Huntington's Disease Association

Magazine

Spring 2021

The Huntington's Disease Association 50th Anniversary Edition

Contents

1970 — A letter in a newspaper reaching out to people with 'Huntington's chorea'

p5

1971 — United Kingdom Committee to Combat Huntington's Disease is established and the first branch is created

1986 — Gained official charity status as 'The Association to Combat Huntington's Chorea'

1990 — Charity is renamed as the 'Huntington's Disease Association'

1991 — The Annual General Meeting is held at the head office and the public are invited to attend

2000 — New Huntington's Disease website goes live

p12

2002 — CHDI Foundation founded

2003 — European Huntington's Disease Network founded

1993 — Specialist Huntington's Disease Adviser roles created and implemented - Formerly known as CASE

p7

1996 — The Huntington's Disease Association's 25th Anniversary

1993 — The Huntington gene is discovered, first predictive test is offered and Princess Diana becomes a patron

p9

2005 — First ever Juvenile Huntington's Disease Weekend

p12

2008 — Charity moves offices from London to Liverpool

2009 — First Certified Huntington's diesease course for professionals

Shane Richie and Tony Hadley become patrons

2010 — Facebook and Twitter pages launched

First Young Adults event - Decisions, discussions and dilemas

First #LightItUp4HD campaign launched. We supported with the filming of Casualty's Huntington's storyline and held the first volunteer awards

p15

2013

The charity wins GSK Impact Award for outstanding work in community healthcare and the Huntington's prevalence report is published

2014

The charity wins the AMRC Judges Discretionary award

2016

2017

Pope Francis blesses families affected by Huntington's at Vatican City

p17

2018

Our first ever Inbetweeners event

2019

Roche and Ionis GENERATION-HD1 trial begins

2020

The charity and Huntington's Community must cope with a global pandemic - COVID-19

2021

p18

GENERATION-HD1 and WAVE trials halted

CONTENTS

A message from the Chief Executive 4

What our Specialist Advisers can offer you 8

My testing journey 10

Understanding Juvenile Huntington's disease 13-14

National awareness campaign for Awareness Month 20

New faces of the Huntington's Disease Association 21

Happy 50th anniversary to the Huntington's Disease Association 21

Get Involved 22

Save the date 23

Looking back over years at the Huntington's Disease Association 24

Support 26

A message from Cath Stanley, Chief Executive

It seems hard to imagine that it's over a year since the first lockdown, and what a year it has been. I know it has been a struggle for so many different reasons. Not being able to see loved ones, not getting the usual support and in some cases, coping with loss and bereavement has meant a very difficult 12 months for so many people. As the flowers bloom and we see ducklings popping up in ponds everywhere, I hope they are a sign that we can begin to open up our homes again, hug our loved ones and value our friends and family thanks to the mass vaccination program.

I know for many, the news of the Roche and Ionis GENERATION – HD1 and the WAVE Life Sciences PRECISION-HD1 and HD2 trial halts have been a real blow when all had dared to hope. However, what has been learned from the trials will help other ongoing trials move forward and so we still do have a lot to be optimistic and hopeful about.

On behalf of the charity, I would like to thank everyone who has continued to support us through this time. As fundraising in the "normal" way, by running marathons, holding bake sales, and all the other things you amazing people do, has not been possible, you have found innovative ways to continue to support us. Many, many thanks to you all. I know our fundraising team has lots of opportunities for you to support us in the coming months, so please get involved.

We are really proud of the series of webinars that we have put in place over the last 12 months. Please keep an eye on our website for more information about the webinars and events to come. If you sign up to our mailing list by visiting 'Get involved' on our website and navigating to 'Join our mailing list', we will be able to keep you up to date on all of the events and opportunities that we run. Finally, the charity is 50 years old this year – we are currently planning a number of ways in which to celebrate this...so watch this space!

Cath Stanley

Chief Executive

1971

Where it all began

The Huntington's Disease Association was first formed in 1971 and was known as the 'United Kingdom Committee to Combat Huntington's Disease'. The tale of how we became the charity we are today is one born out of the desire to learn more about the disease and, as always, help one another.

On a warm summer's day in 1970, our founder, Mauveen Hart, made contact across the pond with the Guthrie family. Marjorie Guthrie was the wife of famous musician Woody Guthrie who sadly passed away from Huntington's disease in 1967. Marjorie had already formed the 'Committee to Combat Huntington's Disease' in the USA and wanted to aid Mauveen in her attempt to find support for those affected by the disease in the UK. Mauveen's father had Huntington's disease and although the Committee to Combat Huntington's Disease was 3000 miles away, she found solace and was relieved to hear that an organisation had been formed to help people like her father.

This struck a spark in Mauveen, urging her to start a similar

organisation here in the UK but she knew little of how to do this and by the winter of 1970 was still pondering the problem.

During the 1970s, when Mauveen was striving to find support for those affected by Huntington's disease, there was widespread ignorance and a stigma around Huntington's even within the medical community. It is said that one consultant spoke to Mauveen and said "The Pilgrim Fathers took it to America and we know no more about it now as we did then" - not the information you want to hear from a medical professional! This just urged Mauveen on.

On Sunday 13 December 1970, an article appeared in a newspaper called 'The People' that featured Mrs Ellen Sibley and her family who were affected by Huntington's disease. This was the first time the disease had been featured in the British press and it surprised both Mauveen and other Huntington's families in the UK. Taking this opportunity in both hands, Mauveen called the author of the magazine. On 20 December 1970, a letter was published in 'The People' calling for other Huntington's families to contact Mauveen.

By January 1971, 76 Huntington's families had written to Mauveen. The next step was to send out a questionnaire to grasp what exactly the families wanted from the group and to choose a name. Mauveen suggested that the group was called the 'United Kingdom Committee to Combat Huntington's Disease' or 'UKCCHD' for short! Armed with 76 members, UKCCHD set off to conquer Huntington's disease and the world.

