Hello,

I'm writing today with concerns regarding bills SF 168 and HF 17. I agree too many patients have difficulty affording their prescription medications, however, **an appointed board of political appointees having the authority to set an upper payment limit is concerning. Mainly, who might save money and who might lose access?**

My son Andrew has PKS or Pallister KIllian Syndrome. PKS occurs in 1:15.4 million in the world. Andrew is 1 of 2 in the state of MN. We spent nearly 5 years thinking our son had a syndrome called Beckwith Wiedemann Syndrome. At a conference in Boston 2007, we showed Dr. Beckwith pictures and a stack of files to prove we had this diagnosis. Andrew was not walking or talking at 4 1/2 years old, and it was clear Andrew's case was much more severe than the other children. Dr. Beckwith encouraged us to have our Geneticist continue looking for an additional diagnosis.

By luck, our Geneticist showed Andrew's picture to a colleague who remembered a familiar face – from a textbook – of someone with PKS. Only because of a clinical similarity did they test for PKS and our puzzle had been solved.

However, PKS is so rare, most doctors had to be educated about this syndrome and we often found ourselves to be the most current source of information and research. Rare has meant a lack of information, lack of advocacy, lack of opportunity, funding, direction, and hope.

PKS is a mosiac, meaning PKS is not in every cell. We have never been able to treat "PKS" but we do our best in daily care and sadly have had to react to crisis and tend to learn more how PKS affects our son during these times. We approached Andrew's care by treating his symptoms.

Initially, Andrew underwent a bilateral femoral osteotomy, a procedure that typically secures a promising future of mobility, but a year later one femur re-fractured with others to follow. There would be four additional re-fractures and bone infections over the course of five years.

During these years of deterioration, Andrew's spine was collapsing. He had lost any ability to sit, stand, walk or transfer. By the time Andrew was 14, we had moved into Palliative Care and our main goal daily was to keep him comfortable. He would spend up to four hours a day sleeping at school or hanging over his wheelchair to decompress the pain from his 110 S curve spine with a 100 degree kyphosis. Our son was in desperate need of a miracle.

Eventually, an Endocrinologist suggested an IV medication specifically made for Fragile Bone Syndrome. Although Andrew had tested negative and there was no previous indication for PKS, this became Andrew's life-changing treatment. After two years of

infusions and no new fractures, the doctor's felt he was ready to undergo a complex spine surgery that was our only hope to stop this ravaging decline to Andrew's health.

Not only was the surgery a success, but our son showed signs of pain relief. He was able to bear weight and started moving in his gait trainer. He would sit on the seat of his gait trainer and stand on his own exploring the room he had not had any interest in six years prior. He was happy, healing and thriving! We had our lives back!!!

Andrew's life doesn't look like yours or mine. His "walking" is in this large contraption where movement forward is considered walking, but his happiness doesn't involve perfection. It is about giving him a chance.

What if his doctor had not suggested that medication due to the fact the UPL reimbursement was below the acquisition price? What if their motives were completely reliant on the fiscal line? How will we ever have drugs recommended and available to our rare cases if these laws are imposed?

I have dedicated my life to combating the challenges this syndrome bestows upon our son and family. While the goal of this bill is worthy, **I worry SF 168 could prevent access to today's treatments especially for those with rare diseases**. I'll leave you with a picture of our family vacation last spring that was taken in celebration of LIVING! It was always a dream to take my family to the all-inclusive dreamy resorts we watched other "normal" families so often do when we often lived an isolated life due to Andrew's complications. Andrew lived his best life that week and enjoyed virgin pina coladas and cheeseburgers pool side. We saw a miracle unfold thanks to this medication our doctor by chance offered to us to strengthen Andrew's bones. **This miracle was only made possible due to Andrew's access to medications and surgery**. After 17 years, we enjoyed our first trip to paradise as a family of five. Please don't take away the opportunity for other Minnesota families to create similar memories.

Humbly,

Nicole Schmidt
President | Founder of Project 12-04
763-482-9284
www.project1204.com
www.operationxmasjammies.com
info@project1204.com