reviewing the Foundation's most fundamental statement of purpose: our mission.

Originally written in 1955, our mission statement has never been substantively altered. Over the course of our nearly 60-year history, one of the Foundation's greatest strengths has been our ability to stay laser-focused on a cure. Yet in light of our recent successes and the changing needs of the CF community, we agreed the time had come to re-examine our purpose and how we can best serve the CF community now and in the years ahead.

The committee analyzed, discussed and deliberated each word of our mission statement at length, tackling important issues at the core of who we are and what we do. In particular, discussions focused on the fundamental word "cure" and the questions it raised: What does it mean to people living with the disease and to all of us dedicated to achieving it? What would a cure actually look like? Would it come in the form of a pill that is taken daily or a therapy we couldn't yet imagine?



The committee concluded that we will have achieved a cure when all those living with cystic fibrosis can live long and productive lives.

We also agreed that — whatever form a cure may take — to reach our ultimate goal we must constantly evaluate, reposition and remain strategic every step of the way.

Reflecting on the way forward, it was obvious to the group that the Foundation must remain committed to world-class science that will produce lifesaving new treatments and a cure. Research will remain at the core of the Foundation’s work to fulfill our mission. The notion that our mission statement needed to include *all* people with cystic fibrosis — not just those with specific mutations or people of certain ages or at certain stages of the disease — was also met with unanimous support. Because of the uniqueness and complexity of CF, the committee also felt it was important to include in the Foundation’s vision for the future a continued commitment to high-quality and specialized care.

Accordingly, the committee agreed to revise and expand the Foundation’s mission statement to reflect these core goals and set the stage for the next phase of our work:

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.

**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.

# Identifying Objectives to Achieve Our Mission

To accomplish our newly expanded mission, and in consideration of the critical issues identified in the strategic planning discussions, the committee agreed on the following key and measurable objectives for the CF Foundation over the next five years:



- **We will develop new, more effective and efficient therapies that address all stages of the disease for all people with CF.**

We have built a productive, respected and successful scientific agenda. Without question, we have been known historically as a highly effective research organization. Through our unwavering commitment to advance drug development and build a robust pipeline of potential therapies, we have achieved remarkable progress. Yet we recognize that we must now respond to new challenges in the world of CF research and care — including the need to target rare mutations — many of which are the product of our success.

- **By 2016, CFTR-modulating therapies will be available to 65 percent of people with cystic fibrosis, and we will have in place a robust pipeline of CFTR-modulating therapies for the entire CF population.**

Finding a cure remains the core of our mission, and we stand firmly committed to address all mutations of the disease. Reflecting the importance of this priority, the Foundation is committed to a significant increase in its annual medical budget, acceleration of pipeline development, and exploration of small molecule compounds and other innovative strategies that show promise.

**All people with CF will have access to the highest quality of care and state-of-the-art therapies.**

As we work to find effective new treatments and a cure that addresses all CF mutations, we also intend to ensure that all patients (children and adults) have access to the best possible care. We will set the bar for our entire Care Center Network to achieve the highest standards of care, and will provide funding to support centers' efforts to improve the quality and delivery of care and facilitate communication among centers and all stakeholders. In addition, as the health care landscape continues to undergo significant changes, the Foundation will continue to advocate strenuously on behalf of all people with CF. It will become the undisputed primary and most trusted source of information about the disease and the issues faced by those living with it.

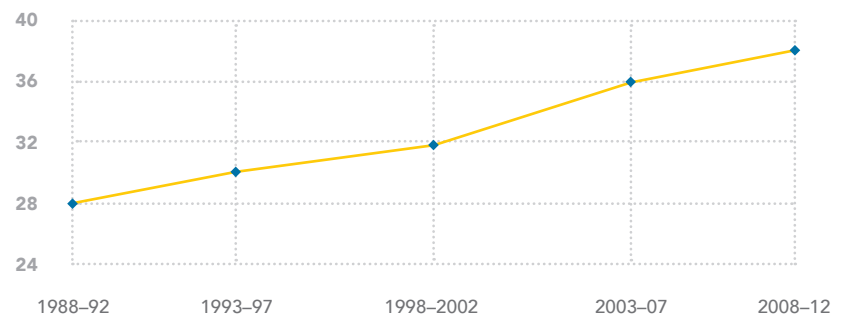
**We will increase adherence with prescribed therapies to 80 percent among at least 75 percent of people with cystic fibrosis.**

Many barriers prevent patients from fully benefiting from the critical CF treatments made possible through our research efforts. Acknowledging the financial, practical and psychosocial issues that contribute to the treatment burden, and understanding the daily challenges faced by the growing number of adults with CF, the Foundation will work to discover effective ways to help people with CF successfully manage their prescribed therapies.

**Few organizations can show the kind of progress that the CF Foundation has achieved in improving health and extending lives. The decades of life we have added for those with CF are proof that our model is working and we are on the right path to curing this disease.**

**ROBERT J. BEALL, PH.D.**  
PRESIDENT AND CHIEF EXECUTIVE OFFICER, CF FOUNDATION

**MEDIAN PREDICTED SURVIVAL AGE**  
1988–2012 OVER 5-YEAR BANDS



SOURCE: CYSTIC FIBROSIS FOUNDATION PATIENT REGISTRY, 2012

# Setting Parameters

Once consensus was achieved on the objectives, the committee moved to define parameters to help guide us as an organization. These parameters will ensure that we concentrate our efforts on our mission and objectives and be used as reference checkpoints if necessary as we move forward.

- 1 The CF Foundation will never rest until a cure is found for all people with CF.
- 2 Every decision we make is for the benefit of the person with CF.
- 3 The Foundation will strive to achieve high levels of effectiveness, efficiency, standards of governance and accountability.
- 4 Foundation funds shall not be used to pay directly for patient medical care.

While the first three parameters were readily endorsed by the committee, the fourth parameter regarding the use of Foundation funds to pay for medical care was the subject of much discussion. Upon careful review of available data and related historical case studies, it was evident that no economic model exists that would enable us to pay directly for individual patient care while continuing to pursue our mission for the benefit of all people with the disease.

These parameters will serve as benchmarks in measuring all we do. While fundraising “efficiency” has long been the benchmark for assessing a successful charity in the United States, today leading charity watchdog groups are encouraging donors to focus instead on “effectiveness” or results. The Foundation’s effectiveness in accomplishing our mission and achieving real results is second to none, and we continue to be measured by the most important yardstick: more and better tomorrows for people with the disease.

# Identifying Strategies for the Future

Once vital groundwork in the process had been laid, the planning committee proceeded to identify and refine five key strategies — an articulation of the bold initiatives through which the organization will deploy its resources to fulfill the Foundation’s mission and objectives.



- 1** We will support and grow our pipeline of innovative therapies that modulate CFTR and treat various manifestations of CF.
- 2** We will develop a multipronged approach to increase adherence that will include enhanced data collection and information sharing.
- 3** We will develop and implement plans so that all people with CF have access to high-quality, specialized and accredited CF care centers and prescribed therapies.
- 4** We will develop and implement a comprehensive communications plan to:
  - MANAGE** expectations among our constituencies.
  - ADDRESS** “mission accomplished.”
  - ADDRESS** real or perceived conflicts related to royalty sales.
  - ENGAGE** the entire CF community.
- 5** We will increase fundraising to achieve our mission and objectives.



## ACTION TEAMS MAP THE WAY

With these five strategies, the planning committee set the stage for the next important phase of the process — moving ideas into action.

Five action teams were created, each charged with addressing a strategy. Their assignment was to devise a plan to make each of these strategic visions a reality and develop possible tactics, budgets and timelines for getting there.

Each action team was composed of volunteers, a person with CF, CF family members, care center professionals, Foundation staff, researchers and other relevant experts. With representatives from all areas of the CF community, the participants brought a diversity of insights and expertise to the teams, challenging each other to look from new perspectives and share experiences through robust and productive discussion. Throughout the process, each of the action teams also sought input from their respective communities and networks to offer an even broader perspective.

From January through June 2013, the action teams worked tirelessly to deliver an operational plan for each strategy — some 125 individuals in all, recording hundreds of hours of face-to-face meetings and hundreds of additional hours in conference calls.

## 6 A SIXTH STRATEGY IS FORMALLY ADOPTED

Along the way, the teams identified the clear need for a sixth strategy focusing on the adult CF community. This need had been identified at the initial planning committee meeting, and became increasingly apparent as the action teams began their work. We recognized that we would be remiss if we failed to add a task force exclusively comprised of adults with CF, whose voices we all agreed must be heard and whose perspectives and experiences were viewed as essential. With close to half of all people with CF now age 18 or older, it was clear we must be more attuned to the priorities and concerns of this growing segment of the CF population.

A 14-member CF Adult Task Force was established to identify issues of importance and to help create recommendations for the Foundation. We heard that adults want to be included in virtually all facets of the Foundation as it exists today, from clinical trials to fundraising, yet they also want to explore new opportunities to contribute their knowledge and experience. We must be unwavering in our commitment to help each person with CF lead a full and productive life, while always taking steps to ensure that both our policies and our actions serve the best interest of all people with CF.

Overall, through a rigorous consensus-building process, each of the action teams generated recommendations that were then translated into detailed action plans that were operational, measurable and assignable. In addition, a high-level cost-benefit analysis was built to accompany each action plan to help the planning committee determine if it was a sound investment.

# From Consensus to Action

With their work complete, the action teams were ready to make their case.

After six months of planning and discussion, the committee reconvened on June 5–6, 2013, in Bethesda, Maryland, to review and act on the 81 action plans, containing more than 550 individual steps presented by the action teams.

