



## Welcome to the Team

We're delighted to welcome two new members to the HDANI team.

First up we have our newest trustee Laura Kane who brings a combination of HD family and professional policy experience to the board. We asked Laura to tell us a little about herself;

I am delighted to have the opportunity to become a Trustee at HDANI. I have a close family connection to Huntington's Disease, with all four on my mother's side of the family succumbing to this illness. I have also been through the testing process, participated in the HD-YAS medical research study and done fundraising events in my workplaces.

My career to date has been based around consumer law and consumer protection and in the last five years has been in policy and strategy. This has given me experience in working with Board members to achieve good policy decisions and outcomes, which has sparked my interest to become a member of a Board myself.

Naturally I have gravitated towards HDANI due to my connection with the illness and the way in which this charity has supported my family/others affected by the illness over the years. I am looking forward to using my professional skills and personal experience to help further the objectives of HDANI.

In my spare time you'll find me studying for a course in therapy, or at the beach with my dog, sometimes taking a dip in the sea if I'm brave enough!



## How many people in Northern Ireland have Huntington's Disease?



There are approx 5  
times as many people  
at risk of inheritance

HUNTINGTON'S  
DISEASE ASSOCIATION  
NORTHERN IRELAND  
*Families at the heart of all we do*



### Contact Us:

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Zelie Clark/Senior Family Support Worker  
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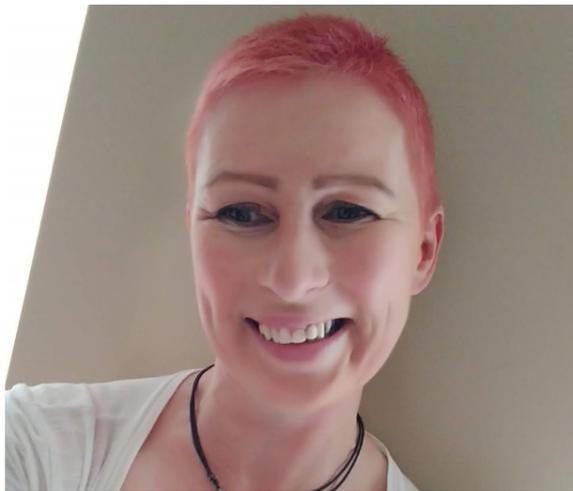
[www.hdani.org.uk](http://www.hdani.org.uk)  
<https://hdfamilymatters.com>



@HDANorthernIreland



@HDAAssocNI



Next we have Glenda Burns who joins us as our new part time Family Support Worker. Thanks to the generous support of a number of funders, in particular The National Lottery People & Community Fund and Garfield Weston, we have been able to extend our support team. Glenda comes to us with a fantastic range of skills in health, social care and wellbeing.

Glenda has over fifteen years of experience working in health promotion and education and also five years' experience working in family support type roles. Glenda has a Postgraduate Diploma in Health Promotion and a wide range of health care and health coaching qualifications and training. Glenda's role includes providing information, emotional and practical support to those affected by HD. She will be working from home from Monday to Wednesday. You will get a chance to meet her at the virtual support groups and she will be reaching out to families to introduce herself once she gets settled in. She can be reached on [glenda@hdani.org.uk](mailto:glenda@hdani.org.uk) and [07921513561](tel:07921513561)

Congratulations!!

**Who do HDANI connect with?**

We are determined to reach as many people as possible to provide services to HD families, raise awareness & improve standards of professional care

As of 29th April 2021

<b>135</b>	People living with Huntington's disease in NI	How many people in Northern Ireland have Huntington's disease? It is estimated that there are 285 people with HD and 1035 people at risk of inheritance.
<b>648</b>	Other HD family members in NI	
<b>3687</b>	Health & Social Care staff	Can you help us grow our numbers? <a href="mailto:info@hdani.org.uk">info@hdani.org.uk</a> <a href="tel:07733935863">07733935863</a>
<b>1473</b>	Other Service Providers & Organisations	
<b>6021</b>	Followers on our Facebook, Twitter, YouTube, Instagram	

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Ashley Clarke has been with us in some capacity for around ten years, as a service user, a volunteer, fundraiser, trustee, intern and most recently as a Youth, Events & Fundraising Officer. We have been so lucky to have her on the team and it's been a pleasure to watch her skills and knowledge develop as she gained her qualifications in Events Management. When her one year contract was coming to an end the board decided they couldn't lose her and appointed her as a permanent member of staff as Youth, Awareness & Fundraising Officer. This involves providing information and opportunities to connect for our young people as well as planning and hosting events. It also means working with other people and organisations to deliver specific support and workshops to our youth group. On the awareness side of things Ashley has already been busy helping us grow our database of professional contacts allowing us to deliver resources and training to health and social care staff across Northern Ireland which will mean greater understanding and better care for patients and their families. The awareness work also involves networking with other organisations and groups, helping with social media, working with journalists and running projects like [#LightItUp4HD](https://www.instagram.com/lightitup4hd). If you are interested in fundraising she can help by providing resources and advice to make the most of your hard work.

