SB841 Public Hearing **25 Oct 2016**

Banking and Insurance Committee, Harrisburg, PA, Senate Hearing Room 1

Good morning. My name is Len Azzarano. I'm here representing the hemophilia community in Pennsylvania, and in these next few minutes I want to share information on 4 important points:

1. **My family**
2. **Hemophilia – What is it? How is it treated? And why these treatments are so different than other medications.**
3. **Cost of treatment**
4. **Outcomes – What is possible for patients with access to safe and effective life-sustaining treatments, and what life can be like without these?**

Family - My wife, Karin, and I are fortunate and proud to be the parents of three *generally* well-behaved boys. Erik is 21 and an excellent student in his third year of college. Nick is 15, a typical high school sophomore, involved in many school activities with his friends. Our youngest son, Kristian, is a 13 year old 8th grader hoping to follow in his brothers' footsteps while maintaining his clearly individual spirit. We are perfectly imperfect. We laugh and cry, we fight and forgive; we work hard but feel like we don't accomplish enough. We love each other and are a "normal" family in many ways, whatever that means. But in at least one way we are very different. Nick and Kristian both have severe hemophilia, specifically Factor VIII deficiency, also known as hemophilia A. Their bodies produce little or none of a key component just about everyone else's does.

Hemophilia - So our kids have it. So what? You may have learned about hemophilia in your high school biology class. It's a classic and fairly easily understood example of a recessive genetic trait. Members of some of the European royal families were afflicted with it in the 19th and early 20th centuries. Scientifically, hemophilia is quite interesting. It is a rare, often inherited disease, sometimes plaguing families for generations. But it can also occur from a spontaneous genetic mutation, with no family history at all. The Factor VIII my boys are missing is only one component of a process called the blood clotting cascade. Imagine a set of dominoes set up to fall in a line. The first one is knocked over into the second, which triggers the third to fall and so on until the last one falls and the process is complete. Clotting factors work in a similar fashion and the result of a missing factor for patients with hemophilia is that their blood doesn't clot like blood does for the rest of us. Or when it does clot, that clot may not last long enough to finish the job it was meant to do, helping an injured body to begin the healing process. People with hemophilia don't bleed faster; they bleed longer, sometimes much, much longer. Without treatment, the bleeding from an injury or even from just a spontaneous bleed can be prolonged and dangerous when they occur in a joint or a muscle, and ultimately can be life-threatening when they take place in the abdomen or even the brain. Although hemophilia patients often share many similar challenges and concerns, the disease can manifest itself in multiple ways because clotting factors other than number VIII can be deficient. However, hemophilia is still a rare disease. Only about 3,000 people in Pennsylvania and about 20,000 total in the United States live with hemophilia. As I explained to our boys, that number is only about half the crowd at <u>one</u> Phillies game in Citizens Bank Park when the

games were consistently selling out a few years back. This disease is so rare that most medical professionals often go their entire career and never treat someone with hemophilia.

What you didn't hear in biology class is the impact hemophilia can and does have on patients and their families. The day our Nickolas was born was a great one for us, one of the best. Our wait was over. He was finally here and seemed perfect in every way. The next day was one of our worst. Karin and I were devastated to learn he had hemophilia like his uncle. What made that knowledge so frightening was the fact that my brother-in-law, Nick's Uncle Erik, had died five years earlier as a result of his own struggle with hemophilia and subsequently AIDS, contracted as a result of receiving supplemental clotting Factor VIII contaminated with HIV. Our pediatrician told us to go home, to enjoy our son and not to worry, because so much had changed. New, safer and more effective treatments were available and we would find a very different situation than Karin's family had suffered through for so long. What a relief.

