Testimony: Right to Try for Individualized Treatments in Kansas

Thank you, Senators, for allowing me to testify today in support of Kansas Senate Bill 250 - Right to Try for Individualized Treatments. This is a very important bill.

My name is **Rich DeAugustinis**. I am the vice chair for The Myositis Association and previously served on the board of the Mesothelioma Foundation.

Given my personal journey and my nonprofit involvements, I am very qualified to authoritatively speak on this issue.

I am testifying today on behalf of my father Augie and my late wife Tara. One is living with a rare disease, and one had her life shortened by one.

My father Augie was diagnosed with Inclusion Body Myositis in 2008. IBM, as it's called, is a rare neurodegenerative disease that slowly wastes away all of the muscles in your body.

My dad is 17 years in, and life for him is now in a wheelchair full time. He can't do much for himself without help. In 2014 he participated in a clinical trial of a gene therapy that promised to **rebuild** the muscle tissue in his legs. It was a phase 1 trial, which as you know is focused on safety, not efficacy.

Miraculously, it was successful and built muscle in my father's legs. He was able to walk for a few more years. Unfortunately, the researchers could not find financial support for a phase 2 trial and my father couldn't continue to benefit from the gene therapy.

Had this legislation been law at that time, **it would have cleared a path for my dad to continue utilizing this effective treatment**. It likely would have delayed the further breakdown of his muscles, and extended quality of life for him.

This legislation also could have helped my late wife Tara. She was diagnosed with mesothelioma in early 2016. Meso is a very rare cancer of the lining of the lung, caused by exposure to asbestos. She fought valiantly for 15 months but passed in 2017 at the age of 47, leaving behind me and our 15-year-old daughter.

Meso is considered one of the **deadliest** cancers in the world. Generally, if you are diagnosed with meso, it's a death sentence. It's not a question of if, but how fast.

Despite that, there are a growing range of experimental treatments that are showing promise with mesothelioma, treatments like CAR-T cell therapy and immunotherapy.

Broader access these treatments and others might just turn mesothelioma into a **chronic disease that can be managed**, rather than a very painful death sentence.

Simply put, this legislation would have given Tara a chance — and just as importantly given her a chance to try some of these experimental treatments.

Senators, there are many people in the great state of Kansas struggling with rare diseases — very likely in **every single one of your districts**. They need new options to improve their quality of life and to extend their lives. **They need HOPE.**

There are treatments are out there that Kansas residents can't access. … Effective treatments that are available in other countries or that could be in clinical trials here in the US.

While this bill won't address every single situation, it will open the door to a lot more opportunities for access to care. **It will open the door to HOPE.**

Please support this bill and let's make it law during this legislative session. Do it for Augie and Tara, and countless others in your districts that need it. Thank you.

Rich DeAugustinis
Vice Chair of the Board, The Myositis Association
deaugustinis@myositis.org
404-547-8153