



# Gateway

**News from Huntington's New South Wales**

**Volume 20 No 2**

**Winter 2017**



## **WALK 4 HOPE 2017**

**CROUDACE BAY SATURDAY 16 SEPTEMBER**

**ORANGE SATURDAY 16 SEPTEMBER**

**CANBERRA SUNDAY 17 SEPTEMBER**

**PARRAMATTA SUNDAY 24 SEPTEMBER**

**REGISTER TODAY**

[Walk4Hope2017](http://Walk4Hope2017)

## Membership Renewal

Thank you to everyone who has renewed their membership for 2017/2018. We are also very grateful for the many donations we have received to date.

It is now possible to renew your membership, join the Association and give donations on-line by visiting our website [www.huntingtonsnsw.org.au](http://www.huntingtonsnsw.org.au)

If you still prefer to fill out a membership form and post it with your payment, you can either download one from the website or contact us on 9874 9777 and we will send one out to you.

The membership fee is still only \$22 (incl GST) per annum.

A strong membership will ensure that the Association continues to be representative of, and relevant to, people affected by HD in NSW and the ACT. So why not encourage another family member or friend to join?

## Southern Sydney & Illawarra Outreach Club

You and your family are invited to attend the next Outreach Club on

**Thursday 17th August 2017** at  
Club on East, 7 East Parade, Sutherland  
**11.30am to 2pm**

A range of meals will be available from \$10.  
We look forward to seeing you there!

The team at the Huntington Outreach  
Service

\*Please **RSVP** to Terry on 8890 9953 or  
0408 625 251



## Central Coast Support Group

This very energetic and enthusiastic group meets quarterly at  
Niagara Park Stadium, Washington Ave  
Niagara Park on  
**Wednesday evenings at 6.30pm.**

Dates for the rest of 2017 are  
**16th August**  
**15th November**

They also enjoy social functions and  
organise fundraising events  
For further information contact  
Dianne Faulkner at  
[huntingtons.centralcoastnsw@gmail.com](mailto:huntingtons.centralcoastnsw@gmail.com)

## West Ryde Carers' Support Group

You're invited for a get together with other carers who, like yourself, are caring for a partner, a family member or a friend with HD. Come along and join us as we share our chatter, laughter, tears and experiences.

Meetings are held on **Wednesdays at 10.30am** at the Huntington's NSW office,  
21 Chatham Rd West Ryde

**16th August**  
**13th September**  
**11th October**

Friday 1st or 8th Dec (TBC), Year-end Get-Together

To RSVP and for further information, please contact Huntington's NSW - 98749777 or  
Cecelia Lincoln, Westmead Hospital –  
8890 6699

**25** Australian Doctors Orchestra  
YEARS



# BOLERO



Conducted by

**Nicholas Milton**



Featuring Guitarist

**Slava Grigoryan**

**Shostakovich: 10th Symphony | Rodrigo: Guitar Concerto | Ravel: Bolero**



**SAVE THE DATE**

Sunday 5th November 2017, 2:00pm  
The Concourse Concert Hall, Chatswood

Tickets: Adults \$50, Concession \$35, Children \$25, Family \$120

Bookings: Web [www.theconcourse.com.au](http://www.theconcourse.com.au) or

[www.ticketek.com.au](http://www.ticketek.com.au)

Call The Concourse 8075 8100 or

Ticketek 1300 795 012

Visit The Concourse Box Office

**All proceeds benefit Huntington's NSW**

## Westmead Hospital Phone Numbers Change



From 1st August 2017, all Westmead Hospital phone numbers will start with 8890.

For example, if the Westmead Hospital number you are currently dialling is 9845 5555, the new number will now be 8890 5555.

The 8890 numbers are now live, so feel free to update your contacts now!