Before taking any action, the committee first reviewed and made minor adjustments to its own work from the launch of the Strategic Planning Initiative (Mission Statement, Objectives, Parameters and Strategies) and evaluated and fine-tuned results statements and action plans to ensure the six strategies could be most effectively operationalized.

With its charge now met, the planning committee formally handed the official Strategic Plan document to the President and CEO. From June through November 2013, the executive management team prepared the plan for presentation to the Board of Trustees for consideration and possible approval.

In doing so, the management team was asked to closely research and evaluate the details associated with implementing the plan, identifying the scope of responsibility assigned with each strategy. After a full evaluation of every aspect of execution, from budget and personnel considerations to questions of technology infrastructure and capacity, the executive team prioritized the goals, keeping in mind those most essential in the initial phase.

In preparing its findings and recommendations for the Board of Trustees, the executive management team observed three guiding principles:

- 1 It would not in any way alter the consensus conclusions of the Strategic Planning Committee.
- 2 It would build a graduated schedule for implementing the plan by reconciling ambition with reality, while also preserving important priorities already in motion.
- 3 It would always attempt to manage expectations, not only with regard to medical programs, but also with regard to fundraising — our network of tireless volunteers being the most important asset we possess.

## MOVING FORWARD

At its November 12, 2013, meeting, one year after the initial planning committee first met, the CF Foundation Board of Trustees unanimously adopted the revised mission statement and accompanying objectives and strategies.

To effectively and fully implement the more than 80 action plans and hundreds of different steps, we are initiating a phase-driven process that will be adjusted annually in alignment with the annual budgeting process.

By working in stages, we can prioritize more effectively and continually refine areas that have significant crossover impact within the Foundation—from technology and communication to finance and operations. As always, when we advance new work, we want to protect those areas that function well and are necessary to continue operations on a daily basis while keeping an open ear to the changes happening around us.

We believe our ability to be flexible and to evolve in our thinking as we face the challenges ahead will mean greater success in the future.


This report offers a detailed overview of the priorities we will address in the initial phase of our strategic work, as well as those required to launch the remaining action plans over the next five years. A list of the complete action plans for each strategy is included in the appendix.

As the Strategic Plan unfolds, we are committed to providing updates to all of our stakeholders — through multiple outlets and modes of delivery — as suggested in our new communications strategy.

We hope that this report provides helpful insight into the six strategies and conveys the Foundation's commitment to achieving success in all we do, both now and in the future.



# OUR SIX STRATEGIES

- 
- 1 Pipeline and Promise**
  - 2 Adherence**
  - 3 Access to Treatment and Quality Care**
  - 4 Communications**
  - 5 Fundraising and Outreach**
  - 6 Engage and Listen to CF Adults**

STRATEGY 1

# Pipeline and Promise



**We will support and  
grow our pipeline of  
innovative therapies  
that modulate CFTR  
and treat various  
manifestations of CF.**

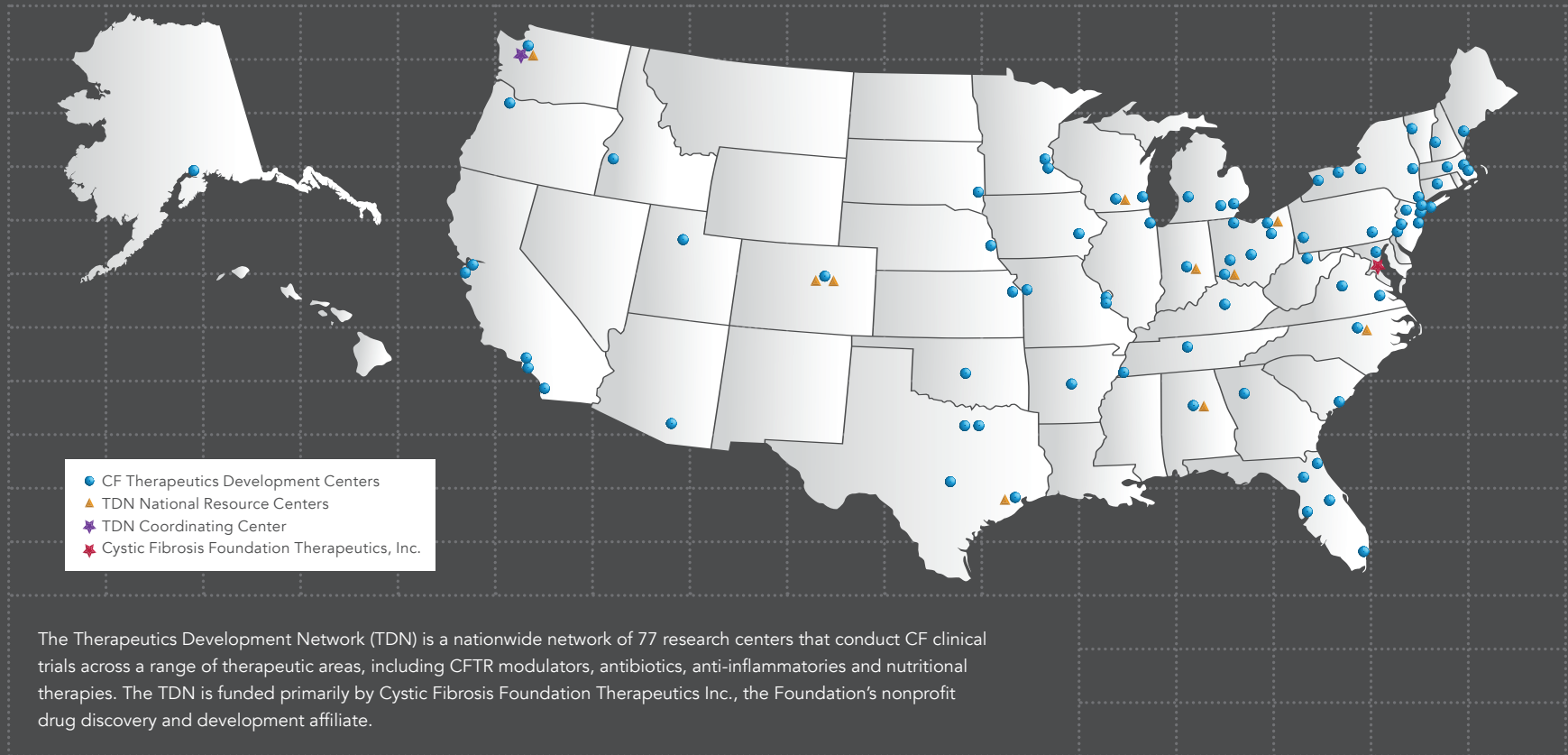
# Pipeline and Promise

In 1989, a team of scientists supported by the CF 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. Since the discovery of the CF gene, the Foundation has laid the groundwork for and helped fund a number of breakthrough new therapies, including ivacaftor (Kalydeco™), the first drug targeting the underlying cause of the disease.


The newly enhanced CF Foundation mission statement promises that we will work to provide all people with the disease the opportunity to lead full, productive lives by funding research and drug development. To address this charge, the Pipeline/Research Action Team set out to focus on seeking the discovery and development of new, more effective and efficient therapies that address all stages of the disease for all people with CF.



# THERAPEUTICS DEVELOPMENT NETWORK







**The remarkable success of the CF Foundation’s medical and scientific programs has been achieved by establishing bold strategic objectives that are accomplished through a collaborative team approach. This strategic plan takes that same focused approach to a new level and applies it to new challenges — assuring us that we should expect great success as a result.**

**PRESTON W. CAMPBELL, III, M.D.**

EXECUTIVE VICE PRESIDENT FOR MEDICAL AFFAIRS, CF FOUNDATION

The development of new symptomatic CF therapies and their integration into clinical care has already brought decades of life to individuals with CF. We will continue to foster good ideas and support expert practitioners by funding basic and clinical research, training programs, and the development of effective symptomatic therapies through our existing Therapeutics Development Program.

The Pipeline/Research Action Team examined the impact of mutations in the CFTR (cystic fibrosis transmembrane regulator) gene on the CFTR protein, how existing pharmaceuticals affect various mutated forms of CFTR protein, and how state-of-the-art approaches may be applied to CFTR mutations that are non-responsive to existing pharmaceuticals. The team then crafted specific results that would identify CFTR mutations that are responsive to existing pharmaceuticals and, for those that were non-responsive, what steps were needed to set in motion appropriately focused drug discovery projects.

The following steps are the initial priority actions we will take toward achieving this strategy. A full overview of the Pipeline Five-Year Action Plan can be found in the appendix of this report.

## **1** We will pursue new and expanded CF projects with leading pharmaceutical companies to discover and develop more potential therapies targeting the root cause of CF for all mutations of the disease.

The action team proposed steps to test approved CFTR modulators (e.g., ivacaftor and possibly correctors like lumacaftor) against rare CFTR mutations. This would be accomplished by collecting epithelial samples from people with rare CF mutations or creating cell lines where patient-derived materials are not available, expanding the size of the samples, testing their response against available CFTR modulators, and understanding why some mutations are non-responsive. Outcomes from these studies would potentially be applicable in helping physicians in the future decide whether an individual should be examined for his or her response to a CFTR modulator based upon genotype. In addition, these studies may help drug discovery companies identify cohorts of patients with rare CFTR mutations to examine in clinical trials.

A significant number of people with CF have a combination of mutations that will not respond to ivacaftor or corrector agents; however, one of their mutations may respond to drugs that overcome premature termination codons (so-called nonsense mutations). For these patients, a number of pharmaceutical companies — some with funding from Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) — are working to discover and develop read-through promoter agents that reduce the impact of the nonsense mutation, resulting in functional CFTR.

