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At Risk for Huntington's Disease

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## Lifting Huntington's families' spirits with the promise of clinical trials

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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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## HD Links

[Huntington's Disease Society of America](#)

THURSDAY, SEPTEMBER 27, 2012

## Lifting Huntington's families' spirits with the promise of clinical trials

Perhaps nothing lifts the spirits of struggling Huntington's disease families more than news about research breakthroughs.

With people emitting oohs and ahs about some of the key developments, you could feel the excitement in the room Monday evening, September 24, as Jody Corey-Bloom, M.D., Ph.D., presented her annual research update to the support group of the San Diego Chapter of the Huntington's Disease Society of America (HDSA).

The director of the HDSA Center of Excellence for Family Services and Research at the University of California, San Diego (UC San Diego), Dr. Corey-Bloom kicked off the update with the hottest topic in the HD research world: gene silencing techniques aimed at attacking the disease at its roots.

"This is a big thing," Dr. Corey-Bloom stated. "There are actually two methods to accomplish this that are under way in research labs around the world.... We want to switch off the gene that is responsible for Huntington's disease. A lot of people feel very good about these techniques."

Before a room packed with some 50 support group participants, Dr. Corey-Bloom proceeded to outline the efforts to set up clinical trials to test RNA interference ([click here](#) to read more) and antisense oligonucleotides (ASO) ([click here](#) to read more) as ways to block the negative effects of the HD gene, which produces a harmful protein.

"If we can get rid of this harmful protein, there's a good chance that we could slow down the disease or even prevent it," Dr. Corey-Bloom explained.

[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](#)  
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## HD Blogs and Individuals

[Chris Furbee: Huntingtons Dance](#)  
[Angela F.: Surviving Huntington's?](#)  
[Heather's Huntington's Disease Page](#)



*Dr. Jody Corey-Bloom at a 2010 HD research meeting (photo by Gene Veritas)*

### **A trial in 12 months?**

“It’s likely that we’ll see both approaches being tried,” Dr. Corey-Bloom said, noting, that, given the current state of the science, it appears that the ASO approach will enter a trial first.

Dr. Donald Cleveland of UCSD and [Isis Pharmaceuticals, Inc.](#), of Carlsbad, CA are nearly “ready to go” with a clinical trial, perhaps as soon as within 12 months, she observed.

“The critical thing to say here is that we really are edging closer to human trials,” Dr. Corey-Bloom emphasized.

The trial site has not yet been determined.

Dr. Cleveland, UCSD’s Ludwig Institute, and Isis will be honored at HDSA-San Diego’s 12<sup>th</sup> Annual [Celebration of Hope Gala](#) on November 16, 2012.

In the coming weeks, I will report in detail on the UC San Diego/Isis project, which is mainly underwritten by the [CHDI Foundation, Inc.](#), the multi-million-dollar, non-profit biomedical research foundation dedicated exclusively to accelerating Huntington’s drug discovery and development.

You can watch Dr. Corey-Bloom’s presentation in the video below.



## Corey-Bloom Research Update 2012

from [Gene Veritas](#)

1:23:54

### The shock of HD's reality

Having tested positive for HD in 1999 and watched my mother succumb to the disease in 2006, I was thrilled to hear the news about the Isis ASO trial.

I have tracked the progress of the project since early 2008, shortly after the start of the CHDI-Isis collaboration ([click here](#) to read more).

Isis had first estimated that a clinical trial could start in late 2010. As with many scientific endeavors, delays have occurred. Now that a trial could realistically begin next year, I can once again fantasize about living free of this scourge that robbed my beloved mother of her ability to walk, talk, eat, and care for herself, leaving her a mere shadow of herself.

The support group meeting proved especially poignant for me because I had not attended for a number of months. After a summer break from my usual intense focus on HD, I once again confronted the reality of my own future in the faces of the HD-affected individuals I saw at the meeting.

Seeing these disabled individuals shocked me into renewing the fight to save them – and myself.

At 52, I am now at mother's age of onset. Once again, I felt extremely lucky to have avoided the noticeable, classic symptoms of HD.

### Imagining a healthy future

I have attended Dr. Corey-Bloom's updates for about ten years. Each time, she manages to provide clearer and more comprehensive information.

Squeezing literally dozens of research developments into an hour-long talk and making it understandable for a general audience is no small challenge. As I told her afterwards, "You just get better every year!"

This year's update especially served like a salve to calm my worries, which had heightened considerably after hugging one HD-affected friend who seemed to have declined since I last saw her a few months ago.

As Dr. Corey-Bloom spoke, I imagined this HD sister becoming healthy again and happily growing old with her family.

Posted by [Gene Veritas](#) at 9:52 AM



Labels: [Center of Excellence](#) , [CHDI](#) , [clinical trials](#) , [drug](#) , [gene](#) , [gene silencing](#) , [hope](#) , [Huntington's](#) , [Isis](#) , [Jody Corey-Bloom](#) , [mother](#) , [oligonucleotide](#) , [onset](#) ,

[protein](#) , [support group](#) , [symptoms](#) , [UCSD](#)

1 comment:

**Anonymous said...**

SO needed to read this!! My bf's mom has passed away this week from this horrid disease.. She had lived with it 15 years. To read this today makes me feel sooo much better/more hopeful.

I thank you so very much for this most informative blog... and I'm praying for you and this cure every day from NYC! THANK YOU.

8:30 PM, October 02, 2012

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