**Please note, this phone upgrade does not affect  
The Children's Hospital at Westmead.**

## HDdenmore — Raising Awareness of HD



On Thursday, 18th May, 2017, on a sunny morning in Rome, a historic event occurred. Pope Francis, the head of the Catholic Church, held an audience with almost 2000 people whose lives have been affected by Huntington's Disease. There were HD family members and researchers from 23 different countries - Dr Elizabeth McCusker, neurologist and I were privileged to be the two Australians at the meeting held in the Paul VI Audience Hall. But the main focus of the event was on families from South America - Venezuela, Colombia and Argentina.



Hddenmore and Factor\_H organisers and HD family members

The morning began with a kind of roll call of people from all of the countries attending—Australia and New Zealand both received hearty cheers, recognising the distance we had travelled to attend the event. Next followed a series of musical acts, with special gestures made for two children who have been impacted by HD. One young boy from Venezuela had been bullied and forced out of school because he came from an HD family. He was sent a video message from his soccer hero Neymar and on stage received a signed shirt and football. A young Argentinian girl with juvenile HD met her musical hero Axel on the stage.

*"For far too long, the fears and difficulties that characterize the life of people affected by Huntington's Disease have surrounded them with misunderstandings and barriers, veritably excluding them. In many cases the sick and their families have experienced the tragedy of shame, isolation and abandonment. Today, however, we are here because we want to say to ourselves and all the world: "HIDDEN NO MORE!"*

- Pope Francis, Vatican City, 18 May 2017

The full text can be found here. [https://w2.vatican.va/content/francesco/en/speeches/2017/may/documents/papa-francesco\\_20170518\\_malati-corea-huntington.html](https://w2.vatican.va/content/francesco/en/speeches/2017/may/documents/papa-francesco_20170518_malati-corea-huntington.html) He then went around the small audience at the front of the stage and met the HD families, one by one. The first greetings were

more formal and restrained, but then people seemed to relax. Many collapsed weeping into the Pope's arms, and it was clear from their faces that this would be one of the pivotal moments of their lives. Being in the audience of this event was an emotional experience, and many of those watching the live stream on the

After this the Pope arrived and gave his speech about HD, which emphasized the historical difficulties HD families faced and the enormous impact of the disease.



Pope meeting Nancy Wexler and Ed Wild

internet were equally moved.

So how did this event come about? According to their website, HDdenmore is the brainchild of "a global coalition of HD advocates", including former NBC journalist and HD family member Charles Sabine, Italian Senator and HD researcher Elena Cattaneo, CHDI Researcher and founder of Factor\_H Dr Ignacio Munoz-Sanjuan, amongst many others.

The HDdenmore (pronounced Hidden No More) initiative is the latest in a long line of attempts to reduce the shame and stigma which have been associated with the disease in the past. Beginning in the 1970s, we have used slogans such as "It's Better to Light a Candle

(Continued on page 5)



(Continued from page 4)

than to Curse the Darkness" to bring the challenges faced by HD families to the wider world, to reduce isolation and to build a stronger HD community.

The current Pope was born in Argentina, and he was approached a couple of years ago to meet with a single Venezuelan family – it was his suggestion to make this a bigger event by bringing several families to meet him, and planning for the HDdenmore event began. Apart from the Pope, why the emphasis on South America? We need to look a little into HD's history to answer that question.

As we all know, HD has been a hidden disease, and the needs of HD families were neglected for much of the twentieth century. In 1972 researchers came together from many countries around the world for the first major conference focussing on the disease. This date was also significant as it marked the hundredth anniversary of the first full description of HD in the medical literature by American physician Dr George Huntington. At the meeting, one doctor reported on an area in Venezuela, at the top of South America, where there was an extremely high incidence of HD.

Over the next 20 years, researchers travelled to the Lake Maracaibo area of Venezuela to study the disease. Slowly they built up a database of family trees and blood samples, and in 1983 researchers discovered that the gene causing HD was on Chromosome 4, more precisely identifying the location and nature of the gene in 1993.