In May 1971, a central committee was formed and their first meeting took place at Mauveen's home in Ashford. This was probably the first-ever meeting of Huntington's disease families in the UK. The first newsletter was sent out the very same month to over 80 members. Little did the group know that within ten years, family membership alone would be over 2000.

Mauveen (left) attending a branch presentation

Sheffield was the first branch to form in 1971. During that year, UKCCHD drew in more Huntington's families along with an army of doctors, social workers, nurses and more. News spread of a doctor from Gloucester Royal Hospital who was especially interested in Huntington's. Mauveen wrote to Dr David Stevens who offered his full support. Later, Dr Stevens wrote family and medical booklets for the charity.

In 1986, the United Kingdom Committee to Combat Huntington's Disease was awarded full charity status as 'The Association to Combat Huntington's Chorea'. This meant that the group, by law, was to be exclusively charitable and were set up for public benefit only. By 1990, it was decided that the charity would be renamed the 'Huntington's Disease Association'.

The rest...is history.

Mauveen (right) with Goddaughter

Photos taken from the 25th anniversary edition of our newsletter in 1996.

1993

The Huntington's community call for specialist support

As we entered the 90s, the 'The Association to Combat Huntington's Chorea' became the 'Huntington's Disease Association'. The charity kept on growing which put more demand on the services we could provide and the support we could offer.

Families that were involved in the charity at that time were asked to fill out a questionnaire entitled 'Finding out about Huntington's disease'. 86% of the community replied and provided feedback stating that they would benefit from professionals with good knowledge and understanding of Huntington's disease.

The 1993 Autumn edition of our magazine stated that the community had several recommendations to help people cope with finding out about Huntington's -

"The first two recommendations are that the HDA should:

- *Coordinate a campaign to 'educate' GPs about HD.*
- *Produce an informative leaflet specifically for teenagers.*

Other recommendations concern ways in which we can:

- *Target publicity to reach families currently missing out on membership and information.*
- *Reduce the problems people have in finding out about the association."*

The results of this questionnaire led to the development of a project known as CASE – Care, Advice, Support and Education. In 1995, CASE was first introduced to the Huntington's community via a magazine piece entitled 'A growing and developing network of CASE'. It read -

"In this issue we feature our growing team of Regional Care Advisers. Operating at a local level they offer help and advice on health and welfare; give practical and emotional support to those living with HD and provide information, study days, workshops and seminars to professionals working in healthcare, education, social services, nursing and residential homes, creating awareness of HD, the needs of HD families and the work of the Association."

This project saw the birth of our Specialist Huntington's Disease Advisory Service (SHDA) which continues to be a valued resource and the backbone of our charity to this day.

CASE Regional Care Advisers -1995

Specialist Huntington's Adviser, Debra Robinson, supports Huntington's family

Specialist Huntington's Disease Adviser, Poppy Hill, supporting Huntington's family

Over the years, our Specialist Huntington's Disease Advisory Service grew and spread across England and Wales leaving us with a comprehensive web of support that we can offer families affected by Huntington's disease today. Our advisers can offer a range of services for a variety of age groups.

What our Specialist Advisers can offer you now

Our Specialist Huntington's Disease Advisory service covers England and Wales and is delivered by a team of experienced and compassionate care management professionals. We understand that whole families may need our support and therefore provide a comprehensive service that supports the whole family unit, friends and professionals who may work with the person affected by Huntington's disease.

We have the only dedicated Juvenile Huntington's disease adviser in the world and offer a Huntington's Disease Youth Engagement Service that provides support to young people aged 8-25 living in a Huntington's family.

Our specialist advisers support anyone who needs help at any stage of their Huntington's journey. They can help people living with the disease, people at risk of inheriting the disease, family members, carers, children and young people. They can even advise friends, neighbours, employers and medical teams.

Our Specialist Huntington's Disease Advisers can help by:

- Visiting people at home to provide practical help and emotional support - Currently carried out online or via phone.
- Delivering a confidential telephone helpline service.
- Coordinating support with medical, health and social care professionals.
- Making referrals into specialist Huntington's disease clinics.
- Advocating for the person with Huntington's disease to get the best support.
- Educating medical, health and social care professionals - meaning better diagnosis and ongoing care.
- Organising and attending local support networks and carers' group meetings.
- Providing a listening ear.
- Providing information resources on symptoms and how to manage them.
- Connecting younger family members with our Youth Engagement Service.
- Giving support for sensitive end-of-life care.

If you are affected by Huntington's disease and need support or advice, please contact us on **0151 331 5444** or email **info@hda.org.uk**. We will put you in touch with one of our advisers.

You can see which adviser currently covers your area by visiting our website, navigating to 'Getting help' then 'SHDA service' and scrolling to 'Find your local Specialist Huntington's Disease Adviser'.

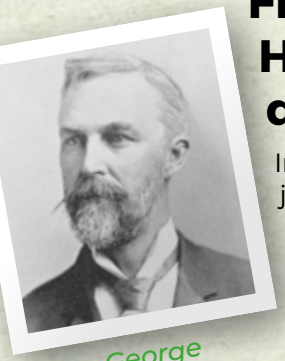
Specialist Huntington's Disease Adviser, Poppy Hill, supporting young man from a Huntington's family

1993

Diana and the gene

1993 was a big year for Huntington's disease and the Huntington's Disease Association. Not only was the Huntingtin gene discovered but Princess Diana became a patron.

Finding Huntington's disease



George Huntington

In 1872, at the age of just 22 years, George Huntington, a third-generation physician from New York State, USA wrote the first known and accurate description of Huntington's disease that he, his father and grandfather had observed in their patients over the years.

George identified the disease as a type of chorea - a movement disorder that causes involuntary, irregular, unpredictable muscle movements - and wrote that Huntington's disease was a nervous disorder marked by incessant and uncontrollable muscle twitches. He also noted the hereditary nature of the disease and the common symptom of mental health problems including suicidal tendencies and psychosis.

It wasn't until 1993, however, that a collaboration of scientists led by Geneticist, Nancy Wexler, discovered the Huntingtin gene that causes Huntington's disease.