New treatment products eliminated many of the dangers faced during the previous decades. These were more effective and often didn't require patients, and their families, to spend so much time in the hospital. Dedicated hemophilia treatment centers were able to focus not only on the medical needs of patients, but in partnership with organizations like the National Hemophilia Foundation and local chapters like ours, were also able to support many of the physical, social and emotional challenges our community faced. Factor could be stored at home, patients could learn to treat themselves and could avoid many of the missed days of school and work my brother-in-law experienced as a boy and young man. Truly an amazing improvement. Still there are things you need to know. These medications are great but not perfect. As with the older products, factor replacement therapy must be administered as an intravenous infusion because it can't be absorbed into the body by any other route and generally it doesn't last very long. Depending on the severity of each patient's condition, treatment is usually required every 2 or 3 days to maintain enough factor in the body to guard against bleeds. In addition, no single product or dosage of these medications will work for every hemophilia patient. The product, the frequency of treatment, and the required dosage must be personalized for each. And since these are biologics, there are no generic or low-cost options for medications of this type. This takes us to my third point.

Cost - Factor replacement therapies are <u>expensive</u>. For each of our boys, the current treatment plan is to infuse their factor 3 times each week, every week. For their body weights, each treatment costs about $4000, approximately $12000/week for each of our sons, and well over a million dollars a year combined. This assumes no injuries, no medical procedures, and no breakthrough bleeds which could require treatments in addition to their regular schedule. And these things do happen.

I have heard proposed cost-sharing for medications in the specialty tiers could be in the range of 25 to 50%. These numbers are truly frightening to people in the hemophilia community. For my family, we meet our out-of-pocket insurance maximum in January or February. Imagine being burdened with a cost of 300 to 600 thousand dollars, every year! Families can't bear this. It's just not feasible. Instead, we need a cap to this cost and not a percentage. We need a dollar amount, and it must be a reasonable amount.

Outcomes - So what do you see when people with hemophilia, young gentlemen like our boys, have consistent access to safe and effective medications like the factor replacement products of today? You see a kid who goes to school, pretty much every day. They don't miss class because of a bleed and the time needed to recover from one. They're able to keep pace with their peers and even excel at academics like our Nick does. You see boys who build great friendships and can participate in athletics without fear. Staying physically fit is just as crucial for people with hemophilia as consistent access to their medication. Healthy bodies foster healthy joints which can reduce bleeds and ultimately reduce medical care and overall cost. Our boys play baseball, volleyball, basketball and even soccer. Nick was a relief pitcher for his grade school baseball team, and Kristian plays goalie for one of his 2 soccer teams. These healthy young men can go to college or a vocational school, compete both academically and athletically, enter the workforce in a career of their choosing, pay taxes, and essentially have a life so many parents want for their children. Specialty tier cost-sharing puts all of this in jeopardy. When patients are forced to choose between the high cost of their medication versus life's other necessities such as food, car payments and rent or mortgages, some will reduce the amount of medication they take to save money, or even stop treating themselves altogether because they just can't afford it. They can then experience more frequent and often more severe bleeding episodes, disrupting life with lengthy and costly hospital stays, placing additional stress on relationships and impacting their ability to hold a job.

My brother-in-law, Erik, died when he was 27. He was a character, an amazing guy, and we're so sorry our boys never had the chance to know their uncle. One of the things that always bothered Erik was that he could never keep a job, even part-time. His body just wouldn't allow it. In Erik's youth, treatment for hemophilia was more responsive than preventative. Because of this he experienced many, many joint bleeds as a child, which limited his ability to be the student he should have been and to stay focused and active for long periods as an adult. The implementation of specialty tier cost-sharing risks taking our patient community back to those days. You can see now that financial burden would start a cascade of sorts, one that ends very sadly, and so it is not an acceptable option. Today, I know many young, and not-so-young, adults with hemophilia. They are professionals, craftsmen, business managers and owners. Some have families. And they lead productive, rewarding lives. My wife and I want the same opportunities for our boys and for all the patients and families in our community.

I'll close with a visual. This is a photo of my family; a family who has consistent access to safe and effective treatment and lives with hemophilia every day, and we are enjoying a truly special time together. We hiked over 5 miles that morning and climbed to an elevation of over 10,000 feet. "Best day ever!" Kristian told us. I agree completely. There were lots of other families on the trail that day. I don't know if anyone else we saw lives with hemophilia, but they certainly couldn't tell that we do, and that's how we want our boys and all of our community to feel.

Thanks for listening. I truly appreciate your time.

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