



**WHITE PAPER**

**Shields Health Solutions**

**Cystic Fibrosis Care Model and Clinical Program**

# How Specialty Pharmacy Can Drive Better Outcomes in Cystic Fibrosis

## KEY TAKEAWAYS

- Cystic fibrosis (CF) is a rare, genetic disease caused by mutations in the cystic fibrosis transmembrane regulator (CFTR) gene. This progressive disease causes lung infections and leads to loss of lung function, pulmonary exacerbations, and eventually respiratory failure.
- This is a multisystem disease that requires prompt recognition and management of CF-related health issues to maintain quality of life.
- Multiple chronic pulmonary and gastrointestinal therapies are available which can contribute to a complex treatment regimen and overall burden for patients with CF and their caregivers.
- A high-touch integrated specialty pharmacy care model can help patients with CF manage the complexity of their treatment regimen and achieve their therapy goals.

CF is a rare, autosomal recessive disease caused by mutations in the cystic fibrosis transmembrane regulator (CFTR) gene that encodes the CFTR protein, an anion channel normally present in the epithelial membrane.<sup>1,2</sup> Absence or reduction in the CFTR protein results in thick mucus in various organs in the airway, gastrointestinal and reproductive tracts, pancreas, and sweat glands.<sup>1,3</sup> When mucus clogs the airways, infections and inflammation occur resulting in pulmonary exacerbations which are associated with a decline in lung function and decreased survival.<sup>4</sup> Given the progressive nature of CF, chronic polymicrobial airway infections are commonly seen and are rarely eradicated with antimicrobial therapy. As patients with CF get older, their need for intravenous (IV) antibiotics to treat their pulmonary exacerbations increases. Other complications associated with CF include liver disease, gastrointestinal disease, bone disease, depression and anxiety, chronic rhinosinusitis and diabetes mellitus.<sup>5</sup>

Malnutrition has been associated with increased morbidity and mortality in CF.<sup>5</sup> Decreased intake, malabsorption and increased metabolic demands contribute to poor growth. Therefore, optimizing nutritional status, educating patients and caregivers about nutrition or enteral feeding options, and potentially adding pancreatic enzyme replacement therapy is key to the overall management of a patient with CF.<sup>5</sup> The goal Body Mass Index (BMI) percentile established by the CF Foundation nutrition guidelines for children aged 2 to 19 years is at or above 50th percentile using the CDC growth curves.<sup>6</sup>

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CF is a rare genetic disease caused by mutations in the cystic fibrosis transmembrane regulator (CFTR) gene that encodes the CFTR protein, an anion channel normally present in the epithelial membrane.<sup>1,2</sup>

CF affects more than 30,000 children and adults in the United States.<sup>7,3</sup> The percentage of CF patients detected by newborn screenings continues to increase and the median age at diagnosis is 3 months.<sup>8</sup> Between 2016 and 2020, the median predicted survival age was 50 years (95% CI: 48.5 - 51.3 years), meaning half of the individuals born from 2016 to 2020 are predicted to live beyond 50 years of age.<sup>8</sup> This prediction does not take into account the potential impact of CFTR modulators on younger CF patients and other improvements in clinical care.<sup>8</sup> The current CF Registry contains data on people with CF from 1986 to 2020, and the improvements in care led to improved survival. For example, the number of adults with CF has increased from 32.1% in 1990 to 57.2% in 2020.<sup>8</sup> According to the CF Registry the median age of people with CF was 20.3 years in 2020.<sup>8</sup>

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CF affects more than 30,000 children and adults in the United States.<sup>7,3</sup> While the number of adults with CF has increased in the last three decades, advancements in clinical care have led to improved survival.

## MANAGEMENT OF CF AND ITS CHALLENGES

Pulmonary management of the CF patient involves clearance of airway secretions and can be accomplished using nebulized agents that thin the mucus, in addition to other airway clearance methods involving chest physiotherapy.

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### PULMONARY MANAGEMENT FOR PATIENTS WITH CF



Airway clearance helps decrease the respiratory bacterial load as an infection control measure.<sup>5</sup>



Nebulized therapies are a critical component to a CF treatment plan. Reports suggest patients and caregivers spend up to 6 hours per day on airway clearance activities and taking oral and inhaled therapies.<sup>9</sup>



Chronic infections can lead to pulmonary exacerbations which are typically treated with antibiotics.

