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As he lay dying: FDA and Huntington’s disease families meet to ponder potential treatments

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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GENE VERITAS

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On Tuesday, September 22, when the U.S. Food and Drug Administration (FDA) focuses on Huntington's disease drug development at a meeting with affected individuals and advocates, HD families must drive home the sorrowful truth: people are dying because of the lack of effective treatments.

About two weeks ago, shortly after pouring out my heart about my family's HD struggles on the FDA's pre-meeting questionnaire, I received a message from the mother of 18-year-old Terry Leach of San Diego: "It's Terry's final days if you wanted to say good-bye."

A couple days later, on Labor Day, I visited Terry, who suffers from juvenile HD, in his bedroom. As Terry slept, his mother Angela and I looked on. Next to us a home healthcare worker prepared a can of liquid food to be administered via a feeding tube attached to Terry's abdomen. Hospice workers are also helping.

"He grew a lot," I said to Angela, amazed at how, despite the particularly cruel toll of juvenile HD, his body had strived to develop. It had been more than a year since I had last seen Terry.

"Yes, he did," Angela said.

I noticed Terry's beard and his healthy head of somewhat wiry, dark brown hair.

"You have a very handsome son," I continued.

"Thank you," Angela said.

HD Links

Huntington's Disease Society of America
International Huntington Association
Huntington's Disease Drug Works
Huntington's Disease Lighthouse
Hereditary Disease Foundation
Huntington's Disease Advocacy Center
Thomas Cellini Huntington's Foundation
HDSA Orange County (CA) Affiliate
HD Free with PGD!
Stanford HOPES
Earth Source CoQ10, Inc.

HD Blogs and Individuals

Chris Furbee: Huntingtons Dance
Angela F.: Surviving Huntington's?
Heather's Huntington's Disease Page

Terry Leach resting at home (family photo)

Unspeakable pain

As Angela nervously shifted her balance from foot to foot, I sensed that she continued to carry the unspeakable burden that comes with juvenile HD: Terry's first symptoms as an infant, his need for a full-time aide in school after losing his ability to walk and talk, the insertion of the feeding tube in 2010, Botox injections into his arms and legs in recent years to relieve pain, and leg and foot operations, among other procedures and hospitalizations.

Although Terry still attended school last academic year and also the summer session, he has declined considerably in the last few months, Angela said.

The night before my visit, he vomited after receiving liquid food through his tube. On the day I visited, the aide would give him only one can instead of the usual two, noting that his body would not accept anything more than that very small amount.

I asked Angela if I could touch Terry.

"Sure," she said softly.

I ran my hand along the top of his hair. I remembered Angela and her family's steadfastness in caring for Terry. I also recalled fondly our collaboration in the cause, starting with an article I wrote about them in 2009. In 2012, Terry emerged as "SuperTerry" in an artist's comic-book like rendition as a hero defeating HD. In 2014, our families participated in the 2014 Team Hope Walk of the San Diego Chapter of the Huntington's Disease Society of America.

SuperTerry in San Diego artist Lee Ellingson's rendition (above) and with Gene Veritas (aka Kenneth P. Serbin) at the 2014 Team Hope Walk (below, photo by Misty Oto)

Seeking feedback from the community

As Terry lies dying, the September 22 FDA event, a public meeting on "patient-focused drug development," will seek feedback from affected individuals and others in the HD community.

The meeting will occur from 9 a.m.-12:30 p.m. at the FDA's White Oak Campus in Silver Spring, MD. Pre-registration for attending the meeting and viewing via webcast is closed, but the FDA will make available a video of the proceedings shortly thereafter.

The meeting stems from the reauthorization of the Prescription Drug User Fee Act in 2012, in which Congress required the FDA to more

systematically solicit input from patient communities with regard to drug development. The FDA hopes this will help its review process.

Huntington's disease became one of just 20 diseases selected by the FDA for a patient-focused meeting through the end of 2015.

'Nothing for neurological disorders'

Responding to the preparatory questionnaire, I revealed my situation as a carrier of the HD genetic defect and my mother's decline and death from HD in 2006.

"I fear that I will become like my mother," I wrote. "She had mild chorea [involuntary movements associated with HD]. I would not mind having chorea as long as I can continue to be myself, work, and not become a burden on my wife and daughter. My mother became a shadow of herself. I have great anxiety about losing my personality and ability to work as a college professor and writer."

Like others, I would like to see a treatment that prevented symptoms, I added.

"If I get symptoms, then I would like a medication that allows me to manage the disease just as other diseases such as diabetes are managed without affecting a person's livelihood, family life, or activities in general," I continued. "There are lots of advances in cancer treatments, for instance, but really nothing in the field of neurological disorders that prevents, halts, or reverses the condition."

I uploaded my response to the FDA's public docket regarding the meeting. Anybody can comment at that link through November 23, 2015.

Speeding up clinical trials

At the meeting, I also plan to urge FDA officials to allow researchers and clinical trial administrators to use new technologies to measure the effect of medicines.

All clinical trials of new drugs taking place in the U.S. must receive the approval of the FDA, considered to have the world's most rigorous standards. Although the drug industry executives I have met recognize the importance of that rigor in assuring the manufacture of safe and effective drugs, they sometimes have also expressed the need for the FDA to be more flexible and allow for faster clinical trials.

Generally, the FDA still does not accept techniques such as brain scans. HD researchers and other scientists are vigorously searching for biomarkers – signs of disease and drug effectiveness – in the blood, cerebrospinal fluid, and other materials taken from the body that can be measured using the scans and other new techniques.

Instead of waiting for a doctor's clinical observation of an improvement, these techniques could potentially allow faster and earlier reading of a drug's effectiveness.

I will stress that the FDA work closely with scientists and the HD community to make clinical trials as efficient and meaningful as possible.

Speed is of the essence for the HD community.

Heartbroken by another loss

As of this writing, Terry is stable, but his prognosis is not positive.

To give me strength as I travel to Maryland on September 21 and take part in the meeting the next day, I will keep fresh my memory of Terry.

I am heartbroken by yet another loss to HD.

As an advocate, I feel I have failed to fulfill the promise of hope presented so often to families such as the Leaches. No 18-year-old should die.

I am comforted to know that Terry is in loving hands – and that he never gave up, always smiling that infectious smile. And I am committed to making sure policymakers know of the people whose lives they could improve and save.

Terry in 2010 as a Hero of the Carlsbad Marathon

Posted by Gene Veritas at 6:45 AM

Labels: advocates , brain scan , clinical trials , drug development , FDA , feeding tube , Huntington's disease , patients , researcher , scientists , symptoms , Terry Leach

2 comments:

Charles said...

YEAH! Terry! Keep Up The GOOD Fight!

Chop-n-Moe

8:13 PM, September 18, 2015

jakby said...

Hi, have you maybe tried Triheptanoin? it is synthetic oil described here: http://en.hdbuzz.net/185 I wonder how to get it.

10:35 AM, September 19, 2015

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