PUTTING ALS ON ICE

Local researchers take up a challenge in order to fund work that may have broader implications.

BY KIM KISER

On a warm August afternoon, Ezgi Tiryaki, M.D., did what so many others did over the summer: got doused with a bucket of ice water to raise awareness of amyotrophic lateral sclerosis (ALS), a disease that affects two to four out of every 100,000 people.

Wearing a red T-shirt with the words "ALS Sucks" on it, Tiryaki, who is medical director of Hennepin County Medical Center's (HCMC) ALS Center for Excellence, was part of a group from the center that answered a challenge issued by the local chapter of the ALS Association. After tipping their buckets and making a donation to the association, they went on to challenge others, including their colleagues at the Minneapolis VA Medical Center, a group from the University of St. Thomas, Minnesota Public Radio’s Cathy Wurzer and the Hennepin Health Systems Foundation Board.

Tiryaki and her colleagues became part of the biggest social media event of the year: the ALS Ice Bucket Challenge. Although it’s uncertain exactly how it began, some credit the friends and family of 29-year-old Pete Frates, a former Boston College baseball star who was diagnosed at age 27 and is now paralyzed by the motor neuron disease (also called Lou Gehrig’s disease), with making it go viral over the summer.

According to the national ALS Association’s website, the challenge raised more than $100 million in the month of August alone. “From a dollar perspective, it shattered everyone's expectations,” says Jennifer Hjelle, executive director for the ALS Association’s Minnesota/North Dakota/South Dakota chapter.

Hjelle says the local office received about 10 online donations in July and more than 1,500 in August. “We’ve seen a tremendous influx of support,” she says.

Now comes the question of how to use that money. Hjelle says the national organization has convened representatives from the local chapters to help decide. She says support at the local level will help fund programs that loan durable medical equipment and assistive devices to people with the disease. “Both programs have waiting lists,” she says. Hjelle also would like to see more go toward research.
An orphan disease

About 30,000 people in the United States and 350 in Minnesota are living with ALS at any one time. “The number doesn't change,” Hjelle says, “because every week two people are diagnosed and two people die.”

Because it affects so few people, ALS is considered a rare or “orphan” disease. “Research has been underfunded,” Tiryaki says. So far, only one drug is approved for its treatment, riluzole. Riluzole delays the disease's progression by about three months, on average.

Tiryaki is involved in the Northeast ALS consortium, which includes researchers from the United States, Ireland and Israel. Faculty from HCMC, the Minneapolis VA Medical Center and the University of Minnesota have joined forces as the Twin Cities ALS Research Group. "ALS is a disease where people have to work together,” she says. "We can't compete.”

Local researchers are currently taking part in a trial of Nuedexta, an FDA-approved drug that’s used to treat pseudobulbar affect, pathological laughing and crying that can occur in conjunction with a number of neurological conditions. She says they are testing it to see if it also helps stabilize speech and swallowing function in people with ALS.

They’re also doing imaging trials. “We're trying to see if we can find a signature of ALS on imaging,” Tiryaki says. Diagnosing ALS is difficult because there is no specific test for it. “One of the theories around ALS is that people lose about 70 to 80 percent of their motor neurons before they have their first symptom. We're wired in a way that we can make up for losses for a long period of time, so it stays under the radar for quite a while. One of the frustrations is we don’t recognize the disease and start treating it until it’s quite progressed”

Tiryaki explains that when people do start noticing symptoms, it usually takes about a year for them to be diagnosed, as those symptoms are often first mistaken for other conditions.

Greater good

Tiryaki believes that ALS research could help people with other neurodegenerative diseases including Parkinson’s and Alzheimer’s disease. “What’s happened over the last five to 10 years is that people have started to realize that the last frontier in medicine is keeping dying cells alive.” She says the money raised by the ice bucket challenge could support research into understanding how to preserve—and even reverse—the death of motor neurons and other nerve cells.

“The biggest thing this has done is offer hope,” Hjelle says. “You can't put a price tag on that.”

Kim Kiser is an editor of Minnesota Medicine.