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A key Huntington's disease trial remedy gets Orphan Drug Designation, as yet another young life is cut short

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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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FRIDAY, JANUARY 08, 2016

A key Huntington's disease trial remedy gets Orphan Drug Designation, as yet another young life is cut short

Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals) has achieved another milestone in its search for a treatment for Huntington's disease: on January 5 the company announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for its gene-silencing drug, currently under study in a clinical trial in Europe and Canada.

The FDA designation, intended to facilitate development of the test drug IONIS-HTT_{Rx} by offering financial incentives and assistance, could not come at a better time. Huntington's disease patients – like 18-year-old Terry Leach of San Diego, who died the morning of January 2 – continue to succumb to this devastating, untreatable disorder.

"Although the toxic protein produced from the huntingtin (HTT) gene in HD patients has been a target of interest for many years, IONIS-HTT_{Rx} is the first therapy to enter clinical development that is designed to treat the underlying cause of this fatal disease," Frank Bennett, Ph.D., Ionis's senior vice president of research, said in a company press release. "The granting of Orphan Drug Designation in both the U.S. and Europe highlights the significant need for a drug that could transform the treatment of HD."

HD-affected Phase I clinical trial volunteers in London received the first dosing of IONIS-HTT_{Rx} in the October 2015 (click here to read more). IONIS-HTTRx could potentially reduce, partly reverse, and even prevent symptoms.

Likely ending in 2017, Phase I is testing primarily for safety and tolerability. If it is successful, Phase II and III trials measuring the drug efficacy's would ensue. Together the three phases of a trial typically take at least five years. If the trial is successful, a drug could become available around 2020.

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Frank Bennett, Ph.D. (photo by Dr. Ed Wild)

Increased dialogue, helpful benefits

"We were pretty excited to get Orphan Drug Designation," Kristina Bowyer, Ionis's executive director of patient advocacy, said in a phone interview on January 5. Ionis is based in Carlsbad, CA.

The designation means that the FDA recognizes "the severity of the disease and the limited population," she noted.

The designation creates an opportunity for increased dialogue between Ionis and the FDA regarding the IONIS-HTT_{Rx} clinical trial, Bowyer explained.

Such extra communication can help resolve the unique issues of orphan disease trials. Because an orphan disease like HD involves fewer than 200,000 patients and very specific approaches to treatments, the design of clinical trials is atypical, Bowyer said. The smaller number of potential volunteers also means that the FDA might have to approve a smaller than normal trial, and sometimes perhaps even a faster trial, she added.

"It's an area where our technology is well-suited," Bowyer continued, referring to the antisense oligonucleotides that form the backbone of all Ionis drugs. "We have been able to focus on several rare diseases with a known target."

By law, as outlined in the press release and in an e-mail from Bowyer, the Orphan Drug Designation includes significant financial benefits for Ionis: seven years of market exclusivity in the U.S. if the FDA approves the drug, tax credits related to clinical trial expenses, FDA assistance in clinical trial design, and a waiver of Prescription Drug User Fee Act filing fees – over $1 million per drug as of fiscal year 2009.

"These benefits help manufacturers recover the costs of developing a drug for small numbers of people," Bowyer wrote. The Orphan Drug Act was signed into law in 1983.

Kristina Bowyer (photo by Gene Veritas)

Trial 'moving along well'

In IONIS-HTT_{Rx}, HTT stands for the gene huntingtin, and Rx for medical treatment. The train has just recently begun, so, Ionis has reported no official update at this time.

"Everything is moving along very well," Bowyer said.

The new name

Ionis has also adapted well to its name change, announced on December 18, 2015, in response to concerns about confusing the name Isis Pharmaceuticals, Inc., with the acronym "ISIS" used in the English-language media for the Middle Eastern terrorist organization, the Islamic State. Isis Pharmaceuticals was founded in 1989.

"We want people when they hear or say our name to think about the incredible drugs we're developing and not a terrorist group," Wade Walke, Ph.D., vice president of communications and investor relations, told the press.

Ionis chose its new name based on employee suggestions.

"It seemed to me that everybody came together and decided that Ionis was a nice-sounding, feeling name, as soon as someone hit on it," said Stanley Crooke, M.D., Ph.D., Ionis's chairman of the board and CEO. The new moniker is a so-called empty vessel name and has no inherent meaning other than what the company does, he added.

Said Dr. Crooke: "We're here for the patients. We're not here for our name."

(Disclosure: I hold a symbolic amount of Ionis shares.)

The Ionis logo

Too late for Terry, other 'HD angels'

I remember that Dr. Crooke spoke the same phrase – "We're here for the patients" – when I met him briefly during one of my first visits to the company in the late 2000s.

I also remember visiting Terry Leach on Labor Day 2015. He was slowly but inexorably slipping away from the ravages of juvenile HD, a particularly devastating form of the disease (click here to read more about my visit).

Nevertheless, I was still shocked by his death on January 2.

What a horrible time to lose a family member. New Year's will forever remind Terry's mother Angela and his siblings of his passing.