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Studies show that one factor associated with poor response to exacerbation treatment may be longer time from symptom onset to treatment, which suggests delayed treatment results in worse outcomes.<sup>4</sup>

CFTR modulators target the CFTR protein resulting in improvement in outcomes such as lung function, growth and a decrease in pulmonary exacerbations. CFTR modulator therapy is expected to change the course of CF by slowing or possibly preventing progression of pulmonary complications.<sup>10</sup> CFTR modulators have been developed for ~90% of persons with CF yet the pricing strategy creates a barrier to effective and equitable treatment such that current estimates suggest 12% of CF patients receive elexacaftor/tezacaftor/ivacaftor worldwide.<sup>11,12</sup>

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Health system specialty pharmacies (HSSPs), which have developed a proactive, integrated, and coordinated approach to address the needs of complex patients, can play a critical role in ensuring timely medication access and disease state management.

One study estimated that universal introduction of elexacaftor/tezacaftor/ivacaftor in 2021 would reduce the number of people living with severe lung disease by 60% and deaths by 15% by 2030.<sup>12</sup> Current US list prices for CFTR modulators are over \$250,000 per year which creates a barrier to the best available agents for CF.<sup>12</sup>

The multitude of therapeutic options for this multisystem progressive disease leaves CF patients with an immense treatment burden. Health system specialty pharmacies (HSSPs), which have developed a proactive, integrated, and coordinated approach to address the needs of complex patients, can play a critical role in ensuring timely medication access and disease state management. It's likely the most substantial impact of CFTR modulator therapy occurs when initiated early in the course of the disease to minimize irreversible lung damage and to allow participation in activities such as exercise, social events and planning for the future.<sup>10,13</sup> HSSPs are positioned to advocate for and gain access to these critical agents while managing other barriers to optimal care such as adverse effects, drug interactions and out-of-pocket costs.

#### HOW THE SHIELDS CARE CONTINUUM HELPS

ShieldsRx has experience with partnering with the top, accredited CF Centers of Excellence institutions to develop innovative patient care models. ShieldsRx proactively works with patients to provide best-in-class therapy management to elicit optimal clinical outcomes, to reduce the incidence of adverse drug events, and to prevent unnecessary health care costs. Core clinical services provided by a team of CF clinical pharmacists consider patient/family needs assessment, comprehensive medication review, medication education, ongoing support through frequent outreach calls, and direct care coordination with the HSSP and CF center liaisons and staff. The CF clinical pharmacists complete advanced training to ensure knowledge and understanding of CF care guidelines and the evolving literature on CFTR modulators.

ShieldsRx has dedicated pharmacy liaisons embedded within health system partner CF Center clinics. The liaisons, with the support of the ShieldsRx centralized Patient Support Center (PSC), performs all benefits investigations, prior authorizations (PAs), and financial assistance for all specialty patients serviced by the CF clinic regardless of the dispensing specialty pharmacy. The liaisons interact with patients or caregivers in the clinics, providing personalized attention, and serve as a single point of contact for proactive refill management. Reminder calls are made to patients at a minimum of seven days before the refill date to ensure timely prescription receipt, and liaisons communicate with the CF Centers when issues with unreachable patients or those with adherence concerns arise. ShieldsRx liaisons also coordinate with the patient and CF provider to facilitate manufacturer hub enrollment for medication support services.



CF patients with access to the Shields Care Model

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The ShieldsRx CF clinical program has an average ER / hospitalization utilization rate of 10.7%, in line with the 14% reported in the 2020 CF Foundation Annual Report.<sup>8</sup>