Nancy was from a Huntington's family - her mother had the disease and her father was a clinical psychologist. This background urged her to study genetics and in 1979, Nancy took her first trip to Venezuela where generations of people had been affected by the disease with no care, consideration and a lot of stigma. For two decades, Nancy led medical teams into remote villages in Venezuela to take blood samples for research and offer support to the communities. Over time, Nancy coaxed scientists to collaborate and find the cause of Huntington's disease. She raised millions of dollars for research and in 1993 Nancy, alongside a team of elite scientists, revealed that they had finally discovered the Huntingtin gene.

Nancy Wexler at APPG - 2006

Our patron, Princess Diana

The UK remembers Princess Diana fondly for many reasons, her philanthropy being one. In the summer of 1993, the Huntington's Disease Association were lucky enough to have the Princess agree to be a patron for a time and have her visit our, then London based, offices.

The edition of our magazine from the same year states that the Princess planned to make at least four official appearances or visits on behalf of the charity. At this point, she had already made one unofficial visit to the head office in Battersea.

Then director of the charity, Mark Payne said "We were told that the Princess just wanted pop round for a cup of coffee. So that's what she did."

On her visit to Battersea, Diana met all members of staff and volunteers and discussed the organisation and the issues that those affected by Huntington's disease face. Mark Payne stated, "Her great concern about the effects of the disease and the plight of sufferers was obvious to all".

According to the 1993 issue, it was clear that Diana already knew a lot about Huntington's disease that she had learnt not only from reading books or leaflets but prior to her meeting with the charity, Diana had visited the Royal Hospital and Home in Putney to meet and talk to patients with Huntington's.

John Heald, former chairman of the Huntington's Disease Association said "We are naturally absolutely delighted by Her Royal Highness agreeing to be Patron. It provides even more recognition for the Association and all those who work so hard for it. Her care and understanding will be a great source of support to those affected by the disease."

(Above) A reception at the Merchant Taylor Hall in London

(Below) Meeting Chairman John Heald

The Princess signs an official photograph of herself

Greeting charity Fundraising and Marketing officer Freda Tozer

My testing journey

by David Holley

My name is David Holley. I'm a driving instructor of 12 years living and working in Sunderland. I live with my wife Judith, we have been together 20 years and been married for four years.

My father was diagnosed with Huntington's disease when I was around 19 years old. It really hit me hard in terms of coming to terms with the illness and the possibility of inheriting the disease from my father, as well as seeing my father go through the disease.

My brother Ian and I both received counselling from excellent genetic counsellors. My brother took the decision to be tested immediately and thankfully tests came back showing he didn't carry the Huntingtin gene. Although very pleased for my brother, my emotions were mixed. I thought, 'did his negative result mean there was more chance I would test positive?' even though I knew this wouldn't be the case. I made the decision to not get tested and tried to live my life to the full, all the while seeing my dad decline physically and mentally as the years passed and always having the possibility of going down the same path in the back of my mind.

David's dad

As years passed, I met my now wife Judith in my early 20s, whose support and understanding was invaluable from the day I told her my Huntington's story. It wasn't until I was in my late 30s that the 'not knowing' became worse than having to deal with a positive Huntington's result. I spent most of my late 20s and 30s analysing everything I did, from the way I spoke, a trip on a paving stone, to a dropped teacup or a forgotten appointment.

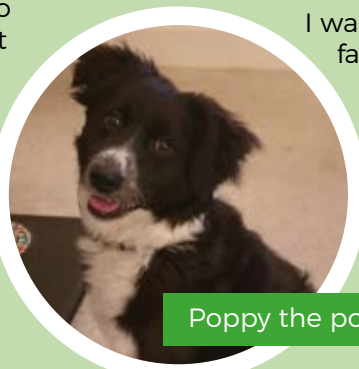
I had gone around 10 or 15 years without speaking to the Huntington's disease counsellors and decided I needed a little help from them. After several sessions with the counsellors, which really helped to set things clear in my mind, I decided to be tested for the Huntington's gene, and my decision to do so, although not taken lightly, felt like the right decision at the right time for me, due to the 'not knowing' seemingly taking over my life.

The wait for the result took a few weeks and in that time I went on a holiday to France that I had booked before deciding to get tested, although it was a holiday that I didn't really enjoy due to the test result awaiting me when I would return.

The day I went to get the result was a difficult day, I knew no matter what the result Judith would support me. I received a negative result meaning, thankfully, I didn't have the gene. It felt like the weight of the world had been lifted from my shoulders and I could live my life without the cloud of Huntington's disease constantly in my thoughts. It took a few weeks to actually sink in that the disease would not continue in my family and that as a family, we were free of the disease and we could concentrate on caring for my dad.

My dad has since passed away after a long battle with Huntington's, but I also feel that I wouldn't be the person I am today without going through the process of the disease with my family.

Since my result, I got married to Judith, which I thought was about time as we'd been together for 16 years, she'd been so supportive throughout and she's the love of my life. We have since tried to holiday as much as possible and now have a little puppy called Poppy that we rescued from Romania.



Poppy the pooch

Banger in action

I also took part in a charity 'banger rally' to raise money for the Huntington's Disease Association, where me and my brother, Ian, drove a car worth £200 from Sunderland to Benidorm, raising over £500 in the process.

Huntington's disease is a very difficult thing to go through and without the support from my family and the amazing help, understanding and support from the genetic counsellors it would've been a real struggle.

I'm still hopeful that with the excellent scientific work that is done behind the scenes a cure can be found.

I wanted to help in any way with families going through the same illness and feel my story, although a positive one, may help someone going through the same experience.

2000

The Huntington's Disease Association goes digital and Juvenile Huntington's takes the lead

In the year 2000, as the millennium hit and the world didn't actually end, the Huntington's Disease Association went digital as we invested in our first website.

The year 2000 issue of our magazine stated - 'We are a technology-friendly charity that has gone with the times and put ourselves on the internet! With so many people using the internet today, it was imperative that we kept up to date, moved with the times and went online.' Below you can see the progression of our website from 2000 - 2021.