Services for people in developed countries have flourished over the past decades, and in Australia we have been well ahead of our counterparts in other countries. One of the first HD clinics in the world was started in Melbourne in the early 1970s by psychiatrist Dr Edmond Chiu and social worker Betty Teltscher. In NSW, in the 1980s,



Dr McCusker & Therese Alting outside the Paul VI Audience Hall.



Young Venezuelan boy receiving soccer kit

Dr Elizabeth McCusker built on the work of Dr David Rail at Lidcombe Hospital. Alongside social worker Fiona Richards and many other devoted staff, services were provided for HD families, with the more formal establishment of a comprehensive HD service at Westmead Hospital in 1995, now headed by Dr Clement Loy. Patient organisations have also been crucial in helping HD families, with our own association, Huntington's NSW, being founded in 1975.

However, families in South America are struggling not only with the effects of HD, but also severe poverty, extremely limited or more often non-existent services and little support or understanding from the wider community. One of the organisers of the event, Dr Ignacio Munoz-Sanjuan has also founded an organisation called Factor\_H, (<https://factor-h.org>) which aims to help HD families in South America. Edenmore brought some of these families to the Vatican in a bid to highlight their very difficult situations. There was widespread media coverage of the event. Articles appeared in La Republica in Rome, the Guardian newspaper in London, the Irish times, and the BBC. There was a story on National Public Radio in the US. There was also coverage in the Latin American Press. The HDdenmore website has a link to all the media.

The team behind HDdenmore hope it continues to play a role beyond the event in Rome. If you wish to know more about the organisation, they can be found here (<http://HDdenmore.com>) and on Facebook, Instagram and Twitter.

*Therese Alting*

*Therese is a Clinical Neuropsychologist working in research at Westmead Hospital Huntington's Disease Service, primarily coordinating the Enroll-HD Study. She did her PhD on the History of Huntington's Disease in Australia, and is a Board Member of HNSW. She would like to thank the Board and the anonymous donor who made possible her travel to the Vatican for this event. Dr McCusker was self-funded.*

## NATIONAL DISABILITY INSURANCE SCHEME – NDIS

### New to the NDIS?

Most of the feedback we've received indicates that the NDIS is making the lives of people with HD a little easier, as there is greater choice and higher levels of support and services available than in the past. Though, the NDIS is a new large-scale change, and we've also heard that some people are having trouble navigating the NDIS processes. *So where do you start?* Begin by:



- Involving your key worker at the HD Service at Westmead Hospital
- Attending an NDIS information session near you. A calendar of available sessions can be found at <http://ndis.nsw.gov.au/events/>
- Register for the NDIS, there are two different pathways

### Currently receiving disability services?

A letter from the National Disability Insurance Agency (NDIA) will arrive at your home introducing the NDIS and advising you a person from the NDIA will phone you to walkthrough your NDIS registering also known as 'completing an Access Request form over the phone'.

**Tip:** decline completing the form over the phone; instead ask for the form to be mailed to you. This way you can complete the form with your key worker, carer and/or family members.

### Not currently receiving disability services?

You may still be eligible to register for the NDIS.

**Tip:** use the 'My Access Checker' on the NDIS website, <https://www.ndis.gov.au/ndis-access-checklist.html>, and involve your HD Service key worker who can assist with obtaining an NDIS Access request form

### What next?

- You'll receive an NDIA letter advising you of the outcome of your NDIS access request.

**Note:** decisions are provided within 21 days of the NDIA receiving all appropriate information; additional supporting information may also be requested separately by mail to help establish your eligibility for the NDIS.