ShieldsRx creates and follows holistic treatment care plans to closely monitor patients' success with a therapeutic regimen and any barriers in achieving goals of therapy. For example, pharmacists evaluate the CFTR mutation to assess if patients are candidates for CFTR modulators thus ensuring patients get started on the most effective therapy to align with the best evidence-based clinical practice. From there, clinical reassessments measure patient progress towards goals of therapy as pharmacists closely assess markers of lung function and nutritional status while monitoring for multisystem organ dysfunction. Pharmacists review respiratory culture results to ensure antimicrobial stewardship which ties into the management of CF-related acute respiratory infections. In response to a patient-reported exacerbation, the pharmacist evaluates the patient as well as data from the Electronic Medical Record (EMR) to determine if the event could be due to medication-related issues, such as non-adherence. In collaboration with the CF prescriber, the clinical pharmacist designs interventions, tailors the care plan frequency, and outlines goals of therapy, which are periodically reassessed. The clinical reassessments include adherence screening, side effect monitoring, screening for absenteeism from work or school, and evaluation of overall impact of the medication regimen to ensure the patient is receiving optimal benefit from the care plan. Additionally, pharmacists conduct medication reconciliation at every touchpoint with the patient to assess drug-drug interactions, eliminate duplicate therapy, and reduce the overall incidence of polypharmacy. Through this thoughtful and detailed follow up, the ShieldsRx clinical care model provides the proper guidance to find balance between this complex disease and maintaining quality of life.

Full access to the EMR, pharmacy dispensing system, and clinical management platform by both the clinical pharmacists and liaisons is key to supporting a best-in-class, fully integrated specialty pharmacy program for CF patients. To provide a seamless continuum of care, all documentation is recorded within the EMR and in the therapy management system. Pharmacists document interventions and patient clinical questions in the EMR and communicate to the prescriber in real-time, often preventing unnecessary clinic or Emergency Department (ED) visits. In an internal review of CF pediatric and adult patients who were onboarded into the integrated care model after 2019, the rate of ED or hospitalization utilization was 10.7% which is in-line with the lower rates of hospitalization seen after the introduction of CFTR modulators.<sup>8</sup> This integration bridges the communication and connection barriers that exist with non-integrated specialty pharmacies which could lead to delays in initiating therapy, medications being dispensed that are no longer appropriate, or confusion for the patient and their caregivers as to whom they should contact when issues or questions arise.

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The ShieldsRx CF clinical program results in timely initiation of therapy, with <6.9 days to therapy on average.

## THE SHIELDSRX IMPACT

In addition to achieving a high Proportion of Days Covered (PDC) of 91%, an indicator that patients receive and take their medications on time, the ShieldsRx CF clinical program results in timely initiation of therapy, with 6.9 days to therapy on average. As a result of the financial assistance support provided by the ShieldsRx model, liaisons within CF clinics have secured more than \$1 million in 2021, resulting in an average per script copay of \$15.00. Alleviating out-of-pocket costs for CF patients reduces financial toxicity barriers for medication access, which ultimately correlates with adherence.

