CYSTIC FIBROSIS FOUNDATION®

April 26, 2023

The Honorable Bernie Sanders
Chair
HELP Committee
United States Senate
Washington, DC 20510

The Honorable Bill Cassidy, MD
Ranking Member
HELP Committee
United States Senate
Washington, DC 20510

Dear Chairman Sanders and Ranking Member Cassidy:

On behalf of the nearly 40,000 children and adults with cystic fibrosis in the United States, we write to provide additional perspectives and recommendations for the May 2nd mark-up to lower prescription drug prices. First, we urge Congress to address the confusing, labyrinthian system pharmacy benefit managers (PBMs) have created and improve transparency for patients. We also ask Congress to ban certain PBM practices, including co-pay accumulators, maximizers, and alternative funding programs. Finally, we urge Congress to codify the decades-long interpretation and practice by the FDA for awarding orphan drug exclusivity by passing the Retaining Access and Restoring Exclusivity (RARE) Act.

The Cystic Fibrosis Foundation is a national organization dedicated to curing cystic fibrosis (CF). We invest in research and development of new CF therapies, advocate for access to care for people with CF, and fund and accredit a network of specialized CF care centers. Cystic fibrosis is a life-threatening genetic disease that causes the body to produce thick, sticky mucus that clogs the lungs and digestive system, which can lead to life-threatening infections. As a complex, multi-system condition, CF requires targeted, specialized treatment and medications. If left untreated, infections and exacerbations caused by CF can result in irreversible lung damage, and the associated symptoms of CF lead to early death, usually by respiratory failure. Transformative therapies—such as CFTR modulators—have been paramount in changing what it means to live with CF. However, PBM cost containment strategies have created a convoluted system that patients struggle to navigate and often results in significant barriers to care.

Pharmacy Benefit Manager Reform

PBMs manage prescription drug benefits on behalf of health insurers, Medicare Part D drug plans, large employers, and other payers. By negotiating with drug manufacturers and pharmacies to determine drug coverage and reimbursement, PBMs can exert significant control over total drug costs for insurers, patients' access to medications, and how much pharmacies are paid.[1] PBMs often focus cost mitigation strategies on specialty drugs because of their high cost but low utilization within the overall population.

PBM practices and the opacity of the system are extremely problematic and burdensome for chronic conditions like CF that primarily use specialty drugs. PBMs and insurance companies both regularly claim that the other entity makes the final determinations on coverage for a therapy, resulting in an avoidance

[1] https://www.healthaffairs.org/do/10.1377/hpb20171409.000178/full/healthpolicybrief_178-1660136543567.pdf

Bethesda Office
4550 Montgomery Avenue, Suite 1100N, Bethesda, Maryland 20814
301.951.4422 800.FIGHT.CF Fax: 301.951.6378
www.cff.org email: info@cff.org

of responsibility from both parties and delays and confusion for the patients they cover. Patients frequently report being "passed back-and-forth" between the two entities when seeking to understand coverage decisions. The result is that people with CF do not know who is ultimately responsible for decisions about their drug coverage, or where to appeal in order to access their essential treatments. The CF Foundation appreciates Congress' attention to PBM reform. We urge Congress to ensure that the legislative proposals seek to improve the experience for patients, in addition to regulating the business and financial structure of PBMs. We provide the following recommendations:

Transparency: CF Foundation recommends Congress direct the FTC and HHS to expand transparency measures for PBMs and insurers to ensure patients receive better information about coverage policies for specialty drugs, including relationships with third-party entities. Specifically, Congress should direct the FTC and HHS to require PBMs and payers to provide enrollees with notices and disclosures on which entity is responsible for coverage determinations and provide clear contact information.

HELP Copays Act: The CF Foundation recommends including the Help Ensure Lower Patient Copays Act (HELP Copays Act, HR 830) into any PMB reform legislation. This bill reduces patient administrative and financial barriers imposed by PBMs and payers by 1) requiring payers to apply third-party assistance to out-of-pocket maximums and other patient cost-sharing requirements; and 2) ensuring any item or service covered by a health plan is considered part of their essential health benefits (EHB) package. Together, these policies would prohibit accumulators, maximizers, and alternative funding programs in federally regulated insurance plans, eliminating some of the most problematic PBM practices for patients. We recognize that PBMs and insurers are trying to mitigate the increasing cost of care but these practices that put financial and administrative burden on patients are not the solution.

Oversight & Enforcement: The CF Foundation supports efforts by Congress to require the FTC to determine whether there is more information about PBMs that should be available to consumers and whether there are any legal or regulatory obstacles the FTC currently faces in enforcing the antitrust and consumer protection laws in the PBM marketplace.

Orphan Drug Exclusivity

The CF Foundation also supports the passage of the RARE Act in a package to address prescription drug prices. As a way to incentivize drug development for rare diseases, the Orphan Drug Act established a term of market exclusivity for orphan drugs approved by the FDA. Orphan drug exclusivity (ODE) protects companies from parties seeking approval for the "same drug for the same disease or condition" for seven years. Importantly, the FDA has historically interpreted this as protecting exclusivity for the "same use or indication" within a disease or condition. The recent *Catalyst Pharms., Inc. v. Becerra* court decision would require the FDA to grant ODE based on "disease or condition," not "approved use or indication" within the disease or condition. Under the *Catalyst* decision, once an orphan drug is approved for a single use or indication, the FDA cannot approve another company's application for the same drug for any additional use or indication within that disease (e.g., pediatric populations).

For cystic fibrosis, some sponsors pursue label expansions to add additional indications, such as new genotypes or age groups, to a drug's label. Each label expansion receives an additional orphan drug exclusivity period—under *Catalyst,* these additional label expansions could block generic drugs from coming to market for the populations included in previous labels, even when those earlier exclusivity periods expire. This means that patients may wait longer for more affordable options. We therefore ask Congress to restore the FDA's long-standing system for awarding ODE by including the RARE Act in the upcoming legislative mark-up to lower prescription drug prices.

Bethesda Office
4550 Montgomery Avenue, Suite 1100N, Bethesda, Maryland 20814
301.951.4422 800.FIGHT.CF Fax: 301.951.6378
www.cff.org email: info@cff.org

****

Thank you for your leadership on these important issues; the CF Foundation stands ready to partner with this committee to pass legislation that will have significant impact on patient access. Please contact David Elin, delin@cff.org, if you have any questions.

Sincerely,

Mary B. Dwight
Chief Policy & Advocacy Officer
Senior Vice President, Policy & Advocacy
Cystic Fibrosis Foundation

Bethesda Office
4550 Montgomery Avenue, Suite 1100N, Bethesda, Maryland 20814
301.951.4422 800.FIGHT.CF Fax: 301.951.6378
www.cff.org email: info@cff.org