- If you're successful, prepare for your first planning session  
As part of your NDIS acceptance letter you'll be advised that an NDIS planner will phone you to complete your first plan as well as paperwork to help you prepare for your first planning meeting, see more at <https://www.ndis.gov.au/participants/firstplan.html>

### Tips:

- 1) decline any requests to have meetings over the phone; instead ask for a personal meeting that includes your advocates like, your key worker, carer and/or important family members;
- 2) ask the planner to do some research on Huntington's Disease;
- 3) tell the planner everything - do not sugar coat what is going on for you – share your worst day;
- 4) remember the carers – carer respite is attained by asking for 'external socialisation for the person with HD';
- 5) ask for 'Support Coordination' – this takes away the burden of managing an NDIS plan, you can then enjoy your life or family members can concentrate on caring for their loved one.

### Providing feedback to the NDIA

Visit the NDIS website at <https://www.ndis.gov.au/about-us/contact-us/feedback-complaints.html>

### Share your NDIS experience with us

We'd love to hear from you! Tell us your good news stories, what hasn't worked for you or any tips you'd like to see passed onto other HD families.

Send an email to [info@huntingtonsnsw.org.au](mailto:info@huntingtonsnsw.org.au).

(Continued on page 8)

## Disability No Barrier for Wendy's Equestrian Pursuits

We all know that feeling when we meet someone who shares our passions. The excited anticipation of meeting a kindred spirit whose love equals your own. This was what happened when Aberdeen local Wendy found her support worker Janell. A love of horses unites the pair and has seen them travel throughout NSW to watch them compete.



### For the love of horses

Wendy suffers from Huntington's disease, a genetic neurodegenerative condition, and was facing the heartbreaking possibility she may have to give up her beloved horses.

"I have had a passion for horses all of my life," Wendy said. "I had been competing in dressage for 20 years, but I have been unable to compete myself since being diagnosed with Huntington's. I have also been breeding horses myself for many years and have had to pay people to train them instead of being able to train them myself before selling them on."

A chance recommendation by her social worker, John Conaghan, from John Hunter Hospital, led Wendy to approach Challenge Disability Services and our support worker Janell, who has experience working with people with Huntington's. And what a lucky chance it was! The camaraderie between Wendy and Janell is clear, and the pair take pleasure in sharing their hobby.

### A helping hand

I don't know about you, but it's rare that I know what I'll be doing from one week to the next. Just like me, no week is the same for Wendy and Janell. That's the beauty of the NDIS, having control of your funding means you have the flexibility to match the services you use to what you require from week to week, or year to year.

While Janell supports Wendy 20 hours per week, the tasks they accomplish depend on what Wendy wishes to achieve at the time. Janell helps with cooking, cleaning, emailing, feeding the horses and also supports Wendy on her equestrian adventures. Just like everyone else, some days Wendy feels tired and doesn't need Janell's assistance. Thankfully, the NDIS funding arrangement allows for this.

### Meeting your needs

I always dread having to go to the doctors; any appointment or meeting fills me with fear. Mainly because I always forget everything I need, or can't seem to describe what's going on accurately.

Janell's support in preparing and attending medical and allied health appointments has allowed Wendy to request help to maintain her independence. A recent occupational therapist assessment has resulted in the trial of a scooter with a specially-designed hay trailer, so Wendy can independently feed her horses. The pair are understandably excited about using the new equipment.

"The scooter allows Wendy to continue to care for her horses without falling in the paddock," Janell said. "Hopefully, she will get permanent approval from the NDIA for this in the next couple of months."

And Janell's efforts are not the only help Challenge provides Wendy. She regularly enlists the support of Challenge Business Services Koora Industries to clean and rake the stables, clean her car and they also whipper snip and mow the lawn.

### Equestrian adventures

The pair are adventurers, following Wendy's horses around NSW's equestrian circuit to great success, including qualification for national and international competitions.

It was an exciting trip when Wendy and Janell visited Sydney in March. Wendy's horses successfully competed in the Elizabeth Farm Horse of the Year Show at the Sydney International Equestrian Centre.