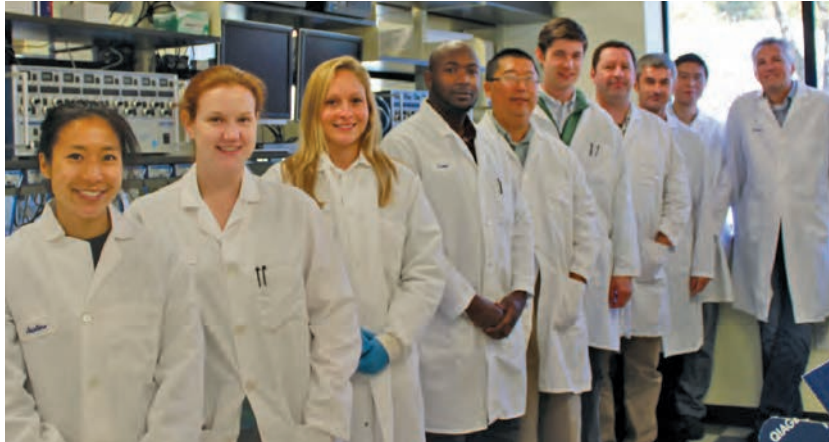
## **2** We will continue to support vital initiatives to identify potential new treatments and strategies that help people with CF better manage the symptoms of their disease and improve their health.

Symptomatic therapies that address airway infection and inflammation and nutrition in conjunction with organized care have brought many more years of life to CF patients, even before the advent of CFTR-directed therapies. We will continue to seek out and assist in the development of such therapies, as they remain an important and vital weapon in clinicians' armamentariums.

## **3** We will actively seek, identify and fund innovative drug development opportunities that aim to replace or repair the defective cystic fibrosis gene.

We know that improving CFTR function yields extraordinary clinical benefits for CF patients, as exemplified by the impact of ivacaftor on a subset of CF patients with the G551D mutation. There are other drugs in the CF pipeline directed toward the most common CFTR mutation, F508del. If current drug discovery/development efforts are successful before 2020, approximately 95 percent of CF patients in the U.S. will have a drug cocktail available that is appropriate for their CFTR genotype.

The number of people with CF whose mutation combinations will not be responsive to a CFTR potentiator, corrector, read-through promoter or combination thereof is extremely small and the number of reasons their CFTR protein is dysfunctional quite large. Therefore, the action team mapped out endeavors to replace these patients' mutated CFTR by seeking to overcome the hurdles to CFTR gene, oligonucleotide and protein delivery.



These therapeutic avenues have been explored in the past but so far have not met with demonstrable success. The team believes it is time to revisit this field, better understand the advances that have been made, and apply them to CF. A byproduct of this work to understand the impact of mutations on CFTR includes a better understanding how cells regulate the amount of CFTR that is made. The outcomes of this work are not only potentially useful for CF patients with mutations in regulatory elements of the CFTR gene but also for other CFTR-focused drug development efforts.

A number of action plans take advantage of tools commonly used today in molecular biology laboratories to create models of genetic diseases by inserting mutations into endogenous DNA. The Pipeline/Research Action Team felt it was important and timely to understand the potential application of this methodology in a directed therapeutic fashion, whereby correcting mutations instead of inserting them could be explored. Were success to be found in these explorations, all individuals with CF could potentially benefit.

Decades of outstanding work in basic science and clinical research by experts around the globe have opened the door for new advancements in CF medicine. We are well-positioned to accelerate our work to target cystic fibrosis from every angle and reach our ultimate goal: to find effective treatments for all people with CF, and every mutation that causes this disease.

**PHIL THOMAS, PH.D.**

PROFESSOR, DEPARTMENT OF PHYSIOLOGY,  
UNIVERSITY OF TEXAS SOUTHWESTERN MEDICAL CENTER

The future may hold even more promising opportunities. CF will be a model of personalized medicine as a patient's genetic makeup, or even perhaps the response of a patient's cells to a particular drug or drug combinations in the laboratory, informs that individual's care. We will pursue ways to eradicate or prevent airway infection, normalize nutrition, reduce inflammation and, ultimately, devise one-time therapies that will correct the basic defect in CF. The action team's strategy is to create a path to success for the entire CF population.

## PIPELINE/RESEARCH ACTION TEAM

### CHRIS PENLAND, PH.D.

Vice President of Research, CF Foundation (Team Leader)

---

### PRESTON W. CAMPBELL, III, M.D.

Executive Vice President for Medical Affairs, CF Foundation

---

### GARRY R. CUTTING, M.D.

Professor of Pediatrics and Medicine,  
McKusick-Nathans Institute of Genetic Medicine;  
Johns Hopkins University School of Medicine

---

### PHIL THOMAS, PH.D.

Professor, Department of Physiology,  
University of Texas Southwestern Medical Center

---

### TED TORPHY, PH.D.

Head of Research Strategy, CF Foundation

---

STRATEGY 2

# Adherence



**We will develop  
a multipronged  
approach to increase  
adherence that will  
include enhanced  
data collection and  
information sharing.**

# Adherence

Among the many difficulties of living with cystic fibrosis is the heavy treatment burden that individuals must manage every day. Daily adherence to prescribed treatments is a significant challenge for the CF community, particularly when people with CF face major life changes.

If effective therapies exist, but patients are not taking them, then we must ask if we have done all we can. In reality, medication adherence has been poorly understood and is very difficult to measure. We have begun to develop ways of measuring adherence and correlating it to clinical benefit to help us understand which interventions or other factors are most helpful.

The challenge set by the Strategic Planning Committee was aggressive:

“We will increase adherence with prescribed therapies to 80 percent among at least 75 percent of people with cystic fibrosis.”



The Adherence Action Team did not hesitate to accept this challenge. It delved into the complexities of adherence and articulated three long-range goals:

- Adherence programs will be integrated and sustained with the Foundation, CF care centers and the CF community.
- Effective and efficient adherence programs tailored to individual patients and families will be disseminated and implemented.
- A technology platform will be created to support and facilitate multilevel, multichannel adherence programs and information-sharing among stakeholders.

The team had concerns about the difficulties of translating adherence research into practice and of sustaining these efforts over the long term. To meet these challenges, team members emphasized the importance of community engagement, pragmatic intervention programs, and practical measures for monitoring progress.

The following initial, priority actions are intended to lay the groundwork for meeting the Foundation's five-year objective of achieving 80 percent adherence to prescribed treatments for 75 percent of people with CF. An overview of the full Adherence Five-Year Action Plan can be viewed in the appendix of this report.

## **1 We will establish a multidisciplinary stakeholder advisory committee to provide input and guidance for the adherence initiative.**

Patients, families, health care providers and drug manufacturers all have a stake in improving adherence among people with CF. Their involvement is imperative to better understand the perspective of each group and the efforts that have already been undertaken to address the problem. Building upon a similar Foundation initiative begun in late 2012, a stakeholder advisory committee consisting of representatives from these groups was established in November 2013.

## **2 We will collect prescription refill data on all people with CF to establish an objective measure for monitoring adherence.**

For a long time, clinicians have been challenged to determine the extent to which an individual is adherent to prescribed therapies. Currently, there is no suitable set of objective adherence measures to determine a person's level of adherence or the effectiveness of future adherence interventions. Prescription refill data is an objective measure that is readily available and can be used as a starting point. A recently completed pilot study demonstrated the feasibility of collecting this data on individuals with CF. The Foundation is currently initiating a beta test with approximately 10 care centers to begin gathering data on patients with CF, and will also create a dashboard that would allow visualization of this data in an accessible format for use in the clinical management of patients.



### **3** We will develop and implement a validated adherence-barriers assessment that can be deployed by all CF care centers.

An adherence-barriers assessment tool will be developed, validated and implemented as part of routine CF care. This tool will be used to identify each patient's barriers to staying on his or her prescribed treatment regimen, and will guide the selection of interventions that are most likely to improve adherence to treatment.

### **4** We will design and implement a series of pilot adherence interventions at a network of CF care centers.

Pragmatic and scalable adherence interventions that address the various barriers to adherence must be designed, tested and evaluated. Interventions such as health coaching, mobile applications and patient portals or personal health records (PHR) have been identified as potential means to educate and motivate patients to become actively engaged in their health care. We will seek stakeholders' input to help us decide whether these and other interventions are worth pursuing.

**CF presents many challenges for patients and families. We are dedicated to creating tools to empower people with CF and their families to successfully take control of their health.**

**DRUCY S. BOROWITZ, M.D.**

DIRECTOR, CF CENTER, WOMEN AND CHILDREN'S HOSPITAL OF BUFFALO;  
PROFESSOR OF PEDIATRICS, SUNY AT BUFFALO SCHOOL OF MEDICINE  
AND BIOMEDICAL SERVICES

### **5** We will plan and implement a multichannel communication campaign to raise awareness and inform people with CF and their families and care providers about the importance of adherence.

The literature clearly shows the importance of CF treatment adherence to the health outcomes of an individual with CF. However, adherence is often not discussed at all or is inadequately addressed by providers and patients. A national campaign is planned to raise awareness of the importance of adherence among patients and health care providers, and to help stimulate discussions about adherence at care center visits and in the home. The "I Am the Key" clinical trials campaign, which helped mobilize participation in CF clinical trials and speed the development of new therapies, could serve as a model for this campaign.

