CYSTIC FIBROSIS FOUNDATION®

5·Y·S·P

FIVE-YEAR STRATEGIC PLAN

Driven by a deep sense of urgency and commitment to people with cystic fibrosis, we will pursue progress with purpose across every area of our mission: Cure, Care, and Community.

Cure

In Cure, we are harnessing the power of science and of the CF community to unlock the potential of genetic therapies, invest boldly in breakthrough opportunities, and build the ecosystem needed to accelerate innovation.

Goal 1: Develop transformative genetic therapy approaches for the lungs for every person with CF
We will advance innovative genetic pulmonary therapies, optimize delivery technologies, and clear pathways for clinical trials for highly effective treatments

Subgoals:

- Establish models and biomarkers that relate laboratory assessment to clinical research to better predict clinical translation
- Assess alternate delivery vehicles to the lung to increase potential options
- Advance pulmonary therapy with potential for high efficacy and a strong safety profile in clinical trials
- Implement an efficient trial design and enrollment approach, paired with a supporting regulatory strategy, to pave the way for genetic therapy development

Goal 2: Pioneer a genetic cure for CF
We will build on the latest science to design the blueprint for a genetic treatment that cures CF both in and beyond the lung

Subgoals:

- Identify tissues and cell types beyond the lung where CFTR restoration is beneficial and needs to be targeted
- Establish enabling thresholds and assessment tools to initiate development of one or more delivery vehicles for prioritized tissues and cells
- Increase understanding of the minimal editing efficiency and maximal off-target rates of CFTR in vivo that translate to therapeutic benefit

Goal 3: Decrease the burden of CF manifestations
We will fuel research and catalyze treatments for GI, infection, CFRD, mental health, and other manifestations of CF

Subgoals:

- Describe the contribution of CF to clinically significant GI manifestations to inform future therapeutic development
- Improve detection and clarify clinical implications of persistent CF-specific infections
- Assess the feasibility of technologies to eradicate CF pathogens, such as *Pseudomonas aeruginosa*
- Expand foundational understanding of CFRD to inform how to leverage diabetes therapies for people with CF
- Assess the role of CFTR modulation in mental health to inform the treatment and management approach
- Explore the feasibility of developing future pulmonary manifestation therapies by combining populations for trials with adjacent diseases

Goal 4: Establish tomorrow's CF science infrastructure

We will prepare our enabling networks, tools, and workforce for the next era of CF research and development

Subgoals:

- Evolve research networks to most effectively advance therapeutic development
- Enable model development for inhaled pulmonary therapies at scale, and prepare for modeling needs for systemic multi-organ therapies
- Identify and support use of fit-for-purpose quantitative endpoints that enable efficient development of pulmonary therapies
- Assess whether the Foundation can play a direct role in therapeutic development for ultra-rare mutations and prepare accordingly
- Develop a skilled, multidisciplinary research workforce capable of advancing critical CF priorities in discovery, translational and clinical research, and therapeutic development

Care

In Care, we are adapting the care center network to deliver personalized care where it is needed most, advancing research to shape the future of care, and modernizing data systems to accelerate research and guide evidence-based decisions for care teams and people with CF.

Goal 1: Innovate CF care delivery

We will reimagine the care network and model to meet the varied and changing needs of people with CF while promoting long-term sustainability

Subgoals:

- Design an adaptable, sustainable CF care network that delivers high-quality, accessible care to all people with CF
- Tailor Foundation support for the care workforce to address increasing breadth of clinical needs, including in gastroenterology and endocrinology
- Characterize and evaluate ways to address care system navigation and other barriers to whole-person care

Goal 2: Drive advances in personalized care

We will help all people with CF receive the right care by facilitating care research on key topics, translating findings into guidance, and enhancing implementation efforts

Subgoals:

- Define and sponsor studies to address key care research topics in partnership with the CF community
- Develop guidance documents for care teams on prioritized topics using available data and evidence
- Strengthen dissemination of best practices, including for advanced disease and transplant, by developing a more customized, scalable model for quality improvement and implementation

Goal 3: Enhance care data capabilities

We will enhance the Foundation's Patient Registry's capacity to support insight-driven care and research via new data capture, analysis, and access capabilities

Subgoals:

- Optimize systems to capture and analyze additional essential data sources to advance discovery and delivery of optimal care
- Improve data availability for care team members, researchers, and people with CF, while continuing to meet the highest standards in privacy protection

Community

In Community, we are amplifying impact by optimizing programs, emphasizing fundraising, and reinforcing our position as a trusted resource and partner for the CF community. This plan is distinct in its commitment to activating the CF community and building more inclusive ways to get involved, while delivering more tailored information and support opportunities to meet evolving needs.

Goal 1: Support people navigating life with CF

We will provide tailored support for people with CF and their caregivers/partners in navigating their unique CF journeys

Subgoals:

- Optimize support offerings to maximize impact in areas where the Foundation provides the greatest value, complementing resources available from other sources
- Offer tailored connection opportunities to support the wellbeing of people with CF through different stages of life
- Enable care centers to guide people with CF and their caregivers/partners to relevant Foundation offerings at local and national levels

Goal 2: Achieve bold fundraising goals

We will accelerate fundraising momentum by transforming our strategy and structure, activating the CF community, and expanding circles of support

Subgoals:

- Design and implement a comprehensive, effective, and sustainable fundraising model and structure
- Evolve fundraising and related volunteer opportunities to be more inclusive, accessible, and inspiring
- Share stories and messages that show the Foundation's urgent need for support, building on impact already made alongside the community

Goal 3: Empower people with CF with trusted information

We will deliver accurate, relevant, and timely information to people with CF to address their evolving needs

Subgoals:

- Develop educational content on priority topics that integrates information across research, care, and day-to-day life with CF
- Increase direct access to information for people with CF, their caregivers/care partners, and care teams
- Tailor outreach to increase awareness and utilization of Foundation resources and offerings

Goal 4: Champion community priorities

We will amplify the voices of the CF community to advocate for the priorities of people with CF across research, care, and support

Subgoals:

- Enable people with CF and their circles of support to advocate on issues of importance to the CF community
- Harness community insights and data to effectively inform and shape the Foundation's work