"Due to my condition I am on a restricted license

*(Continued on page 8)*



(Continued from page 6)

### **NDIS advocacy for the Huntington's community**

Huntington's Australia is part of the Neurological Alliance Australia (NAA). In March 2017 the NAA submitted a joint position statement to the NDIS Cost Review, see [http://www.pc.gov.au/\\_data/assets/pdf\\_file/0018/215262/sub0030-ndis-costs.pdf](http://www.pc.gov.au/_data/assets/pdf_file/0018/215262/sub0030-ndis-costs.pdf)

The NAA acknowledges that the NDIS is integral to improving the lives of people living with progressive neurodegenerative diseases. In this Statement the NAA highlighted some of the difficulties faced by people when needing to engage in the NDIS and also made recommendations for more appropriate approaches to improve NDIS outcomes for people living with progressive neurodegenerative diseases.

In June, the Productivity Commission released the draft NDIS Costs position paper for review, refer to <http://www.pc.gov.au/inquiries/current/ndis-costs/position>. The NAA submission was acknowledged on a number of occasions and it was pleasing to see the following statements:

- "Phone planning conversations are not appropriate for some participants, including some participants with particular accessibility requirements, mental illness, cognitive impairment and neurodegenerative diseases or people of culturally and linguistically diverse backgrounds." and;
- "Planners should, at a minimum, have a general understanding about different types of disability. The Commission recommends specialised planning teams for some types of disability, such as psychosocial disability." (NDIS Productivity Commission Position Paper Overview & Recommendations, p.26 & 29)

The final position paper will be released in September this year.

(Continued from page 7)

and need to stop every hour, so it was a long trip to Sydney," Wendy said.

"The winners of this competition go on to the national competition in Melbourne, it was great to be there as one of my horses qualified for the national competition. One of my other horses also competed in Richmond recently and at an international competition in Sydney in April."

Hopefully, Wendy and Janell can keep us updated on their equestrian adventure, I know I want to find out more!

Not content with only caring for her horses, Wendy is known for looking after the Aberdeen community too. Volunteering at the Upper Hunter Riding for the Disabled Association and local Lions Club activities are both on her regular agenda.

She also participates in an international study at Westmead Hospital in Sydney for people with Huntington's, and encourages others living with the disease to "hang in there" until they find a cure.

"While there is no cure, I want to get the word out to other people with Huntington's about the services being offered by Challenge Disability Services under the NDIS," Wendy said.

"With the support we provide we can help Wendy, and others suffering from Huntington's disease, to continue to do the things they love," Janell said.

Want help to pursue your passions? Check out the Challenge Disability Services website for more information on how the NDIS can provide you with more independence, or fill out the enquiry form to arrange for one of our team members to get in touch with you.

*Acknowledgement: Katrina Warmoll  
<https://www.challengecommunity.org.au>*

### **Do you have a story to share?**

If you have a story that you would like to share in "Gateway" please contact Robyn at [robyn.kapp@huntingtonsnsw.org.au](mailto:robyn.kapp@huntingtonsnsw.org.au)



## Update confirms Huntington's disease 'gene silencing' trial on track

Ionis says its trial of HTRx, intended to lower huntingtin protein, is fully recruited and plans to extend it

*By Dr Jeff Carroll on June 22, 2017 Edited by Dr Tamara Maiuri*

Ionis Pharmaceuticals launched the first ever trial of a huntingtin-lowering drug – sometimes called a 'gene silencing drug' – in late 2015. In a significant update, the company has announced two important milestones: the trial is now fully recruited, and an 'open-label extension' will be activated for the volunteers in the current trial. While nothing is guaranteed, this bodes well for the future of this important program.

### A quick recap on huntingtin lowering

The idea of 'switching off' the cause of Huntington's disease was one of the first thoughts that popped into researchers' heads when the HD gene was discovered in 1993.