You can reach out to Ashley Monday-Friday on [ashley@hdani.org.uk](mailto:ashley@hdani.org.uk) or [07810330949](tel:07810330949)



Having been with us for over three years, Zelie, has proven to be an invaluable asset bringing a wealth of personal and professional experience to the role she has undertaken every opportunity to learn and develop a strong understanding of Huntington's disease and how it affects the whole family. In recognition of her expertise, commitment and work to date she has recently been appointed to the role of Senior Family Support Worker. I know she has been a great source of support, advice and advocacy to many of you and will prove to be a great port of call for the new staff joining us in the coming months. We were also delighted to support Zelie with her continued studies and congratulate her on her acceptance to a Diploma in Social Work at Queen's University starting this September. All our staff will also be undertaking a specialist course at University of Stirling entitled and Enabling Approach to Huntington's Disease which will be delivered remotely over 14 weeks by staff at the university and colleagues at the Scottish Huntington's Disease Association. You can contact Zelie Monday- Friday on [zelie@hdani.org.uk](mailto:zelie@hdani.org.uk) or [07733935863](tel:07733935863)



## Report from the CEO

*Hi to all our service users and friends,*

It has been a difficult and scary time for many of you over the past year but hopefully, with the roll out of vaccines, the end is in sight. While vaccines offer some degree of reassurance and will allow the world to return to a degree of normality, a lot has still to be learnt about how effective they are at preventing infection and transmission so we urge you to continue to follow public health advice on social distancing, cough and hand hygiene. It is unlikely that HDANI will return to hosting face to face gatherings for the remainder of the year but we will continue to work as hard as ever providing online support, telephone advice and information to our HD families as well as training to our health and social care professionals. Over the past 6 months over 500 health and social care staff have received training on Huntington's disease via Zoom and follow up resources, something that we could never have imagined pre-Covid. Unlike a lot of charities, HDANI, have been able to continue to deliver our services throughout the pandemic and I know many of you have expressed how much you appreciated the support and advice you received as well as care packages and check ins. We have been so lucky to have Zelie who works incredibly hard on your behalf to ensure you get the best help possible. Ashley has been worth her weight in gold this past year too and has been made a permanent member of staff as our Youth Events, Awareness & Fundraising Officer. One of the things she has been working on as fundraising quietened down was building up our contacts database so we can ensure the information we have on families is up to date but also that we can reach more and more health and social care staff and provide them with information about HD. You will see what she has been working on with the youth later in this newsletter.

We have received a mixed bag of news with regard to drugs trials some bad but also some good and you can read about the latest news in our research section. I have been busy engaging with policy makers and officials in the Dept. of Health to encourage them to expand neurological services to take into account new drug developments that may come in the next five years to ensure that the equipment and

staff are there so that people in Northern Ireland can access these treatments in the same way as people in other parts of the UK. On a lighter note, some of you have been taking part in our exploring ways to improve the lives of people with HD through the use of weighted blankets and virtual reality gear and we look forward to sharing the findings in due course. Others have been learning about the benefits of mindfulness and self-care through our Covid packs, and many of you have been enjoying our chair yoga sessions with Natalie. Zelig is currently busy planning the next years' worth of virtual support group sessions so if you've any ideas of talks or activities you would like to try please drop her a line. Similarly, Ashley would love to hear from you with any thoughts on what our children and young adults would like to do virtually and once we can meet up again.

As I've explained before, a lot of my job involves trying to identify and apply for appropriate funding which involves designing and planning services, gathering evidence of need and proving our ability to deliver. Although it has been incredibly stressful at time we have been very fortunate to secure almost £350,000 over the past few months. That is a huge amount of money and will allow us to both secure and expand our services over the course of the next three years. To fully complete our plans, we need to raise a total of half a million pounds which will cover all of our staff and service costs for three years. The design of the project is based on the evaluation we carried out last September where our families and the professionals we work with told us what was working well and what they wanted to see more of. In practice it will mean another full time and a part time family support worker, access to counselling and holistic therapies and more health and social care training sessions. We still have money to raise but have more than enough to continue with our existing work and hire new staff. So huge thank you to those funders who have contributed so far The

National Lottery Community Fund, ADDOR, Roche, Garfield Weston, Rank Foundation, NI Government Covid Fund, Alpkit, UniQure and the Belfast, Western & South Eastern Trusts Carers Funds.

In other news you will read about the huge HD Awareness campaign I've been working on with colleagues in Ireland, Scotland, England & Wales thanks to generous sponsorship from Roche pharmaceuticals. Thanks so much to those of you have contributed your time, stories and memories to help increase understanding about how Huntington's impacts on families. If you've not had a chance to submit contributions you can still help massively by sharing our stories across social media, or simply showing to friends, family and colleagues. It might provide to be the icebreaker you've been looking for to help explain HD to someone.

On a personal note I'll be taking a break from HDANI for a while as we prepare to welcome a little sister for Sáerla. It's comforting to know that the future of HDANI is financially secure, that our families will be supported, that progress is being made on improved services and that the board will ensure things continue to tick along, while I'm off.

