PIPC Patient Blog: ICER Report Adds Insult to Injury for Cystic Fibrosis Patients

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My daughter, Tess, lives with cystic fibrosis (CF). She is twenty-five years old. Not too long ago that would have been unimaginable for a CF patient. I’m so grateful for the medical advances that allow my child to have a full life.

Tess – like the majority of her peers with CF – spends hours every day following a complex medical regimen, trying to stave off lung function decline and death. In the CF community, we know too well what a capricious disease it is. One bad infection can create the “death spiral,” and we are still losing people at tragically young ages. In recent years, halting a decline in lung function was considered a win. Yet today, thanks to a new disease modifying therapy, 90% of those with CF have the potential to improve their ability to breathe. Trikafta, a breakthrough therapy for CF, treats not just the symptoms but the underlying cause of the disease. The availability of this type of treatment means my daughter, and many patients like her, have hope and a real opportunity for the future.

As you can imagine, the CF community has been particularly on edge during the current COVID-19 crisis. The virus, which attacks the respiratory system, poses an extreme risk for CF patients. We have been hunkering down to care for our community. As the executive director of Cystic Fibrosis Research Inc. (CFRI), I have been focused on ensuring that CF patients are still able to access medications while sheltering in place and advocating to ensure CF patients are not subject to discrimination in favor of healthy people should they contract the disease and require hospital services and medical equipment that is in short supply. It has been a trying eight weeks.

To add insult to injury, in the middle of this crisis, the Institute for Clinical and Economic Review (ICER), a watchdog group that purports to determine whether a drug is worth its cost, released its “Evidence Report” on the ‘value’ of Trikafta. ICER’s reports are based on a discriminatory metric, the Quality-Adjusted Life Year, which quite simply measures the lives of people with disabilities and chronic illnesses, like my daughter, as less valuable than those of “healthy” people. This means ICER’s reports consistently undervalue treatments for people with diseases like CF.

ICER’s report found that Trikafta had clear clinical benefit. Indeed, they gave Trikafta an “A,” their highest rating for comparative clinical effectiveness, which, in the words of ICER staff indicates there is “high certainty that the treatment delivers substantial health benefits.” Without question, this drug is a breakthrough. For many patients it can turn CF from a definitive death sentence into a chronic life-threatening disease. Despite this clear clinical benefit, ICER also found that the drug is “not worth the cost,” even if it were a curative therapy.

Put bluntly, ICER believes that my daughter is not worth treating. Her life – and the lives of thousands of individuals suffering the impact of CF - is not worth improving. I find it horrifying that this organization is able to make decisions so divorced from humanity. Cystic fibrosis-related complications are numerous, including lung infections requiring IV intervention, hemoptysis (in which the lungs flood with blood) and pneumothorax. Many face respiratory failure, in which a risky lung transplant is the only option to potentially extend life. These are painful, and I must add, very expensive. ICER’s methodology calculated the financial costs of these heartbreaking experiences and concluded that Trikafta, which can ameliorate
this expensive suffering, was simply not worth it. This conclusion is dehumanizing and beyond comprehension.

In the best of times, it is difficult for patient groups to make their voices heard in ICER’s reports. ICER claims it wants to engage the patient community, but consistently minimizes our voice, despite a tremendous investment of time on our part. In this moment, taking the time to focus on ICER means taking time away from patients when they need us most.

ICER typically has a public meeting, which is the one opportunity patients have to speak in a public forum and make clear whether they, the indicated population, find value in the treatments under question. Due to COVID, ICER postponed its CF public meeting, but still released their evidence report on Trikafta. The report should have been put on hold as well, until patients could be heard in conjunction with its release. This feels calculated, as if ICER is taking advantage of the crisis to silence those who oppose their philosophy and methodology. This includes CF patients, family members, caregivers, and advocacy groups, who at that public meeting would point out that ICER’s economic model is flawed, relying on very questionable quality of life inputs and incomplete economic measurements of CF impacts. It does not consider the non-medical costs of the disease like lost caregiver productivity and CF patients who can have full productive careers if allowed to survive to adulthood. It refuses to consider Trikafta a treatment for a rare disease when CF is considered rare under any federally accepted definition.

ICER’s reports are both flawed and insulting, but my greatest concern with them is that they serve as a tool to block access to life-saving therapies. Payers, who fund ICER, look to these reports in making their coverage decisions, as do state Medicaid programs. This is ICER’s second review of drugs for CF. After the first review, New York’s Medicaid program, relying on ICER’s results, attempted to not cover one of the drugs in question, leaving patients who had been waiting years for an effective treatment to wonder if they could access it. This is not an anomaly. We are seeing more states look to ICER for guidance, from Oklahoma to Massachusetts, which leaves me with deep concern for the future of my daughter and patients like her. In the treatment of CF, every day matters. Delays in access mean decreased lung function. We are already subject to arduous preauthorization policies that cost us valuable time, and ICER’s reports continue to encourage payers to subject us to delays, or worse, noncoverage of the treatment.

As we as a nation grapple with rising healthcare costs and the topic of value continues to surface, I encourage policymakers and payers to talk to the real experts - the patients who suffer from chronic diseases and the providers who treat them daily – to determine what works and what has value, instead of giving any credence to a self-appointed entity like ICER.