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Answer to a Question for the Record for the hearing:
"Improving the Medicaid Program for Beneficiaries"
Committee on Energy and Commerce, Subcommittee on Health

The Honorable Representative Renee L. Ellmers

1. **I'm concerned that lack of access to appropriate care often times leads to more significant costs to beneficiaries and the program, especially those with chronic conditions such as diabetes. Have you examined and/or do you have experience with the impact of access to care on cost, care needs, and mortality?**

The Cystic Fibrosis Foundation supports a wide range of activities aimed at ensuring that individuals with cystic fibrosis (CF) receive access to appropriate care according to clinical guidelines or standards and without delay.

The CF Foundation has developed clinical practice guidelines for routine care and screening for individuals with CF. These standards guide care for individuals with CF during infancy, childhood, and adulthood. For those over age 6, quarterly office visits and two pulmonary function tests each year are recommended. Guidelines have also been developed for use of chronic pulmonary therapies, which are a major component of the treatment regimen for those with CF; these guidelines govern pulmonary therapies for those who are age 6 or older. Goals have also been set for nutritional outcomes for those with CF. Finally, CF experts follow standards for the management of the complications of CF.

These guidelines and standards encourage the proper use of the many new therapies that have been developed for the management of the symptoms of CF. The CF treatment regimen is complex and therefore poses challenges for patients and their families. Gaps in patient adherence mean that the benefits of CF treatments and treatment guidelines benefit many but not all.

Sixty years ago, the average life expectancy of a child with cystic fibrosis was five years. Because of new CF treatments, constant quality improvement of the CF care system, and strong disease self-management average life expectancy of individuals with CF is 40 years. And for the first time in the history of the disease more than half of people with CF in the US are age 18 or older.

More recently, CF treatments have expanded to include two therapies that address the underlying cause of CF. These treatments were developed for and are effective in patients with specific CF mutations, making them the first "targeted" therapies for CF. The CF Foundation has developed strong initiatives to ensure that those who might benefit from these new medications have prompt access to them.

The Cystic Fibrosis Patient Registry data indicate that CF patients rely on private insurance, Medicare, Medicaid and other state programs, and Tricare or other military plans; and often patients have more than one plan covering their care and treatments. A very small percentage of those with CF are uninsured. The reliance on specific types of health insurance varies by age. For example, a large portion of children with CF use Medicaid or state programs, including over 50 percent of children under age 10. A majority of people with CF ages 18 to 25 received health insurance through their parents' plans in 2014.

While complex, time-consuming treatment regimens have greatly contributed to increasing length and quality of life for people with CF, the financial burden of the disease is a critical challenge for patients and families. Health insurance coverage does not necessarily eliminate all problems in obtaining access to CF care, including medications. There are programs in place that offer financial assistance but even so, CF patients may experience difficulties in obtaining coverage and proper payment for new CF medications. Patients also report burdensome cost-sharing requirements that may force them to forgo treatment or delay or reduce doses of medications. This means that because of financial challenges, patients may receive less than the optimal standard of care. We do not have data on the impact of these changes in care on overall health system costs.

The CF community has observed socioeconomic status-related disparities in CF health outcomes. Investigators have undertaken analyses of the Epidemiological Study of Cystic Fibrosis (ESCF) to determine if a differential use of health services or differential prescription of chronic therapy explains the disparities in CF outcomes. In other words, CF researchers have sought to understand if differential access to care explained SES-related disparities in health outcomes. Studies have found no evidence that disparities in CF outcome are due to discrepancies in care, including treatment of pulmonary exacerbations, use of chronic therapies, and outpatient monitoring. [1] Investigators have suggested that future studies consider the differences in disease management by patients and families and environmental exposures in efforts to understand disparities in CF outcomes.

[1] Schechter MS, McColley SA, Regelmann W, et al. Socioeconomic status and the likelihood of antibiotic treatment for signs and symptoms of pulmonary exacerbation in children with cystic fibrosis. J Pediatr. 2011 November; 159(5) 819-824. Schechter MS, McColley SA, Silva S, et al. Association of socioeconomic status with the use of chronic therapies and healthcare utilization in children with cystic fibrosis. J Pediatr 2009 November; 155(5): 634-9.