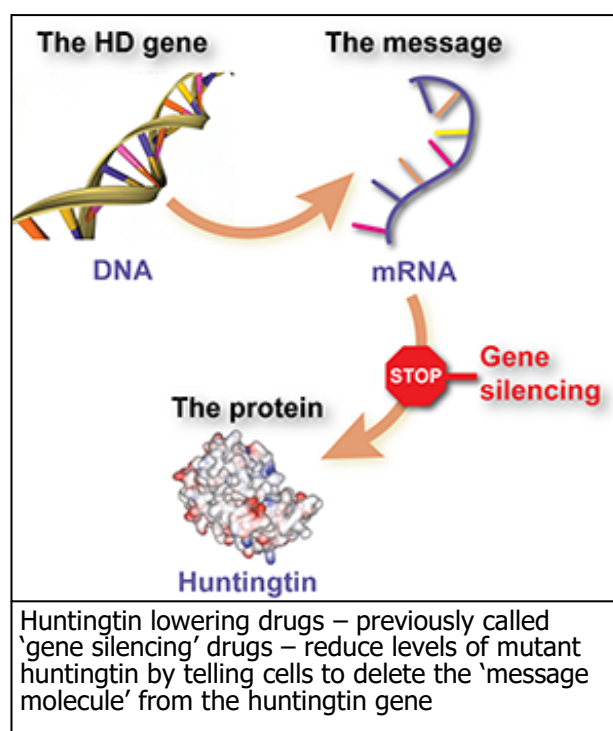
The first major progress in achieving this came around a decade ago, when several mouse studies reported success in reducing the activity of the gene. Doing that lowered levels of the protein for which the gene is a recipe: **mutant huntingtin**. In each case, lowering huntingtin improved symptoms of the disease in the HD mice.

These huntingtin-lowering drugs all act in similar ways – by 'shooting the messenger'. A gene is a recipe for a protein, but in between gene and protein, a chemical messenger is made that is essentially a working copy of the gene. Getting rid of the messenger breaks the production line, so less of the protein is made.

Huntingtin-lowering drugs are made from molecules similar to DNA, the stuff that makes up our genes. These kinds of chemical have the special ability to stick to each other strongly, if the sequence of genetic 'letters' matches up properly. Drug-makers can build designer molecules with a sequence that will stick to the messenger molecule of the huntingtin gene, but not to other messengers.

When the cell sees that the messenger has a drug molecule stuck to it, it recognises that something unusual has happened, and responds by deleting the message molecule. Eventually, this lowers the level of the protein.

The drug molecules themselves come in many different flavors. Some are made from RNA (the stuff the messenger is made from), while others are made from DNA (the stuff our genes are made from).



One quick word on terminology. Many people, including HDBuzz, have referred to these as 'gene silencing' drugs. That slightly suggests we're trying to switch off the gene altogether, which is probably not possible or desirable. So, increasingly, you'll hear us call them 'huntingtin lowering' drugs. We'll still use the phrase 'gene silencing' from time to time, so that people can find the latest article if they Google the better-known term.

### The current trial

When they started working on HD over a decade ago, the California-based company behind the current trial was called Isis Pharmaceuticals. A lot has changed since then, including the name of the company – since 2015 it's been called Ionis.

*(Continued on page 10)*

(Continued from page 9)

Ionis' drug is an **antisense oligonucleotide**, or ASO. That means it's a single strand of chemically modified DNA, designed to stick to the message molecule from the huntingtin gene.

Just to keep things exciting, the drug itself goes by two names. It was initially called **ISIS-443139**, but lately it's been going by **IONIS-HTTRx** or just **HTTRx** – a combination of *HTT*, the abbreviation scientists use for the huntingtin gene, and *Rx*, a symbol used in the pharmaceutical industry meaning a treatment or prescription.

(Fun fact: the origin of 'Rx' is unknown, but it may come from the Latin word 'recipe'.)

Ionis' trial, led in partnership with Prof Sarah Tabrizi of University College London, was the first time a targeted huntingtin-lowering drug had been tested in humans. As with the first trial of any new drug, the main focus of the trial is **safety**.