By 2005 the charity was growing, our digital impact was improving and we decided to put a special focus on Juvenile Huntington's disease with our first ever JHD Weekend. The Huntington's Disease Association has always taken the lead when it comes to Juvenile Huntington's - we have the only dedicated Juvenile Huntington's Disease Adviser in the world and have been proudly hosting this weekend for many years, giving children with the disease an opportunity to experience rock climbing, abseiling, canoeing and more! Our JHD Weekend allows families to join together and enjoy these experiences. Our Saturday night fancy dress parties go down in history.

To find out more about Juvenile Huntington's, please read on for a useful and informative article on the ins and outs of the disease.

2000

2005

2010

2014

2021

JHD Weekend 2005

Understanding Juvenile Huntington's disease

Juvenile Huntington's disease is basically the early onset of Huntington's disease. As Huntington's is a hereditary disorder, children of those who carry the Huntingtin gene have a 50% chance of inheriting the disease. In some very rare cases, this appears in the juvenile form and affects children and young people up to the age of 21. About 5-10% of all people with Huntington's disease have juvenile-onset. This can make dealing with the disease an isolating experience for the young people, their families and professionals involved with their care including teachers.

Why do younger people get juvenile-onset Huntington's disease?

The gene responsible for causing Huntington's disease is called the Huntingtin gene. We all carry two copies of this gene whether affected by Huntington's or not. The Huntingtin gene is made up of a DNA segment called CAG which repeats itself within the gene. Those who are not affected by Huntington's disease will have a CAG repeat of 26 and lower whereas those with 40 CAG repeats and above will develop Huntington's disease at some point in their lives. However, when you reach 60 plus CAG repeats you enter into the realms of Juvenile Huntington's disease. Therefore, the CAG expansion within the inherited Huntingtin gene dictates whether someone will develop early-onset Huntington's disease or not. It cannot be predicted what CAG expansion people will get if they inherit the gene.

Signs of Juvenile Huntington's in young people and children

Juvenile Huntington's affects people slightly differently compared to Huntington's disease. Therefore, some of the common signs and symptoms may vary. Some typical early signs and symptoms of the disease include:

- Seizures (In 25-30% of cases, there is a tendency to epileptic seizures - something almost never seen in adults with Huntington's.)
- Stiffness of the legs
- Clumsiness of arms and legs
- Decline in cognitive function
- Changes in behaviour
- Speech problems
- Behavioural disturbances

Many of these symptoms will be noticed during education as things like speech issues, behavioural problems and general changes in behaviour can be more obvious in a school environment to teachers and other members of staff.

Diagnosing Juvenile Huntington's

The diagnosis of Juvenile Huntington's can be tricky. In the UK, you cannot test for Huntington's disease until you are 18 years old or over. It is deemed against someone's rights to test them for the disease without their consent and being of age. Therefore, testing children and people under 18 must be done with caution and only if medical professionals are sure they're showing signs of Juvenile Huntington's. This means that behavioural or speech problems may not warrant testing and can sometimes lead to a lengthy and frustrating diagnosis process. Our Specialist Juvenile Huntington's Disease Adviser, Helen Santini, can support and advise through the whole process. If you're affected by Juvenile Huntington's and need support, please contact Helen at **helen.santini@hda.org.uk**.

Helen Santini

Support for those affected by Juvenile Huntington's disease

There are many professionals who can offer help to someone affected by Juvenile Huntington's disease and their families. These include:

- Neurologists
- Physiotherapists
- Occupational therapists (OT)
- Speech and Language Therapists (SALT)
- Dietitians
- Palliative care teams

Other forms of support that those affected by Juvenile Huntington's disease can access range from local council support to NHS funding and branch and support groups. To learn more about these services, please visit our website and navigate to 'Getting help' then 'If you have Juvenile Huntington's disease and finally, 'Getting the care you need'.

Juvenile Huntington's disease and school

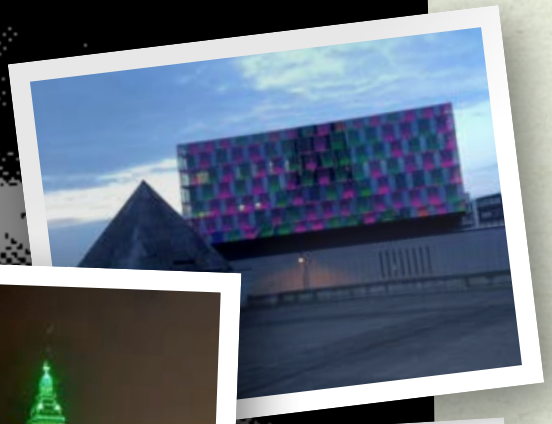
All schools are required, under the Equality Act 2010, to make "reasonable adjustments" for disabled children. These can include anything from adding ramps or lifts to providing extra support and aids such as specialist teachers. It is the responsibility of local councils to ensure these adjustments happen.

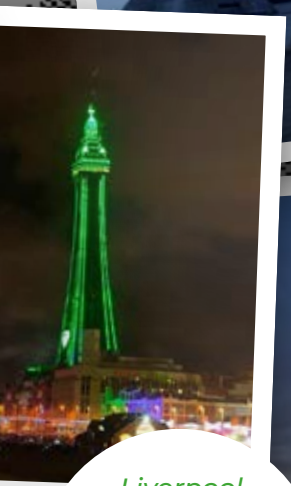
As Juvenile Huntington's is such a rare disease, teachers are unlikely to have encountered any pupil with it before. They may need some guidance on how best to help. Further information and resources can be found by visiting our website, navigating to 'Getting help' then clicking 'Information resources' and 'Guides, leaflets and forms'.

There are other things on offer to those affected by Juvenile Huntington's along with certain things to consider such as choosing the best school for the child, getting emotional support and getting the most out of life with Juvenile Huntington's. You can learn more about this support by checking out our 'If you have Juvenile Huntington's disease' webpage by visiting our website, navigating to 'Getting help' and then clicking 'If you have Juvenile Huntington's disease'.