## ADHERENCE ACTION TEAM

### CINDY GEORGE, M.S.N., R.N., N.P.

Senior Director of Patient Engagement, CF Foundation  
(Team Leader)

---

### DRUCY S. BOROWITZ, M.D.

Director, CF Center, Women and Children's Hospital  
of Buffalo; Professor of Pediatrics, SUNY at Buffalo  
School of Medicine and Biomedical Services

---

### WENDY BULLINGTON, PHARM.D.

Clinical Associate Professor, Medical University  
of South Carolina CF Center

---

### BRIAN CALLANAN

Adult with CF, Vermont  
Executive Director, Cystic Fibrosis Lifestyle Foundation

---

### CAROL CHACE, M.S.W., L.I.C.S.W.

Social Worker, Children's National Medical Center  
CF Center

---

### MICHELLE EAKIN, PH.D.

Assistant Professor, Pulmonary and Critical Care Medicine,  
Johns Hopkins Adherence Research Center

---

### ALEXANDER ELBERT, PH.D., P.M.P.

Director of Patient Registry Program,  
CF Foundation Therapeutics

---

### ALIZA FINK, D.SC.

Director of Epidemiology, CF Foundation Therapeutics

---

### DEE ANNE EVANS, M.S.N.

Genentech

---

### DANIEL KLEIN, M.H.S.

Senior Vice President of Patient Access Programs,  
CF Foundation

---

### DAVID MCLOUGHLIN, M.B.A.

Senior Vice President of Operations, CF Foundation

---

### ALEXANDRA QUITTNER, PH.D.

Professor of Psychology and Pediatrics; Director,  
Child Division; Provost Research Scholar  
and Cooper Fellow, University of Miami

---

### KRISTIN RIEKERT, PH.D.

Associate Professor, Division of Pulmonary and Critical  
Care Medicine, Johns Hopkins School of Medicine;  
Co-Director, Johns Hopkins Adherence Research Center

---

### GREGORY SAWICKI, M.D., M.P.H.

Associate Director, Boston Children's Hospital CF Center;  
Assistant Professor of Pediatrics, Harvard Medical School

---

### REBECCA SCHROEDER

CF parent, Idaho

---

### JOAN ZUKOSKY, R.N., B.S.N.

Nurse Coordinator, Adult Cystic Fibrosis Program,  
Barnes-Jewish Hospital, Washington University  
School of Medicine

---



STRATEGY 3

# Access to Treatment and Quality Care

**We will develop  
and implement plans  
so that all people  
with cystic fibrosis  
have **access** to high-  
quality, specialized  
and accredited CF  
care centers and  
prescribed therapies.**

# Access to Treatment and Quality Care

A key objective identified by the Strategic Planning Committee — that all people with CF will have access to the highest quality care and state-of-the-art therapies — was the focus of the Access/Quality Care Action Team. While the Foundation has a strong history of advancing clinical care, beginning with the establishment of the Care Center Network, several converging factors necessitate an expansion of the Foundation's efforts to ensure that people with CF have access to quality, specialized care.

First, clinical and therapeutic advances are changing the face of cystic fibrosis. Nearly half of all people with CF in the United States are adults, and we predict that proportion will steadily increase, approaching nearly two-thirds in 20 years. This dramatic demographic shift requires new thinking about the care needs of the adult patient population, as well as the resource needs of their health care providers.





Second, the definition of quality is evolving to encompass patient-reported outcomes (e.g., experience of care and quality of life) and the concept of value (i.e., the efficient delivery of high-quality care). While the Foundation has been successful in driving dramatic improvements in the key medical outcomes, such as FEV<sub>1</sub> and BMI, we must now incorporate emerging quality metrics into the CF care model.

Third, the national debate about controlling health care costs, the rapidly evolving health care delivery system, and the high cost of CF care, particularly therapies addressing the basic defect, require the Foundation to expand its role as a leader in advocacy for the care and therapies needed to treat the disease. The Access/Quality Care Team's action plans addressed these challenges, using a combination of new approaches, and repurposed and/or reinvigorated efforts already established.

The Access/Quality Care Action Team created plans to develop the infrastructure to collect patient-reported outcomes, including patients' experience of care, and to analyze the comprehensive costs of CF care. When coupled with the medical outcomes from the CF Foundation's patient registry data, this will provide a broad and well-balanced assessment of the quality of CF care. The team proposed taking initial steps to develop a comprehensive plan to define and measure value-based CF care and formulate strategies to adapt the CF care delivery model to this emerging and rapidly evolving approach to health care delivery and payment.

Collectively, these efforts will best position the Foundation to define value-based health care for CF by understanding what therapies drive health improvements for people with CF, and how to better advocate for access to the highest quality, specialized treatment and care. The following steps are the initial priority actions we will take toward achieving this strategy. A full overview of the Access/Quality Care Five-Year Action Plan can be found in the appendix of this report.

# 1 We will provide additional support to Foundation-accredited care centers — enabling their teams to spend more time providing quality health care to people with CF and their families.

The Access/Quality Care Action Team acknowledged the heavy administrative burden that the current health insurance system places on people with CF, their families and health care providers. By expanding the Foundation’s capacity to be a reliable and up-to-date source of support, information and expertise related to care and coverage, these resources will enable care center teams to spend more time doing what they are trained to do — provide quality health care to patients and families. It also will position the Foundation to better address the needs of those with CF and their families by centralizing information about clinical care and coverage assistance.

Recognizing the critical role that people with CF and their families play in maintaining health, the action team proposed a demonstration project to assess the impact of providing patients with (1) tools to report their symptoms and quality of life in response to medical treatments, and (2) opportunities for social networking with other patients and care center staff on health outcomes, quality of life and the experience of care.



**2** We will strengthen the nationwide network of CF clinical care by ensuring that all Foundation-accredited care centers adhere to the same standard of care and by providing a framework for close collaboration with centers that are struggling to meet these standards.

To strengthen the nationwide network of care for all people with CF, the action team engaged in a comprehensive evaluation of the CF Foundation's CF Care Center Accreditation process. It focused on refining the process so that all care center programs, including affiliates, are held accountable to the same standards. The team also recommended the creation of a "rescue" program targeting care centers struggling to meet standards of care. The goal of this plan is to provide a framework for close collaboration with a struggling care center to develop concrete plans to correct deficiencies on a timeline and with accountability if milestones are not met.

The team also emphasized that work to develop and strengthen the Foundation's evidence-based clinical and quality improvement efforts across the CF care delivery system by engaging people with CF, their families and care providers must be accompanied by robust efforts to ensure that this high-quality care is covered by the health care system.

High-quality care in cystic fibrosis has always depended on teamwork. Working together, we are entering a new era of specialized CF care and discovering new ways to support the health and well-being of all people with CF. It is an amazingly exciting time.

**KATHRYN A. SABADOSA, M.P.H.**

CF PARENT, VERMONT; RESEARCH ASSOCIATE,  
THE DARTMOUTH INSTITUTE FOR HEALTH POLICY  
AND CLINICAL PRACTICE



### 3 We will expand our efforts to continue advocating on behalf of those with CF and their families to ensure that high-quality, specialized care is covered by the health care system.

The action team identified the need to expand the Foundation's public policy efforts to more rapidly identify and respond to health coverage policy and regulatory proposals obstructing access to quality CF care. This includes the development of a payer-relationship program to facilitate more open communication between the Foundation and third-party payers and an improved understanding of the processes and standards that payers utilize in making coverage and payment decisions. This effort will enhance the Foundation's ability to anticipate possible negative coverage determinations and its ability to influence or change those decisions.

Action plans focus on strengthening the Foundation's capacity to conduct policy and regulatory analysis at the state level. The team also recognized the need to increase the CF community's involvement in state advocacy efforts, where many health policies impacting CF care are implemented, and where the Foundation has had limited involvement and influence previously.

Because of the Foundation's many investments in care and research we are facing an exciting new chapter in the fight against cystic fibrosis. The action team's plans will help position the Foundation to tackle the next challenges in developing a clinical care delivery system that provides the highest-quality, specialized care and moves toward better outcomes for all people with cystic fibrosis.



As a result of the work of both the Adherence and Access/Quality Action Teams, the Foundation established a new Patient and Community Affairs Department in November 2013, incorporating public policy, current access initiatives and patient engagement activities, including a permanent CF adult advisory committee.

## ACCESS/QUALITY CARE ACTION TEAM

### **BRUCE C. MARSHALL, M.D.**

Senior Vice President of Clinical Affairs, CF Foundation Therapeutics (Team Leader)

### **MARY DWIGHT**

Senior Vice President of Public Policy and Community Affairs, CF Foundation

### **ALEXANDER ELBERT, PH.D., P.M.P.**

Director of Patient Registry Program, CF Foundation Therapeutics

### **ALIZA FINK, D.SC.**

Director of Epidemiology, CF Foundation Therapeutics

### **TRINA FONTAINE**

Senior Project Manager, Greater New York Chapter, CF Foundation

### **ELIZABETH GOSS, J.D.**

Policy Expert, Turner & Goss, LLP

### **LESLIE HAZLE, M.S., R.N., C.P.N.**

Director of Patient Resources, CF Foundation

### **KAREN HOMA, PH.D.**

Improvement Specialist, Leadership Preventive Medicine Residency Program, Dartmouth Hitchcock Medical Center

### **KAREN MAGUINESS, R.D.**

Dietitian, Riley Cystic Fibrosis Center at Indiana University Health

### **SUSANNA MCCOLLEY, M.D.**

Head, Pulmonary Medicine; Associate Chair for Clinical Affairs; Co-Director, CF Center, Ann and Robert H. Lurie Children's Hospital; Professor of Pediatrics, Northwestern University Feinberg School of Medicine

### **CATHERINE C. MCLOUD**

CF parent, Florida; Chair, Board of Trustees, CF Foundation

### **PETER J. MOGAYZEL, JR., M.D., PH.D., M.B.A.**

Director, CF Center; Medical Director, Pediatric Specialty Clinic; Professor of Pediatrics, Pulmonary Medicine, The Johns Hopkins Hospital

