31 January 2024

**Regarding LD1829, An Act to Reduce Prescription Drug Costs by Requiring Reference-based Pricing**

Dear Members of the Health Coverage, Insurance, and Financial Services (HCIFS) Committee,

I live in Kittery and have since 1997. Back then we were newly married, one dog, first house purchase, and then, yes, first child born. York Hospital, 1999. We named him Trevor. Exactly 4.5 months later he was diagnosed with cystic fibrosis (CF) and our idyllic world flipped completely. No family history of CF could be found, we were blindsided. When Trevor was born the median lifespan for people with CF was 29. I was 32 when this happened.

CF is characterized by a simple salt and water problem intracellularly which causes a giant body-wide problem. Misfolded protein causes lungs to get mucus laden and infected, pancreases don't secrete enzymes to breakdown and use food, sinuses get loaded with polyps, and on and on. We/he spent hours daily doing breathing treatments and chest percussion to keep mucus from staying, and equally as much time preparing meds and cleaning treatment tools. Daily, with no days off. This was how it was. It was exhausting and expensive—even with insurance. When Trevor was born we had an HMO that capped lifetime insurance payments at $1 million dollars. I would mourn inside knowing that one hospitalization cost a quick $50,000. I would do the math. It was unsustainable.

When the Affordable Care Act was saved in 2010 I could breathe a little bit. That was historic and lifesaving. And the right thing to do.

As you can imagine, his childhood was typical macroscopically—but not microscopically. Many doctor appointments, many hospitalizations, surgeries, bowel issues, sinus issues. Never ending issues and hours and hours of daily treatments only to wind up in the hospital despite doing it all just right. All the time, every day.

We did walks, bake sales, runs, golf events, car washes, poker tournaments, everything we could do to raise money to support the CF Foundation, the driving force behind early stage clinical research. We read and educated ourselves so that we could understand the 'why' behind where the science and research were going. We prayed in our hearts for science to save the day. We followed the research and then prayed some more.

And then the most amazing thing happened. A little company had been chugging along working on correcting the CF cellular protein misfolding that was causing all the CF troubles. And they spent years on this. I first heard mention about it back in 2003 in while in Washington, DC at a CF Foundation leadership event. They referred to it as a 'silver bullet.' It takes *a long time* to bring a drug to market. And it costs *a lot* of money. What this company did has literally been called "The greatest story in medicine." And it is. Because they did do it. They 'fixed' CF with pills. Not treatments that would take hours every day on end. Pills. Correcting the misfolded protein inside the cells. Incredible.

There are now a small handful of medicines developed that have changed the landscape of what it means to "live with cystic fibrosis." Their incredible innovation and talent allowed a rare disease to be redefined. There are people living with CF and *thriving* now. Because of these medicines. It has given patients and their caregivers so much more time TO LIVE!

These medicines, born of decades of research and designed for such a small population, consequently cost a lot of money. But they should. These medicines require high per-patient prices while they're on-patent to incentivize their development. If you cap the prices with upper payment limits you will kneecap the patients who need to access these amazing, life-altering medicines.

I appreciate and can understand why states would be tempted to create PDABs with limits but beg Maine to reconsider. These innovative, cutting-edge medicines are that way for a fixed time. We can't stifle creativity and high-tech drug innovation that will redefine diseases as we know them. No company will willingly enter the Rare Disease realm and front the incredible sums of money it takes to bring a drug to market without being able to make money. And frankly, there is so much nuance in each rare disease space that all a state may see is a price tag and miss the rest of the story. Lifesaving. Life giving. Profoundly so.

I am including a link to an op-ed written by Gunnar Esiason, son of Boomer Esiason. Gunnar has CF. Colorado attempted to put upper price limits in place and realized for the CF population the medicines were cost-effective. He is slightly older than Trevor and he can express what this means more eloquently than I can. But we both mean it.

Gunnar Esiason STAT News Op-ed
URL-- https://www.statnews.com/2023/11/03/trikafta-cystic-fibrosis-price-colorado-prescription-drug-affordability-board/

Sincerely yours,

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