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MAKING TREATMENT ACCESSIBLE FOR CYSTIC FIBROSIS PATIENTS

A photograph showing a doctor on the right, wearing a white coat, using a stethoscope to examine the back of a patient on the left. The patient is wearing a grey t-shirt and has his hand near his mouth. The background is a plain, light-colored wall.

OVERVIEW

Cystic fibrosis is a lethal genetic disease that causes excessive mucus in vital organs, most notably the lungs. Patients struggle to breathe and face chronic and debilitating lung infections. Over time, cystic fibrosis damages the lungs and organs, and patients can suffer from severe digestive problems, diabetes, liver disease, and male infertility. The leading cause of death is respiratory failure.¹

Cystic fibrosis was identified as a disease in 1938, but practical tests for it weren't developed until the 1950s.¹ Until that point, most cystic fibrosis patients didn't live to reach elementary school. Even today, while modern medicine has greatly improved the life expectancy of cystic fibrosis patients, the median predicted survival age is still only 41 years old.²

For decades, doctors have been able only to mask the symptoms of cystic fibrosis. Now, recent

breakthroughs have produced treatments that actually address its cause. In 2012 the U.S. Food and Drug Administration (FDA) approved new medications to target specific cystic fibrosis mutations. Yet challenges remain. The drugs are mutation specific and cannot work for all cystic fibrosis patients. For patients who can benefit from them, cost and coverage barriers keep the drugs from being broadly available.

LIFE WITH CYSTIC FIBROSIS

The most common fatal genetic disorder in North America, cystic fibrosis affects approximately 30,000 Americans and more than 70,000 people around the globe. The disease can impact nearly every aspect of these patients' lives.

Reduced lung capacity leaves patients coughing, struggling with shortness of breath and repeatedly fighting off pneumonia and bronchitis. Avoiding germs is a constant worry, and oral, inhaled and IV antibiotics are a fact of life for many patients. Meanwhile, mucus buildup in the pancreas can interfere with the body's ability to absorb nutrients from food, stunting growth. Patients may struggle to maintain normal body weight.

Those suffering from cystic fibrosis endure not only the disease itself but also daily, time-consuming treatments and a lifetime of medication. Symptoms and complications from cystic fibrosis affect young patients' involvement at school and can impact adult patients' ability to maintain regular employment. Physical activity carries added challenges.

Symptoms and treatment can also impact patients' emotional well-being and interpersonal relationships. Males with cystic fibrosis may be unable to have children.

SYMPTOMS



coughing, shortness of breath



need for oral, inhaled & IV antibiotics



stunted growth



struggle to maintain normal body weight

IMPACT OF CYSTIC FIBROSIS

QUALITY OF LIFE



daily, time-consuming treatments



possible male infertility



impact on school involvement



difficulty maintaining employment

OPTIONS FOR PATIENTS

Until recently, medical science has been able to treat only the symptoms of cystic fibrosis.

Patients take a combination of mucus-thinning drugs such as hypertonic saline and dornase alpha; and bronchodilators such as albuterol. Additionally, cystic fibrosis patients use “high-frequency chest wall oscillation devices,” vests that vibrate the chest cavity to help clear mucus. While these treatments extend life, they do nothing to address the underlying cause of the disease.

ORPHAN DISEASES

Lack of treatment options is due in part to the rarity of the disease. Cystic fibrosis is classified as an “orphan disease,” meaning that it affects fewer than 200,000 people around the country.³ As a result, pharmaceutical companies have historically been hesitant to research or manufacture therapies given the financial risks. Other orphan diseases include Lou Gehrig’s disease, Lupus nephritis, and albinism.⁴

In 1983 the U.S passed the Orphan Drug Act, which incentivized manufacturers to pursue treatments for rare diseases. Since then, the industry has developed more than 250 “orphan

drugs” (compared to 10 developed in the preceding decade), providing treatment to more than 13 million Americans.⁵

BREAKTHROUGH TREATMENTS

One of these cutting-edge therapies was ivacaftor, approved by the FDA in 2012 to treat cystic fibrosis. Ivacaftor targets certain cystic fibrosis mutations and treats a small portion, about 5 percent, of cystic fibrosis patients. Yet unlike previous treatments, ivacaftor treats the underlying causes of cystic fibrosis rather than simply treating the symptoms.

Clinical trials showed an average of 10-12 percentage points’ improvement in lung functioning after only 24 weeks of treatment.⁶ For context, the average cystic fibrosis patient over the age of six years loses 1-3% of his or her lung function every year.

The FDA has since approved a new combination drug, ivacaftor/lumacaftor. The therapy has proven effective for patients with two copies of the most common mutation, F508del.⁷



ACCESS CHALLENGES

While the Orphan Drug Act successfully increased the research and production of orphan drugs—which made them available in the marketplace—it did not necessarily make them accessible to patients with cystic fibrosis.

Certain barriers stand in the way.



MEDICAID BARRIERS

Given the high cost of managing cystic fibrosis, about 45% of these patients depend upon Medicaid for their health care.⁸ Typically, Medicaid covers doctor visits and medication for cystic fibrosis patients. According to federal law, state Medicaid agencies are obligated to pay for treatments that are approved by the FDA. However, because Medicaid is administered on the state level, coverage varies by location.