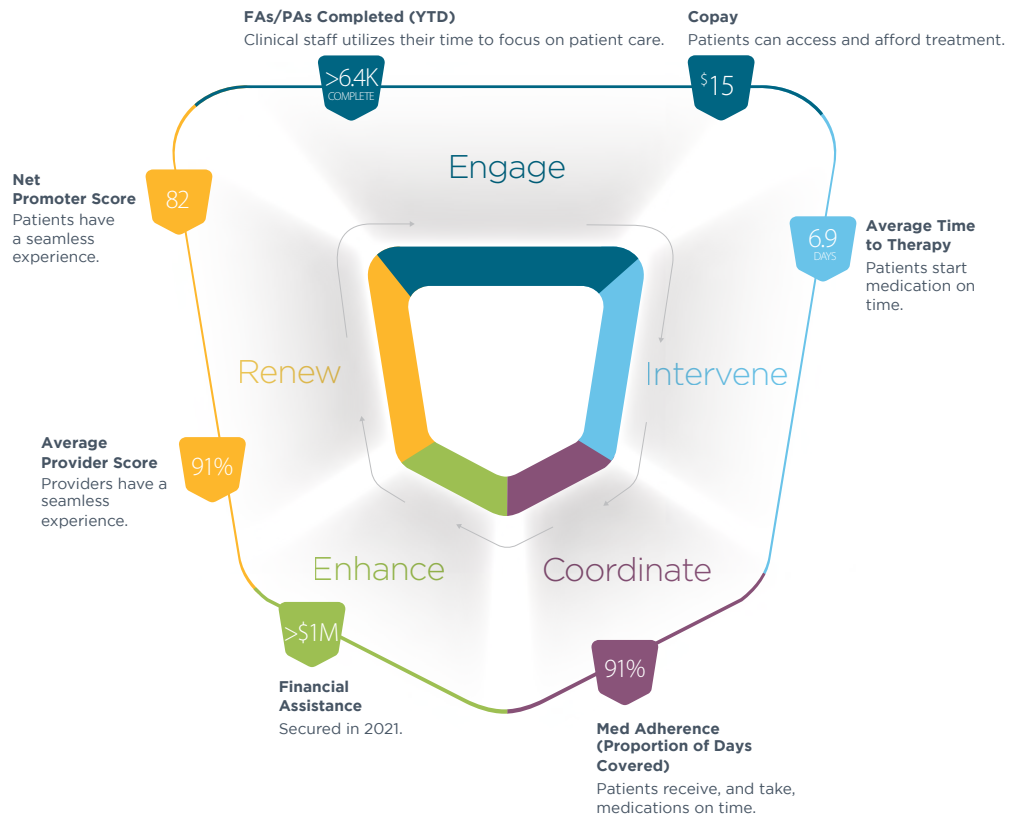
Pharmacists perform clinical interventions to optimize medication therapy and the overall patient care plan. In general, the intervention acceptance rate across the Shields network is >95%, meaning health care providers rely and act upon the expertise provided by the pharmacists. Examples of clinical interventions include identification of drug interactions, prevention of adverse events, elimination of drug wastage, and avoidance of urgent care or ER visits. Additionally, pharmacists guide patients towards achieving nutritional goals. In a brief one-year review of the pediatric population enrolled in the Shields Health Solutions Care Model, 79% (79/100) of CF patients achieved goal BMI percentile of 50 or greater which aligns with goals established by the CF Foundation nutrition guidelines.

Furthermore, 98% of CF patients report their treatment works well or very well to improve their condition, according to a ShieldsRx measure termed Patient Reported Treatment Efficacy (PRTE). All components of the ShieldsRx CF clinical program contribute to high patient and provider satisfaction scores, as indicated by a Net Promoter Score of 82 and Average Provider Score of 91%, respectively.



ShieldsRx CF clinical program  
Net Promoter Score

# SHIELDS CARE CONTINUUM



\*Calendar Year 2021

## CONCLUSION & SUMMARY

CF is a complex disease, and ShieldsRx in partnership with HSSPs and providers eases the burden of this progressive disease through faster time to therapy, high medication adherence, and low out-of-pocket costs for medications. Our goal of providing safe and appropriate use of prescribed therapy regimens through multidisciplinary collaboration is reinforced by a high PDC of 91%, low copays, and a high clinical intervention acceptance rate by CF providers.

The ShieldsRx CF clinical program and care model is designed to support the most innovative, best-in-class patient services in the industry through partnerships with accredited CF care centers nationwide. Through these partnerships, the ShieldsRx CF care model will enhance the best care, treatments and support for CF patients offered at these leading centers of excellence. The program is continually evaluated to ensure services and care protocols are supported by the most recent evidence-based guidelines, stakeholder input and collaboration, patient-reported outcomes, and real-world evidence. Our objective is to consistently promote clinical and economic outcomes while improving patient quality of life.

The ShieldsRx CF clinical program and care model is designed to support the most innovative, best-in-class patient services in the industry through partnerships with accredited CF care centers nationwide.

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### ABOUT SHIELDS HEALTH SOLUTIONS

Shields Health Solutions (ShieldsRx) is the premier specialty pharmacy accelerator in the country. The Shields Performance Platform, an integrated set of solutions, services and technology, is intentionally designed to elevate payer and drug access for specialty pharmacies, elevate health outcomes for complex patients, and elevate growth throughout the entire health system. As the foremost experts in the health system specialty pharmacy industry, Shields has a proven track record of success including access to over 80 percent of all limited distribution drugs (LDDs) and most (health insurance) payers in the nation; and a clinical model proven to lower total cost of care by 13%. In partnership with more than 70 health systems across the country through national-scale collaboration, ShieldsRx has a vested interest in delivering measurable clinical and financial results for health systems.



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