2016

We #LightItUpForHD, honour our fundraisers and work with the BBC





Liverpool Engineering Building, Blackpool Tower, and Spinnaker Tower

2016 proved to be a time for firsts and an exciting year for the Huntington's Disease Association. We joined the global #LightItUpForHD awareness campaign for the first time, hosted our first ever Volunteer Fundraiser Awards and consulted on a Huntington's disease storyline in the popular BBC TV show, Casualty.

#LightItUp4HD

A campaign initially started by the Huntington Society of Canada, #LightItUp4HD is an awareness campaign that calls on buildings to light up in Huntington's charity brand colours throughout Huntington's Disease Awareness Month in May. In 2016, we joined #LightItUp4HD and started our own campaign called 'Shine a light on HD' in which we shined a light on all those affected by Huntington's disease in England and Wales. Many buildings lit up in the pink and green colours of the Huntington's Disease Association, including Blackpool Tower, Emirates Spinnaker Tower in Portsmouth and the Liverpool Engineering building.

Please see page 20 for this year's Huntington's Disease Awareness month plans.

Celebrating our incredible volunteer fundraisers

The Huntington's Disease Association has always been so proud and thankful for our volunteer fundraisers. Without them, we could not carry out the work we do. In 2016, we decided that the time had come to throw them a real celebration and, in honour of Volunteers' Week, hosted our first ever Volunteer Fundraiser Awards.

BBC Radio Merseyside presenter, Roger Phillips, hosted the evening presenting two awards, 'Most Inspiring Volunteer' and the 'Friend of the HDA' award. The winners were voted for by the public.

Volunteer Awards 2016

Casualty and Huntington's disease

In 2016, BBC One ran a storyline on Casualty based on Huntington's disease. The plot saw adopted brothers and doctors at the hospital, Cal and Ethan, reunite with their biological mother Emilie who was affected by Huntington's disease. The storyline followed Cal and Ethan's emotional journey through the genetic testing process. The brothers get to know their mother better as Huntington's disease slowly takes hold. The story culminates with Emilie sadly passing away and the brothers coming to terms with the fact Ethan carries the Huntington's gene.

We were delighted to work with the researchers and scriptwriters on Casualty to ensure that Huntington's disease was accurately portrayed. The Casualty script team appreciated and recognised the importance

of correctly portraying the disease and the impact it has on those affected. With this in mind, BBC One researchers reached out to us at the very beginning of script development, advice was then provided by our Chief Executive, Cath Stanley, over a number of months to ensure an accurate depiction.

This was an incredible way to raise awareness around Huntington's disease with viewing figures for these specific episodes reaching over five million households in the UK, that's 10% of the UK population!

Now in 2021, The Huntington's Disease Alliance UK and Ireland, consisting of Huntington's disease charities representing England and Wales, Scotland, Northern Ireland and the Republic of Ireland, have teamed up to develop a national awareness campaign entitled Family Matters. This will run throughout Awareness Month this May. You may spot some familiar faces fronting our campaign as George Rainsford who played Ethan in Casualty has kindly agreed to be a celebrity ambassador, devoting time to doing all he can to raise awareness of the disease. Thank you, George! You can learn more about Family Matters and how to get involved on page 20.

Shots from Ethan's Huntington's storyline on Casualty

2017

Huntington's disease and the Pope

In 2017, the largest global gathering of the Huntington's community visited Vatican City for an audience with Pope Francis. This was the first time in history that such a prominent world leader recognised the difficulties faced by those affected by Huntington's disease.

The trip to the Vatican was arranged by HDdennomore, a global coalition of advocates dedicated to raising awareness of Huntington's and ending the stigma around the disease. HDdennomore is headed up by ex-war reporter Charles Sabine, who has Huntington's disease.

HDdennomore's website states 'The global gathering was inspired by the plight of families from South America most ravaged by HD. Families from Colombia, Venezuela and Argentina travelled to meet with Pope Francis and join an audience of thousands at the Vatican to drive awareness of HD and to lift the stigma around the disease.' HDdennomore also invited representatives from Huntington's charities in the UK, our Chief Executive, Cath Stanley attended alongside several family members that we supported at the time.

"May none of you ever feel you are alone" was the powerful message given by Pope Francis to the Huntington's community. Kevin and Gloria Orrick from Liverpool were among the families from the UK who were blessed by the Pope. Gloria said: "When our son Kevin was diagnosed with Huntington's it was devastating news, both for Kevin and also the family. Learning about the trip to the Vatican was brilliant. It means the world to us and Kevin, as it provided us with the chance to meet other people who are affected by Huntington's disease. It is a constant reminder that there are other families in the same situation as us. It gives Kevin and our family hope and faith for the future. No one should have to face Huntington's alone and it is important for everyone to reach out for support."

This was a huge step for the Huntington's community around the world. HDdennomore made a documentary about the trip entitled 'Dancing At The Vatican' which premiered early 2020 in London. You can watch the full film by visiting dancingatthevatican.com.

Cath Stanley (Chief Executive) greets the Pope

Pope Francis blessing Kevin Orrick

Cath Stanley (Chief Executive), Theresa Westhead (Specialist Adviser) and Emma Burnip (Huntington's researcher) meet Pope Francis

2019-2021

The GENERATION-HD1 and WAVE trials halted and GEN-EXTEND trial paused - Why and what's next for Huntington's disease research?

Professor Hugh Rickards

Professor Hugh Rickards with Huntington's disease patients

In 2019, Roche and Ionis began their GENERATION-HD1 trial. A trial that invited those with early Huntington's disease symptoms to put themselves forward to be injected with the antisense oligonucleotide (ASO) drug, tominersen also known as RG6042.

ASOs are a short string of DNA like molecules that can stick to huntingtin RNA and stop the huntingtin protein from being produced. RNA is a message molecule, based on DNA, used by cells as the final set of instructions for making a protein. The aim of this trial was to show whether tominersen could slow down the progression of Huntington's or improve symptoms for people with the disease.

