February 5, 2025

House Health and Government Operations Committee
State of Maryland
100 State Circle
Annapolis, MD 21401

RE: OPPOSITION TO HB0424– Prescription Drug Affordability Board

Dear Representative,

On behalf of the Cystic Fibrosis Research Institute (CFRI), which provides services to those impacted by cystic fibrosis across the US, including in Maryland, and as the mother of an adult living with cystic fibrosis, I write to express our concerns regarding HB0424, a bill to expand the authority of the Prescription Drug Affordability Board (PDAB) by enabling a process for the PDAB to set upper payment limits on prescription drugs in the State of Maryland. We respectfully request that the House Health and Government Operations Committee oppose the legislation, as it has the potential to restrict access to rare disease medications, in particular the CFTR modulator drug, Trikafta, and reduce access to other crucial therapies.

As a community with very complex chronic medical issues, those impacted by cystic fibrosis know very well that the cost of health care is far too high, and we applaud the intent to lower costs to protect patients and their families. However, we are concerned that with the proposed expanding authority of the Maryland PDAB there is no carve out for rare disease medications – including Trikafta – and that these drugs will be judged under the same lens as a medication that can treat hundreds of thousands of people.

Cystic fibrosis is a genetic disease with no cure that has been diagnosed in less than 1,000 people in Colorado, and 40,000 nationwide. Most known for causing progressive lung damage, CF impacts every organ system including the pancreas, liver, reproductive organs, sinuses and beyond. Cystic fibrosis is a rare disease, defined as one impacting 200,000 people or less in the United States.

Not too long ago, those with cystic fibrosis were not expected to reach adulthood. Life expectancy has steadily increased thanks to new – but expensive – therapies. This includes Trikafta, a CFTR modulating medication that addresses the root problem of cystic fibrosis. A simple Google search of the impact of Trikafta will lead to numerous scientific research articles and countless personal testimonials from patients about the positive impacts of Trikafta. These include people waiting for double lung transplants who are now off the wait list and even off supplemental oxygen for the first time in years. People have been able to end long-term disability and return to work. I have a friend who was able to return to her work as a practicing physician after many years on disability. My own daughter was able to stop injecting insulin

after 15 years of struggling to control her cystic fibrosis related diabetes. Beyond health improvements, there are emotional and economic benefits as well.

There are currently no generic alternatives to these mutation specific modulator therapies – indeed no alternatives whatsoever - and we are concerned that hospitals and specialty pharmacies will be penalized if they do not purchase medications under upper price limits established by the Board.

While quality of life and life expectancy have improved for many, CF remains a fatal disease, and half the individuals who died from cystic fibrosis complications last year were under 40 years old. Those who battle CF face hours of daily respiratory therapy, and require multiple inhaled, oral, and IV medications.

We are well aware of egregious price hikes imposed by some pharmaceutical companies. We are concerned that the expanded authority of a PDAB will fail to consider patient experience or needs in decisions intended to address prescription drug "affordability." While we appreciate the intent of HB0424 in trying to protect families, people with rare diseases, and others from having to decide between the treatments they need and basic necessities, PDABs in other states failed to provide substantial savings for patients.

Approximately 95% of rare diseases currently have no FDA-approved therapies and we fear that the complexities of developing rare disease medications may not be fully considered before price caps are established, and those desperately needing these therapies will lose access. We are fearful that the targeting of rare disease drugs will stifle innovation and drug development in the rare disease biotechnology realm.

While I represent the CF community, this bill has the potential to impact other Maryland rare disease communities who finally have new therapies, including Sickle Cell, Duchenne and Spinal Muscular Atrophy. Unfortunately, these medications are very expensive as well.

Thank you for ensuring access to therapies for Maryland residents.

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CC: Members, Health and Government Operations Committee