To reach the brain, ASO drugs like HTTRx have to be injected into the spinal fluid using a thin needle. This is called **intrathecal injection**.

While this may sound gruesome, it is a commonly-used method for treating the brain in other conditions like cancer. A closely related procedure – lumbar puncture or spinal tap – is very widely used, and indeed many hundreds of volunteers from Huntington's disease families have undergone this procedure to donate valuable spinal fluid to help with HD research.

The current HTTRx safety trial involves patients with early symptoms of Huntington's disease. After extensive clinical evaluation, each volunteer receives four injections of the drug at monthly intervals, followed by a final spinal fluid collection.

Throughout the trial, a **placebo** arm has been used, to help distinguish drug effects from the effects of being in the trial, such as the injection procedure. The trial has a **dose escalation** design: very low doses were given at first, with later volunteers getting higher doses

### What's new

A recent press release from Ionis – the first official update since the trial began – brings cautiously optimistic news. It contains two important announcements. First, recruitment into the current trial is complete. And second, the company is launching an **open label extension** to the current trial.

Completion of recruitment is an important milestone for any trial, but for this one it's particularly significant. The trial involved a number of 'firsts' – the first ASO drug given to HD patients, the first time intrathecal injection had been used in HD – and each dose escalation brought both the hope of stronger benefits and a greater risk of unwanted effects.

Throughout a trial like this, an independent committee of experts regularly reviews all the safety data to look for any sign of danger or harm. News that the trial is fully recruited and the final patients are going through the procedures is a strong suggestion that even at the highest

doses, the drug's safety looks good. Despite exhaustive safety testing before going into patients, any drug can produce unwanted effects, so that's really the best news we could be hoping to hear at this stage.

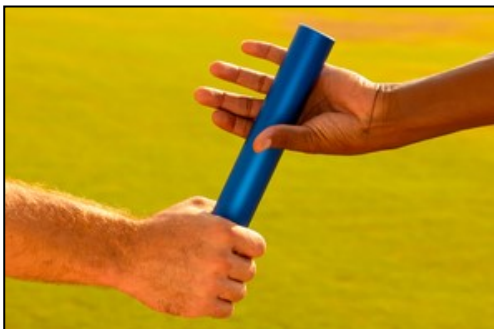
In the words of the press release: "The safety and tolerability profile of IONIS-HTTRx in the completed cohorts of the Phase 1/2a study supports its continued development". Thumbs up to that from us.

With recruitment complete,

Ionis is now in a position to set out a timetable for completion of the trial and the first release of its results. "Top line results" are expected **around the end of 2017**. In our experience, the results of a trial like this may not come out all at once. The safety data may come first, but information about whether treatment with HTTRx lowered the level of huntingtin protein in the spinal fluid – a much-anticipated 'biomarker' outcome – may take a little longer to materialise.

### An open-label extension

The current HTTRx trial has a **blinded** design: some volunteers received four injections containing no active drug – and neither the



Developing a drug is like a relay race - it happens in stages and each one has to complete successfully for the next to start. This announcement is a good sign that the next stage – an efficacy trial to test whether HTTRx slows down Huntington's disease – may be round the corner

(Continued on page 11)

(Continued from page 10)

patients nor the trial personnel knew who was receiving drug or placebo.

In an **open-label extension** trial, or **OLE**, the volunteers from a blinded trial are invited to come back for further doses, and every volunteer gets the active drug rather than some receiving drug and some the placebo – usually at the highest dose that was safely tried in the blinded trial.

Ionis had previously said that an open-label extension **may** be implemented if the data from the safety trial looked good. We don't want to read too much into a brief announcement, but running an OLE isn't cheap for a trial sponsor, so this announcement certainly gives us optimism about the whole HTTRx program.

The open-label extension study will only be available to volunteers in the current trial. That means it will run in the same study centres in the UK, Canada and Germany. The length and exact design of the OLE have not yet been announced, but it will provide a wealth of information that Ionis and its partner, Roche, can use to plan their next steps.

