COMMENTARY

Families with incurable disease relying on RI's policymakers

Your Turn

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Guest columnists

For all parents, time goes too quickly. It often feels like you just put your kindergartener on the bus for the first day of school, and next thing you know, they're off to college.

For parents of a child with an incurable disease, the feeling of fleeting time is amplified in a way most of us can't comprehend. Every day with that child is a gift, and also in many cases, a very tough challenge.

Listening to these families' stories, there's one common denominator that keeps them going – hope. Hope for a new treatment, hope for a cure, hope for a better, longer life for their child.

And as intangible as hope can be, hope for these families is rooted in the most tangible of things – science.

Scientific advancements and medical breakthroughs are helping those living with some of the most devastating diseases, including one that was the focus of a recent event for Rhode Island policymakers and their staffs – Duchenne Muscular Dystrophy.

DMD is a genetic disorder that is characterized by a progressive loss of muscle. The disease primarily affects boys; one in 5,000 will be born with Duchene. Those with DMD lose the ability to walk, and the life expectancy of a person with DMD is 20 to 30 years old.

Not long ago life expectancy was closer to late teens, so we're making progress, but we have a long way to go.

That is why recently our two organizations came together to raise awareness of the ongoing need for scientific innovation and breakthroughs, and to urge support of the continued good work happening within Rhode Island's biotech sector.

More than 400 biotech companies and organizations call Rhode Island home. Within striking distance of similar science-based hubs in Boston/Cambridge, New Haven and New York, along with prestigious academic Institutions, the biotech industry is thriving here.

Some of the very research that the families living with DMD are counting on is happening right here in Rhode Island.

Though the origin of the genetic cause at the root of DMD have been known for many years, only recently with advancements in gene editing and other new technologies is there renewed hope for these patients. Clinical trials are ongoing for multiple gene editing treatments for DMD. The goal of these gene editing strategies is to replace the missing dystrophin protein that occurs in DMD patients.

And while this new era of science is exciting, our discussion also included the need for lawmakers and public health policy leaders as well as industry and patient advocates to come together, to ensure equitable and affordable access to new treatments when they are ultimately available.

Bringing these innovations to the patients is a long and expensive path. Lawmakers must bear that in mind when setting public health policies and looking at proposals that could put innovation in jeopardy.

On behalf of both of our organizations, we are dedicated to continuing the conversation about the amazing work that researchers and scientist do each day, the hope it gives patients and the obligation for policymakers to support pro-science and pro-patient legislation.

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