### **AMY MUELLER, L.C.S.W.**

Adult CF Program Social Worker, Hartford Hospital

### **KIM NUNNARI, B.S.N.**

Adult with CF, Massachusetts

### **GERRY PANDZIK, R.N.**

Director, Chronic Care, Physician-Hospital Organization, Cincinnati Children's Hospital Medical Center

### **KRISTOFER PETREN**

Patient Registry Analyst, CF Foundation

### **REBECCA PETTIT, PHARM.D., M.B.A., B.C.P.S.**

Pharmacist, Riley CF Center at Indiana University Health

### **BOB REID**

CF parent, New York; Pro-bono consultant, Reid, McNally & Savage

### **KATHRYN A. SABADOSA, M.P.H.**

CF parent, Vermont; Research Associate, The Dartmouth Institute for Health Policy and Clinical Practice; Quality Improvement Program Manager, CF Foundation

### **JOE SCHACHLE**

Chief Operating Officer, Parion Sciences

### **CHRISTINE SINGH, C.N.P.**

Certified Nurse Practitioner, CF Center, Akron Children's Hospital

### **STEVEN STITES, M.D.**

Vice Chancellor and Senior Vice President for Clinical Affairs; Professor of Medicine, Director, Adult CF Program, University of Kansas Medical Center

### **BETH SUFIAN, J.D.**

Adult with CF, Texas; Director of CF Legal Information Hotline

### **MARIA THOMAS-WENZEL, M.H.A.**

Director of Patient Access Programs, CF Foundation

### **ELIZABETH TULLIS, M.D., F.R.C.P.C.**

Division Head, Respirology; Professor of Medicine, University of Toronto; Director, Adult Cystic Fibrosis Programme, St. Michael's Hospital

### **PHILLIP WEICHEL**

Director of Field Management, CF Foundation

### **LAUREN WILLIS, R.R.T.-N.P.S.**

CF Center Program Coordinator, Arkansas Children's Hospital



STRATEGY 4

# Communications

**We will develop  
and implement  
a comprehensive  
communications plan  
to better address the  
needs and concerns  
of the CF community.**

# Communications

The Communications Action Team was charged with managing expectations among our diverse constituencies and addressing the misperception that our mission has been accomplished, as well as any issues related to royalties received from our investments. The action team worked to identify ways to engage the entire CF community.

The Communications Action Team identified three fundamental shifts affecting the Foundation's communications strategy:

- CF has changed from a largely pediatric disease to one that also affects an increasing number of adults.
- With the breakthrough approval of ivacaftor, we have entered a new era in drug development, which has brought tremendous hope to our community while also raising complex issues related to costs, royalties and conflict of interest, and increasing the need for medical information and patient assistance resources.
- The advent of social media and other emerging technologies has transformed the way the CF community creates, consumes and shares information, presenting new opportunities for the Foundation to engage people with CF and the general public.



**Digital media is changing the way we communicate. We have more tools at our disposal than ever before to listen to and connect with the people at the heart of the Foundation’s mission. Our challenge is to harness new technology to inspire and activate the entire CF community.**

**MICHAEL L. BEATTY, ESQ.**

CF PARENT, COLORADO; CF FOUNDATION TRUSTEE

Through an extensive, collaborative process, the Communications Action Team concluded that the next step is to conduct both quantitative and qualitative research to assess the needs and concerns of the CF community. To ensure that all recommended actions are the result of a robust listening process, the team incorporated an initial survey/assessment as a first step into the majority of its action plans. In addition, the action team believed strongly that one critical business question should be asked when assessing any proposed action step: Will taking this step make a meaningful improvement in the lives of people with cystic fibrosis or their families?

The Foundation will expand our strategic communications efforts to address these fundamental shifts and our enhanced mission, taking the initial, priority action steps detailed below. A full overview of the Communications Five-Year Action Plan can be found in the appendix of this report.

**1 We will coordinate all Foundation communications efforts, including fundraising and medical communications, so that we are effectively communicating with one voice to respond to evolving issues and the emerging needs of the entire CF community, and to maximize the power and consistency of the Foundation’s brand and messaging across all channels.**

As the Foundation has shifted its mission to more fully support the ability of those with CF to lead full, productive lives, we must expand our medical education and information and strengthen our way of working collaboratively — across disciplines and departments and with greater input from the community we serve.

The action team agreed to conduct an initial survey of needs and concerns; create a Medical Communications Team that will liaise with medical staff to develop meaningful content to share with people with CF and families; and create more “points of engagement” or new opportunities to better interact with, listen to and engage diverse segments of the CF community in order to more effectively communicate relevant and consistent information and messaging.



**2** We will strengthen and enhance the Foundation’s current online presence and invest in new channels to reflect the changing media consumption habits of the Foundation’s existing and future audiences, more fully engage the entire CF community with an emphasis on people with CF, and address the new challenges presented by the infection prevention and control guidelines.

We believe that the Foundation must build the infrastructure, staff, and education and information material to successfully engage the entire CF community. The CF community needs more and better information on, among other things, care access, research and patient assistance. We have begun a redesign of our website and our mobile access. We are also expanding our video capabilities to create content that will engage the CF community, especially in light of new infection prevention and control recommendations that call for new ways to welcome participation of those with CF at Foundation events.

**3** We will manage expectations and address “mission accomplished” by accurately communicating the risks and uncertainties of the drug development process in a way that strengthens the CF community’s dedication to fundraising, advocacy, clinical trial participation and volunteerism, conveying the Foundation’s commitment to cure cystic fibrosis and to provide all people with the disease the opportunity to lead full, productive lives.

The Foundation must position itself unequivocally as a proactive advocate for people with CF and their families and as a leader in venture philanthropy, and must be prepared to address controversial issues related to its business model in the media and elsewhere. As in the past, the Foundation must “lean in” and lead in the CF community and beyond. The Foundation must position itself as a thought leader among other stakeholders, prompting public dialogue on issues of pressing concern for our constituents and the wider health care and policy communities.

Successfully addressing rising expectations, “mission accomplished” and issues related to conflict of interest will only be achieved through proactive education. The action team agreed that we must develop the message and the messengers that can persuasively explain the complexities of the drug discovery, development and approval processes. The team also believes addressing these issues effectively requires ambassadors from the CF community to convey the gains made and the distance yet to go.

## COMMUNICATIONS ACTION TEAM

### MICHAEL L. BEATTY, ESQ.

CF parent, Colorado; CF Foundation Trustee;  
Partner, Beatty & Wozniak, P.C. (Team Leader)

---

### CINDY CAPEN, M.S.N, R.N.

CF Center Coordinator; Assistant Director,  
Pediatric Pulmonary Division, University of Florida

---

### AMY DEMARIA

Senior Vice President of Communications, CF Foundation

---

### CHRISTIE DERBIN

Executive Director, Gateway Chapter – St. Louis, Missouri,  
CF Foundation

---

### ABIGAIL ESHBAUGH

Director of Web and Social Media, CF Foundation

---

### LAURIE FINK

National Director of Media Relations, CF Foundation

---

### JOEL FINKELSTEIN

CF parent, Washington, D.C.; Director of Strategic  
Communications, Climate Advisors

---

### ELLEN HARRIS

Director of Field Management, CF Foundation

---

### JUDY KIRPICH

Chief Executive Officer, Grafik

---

### EMILY KRAMER-GOLINKOFF, M.B.E.

Adult with CF, Pennsylvania; Project Manager, Social  
Media and Health Innovation Lab at the Center for  
Health Care Innovation, Penn Medicine

---

### EMILY MCCORMACK

Web/Social Media Specialist, CF Foundation

---

### MARYBETH MCMAHON, PH.D.

Vice President of Communications, CF Foundation

---

### JERRY MICHAUD

Senior Director of Special Projects, CF Foundation

---

### AMY PATTERSON SLENK

Associate Executive Director, Greater Illinois Chapter,  
CF Foundation

---

### JENNIFER TAYLOR-COUSAR, M.D.

Assistant Professor, Associate Adult Program Director,  
National Jewish Health

---



STRATEGY 5

# Fundraising and Outreach



**We will increase  
fundraising to  
achieve our mission  
and objectives.**

# Fundraising and Outreach


The Revenue Action Team began by analyzing all aspects of the Foundation's traditional revenue streams of public support as they existed at that time. (Team members were not asked to address any revenue generated through royalties.) The team also evaluated other potential sources of revenue that have not yet been explored. Substantial time was dedicated to addressing concerns of donor and volunteer fatigue. The team took stock of our organization's incredibly valuable and time-tested chapter/field structure — one of the nonprofit sector's best. They also debated systemic issues they felt must be addressed in order for the plan to generate more revenue on a long-term basis.



Supported by a dedicated community of donors, volunteers and friends, the Foundation has a long and successful history of raising the funds needed to fuel our mission. Through the years, we have raised and invested many hundreds of millions of dollars to help develop CF drugs and therapies, and we take great pride in being one of the most efficient and effective organizations of our kind. As the Foundation expands its efforts to provide more services to people living with CF and accelerate progress toward a cure, our growth in fundraising revenue is key in generating the resources to fund these important initiatives.

The team came to the conclusion that we conduct a number of fundraising programs quite well across numerous platforms, but particularly in the area of special events. The Foundation is a mature fundraising organization with a laser focus on efficiency. Our field structure has served us well, and we continually make efforts to grow revenue by improving communication, sharing best practices and hiring the right professional staff. We have a strong major gifts program, with extraordinary volunteer leadership and a developing annual fund program, all of which helped the team realize that the “individual giving” space holds significant opportunity for growth.