Some states, like Pennsylvania and Nebraska, have clear criteria for determining what Medicaid will cover. They involve medical experts in their decision making. Others, such as Ohio and Arkansas, do not have a clear decision-making process and are not required to consult with stakeholders or medical professionals when determining coverage.

Often, internal and opaque bureaucracies such as Drug Utilization Reviews and Pharmacy and Therapeutics committees are tasked with approving

the use of drugs within a particular agency or institution. They can delay or deny the use of a particular drug. This lack of communication between actual patients and disease experts and the policymakers who determine access to treatment can result in policies that are outdated and inconsistent with current practices. Such a model hurts patients.

For example, in a 2014 court case three Arkansas families alleged that state Medicaid officials blocked their children's access to ivacaftor due to cost. The state imposed a 12-month waiting period and required patients to show proof their condition was worsening—effectively saying that the children had to become sicker before they received the drug. Arkansas ultimately covered the treatment, but only after a lengthy court process during which all the plaintiffs were hospitalized at least once for cystic fibrosis-related issues.⁹



PRIOR AUTHORIZATION

High-cost, high-value medications can present access barriers even for patients with adequate insurance coverage. **One common health plan barrier is prior authorization, where the insurance provider must sign off on the treatment prescribed by a patient's physician.**

Obtaining this authorization can be time-consuming and difficult. Often, patients are denied coverage altogether. Requirements can include anything from simply submitting documentation to running a full gamut of tests or treatments—some of which may be redundant or unnecessary—before getting approval.

In some cases, public or private health plans require evidence that patients are suffering before they will provide coverage. Consider California, for example. There, the state's Genetically Handicapped Persons Program requires that patients 21 years and older who are prescribed ivacaftor/lumacaftor show proof of hospitalization in the past 12 months if their FEV1, a measure of lung capacity, falls within a certain range. Otherwise, patients must navigate the state's exception process, taking on a time and administrative burden that delays their access to medication—if, in fact, they do finally receive the treatment.



FAIL FIRST

In other cases, prior authorizations include step therapy. Patients must demonstrate that they have failed on an insurer-preferred treatment for a specific period of time before getting the prescribed medication. The medications may be older or less effective, but they are less expensive upfront for the insurer.

This process, also known as “fail first,” can be aggravating for doctors and patients in any situation. But in the case of cystic fibrosis, delaying treatment can have long-term or permanent effects. **Cystic fibrosis is a progressive disease, so every day without treatment represents another day of progressive damage. If left unchecked for too long, the damage can be irreversible.**

Furthermore, the combination of unnecessary tests and prolonged lack of treatment—which often leads to more complications, doctor visits, and hospitalizations—can be more expensive than simply approving the medication in the first place. This hurts patients, wastes doctors' time, and costs insurers more in the long run.



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MOVING TOWARD SOLUTIONS

Prior authorizations, step therapy, and bureaucratic red tape all effectively do the same thing: come between physicians and the treatment they know their patients need. To address this, legislation at the federal and state levels must level the playing field for patients.

In 2012, Congress passed the Expanding and Promoting Expertise in Review of Rare Treatments (EXPERTT) Act, which mandates the involvement of external experts in the FDA's approval process for new drugs targeted at rare diseases.¹⁰ Additionally, the bill helps expedite the approval of medications for rare diseases and encourages knowledge sharing within the government. Several states have implemented similar approaches. With Congress' encouragement, more state Medicaid systems could adopt open, transparent procedures that invite patients and stakeholders into the coverage decision-making process.

In addition, some state legislatures, such as those in California, Illinois, and Indiana, have begun to address step therapy and prior authorization. Most recently, in January 2017, New York passed legislation to specifically address step therapy. The legislation requires that insurers' review criteria reflect science-based guidelines and are not principally focused on cost control. Additionally, New York now requires insurers to provide a clear and expedient process for overriding step therapy requirements.¹¹





CONCLUSION

Only a few decades ago, a diagnosis of cystic fibrosis was a death sentence. Now, modern medicine has found methods to treat not only the symptoms of the disease but also the underlying cause for patients with certain mutations.

Yet these advances have limited impact unless they can reach patients. Granted, innovative medicine is expensive. Yet prior authorizations, step therapy requirements, and slow-moving approval processes all impede patients' access to treatment, which harms patients and ultimately costs everyone more time and money. Processes and bureaucracies must not put finances above doctors' judgment about what is best for their patients.

In some states, legislators have passed laws to protect the needs of patients by regulating the requirements insurers can impose. Until all states have these laws, however, the nation faces a patchwork of coverage so that a patient in one zip code may not have the same opportunities as someone just over the state line.

Tackling complex and rare diseases like cystic fibrosis requires cooperation. Pharmaceutical companies, insurance providers, and state Medicaid systems need to work with patients, families, and physicians to find commonsense solutions that prioritize the health of cystic fibrosis patients—and encourage continued treatment breakthroughs for this and other rare diseases.

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serves as a leading educational and advocacy organization focused on policy matters impacting cystic fibrosis patients' access to optimal care.

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