Now in 2021, we were hit with the difficult news that Roche and Ionis had halted GENERATION-HD1 and paused their open-label extension study, GEN-EXTEND. WAVE Life Sciences also ended their Huntington's trial within weeks of the news from Roche. This came as a shock to the Huntington's community and research professionals worldwide. So, why have the trials been halted and what does this mean for future Huntington's research? We reached out to Neuropsychiatrist, Professor Hugh Rickards for some answers.

"I came to work a couple of weeks ago to see a patient for the Roche/Ionis trial, GEN-EXTEND, only to find out it had been "paused" the previous evening. My patient's husband had discovered this on HD-Buzz before me and the other members of the research team. Naturally, this was a blow for the whole community. As the days have passed, a list of questions came up, some of which I'm going to try and answer here."

Why were the trials halted?

"Firstly, there are some questions about the process. All clinical trials should have a 'data monitoring committee'. These are a small group of people, independent from the company (Roche/Ionis), who look at the data at different periods throughout the trial. Only they can see the data divided into groups according to who has had which treatment. As we now see, these are a pretty powerful group of people and it's a really good thing that they are independent of the company.

I've been able to analyse some of the data that the committee looked at and it's pretty clear that the groups which were receiving the active treatment (tominersen) were gradually, but clearly, starting to go downhill compared to the group who had been administered the placebo. It looks like the more often treatment was given, the larger this worsening

effect appeared to be. My best guess at the moment is that the treatment is still doing what it's supposed to do (lowering mutant huntingtin protein) but it may be doing some other, less helpful, things at the same time. There are a lot of possible reasons for this. It's possible that the huntingtin protein was lowered too much or that the treatment caused some inflammation in the brain. I don't think we'll know the full answer to that for some months.

Some people (including me) were puzzled and frustrated that the instruction to stop dosing in the trial was publicised with no advanced warning to the Huntington's disease community. The reason the Roche/Ionis gave for this was to prevent people with prior knowledge from taking advantage of that knowledge on the stock market. I do understand that position, although it is very frustrating.

The WAVE Life Sciences study has also stopped but at this stage, I don't have information to be able to explain the reasons for this. The only thing I can say is that it's not related to GENERATION-HD1. The fact that these two things happened at the same time is a coincidence."

What did we learn from these trials?

"Amidst all of this difficult news, it can be hard to hold on to the fact that we've still accomplished something new and radical in Huntington's research; we've successfully reduced the amount of mutant huntingtin in the brains of humans with Huntington's disease. It's still possible that there may be a dose of tominersen which will lower huntingtin enough without any of the "downsides" we've seen in the GENERATION-HD1 and GEN-EXTEND studies. There's more data to come on this."

What does the future hold?

"Roche and Ionis are going to look further into their data from the GENERATION-HD1 and GEN-EXTEND studies and they will have to make a decision about whether or not to start another trial of their drug (perhaps at a lower dose or a variety of lower doses).

Uniqure has released preliminary information about the first ten patients to receive their drug directly into the brain. This drug (AMT-130) only has to be given on one occasion and, so far, it's been well-tolerated in patients, who have to undergo an eight to ten-hour operation. Animal models show that this drug can reliably reduce the huntingtin protein. There are also a number of other products in the pipeline that can be given into the brain or spinal fluid.

Beyond that, there are a range of drugs that can be given by mouth currently in development. Pridopidine, which acts to protect nerve cells, is being re-tested in a study called PROOF-HD, including in some sites in the UK. Another drug, Branaplam, which has been used in some other similar neurological disorders, is being tested in people with Huntington's disease now. Elsewhere in the pipeline drugs are being developed which directly target the DNA rather than the messenger, as the Roche/Ionis product does. There are also drugs in development that target other genes apart from the huntingtin gene - genes that seem to modify the effect of the huntingtin gene and so they are useful targets.

Ideas about treatment in Huntington's have revolutionised in the last decade. For the first time, we have a queue of drugs which all have the potential to modify the disease and a wide range of major biotechnology industries working with the scientific community. This was barely imaginable ten years ago. However, there's a mismatch between the speed of change that scientists experience (which seems incredibly fast) and the speed as experienced by patients and families (progress is still really slow in the context of an individual's lifespan). It's probably the time for some reflection, more understanding and considering what lessons we have to learn. We also have to grow in our understanding of how biotechnology and pharmaceutical companies operate (something which is relatively new for us) so we can maximise the benefit from their work."

A huge thank you to Hugh for providing us with this enlightening information and giving a much better insight into why these trials ended and what other trials we have to look forward to. As Hugh says, it is difficult to keep positive in times of disappointment like this, however, we did learn a lot from the Roche/Ionis trials and there is lots of other impressive research going into Huntington's disease around the world.

If you are affected by Huntington's and need support or advice, please contact us on **0151 331 5444** or email **info@hda.org.uk**.

National awareness campaign for Awareness Month

We are extremely excited and proud to reveal that we're working as part of The Huntington's Disease Alliance UK and Ireland which consists of Huntington's disease charities representing England and Wales, Scotland, Northern Ireland and the Republic of Ireland. This awareness month, we will be bringing you a national awareness campaign entitled Family Matters.

What is Family Matters?

Funded by pharmaceutical company Roche, Family Matters is running throughout Awareness Month in May and aims to raise awareness of Huntington's disease across all five countries. The alliance alongside our handpicked PR company, M & F Health will work hard to raise awareness in a variety of ways, including -

- The Living History project - a digital space for the Huntington's community to share pictures, words and thoughts on how they feel about living with the disease
- A large social media campaign
- Celebrity ambassadors
- The creation of videos that feature interviews with Huntington's disease families
- Surveys to gain important data from the Huntington's community
- Conversations with decision-makers
- The launch of a campaign website
- Raising awareness by sharing information with the press

How can you get involved?

You can get involved in Family Matters by sharing your story to the Living History project. We want you to contribute in a way that is meaningful to you. You can contribute as an individual or as a family. You can send us a photo, some of your own words, a poem, a drawing, or something else altogether!

If you'd like to contribute, please email our PR agency M & F Health at **huntingtons@mandfhealth.com** with your story.