A special task force committee of experts convened on the subject of direct mail to review the current environment and set strategic goals. The Foundation has had a long and successful history with direct mail, but the team recognized that we likely need to reposition our traditional direct mail program, as a result of both current and anticipated trends in this sector of fundraising. Its efficiency has been decreasing, yet it remains a valuable source of opportunity for donor conversion, program service needs and planned giving.



**Unlocking the pathway to a cure requires touching the hearts and minds of communities throughout the United States. Energizing a new generation of passionate, motivated CF Foundation volunteers and fundraisers is crucial to fulfilling our mission.**

**PAM SPITZER**

EXECUTIVE DIRECTOR, MASSACHUSETTS/RHODE ISLAND CHAPTER,  
CF FOUNDATION

As one would expect, there was considerable discussion regarding the pros and cons of changing/modifying our field structure and the effects these changes may have on the Foundation and the CF community. Also, the need was raised throughout discussions to more regularly and effectively include people with CF in our revenue plan.

After multiple meetings and hours of robust debate, the action team found consensus on three fundamental principles that would be necessary to substantially increase fundraising in a changing marketplace. These include:

- Enhancing current fundraising programs to generate increased revenue.
- Venturing into new territory — even territory where we may be uncomfortable, or previously have opted to avoid — to establish new fundraising initiatives and create additional revenue sources.
- Creating the structure and infrastructure needed to successfully achieve the two aforementioned principles.

Initially, to meet the strategic objectives of the Revenue Action Team, the following priority action steps will be taken. A complete overview of the Five-Year Revenue Action Plan can be found in the appendix of this report.



## 1 We will revise the crediting process to support collaboration and prospecting, as well as volunteer and donor development.

A Crediting Task Force has already been developed to create a donor-centered, collaborative culture that facilitates shared successes through national protocol and procedures for identifying, stewarding and growing our donor relationships within a professional donor-centric framework. These recommendations will impact volunteer engagement, staff incentive plans, recognition and training, corporate development, individual giving and care centers — all with a primary focus on achieving fundraising goals and rewarding accomplishment, while supporting the donor's intention.

## 2 We will reposition Great Strides as a broader national fundraising campaign that allows for participation in traditional walk events, as well as non-traditional events.

Great Strides is the Foundation's largest signature national fundraising event. Each year, tens of thousands of co-workers, friends and family come together as one community by walking to support our mission. Revenue from this event is projected to raise \$42 million in 2014. By repositioning Great Strides to be a more inclusive campaign, providing "do-it-yourself" fundraising tools, and greatly enhancing our online, mobile and texting platforms, the Revenue Action Team believes that innovation and customization will decrease donor fatigue and reach out to new participants to improve our fundraising outcomes. These plans allow us to communicate our long-term commitment to the community we serve and at the same time demonstrate our flexibility, without abandoning our core that has served us so well.

### 3 We will build a creative outreach and awareness campaign targeted to young professionals that will engage CF adults and generate fundraising.

Charities are typically successful when a personal connection is made to others who know someone affected by the issue — i.e., “the inner circle.” The incredible success of the CF Foundation has been largely driven by the passion and commitment of our inner circle. Because CF is a rare disease, the pool of potential donors who have a direct personal connection to the disease is relatively small. We need to expand the conversation to a broader population and expand the inner circle of caring.

A new outreach and awareness campaign is being developed in collaboration with a number of young CF adults to bring new friends into the inner circle. It is based on the following concept:

- Every one of us takes about 15 breaths per minute.
- That comes to about 21,000 breaths per day.
- People with cystic fibrosis are just like me — except that each of their 21,000 breaths is a struggle.
- So, what am I doing with my 21,000 breaths?

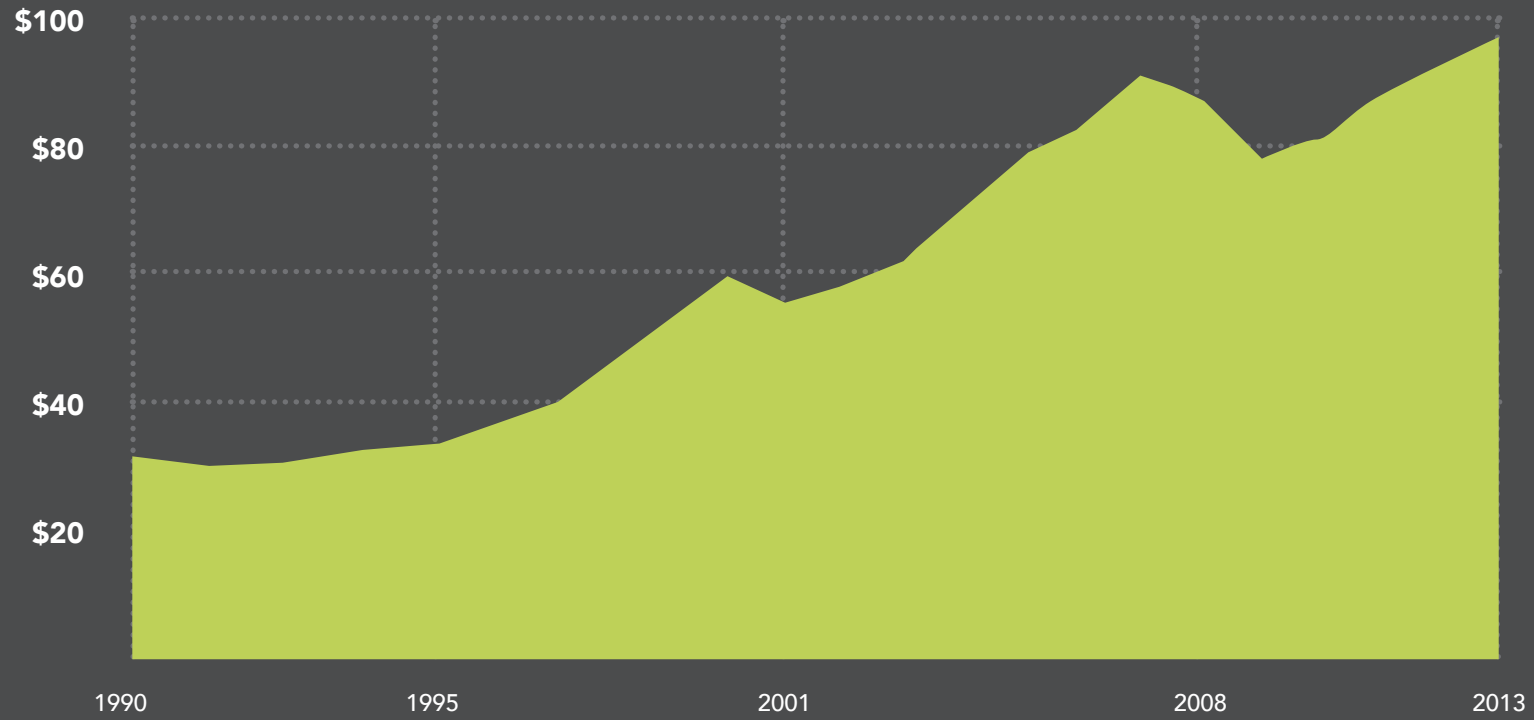
Through the power of social media storytelling and personalization, the program can reach out to individuals, one by one, and introduce them to someone with CF who’s just like them — same age, similar place in life, maybe even similar interests — and make an immediate, personal connection that inspires them to use their 21,000 breaths every day to help find a cure.

### 4 We will integrate donor cultivation and stewardship (Annual Fund, Major Gifts, Corporate, Direct Mail and Planned Giving).

To get from where we are today to where we want to be, we must shift our culture from one that focuses primarily on revenue growth to one that also significantly recognizes teamwork, retention and a deeper engagement of donors and volunteers. Additionally, we must become better at addressing donors in segmented groups often guided by their personal interests. This represents a shift from the single-channel marketing and communications that helped build our organization. Our aim is to build a “Donor-Centric Model” — nurturing long-term relationships, providing information on the difference a donation makes, recognizing and appreciating loyalty and supporting donor cultivation and stewardship.

In summary, the Revenue Action Team’s efforts are aimed at breaking down internal silos that stymie collaboration and creating more mechanisms for evaluating and rewarding staff for retaining and cultivating donors and volunteers. To do this we want to creatively and dynamically meet their needs through expanding online giving platforms, and streamlining our individual giving program (with a new focus on planned giving), as well as maximizing our existing event portfolio. Activities that will ultimately take us to the outer core of larger support include targeting new donors and volunteers and investing in an innovative outreach program.

