Family affected by fatal illness supports UMMS study testing efficacy and safety of a “Trojan Horse” therapeutic

The deadly odds are 50-50: If a parent has Huntington’s disease (HD), then each of his or her children has a 50-50 chance of developing this fatal disease. For many years, Michael Berman’s family thought they had beaten the odds.

Jocelyn Topper, Michael’s life-partner, lost her father to Huntington’s. She had been tested after an automobile accident, she learned otherwise. After a battery of more sophisticated tests following the gene that causes the disease. Then in 2014, he was diagnosed and was told she did not carry the gene. This meant neither did her children. The odds went to 50-50: If a parent has Huntington’s, then each of his children has a 50-50 chance of developing the disease.

“Huntington’s became the focus of my life,” he said. “I wanted to learn everything I could about the disease, get the lay of the land for ongoing research, and see how we could make a contribution to finding a cure.”

Beginning with Jocelyn’s medical team in the couple’s home state of California, Berman reached out to HD experts across the country seeking information and guidance. That led him to Neil Aronin, MD, co-director of the Neurotherapeutics Institute at UMass Medical School.

“Michael called me, and I could hear the pain in his voice,” Dr. Aronin said. “I knew just 40 minutes on the phone wasn’t going to be enough to help him, so I agreed to meet with him and his family.”

Over the course of three days, Aronin, who is also a professor of medicine, cell & developmental biology, and microbiology and physiological systems at UMMS, delivered an intensive tutorial on human genetics and the disease. He explained how genes coded in DNA send messenger-RNAs to direct protein production in cells. People inherit two copies of most genes—one from each parent—and if one copy of the Huntington gene is mutated, it sends errant messenger-RNAs that cause the mutant Huntington protein, which is the hallmark of the disease.

Aronin also explained that much remains unknown about how the mutant Huntington protein actually damages nerve cells. So the focus of the Aronin lab is to go “upstream” and target the defective gene rather than the already damaged nerve cells. The focus of the Aronin lab is to go “upstream” and target the defective gene itself to try to “knock down” production of the errant messenger-RNAs and toxic proteins, thereby preventing nerve cell damage.

“Trojan Horse” therapeutic designed to infiltrate nerve cells in the brain and release an RNA-based construct that will reduce production of the mutant Huntington protein.

“We are so grateful for Michael’s generous philanthropic contribution and the backing of his family,” Aronin said. “Private support like this is critical for the implementation of essential early-stage studies such as the ‘Trojan Horse’ project. If it’s successful, then we can take the idea to the next level...”

Berman subsequently traveled to Worcester to meet with Aronin and colleagues from the UMMS Neurotherapeutics Institute. Impressed with their work, Berman decided to support a significant new project at UMMS to test the efficacy and safety of a “Trojan Horse” therapeutic designed to infiltrate nerve cells in the brain and release an RNA-based construct that will reduce production of the Huntington protein.

“I spent a career making decisions based on a bunch of facts and on the quality of people involved. I believe I know a good thing when I see one. What’s happening with Neil and the team at UMass Medical School is extraordinarily exciting,” Berman said.

Back home, Berman said his family is adjusting to their new reality. Jocelyn is doing well and has no HD symptoms. Her children are getting counseling to grapple with the possibility of HD arising in the years to come. They all remain hopeful that supporting the HD research at UMMS will accelerate development of a cure, not just for their family, but for everyone affected by the disease.

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“Trojan Horse” therapeutic
UMMS study testing efficacy and safety of Huntington’s disease

Family affected by fatal illness supports UMMS study testing efficacy and safety of a “Trojan Horse” therapeutic

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A retired CEO of a major investment bank, Berman understood how to approach complex issues and focus on reasonable solutions—and knew he would be drawing on these skills to address this sudden and vital new priority.

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