### Thanking our heroes

An important upside of an open-label extension is that it rewards the volunteers who took part in the original study by giving them guaranteed access to the active drug. We're particularly pleased about this aspect of the announcement. The participants in this trial were all Huntington's disease family members who gave up significant time to receive spinal injections of a drug that, at the start of the trial, had never been given to humans before. Those enrolling later were given higher doses than anyone had previously received.

Whatever the outcome, these volunteers accepted personal risk, and in most cases did so to help others rather than themselves. Our whole community owes these heroes a debt of gratitude for their bravery and service. Please join us in saluting them on Social Media using the hashtag #HDBuzzHeroes.

What's next?

The open-label extension will begin soon, likely in the next few months. Volunteers in the safety trial will be contacted by the study sites, with information about how and when they can enroll for the OLE. Meanwhile those participants still in the blinded trial will need to finish their involvement before rolling over into the open-label extension.

If you weren't in the blinded safety trial, you will not be able to sign up for the open-label extension. **Please do not contact study sites unless you were in the original trial**

Towards the end of 2017, we can expect to hear an official announcement about the results of the blinded trial. Don't be disappointed if that is confined to safety data in the first instance. "Safe and well tolerated" should be music to the ears of HD family members after a trial of this kind!

Later, maybe in early 2018, there may be a separate announcement about some of the more experimental results of the safety trial - things like whether HTTRx lowered the huntingtin level in the spinal fluid.

The next big news, if all continues to go well, may be an announcement of an efficacy trial, testing whether HTTRx for long enough so determine whether it slows progression of the disease. Roche now has the right to opt in, take over development of the drug and run such a trial based on its own decision-making process. With a bit of luck, we may hear about that in the coming months too.

Emphasising their desire to move the drug forward, in a separate announcement made directly to the HD community, Ionis said "Upon study completion, the next step for this program will be to conduct a study to investigate if decreasing mutant huntingtin protein with IONIS-HTTRx can slow the progression of this terrible disease."

As yet, we have no idea when the next big study will start, but our best guess would be late 2018 or early 2019. As to when, and how many patients, and what countries and sites – it is just too early to be sure, but Ionis says "Future studies for the program will be conducted globally and will include US study sites". As to the rest, you can be sure you'll read about it first on HDBuzz.

Nothing happens quickly enough for people whose families are impacted by HD – but this is a moment to pause and be glad that, for this important huntingtin lowering program, the news so far is all good.

Acknowledgement: <https://en.hdbuzz.net/>





## Huntington's New South Wales

PO Box 178, West Ryde, NSW 1685  
21 Chatham Road, West Ryde, NSW 2114  
Telephone: (02) 9874 9777  
Free Call: 1800 244 735 (Country NSW only)  
Email: [info@huntingtonsnsw.org.au](mailto:info@huntingtonsnsw.org.au)  
Web Site: [www.huntingtonsnsw.org.au](http://www.huntingtonsnsw.org.au)

### Huntington's NSW

The Australian Huntington's Disease Association (NSW) Inc, trading as Huntington's NSW, is a not-for-profit organisation established in 1975.

### Our Mission

The energies and resources of the Australian Huntington's Disease Association (NSW) Inc are directed towards satisfying the needs of people with or at risk for Huntington's Disease and their families in NSW and the ACT by providing and/or facilitating delivery of a range of quality services.

### Our Philosophy

People with Huntington's Disease and their families are individuals with equal value to all other members of Australian society, with the right to treatment and care by knowledgeable professionals and care givers, the right to appropriate support services and the right to have the best quality of life possible.

### Our Services

These include education and information; advocacy; counselling and referral; holiday programs; family support; rural outreach and client services.

### Our Board

President: Brian Rumbold  
Vice President: Deb Cockrell  
Treasurer: Stephen Guthrie  
Secretary: Therese Alting  
Members: Richard Bobbitt  
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