## NET SPECIAL EVENT INCOME (IN MILLIONS)



## REVENUE ACTION TEAM

### SHANNON GUBBELS

Executive Director, Nebraska Chapter, CF Foundation  
(Team Leader)

---

### KRISTIN DUNN

Adult with CF, New Jersey; Financial Analyst,  
Chubb Group of Insurance Companies

---

### GLEN GOLDMARK

Vice President of Human Resources, CF Foundation

---

### SUE HOOK

Vice Chair, CF Foundation Board of Trustees

---

### STEPHANIE KARLO

Executive Director, Greater New Jersey Chapter,  
CF Foundation

---

### JOHN MCKENNA

CF Parent, New York; Managing Director, Miller Buckfire;  
Board President, Greater New York Chapter,  
CF Foundation

---

### LISA MURPHY

Executive Director, Florida Chapter –  
Central Florida Office, CF Foundation

---

### ELIZABETH NANCE, PH.D.

Hartwell Foundation Postdoctoral Fellow,  
Anesthesia and Critical Care Medicine,

Johns Hopkins Medical Institutions

---

### ANN PALMER

Senior Vice President of Field Management,  
CF Foundation

---

### DEBORAH POWELL

Director of Graduate and Adult Business Programs,  
Mount St. Mary's University

---

### REGINA SCHEWE

Vice President of Major and Planned Giving,  
CF Foundation

---

### PAM SPITZER

Executive Director, Massachusetts/Rhode Island Chapter,  
CF Foundation

---

### ALLISON STEINHAUSER

Director of Major Gifts, Midwest Region, CF Foundation

---

### SUSAN WASSERMAN

CF parent, North Carolina

---

### RICK WILLIS

Senior Director of Corporate Development, CF Foundation

---

### DANA WILSON

Executive Director, Heart of America Chapter –  
Kansas City, Kansas, CF Foundation

---





STRATEGY 6

# Engage and Listen to CF Adults

**We will strengthen  
our relationship with  
the growing CF adult  
population and give  
them a **voice** within  
the Foundation.**

# Engage and Listen to CF Adults

Adults with cystic fibrosis bravely and graciously teach us about what it means to have CF. They encourage us in our efforts to achieve our mission and inspire us every day.

Throughout the course of our strategic planning process, because of the active participation of CF adults, it became clear that we have more to do to fully serve and engage our community of adults with CF. In recognizing this, we have made it a priority to focus on engaging, listening to, and learning from the CF adult community.

During the action team process, the Foundation invited a number of adults with CF to join a special task force to make sure that their voices were being heard during the strategic planning initiative. The task force was charged with identifying and discussing issues of unique importance to members of the CF adult community, including networking, access and compliance programs, community involvement, and support of the overall mission of the Foundation.





Cystic fibrosis is a struggle; a struggle to breathe, to find time to do treatments and to find a balance between life and an illness that won't go away. Yet, as adults with CF, we continue to beat the odds and persevere. We are fighters and we're uniquely positioned to contribute to the search for a cure and help find new ways to support others with CF at every age and stage of the disease.

**PIPER BEATTY**  
ADULT WITH CF

The 14 members of the task force represented a diverse cross-section of adults with CF and fully embraced the work of representing this important constituency. Of even more significance, this is the first time the adult community has been tapped in this way to offer insight on a national level.

The task force began meeting via phone and through online portals in early April 2013, with a series of group conference calls 1–4 hours in length. The task force began by identifying several key issues of concern for adults living with cystic fibrosis, ranging from addressing isolation with mutual support and building an engaged community, to medical adherence and age-appropriate information sharing about new developments in CF research or public policy.

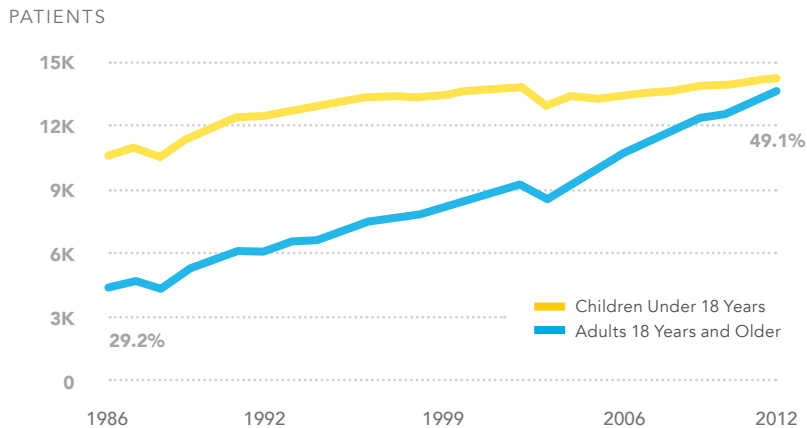
Particular attention was paid to areas — such as communications, outreach and information — where we felt the task force could best support the mission of the Foundation while also making available valuable resources and experiences for people with CF. The task force worked diligently throughout the identification process to remain cognizant of important relationships (such as the doctor/patient priority) and the shared goals of the Foundation and the adult CF community.

Over the course of its meetings, the task force discussed many strategies for strengthening the role of adults with CF within the Foundation. By consensus, they determined that communications both with and from the Foundation, adherence/access issues, and continued safe interaction between members of the CF community were high priorities for many people living with cystic fibrosis. They then sought to identify steps and strategies to help facilitate attention to these issues through a series of recommendations.



One recommendation has been immediately implemented because doing so ensures that this conversation will live beyond the strategic planning process. The establishment of a formal advisory group with a seat on the CF Foundation Board of Trustees will be the beginning of an important new era in which adults living with cystic fibrosis have a significant voice within the Foundation. The complete list of recommendations follows.

### NUMBER OF CHILDREN AND ADULTS WITH CF 1986–2012



SOURCE: CYSTIC FIBROSIS FOUNDATION PATIENT REGISTRY, 2012

- 1 We will enhance the Foundation’s feedback process from people with CF and their families concerning care centers.
- 2 We will update the CF Foundation website to include why a person with CF should use specific therapies.
- 3 We will make available a list of recommended therapists, social workers and other mental health professionals.
- 4 We will establish an Advisory Group with a rotating membership.
- 5 We will establish a formalized mentor program.
- 6 We will create new communication outreach emails, targeting adults with CF and including information on how they can be involved with the Foundation in more ways than just fundraising.
- 7 We will develop innovative means for involving people with CF in fundraising, in light of the updated infection prevention and control guidelines.

## CF ADULT TASK FORCE

PIPER BEATTY, J.D.

Colorado (Team Leader)

---

JENNIFER O'MAHONEY

Pennsylvania (Team Leader)

---

MARISSA BENCHEA

Tennessee

---

LINDA BOWMAN

Florida

---

JOAN FINNEGAN BROOKS

Massachusetts

---

CARLA DEMP

Wisconsin

---

NICOLE DRISCOLL

New Jersey

---

PAUL DRURY

Connecticut

---

KRISTIN DUNN

New Jersey

---

JILLIAN GOODWIN

Virginia

---

ERIN KUHLEERS

Texas

---

JOSH MOGREN

Minnesota

---

BRENT PACE

South Carolina

---

KATHARINE SCHILDT SCRIVENER

Maryland

---

**As I worked side by side over the past year with so many extraordinary people, I was struck by the strength of passion and dedication to this lifesaving mission that we all share. Our vision has never been clearer. Now, we must apply this collective energy to achieving our ultimate goal.**

**LAURA GORDON**  
CF PARENT, MARYLAND



# Appendix

STRATEGIES 1–6

Complete Action Plans

## STRATEGY 1

We will support and grow our pipeline of innovative therapies that modulate CFTR and treat various manifestations of CF.

---

### Evaluate the response of rare mutations to existing CFTR modulators

#### ACTION PLANS

**CONTINUE** to effectively implement existing therapeutic programs that enhance all CF Foundation pipeline targets.

**ASSEMBLE** an expanded collection of CFTR missense mutations expressed in at least two cell types and group mutations by “theratype.”

**UNDERSTAND** the utility of human CF intestinal organoids to identify and predict responsiveness of rare CFTR mutations to CFTR correctors and potentiators.

**COLLECT**, catalog, expand and study drug responsiveness of human nasal epithelia from CF patients with rare CFTR mutations.

**EXPLORE** the potential of innovative CFTR gene engineering technologies to create rare CFTR mutations containing CF HBE cells or modify CFTR null cells to express wildtype, common and rare CFTR mutations.

---

## Develop nonsense mutation therapies which will treat most of the remaining untreated patients

### ACTION PLANS

**DETERMINE** the RNA stability of CFTR harboring a premature truncation mutation and whether readthrough stabilizes RNA.

**GUIDE** drug discovery programs to investigate readthrough alone or readthrough plus inhibition of nonsense-mediated RNA decay based upon the stability of CFTR mRNA harboring premature truncation mutations.

---

## Develop therapies that replace CFTR

### ACTION PLANS

**TACKLE** the issue of gene, oligonucleotide and protein delivery to human airway epithelia in a CF-like environment.

**ACTIVELY SEEK**, identify and, as appropriate, fund CFTR replacement programs.

---

## Repair mutated CFTR Gene

### ACTION PLAN

**EXPLORE** the potential of innovative CFTR gene repair technologies to correct CFTR function.

---

## Continue to develop tools and knowledge that will increase success of any program

### ACTION PLANS

**DETERMINE** the genetic sequence of the CFTR gene from DNA samples acquired in the F508del extremes of phenotype gene modifier study and from F508del DNA samples not in the extremes of phenotype.

**DETERMINE** the genetic sequence of the CFTR gene from HBE samples used by Vertex Pharmaceuticals in characterization of VX-809.

**DETERMINE** the genetic sequence of the CFTR gene from HBE samples owned by CF Foundation Therapeutics.

**COLLECT** and catalog human airway epithelia from CF patients with rare CFTR mutations.

**INCREASE** CFTR gene expression to aid multiple drug discovery programs.

**UNDERSTAND** the regulation of CFTR gene expression to aid multiple drug discovery programs.

## STRATEGY 2

We will develop a multipronged approach to increase adherence that will include enhanced data collection and information sharing.

---

**Adherence programs will be integrated and sustained within the Cystic Fibrosis Foundation, CF care centers and CF community.**

### ACTION PLANS

**DEVELOP** a roadmap that uses best practices for improving adherence and health outcomes for people with CF.

**IMPLEMENT** a communications campaign to publicize the importance of adherence in improving the health and well-being of people with CF.

**MAKE** an ongoing commitment, including personnel and funding, by the Foundation and other funding sources to sustain the adherence strategy.