My immediate reaction that morning was one of intense anger.

No 18-year-old should die!

Having had the privilege of knowing Terry and his family, I felt that I had failed as an advocate to speed the progress towards treatments.

Why hadn't the Ionis trial come in time to save Terry and the other "Huntington's disease angels" who have passed in recent weeks?

I know that HD researchers may have similar thoughts, as they work with the specter of this killer disease ever looming.

Terry Leach (family photo)

Keeping the faith

Later in the day, knowing that I needed to transcend my anger and sadness, I recalled that a new year always brings hope. As I took a long, strenuous walk with my dog through our hilly neighborhood, I renewed my resolve to fight HD in 2016.

I spoke to Angela a few times that weekend. Instead of a wake and church service, the family will hold a remembrance for Terry at the family home on January 16. A Christian minister will preside.

At Angela's request, I wrote some words for the back of the remembrance cards she's having made: "With his infectious smile and fortitude, Terry set an example for all to follow. His life was short, but full of love and joy. He is now free to walk with the Lord."

Understandably, Angela was too drained to talk much. She did confirm that Terry – despite his inability to talk, his confinement to a wheelchair, and years of ingesting food through a feeding tube – had achieved his high school diploma. He had attended school through mid-2015, receiving assistance in a program for the disabled.

I asked Angela if she had any words for the HD community.

She said: "Just to keep their faith."

Angela Leach in 2012 holding artist Lee Ellingson's drawing of her son Terry as "SuperTerry," the superhero who knocks out Huntington's disease (photo by Gene Veritas)

Posted by Gene Veritas at 12:40 PM

Labels: clinical trials , drug , FDA , gene-silencing , huntingtin , Huntington's disease , Ionis Pharmaceuticals , IONIS-HTT-Rx , Isis Pharmaceuticals , orphan disease , Orphan Drug Desgination , symptoms , Terry Leach , treatments

2 comments:

Anonymous said...

Very interesting blog. Together with the family we look forward to a new drug that will free us from this disease.

Greetings from Poland!

6:28 AM, January 09, 2016

Anonymous said...

Gene your contributions towards encouragement and research in the HD field are great.

What horrifys me is the uneeded suffering of ones LIKE Terry(don't know his history)! Excluding a limited number of those born post the 90s where parents truly had no possible knowledge of HD in their family or a late diagnosed , unplanned pregnancy there ought not be hardly any one born positive or at risk for the HD gene.In 1993 the gene test for HD had been found.

While an individual has a right to be too afraid too find out

their at risk status, it's unethical with recent options to procreate children with this HD gene risk. Just think if an at risk person finds just sustaining awareness of the disease affecting themselves how can such dare risking a child becoming a victim of this disease?

What about promoting the reduction of this disease via explaining the various levels of gene testing re embryos,pre-implantation etc avoiding divulging parent's at risk status for the extra afraid, suggesting no children or adoption or donor sperm/eggs for super afraid and explaining the benefits of finding out ones at risk status for the understandably anxious though more open minded.

The other facts are that research has areas where pre symtomatic at risk persons, the earlier the better can be essesntial to furthering potential treatments. Furthermore, the reality is that any treatments that may become relatively effective will be so applied as early as possible as much of the time more established damage is generally resistant to being undone to any great extent.

HD will never have a true cure.Why?
Because no fetus, infant or unsymptomatic child who is at risk may ever are identified to apply any most effective treatment as the differences of HD gene positive persons means the porential pathologies are already on their course.

Eventually some treatments will possibly arrive that may provide some level of modifying the disease course in various numbers for various persons and earlier stages.Still 10 - 15 yrs away at best. Plus with the most treatable not daring to be identified as at risk. More likely the best treatments for those with an established level of HD might just slow down a bit some parts of the disease in their progression. A small difference, though a valuable gain for an individual, parallel to a certain cancer treatments mesured by up to 5yrs extra life.

It's sad an irrational that testing for HD gene status is a taboo topic,even for scientists to broach. Years go so fast that a persons late 30's -40's is really quite brief when becoming symtomatic will reveal one's avoided gene status.So avoidance got one a spouse who in modern times can go as soon as your symptomatic, got kids one with JDH, another who kills themself on drugs afraid of having the disease as no reason was ever applied to dealing with HD, no financial plans made re cut earning times. In top of that alone and declining you can't access any facilities and care that ought to and could have been more there because the ones like you who had shame and fear of being known to have HD in you or kin hid the true numbers

of HD persons, an outdated legacy.

The fact HD can squeeze by as an orphan disease is shameful but realistic through the hidden real number. Wheras the true suspected number of at risk and persons who have even died of HD either purposefully or via misdiagnosis available in figures the actual number is assumed to be multiple times higher.This is emerging though not used yet as data re HD incidence % occurance in populations since in the last 20 yrs GPs and psych services won't collude with avoiding a HD diagnosis re ethics around promoting the option re information for potentially at risk kin.

8:34 PM, February 26, 2016

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