To learn more, visit our Awareness Month webpages by accessing our website, navigating to 'Get involved' and clicking on 'Awareness Month - May 2021'.

New faces of the Huntington's Disease Association

We have been lucky enough to hire two new staff members this year. A Social Media Officer who will be running our social channels and a new Operations Assistant who will be assisting our Operations Team with answering phone calls from the Huntington's community and general administration duties. Keep reading to learn a little about them both and why they chose to work for the charity.

Jordan Barbrooke -
Social Media Officer

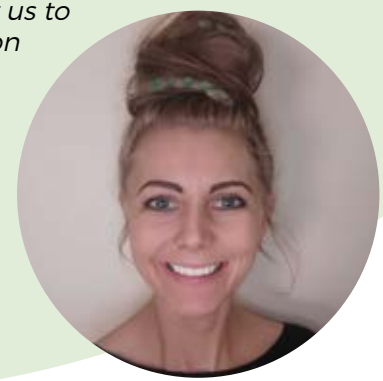
"Moving into the charity sector has been an ambition of mine for a while. I have worked in social media for a number of years but decided that I wanted to use the platforms in a way that was rewarding for both myself and those reading what I share.

This job really stood out to me and ahead of my interview I had already learnt so much about Huntington's disease but was eager to learn more. My head was filled with ideas, so I knew that it was the job for me.

My first month here has been nothing short of amazing. I am an inquisitive character and the other members of the team have taken the time to answer all my questions and educate me about the disease.

I am really excited to get fully involved with the charity, to help increase awareness of the disease, share the stories of those affected by it, and also to highlight the amazing things that the Huntington's community do to raise valuable funds. Social media is a powerful tool and is really important for charities. It provides a platform for us to get important information across, it brings the community together and it is a place where people can talk to one another and feel a little bit less alone."

Social Media Officer -
Jordan Barbrooke



Laura Shaw -
Operations Assistant

"My name is Laura, I have recently relocated from Stafford to the lovely city of Liverpool. In my previous role, I worked within the programmes team for a charitable organisation. Through searching for job roles in the Liverpool area, I came across The Huntington's Disease Association. I quickly found the website and social media pages and saw what an incredible organisation it was. The role of Operations Assistant enables me to be part of helping so many people and their families. I jumped at the opportunity and hit send on my application. I'm really excited to get to know the team and to get stuck in my new role.

In my spare time, I enjoy knitting, building Lego and fantasising about pets I don't yet own!"

Operations Assistant
- Laura Shaw

Happy 50th anniversary to the Huntington's Disease Association

2021 is the Huntington's Disease Association's 50th Birthday! It has been 50 years since the first group of people seeking support for Huntington's disease gathered and decided to start a charity. Later in the year, we will be celebrating this in an array of ways. So far, we have decided on a celebratory pin badge that will be available for purchase through our shop. So! Keep an eye on our website and social media in the coming months for more information on how the whole Huntington's community can celebrate our 50th anniversary.

#HDHike

Around 8000 people in the UK have Huntington's disease with a further 32000 estimated at high risk of inheriting it.

This year we're hosting a fundraising initiative called #HD8000 aimed at raising funds for our work, ensuring that we can continue offering support to people affected by Huntington's disease. Throughout the year, we're holding different and exciting fundraising challenges.

In May, we're calling on supporters to step forward for the 8000 people living with Huntington's disease and join us for our new challenge #HDHike.

There are different ways to get involved in #HDHike, you can organise your own socially distanced 8km sponsored walk or you can set yourself a challenge of covering 8000 steps per day throughout May.

All we ask is that you aim to raise £100 during #HDHike to help us continue our work supporting people affected by Huntington's disease.

To take part, simply register by visiting our website, navigating to 'Get involved', then 'Fundraising' and clicking '#HD8000'.

Digital Fundraiser Volunteer awards

This year, we're hosting a Digital Fundraiser Volunteer awards to celebrate the brilliant fundraisers who took on amazing challenges in 2019 and 2020 in support of the Huntington's Disease Association.

The awards ceremony will be taking place online on Friday 11 June 2021, 3.00 pm - 5.00 pm. It is open to all and information on how to attend will be released at a later date.

Please visit our website, navigate to 'Get involved' and click 'Digital Volunteer Fundraiser Awards' to learn about the nominees for this year.

Jo Hague, Eli and Isaac Greer and Rosie Allen, winners of our 2019 awards

Save the date

Understanding Huntington's disease - a certificated course for professionals

When - Monday 21 June 2021 to Wednesday 23 June 2021
Where - Online
Registration deadline - Friday 11 June 2021

This three-day course is aimed at health, social and care professionals working with people affected by Huntington's disease and offers a unique learning and networking opportunity. The course will help provide a greater understanding of the disease, an opportunity to share experiences and ideas and discuss the management of complex situations.

Leading Huntington's disease experts will present online over three days on topics such as genetics, neuropsychology, psychiatry, physiotherapy, occupational therapy, speech therapy and palliative care. On finishing the course, attendees will receive a certificate confirming completion of training approved by the Huntington's Disease Association.

If you are a Huntington's professional who is interested in our course, please visit the Events section of our website for further information and to see the registration process.

If you are a family member who knows a professional who may benefit from this course, please pass along course details to them.

We also hold regular virtual carers meetings, Juvenile Huntington's disease Zoom meetings and online coffee mornings, please contact us on **0151 331 5444** or email **info@hda.org.uk** for more information on these.

Young adults event

When - Thursday 20 May 2021, 6:00pm - 8:00pm
Where - Online

Our Young Adults event will be held online and is aimed at young people aged 18-45 years old. This two-hour evening event is open to people living at risk, people who have had a positive test and their partners. The session will include personal journey stories, smaller group discussion sessions and updates from the recent Huntington's Disease Youth Organisation congress.

Please visit the Events section of our website to learn more and register for this event.

Family event and AGM

When - Saturday 16 October 2021
Where - Online

We will be hosting our annual family event and AGM* which will include some keynote talks and workshops, but this year, due to the uncertainty of large social gatherings during the COVID-19 pandemic, we will be taking it all online. Further information will be released closer to the event date, so keep an eye on our website in the coming months.