**ESTABLISH** participatory implementation processes to engage all stakeholders in the adherence initiative.

**INCORPORATE** adherence-related outcome metrics and intervention strategies into the Foundation's quality improvement program.

**COMPLETE** an analysis of legal and ethical requirements and ramifications for collecting and sharing adherence-related data in order to determine risks and consents needed.

---

**Effective and efficient adherence programs tailored to individual patients and families will be disseminated and implemented.**

### ACTION PLANS

**TEST** and disseminate adherence interventions through a network of CF Foundation-accredited care centers.

**IMPLEMENT** a systematic process for identifying, selecting, designing and prototyping promising interventions that address barriers to adherence.

**DEVELOP** and implement a comprehensive and systematic means to identify patient, provider and health care system barriers to adherence.

**IMPLEMENT** a systematic approach to addressing the mental health needs of people with CF and their families as it affects adherence.

**IMPLEMENT** best practices for assisting all stakeholders to efficiently access recommended treatments for people with CF.

**IMPLEMENT** training on a broad range of adherence-related topics for CF care providers.

A technology platform to support and facilitate multilevel, multichannel adherence programs and information sharing among stakeholders will be created.

#### ACTION PLANS

**DEVELOP** and adopt a comprehensive set of adherence metrics and measures.

**COLLECT** and verify adherence-related data on an ongoing basis for all people with CF who provide consent.

**FINALIZE** an adherence information systems requirements document.

**DEVELOP** and test Version 1.0 of an adherence information system.

## STRATEGY 3

We will develop and implement plans so that all people with CF have access to high-quality, specialized and accredited CF care centers and prescribed therapies.

### Outcomes

#### ACTION PLANS

**INCORPORATE** the functionality to collect patient-reported outcome measures into the CF Patient Registry to provide a broader and better balanced assessment of quality of care.

**DEVELOP** and test an intervention program aimed at improving the patient experience of care at CF Foundation-accredited care centers in an improvement collaborative.

**CONDUCT** a comprehensive cost analysis of CF care to support an evaluation of existing and potential CF care delivery and payment models.

**IDENTIFY** the steps necessary to develop a value-based health care delivery model for CF care to prepare the CF Foundation for upcoming challenges and opportunities in the evolving health care landscape.

### Productive Interactions between Patients, Families and Care Teams

#### Education and Assistance Resources

#### ACTION PLANS

**ESTABLISH** a national resource network to assist people with CF, families and care center staff in finding CF resources, programs and services related to access and high-quality CF care.

**DEVELOP** expanded resources and make them available to people with CF, families and care center staff to provide the information, support and guidance needed for high-quality CF care.

**EXPAND** CF case management services to strengthen state-specific expertise and support related to coverage, reimbursement and health care policy to patients, families and care center staff.

## Productive Interactions between Patients, Families and Care Teams (continued)

### Patient Engagement

#### ACTION PLANS

**EXPAND** the public reporting of center-level data on [www.cff.org](http://www.cff.org).

**PROVIDE** online access to individualized registry reports, guidelines recommendations and resources to patients and families to promote engagement and facilitate self-management of care.

**CONDUCT** a demonstration project to provide patients with tools to report their symptoms and quality of life in response to medical treatments, and opportunities for social networking with other patients and care center staff with the aim of improving health outcomes, quality of life and the experience of care.

### Quality Resources for Care Centers

#### ACTION PLANS

**DEVELOP** “toolkits” for best practices (including clinical tools, care checklists and measurements) to support quality improvement at CF care centers and help people with CF, families and care center staff work together toward optimal health outcomes.

**DEVELOP** a template explicitly describing the components and characteristics of a high-quality, specialized care center to guide quality improvement efforts at all centers.

## Organization of Health Care

### Accreditation

#### ACTION PLANS

**ESTABLISH** revised standards that all CF Foundation-accredited care center programs (including affiliates) must meet to ensure delivery of high-quality care.

**DEVELOP** a database of information (e.g., site visit critiques and center responses, annual progress reports, etc.) on all accredited CF care centers to streamline the accreditation process and improve oversight of the care center network.

**DEVELOP** an assistance program to incentivize and hold accountable CF care centers having difficulty meeting accreditation standards.

---

## Organization of Health Care (continued)

### Decision Support

#### ACTION PLAN

**DEVELOP** a center-level dashboard with benchmarks (including medical outcomes, patients' experience of care and health care utilization), and patient-level clinical decision alerts and reports from CF Patient Registry data to provide a 360-degree view of care center performance and to guide centers in their improvement of care delivery.

### Adult-Specific

#### ACTION PLANS

**CONVENE** an advisory group to develop recommendations for improving the capacity and quality for age-appropriate CF adult care across the country.

**DEVELOP** a strategy for continued CF care center engagement of CF patients who have received a lung transplant.

---

## Advocacy and Public Policy

#### ACTION PLANS

**DEVELOP** a third-party payer relationship program to educate insurers about cystic fibrosis and to encourage favorable decisions related to coverage and payment for CF therapies.

**DEVELOP** state-level resources to conduct policy and regulatory analyses on proposals impacting access to quality CF care.

**EXPAND** grassroots advocacy capacity and develop relationships with targeted federal and state officials in order to influence health coverage policies and regulations that impact access to high-quality, specialized CF care.

**DEVELOP** and implement a comprehensive advocacy communications and engagement strategy to educate volunteers, donors and staff on health coverage policy issues and direct their advocacy efforts.

**USE** comparative effectiveness research more effectively to support evidence-based decision making in the prioritization of coverage and policy objectives.

## STRATEGY 4

We will develop and implement a comprehensive communications plan to:

- Manage expectations among our constituencies.
- Address “mission accomplished.”
- Address real or perceived conflicts related to royalty sales.
- Engage the entire CF community.

### ACTION PLANS

**COORDINATE** all CF Foundation communications efforts, including fundraising and medical communications, so that we are effectively communicating, with one voice, to respond to evolving issues and the emerging needs of the entire CF community, and to maximize the power and consistency of the Foundation’s brand and messaging across all channels.

**PROACTIVELY COMMUNICATE** the CF Foundation’s role as both a steadfast advocate for CF patients and families and a leader in venture philanthropy, and prepare to address controversial or sensitive issues by communicating the facts and correcting inaccuracies and misperceptions as they arise.

**STRENGTHEN** and enhance the CF Foundation’s current online presence and invest in new channels and technologies to reflect the changing media consumption habits of the Foundation’s existing and future audiences; to more fully engage the entire CF community, with an emphasis on people with CF; and to address the challenges presented by the infection prevention and control guidelines.

**MANAGE** expectations among CF Foundation constituents and address “mission accomplished” by accurately communicating the risks and uncertainties of the drug development process in a way that strengthens the CF community’s dedication to fundraising, advocacy, clinical trial participation and volunteerism, and conveys the Foundation’s commitment to its mission.

**WORK** to engage adults with CF more deeply and ensure their specific concerns are being heard, valued and addressed through ongoing two-way processes.

**PROVIDE** timely and accurate communications to CF Foundation chapters and enhance its support of chapter communication.

**PURSUE** new opportunities to engage and communicate with care centers and all parts of the CF community they serve.



## STRATEGY 5

We will increase fundraising to achieve our mission and objectives.

### ACTION PLANS

**REVISE** the crediting process to support collaboration and prospecting, as well as volunteer and donor development.

**INCREASE** retention rates for donors and volunteers.

**CREATE** a marketing function to support and enhance fundraising.

**FORM** a national volunteer leadership cabinet to identify and cultivate high-potential individuals to support Individual Giving and Corporate Development.

**DEVELOP** an integrated and coordinated approach to donor cultivation, stewardship and solicitation for Annual Fund, Major Gifts, Direct Mail and Planned Giving.

**REPOSITION** Great Strides as a broader national fundraising campaign that allows for participation in traditional walk events, as well as non-traditional events.

**FOCUS** the CF Foundation's special event portfolio on high-net, high-return events and eliminate less efficient events.

**CREATE** and implement a comprehensive, well-defined Planned Giving program throughout the Foundation.

**IMPROVE** the efficiency and effectiveness of the Direct Mail program or eliminate inefficient components of the program.

**INTEGRATE** a corporate sponsorship program into the Foundation's overall fundraising strategy, and set corporate sponsorship goals for the National Office and chapters.

**EMBRACE** and build out the "Adding Tomorrows" cause-marketing campaign, positioning it to offer clear value to participating corporations.

**DEVELOP** and implement a comprehensive strategy to engage, steward and retain "young professionals" (25–40 year olds).

## STRATEGY 6

Please see page 60 for the complete list of recommendations for CF Adult Task Force Action Plans.

## CONCLUSION

This report represents a living and changing, multiyear effort that we hope will allow us the greatest flexibility to meet foreseen and unforeseen changes ahead of us.

In all we do, we remain committed to achieving the Foundation's mission as speedily as possible and to the benefit of all people with the disease.

If you have questions or would like to offer comments, please feel free to share them directly with C. Richard Mattingly, executive vice president and chief operating officer. Rich has played a leadership role in researching, launching and guiding the strategic process and is committed to seeing that your thoughts are shared with the appropriate teams and subject experts. His contact information is: [crmattingly@cff.org](mailto:crmattingly@cff.org).



The CF Foundation wishes to thank the people with CF and their families who contributed their photos to this report.  
PHOTOGRAPHY Cade Martin, Derek Smith, Dakota Fine, limepop photography DESIGN Catalone Design Co.



6931 ARLINGTON ROAD  
BETHESDA, MD 20814  
1.800.FIGHT.CF  
[WWW.CFF.ORG](http://WWW.CFF.ORG)