*For official notice of our Annual General Meeting 2021, please visit our website, navigate to 'About us' and click 'Notice of Annual General Meeting 2021'.

Webinars

Since COVID-19 hit, we have expanded our digital resources, offering regular and informative webinars. These webinars cover an array of topics related to Huntington's disease and are always hosted by an expert in the field. Visit the Events section of our website to see any upcoming webinars and register today!

Looking back over years at the Huntington's Disease Association with Treasurer, Nick Heath

Nick Heath

Nick Heath has been the Huntington's Disease Association's treasurer for 30 years. Nick has watched the charity develop and grow into what it is today. In honour of this, we've asked Nick to provide an interview discussing his time at the charity and sharing his most memorable moments with us.

Why did you first join the Huntington's Disease Association?

"In the summer of 1991, I was approached by my firm's senior partner and asked whether I would become the treasurer of a small charity operating in Battersea. Apparently, it would only involve a few meetings during the year and a few evenings to prepare the annual accounts. Since I was about to marry his PA, I could hardly refuse!"

How has your role at the charity developed?

"When I first started, it was actually a baptism of fire since the charity was struggling to establish its role. There was no clear direction, the care home we had at the time and two local social workers who were attempting to support those with Huntington's disease were struggling to deliver support to the community. There was a major battle between those wishing to keep the care home, which had been established by a dedicated team, and those who believed it had served its purpose and was a drain on resources. The weakness in the governance of the charity was cruelly exposed by the decision to close the care home. I certainly learnt the importance of the Executive Committee agreeing on a course of action after this decision was made."

How has the charity grown since you first joined?

"As the treasurer, I have seen income grow from £1/4m to £1.75m so that the charity is able to do so much more for those we support. When Sue Watkin took the position of Chair, we had difficult decisions to make to implement change. We had to dispense with a top-heavy organisation and for some years the Executive Council ran the organisation without an admin team. As income grew, we had to invest in a structure to support the Advisory Service which we had conceived and established. I think the Advisory Service concept was key to the development of the Huntington's Disease Association as was moving the charity's head office to Liverpool and the appointment of Cath Stanley as Chief Executive.

Now we have a dynamic group of people dedicated to delivering, we have become more of a community supporting each other through the highs of gene discovery and drug trials and lows of treatment setbacks and income loss in recessions."

Nick Heath and Princess Diana at the offices in Battersea

What are your highlights of working with the Huntington's Disease Association?

"My highlights were, as you can guess, related to the patronage of Princess Diana. Her visit to Head Office was special and her personal contribution to individuals so apparent. After Diana moved on from being our patron, I kept the photograph taken at the offices in Battersea. When the news of her tragic death came through, I was lucky enough to be invited to the Princess' funeral. I accepted the invitation and walked into Westminster Abbey directly behind Margaret Thatcher ensuring immortality on the BBC video of the event. The emotional outpouring was extraordinary especially when the crowd outside clapped Earl Spencer's speech and the applause spread up the Abbey from the door in a wave. Being opposite Elton John when he performed was something to remember as was the procession of so many famous faces leaving after the service.

We also had a reception at number 10 Downing

Nick Heath meeting John Major

street hosted by John Major. To walk up the famous staircase with the pictures of past Prime Ministers was something special.

On a personal basis, the opportunity for my children to meet families affected by Huntington's disease has had a deep personal impact upon them, of which I am proud. It has inspired my daughter to take up a career in science after seeing what needs to be done."

What's in store for the future?

"Going forward we must maintain our care activity but also recognise the importance of communication and social media to maintain and enhance the influence, profile and success of our organisation."

A huge thank you to Nick for sharing his experiences with the Huntington's Disease Association, we hope it gives some insight into how the charity has grown over the years.

Here's to another 50 years of supporting friends and families!

Support us

We have a fantastic selection of Huntington's Disease Association merchandise available via our online shop. From practical water bottles and bags to cosy hoodies and pin badges to show your support, there's something for everyone! And, all the proceeds go to supporting our work. Simply visit our website and navigate to 'Shop' in the top right-hand corner to make a purchase. If you do not have access to a computer or the internet, you can give us a call on 0151 331 5444 to order over the phone.

Contact us

If you need advice, information or support about Huntington's disease please contact us on 0151 331 5444 or email info@hda.org.uk

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131 Mount Pleasant, Liverpool, L3 5TF

Website - www.hda.org.uk
Facebook - @hdauk
Twitter - @HDA_tweeting
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We'll be there

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50% off on face coverings

We are holding a mass sale on our awesome face masks and snoods! Now that restrictions are lifting, the masks and snoods are perfect for when out and about to protect you and others. The snoods are also great to wear around your neck for that little bit of extra warmth.

Masks - Were £4 now £2
Snoods - Were £10 now £5

To purchase a face covering, visit our website and navigate to 'Shop' in the top right-hand corner to make a purchase. Alternatively, you can give us a call on **0151 331 5444** to order over the phone.

Why your donation is so important

COVID-19 has had a big impact on the way we support people affected by Huntington's disease, but COVID-19 doesn't mean Huntington's disease stops. Our advisory service has seen an increase in demand with our staff working with multiple agencies to ensure families receive accurate and up-to-date advice and get the support and care they need. Despite not being able to meet in person, we are still there to provide support to those that need us.

"The Zoom meetings each month have been fabulous in bringing our support group together. My husband was admitted to a care home just before the first lockdown and I have only seen him twice since then. I had to admit that I couldn't care for him any more and my Specialist Adviser has helped me come to terms with this over the months. I now feel less guilty and I'm able to support others through our support group meetings who are in a similar position to me."

Your donation today will help us continue providing advice and support through our Specialist Advisory Service, informative and educational website content, supportive social channels, welfare grants and much more to anyone affected by Huntington's disease. You can donate by using the button below, by visiting the homepage of our website or by calling us on **0151 331 5444** or email **info@hda.org.uk**.

Donate here