I look forward to seeing you all soon,

**Sorcha**  
Sorcha McPhillips  
Chief Executive



Sorcha McPhillips  
Chief Executive

## HDANI Support Group

Over the last year, we have been keeping our services users in touch through WhatsApps groups and amalgamated the Belfast, Newry, Omagh and Maghera HDANI support groups' to run virtually on zoom. Keep an eye on our Facebook page to keep track of dates <https://www.facebook.com/HDANorthernIreland>.

This group is open to anyone living in NI who is affected by HD, the group provides an opportunity to come together, share experiences, support each other, get involved with arts and crafts and a monthly quiz, as well as learning more about HD in a relaxed and confidential setting. We end each session with a mindfulness relaxation exercise. We also run a monthly HD Chair Yoga Session with Natalie from Sacred Mountain Yoga.

### MAY IS HUNTINGTON'S DISEASE AWARENESS MONTH

We are working with the Huntington's Disease Association NI to deliver self-management support to those living with Huntington's Disease in Northern Ireland



### Get involved with upcoming events.

Versus Arthritis are offering a 6 week condition management course that will help empower participants to manage their condition and its symptoms well.

The course will be running every week from 26th May to 30th June from 2.00 – 3.30 and will be delivered online using Microsoft Teams.

The course covers subjects including:

- Techniques to deal with problems such as frustration, fatigue, isolation, and poor sleep
- Appropriate exercise for maintaining and improving strength, flexibility, and endurance
- Appropriate use of medications
- Communicating effectively with family, friends, and health professionals,
- Nutrition
- Pacing activity and rest

The course is delivered by two trained volunteers who live with a long term condition themselves and through their lived experience they can help you to understand your condition more and set weekly action plans that work for you and your symptoms.

The course is open to anyone living in NI affected by HD. To sign up please contact Zelig our Family Support Worker **07733935863** [zelie@hdani.org.uk](mailto:zelie@hdani.org.uk)



### Easter Baking March 2021

HDANI Families and Youth all got involved with our virtual "Bake Off" making lots of tasty treats to enjoy over Easter.



## Christmas Crafting 2020

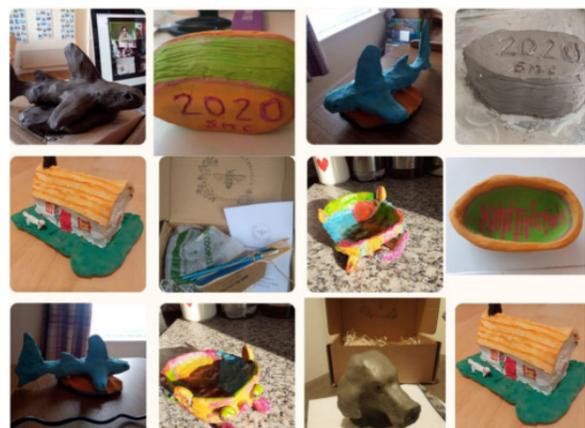
HDANI service users getting into the festive spirit at a virtual crafting session before finishing for Christmas.



Beautiful crafts made by the Byrne Family as part of crafting session.

## HDANI Art Therapy Project 2020 - Busy Bee Ceramics

HDANI service users really enjoyed making their Busy Bees Ceramics as part of Covid19 virtual art therapy project, fantastic work everyone.



## Marie Murphy Retirement

HDANI would like to mark the retirement of Marie Murphy who many of you would have encountered over the past 17 years as she ran the Huntington's disease department at Belfast City Hospital. Marie was more than a faceless someone who answered calls and booked appointments, she was a friend and confidante to many of our HD family and will be sorely missed by all who knew and worked with her. HDANI sent a little token of our appreciation to recognise her invaluable contribution over the years and wish her every joy in her well earned retirement.

Marie said " I was very fortunate to have loved my job and to work for Majella, who was an



inspiration and also a wonderful friend. I have many memories from the past 17 years, and have met so many incredible people and made lasting friendships with people whom I am so in awe of. Huntington's family will always have a special place in my heart. Please extend my sincere thanks to all the staff, trustees, and families at HDANI."

## The HDANI book club is a great way to borrow books for FREE!

If you would like to borrow a HDANI book please message Ashley with the name of the book you would like, your full name and your address. If the book is available Ashley will pop it into the post.

Contact Ashley on [07810330949](tel:07810330949) // [ashley@hdani.org.uk](mailto:ashley@hdani.org.uk)

\*Please ensure you contact Ashley directly as comments/messages on Facebook are not always monitored\*

### Terms and Conditions:

1. You will have the book for 1 month from the date it arrives with you. Please let Ashley know the book has arrived. A reminder will be sent when your month is coming to an end.
2. If the book you want is unavailable you will be placed on a waiting list, and it will be sent to you as soon as possible.
3. Please allow for a three day turnaround period for Ashley to get the book to the next person.
4. All books must be kept clean, safe and in good condition.
5. If after a few chapters you do not like the book, please contact Ashley and return the book early.
6. Prepaid postage will be sent along with the book to return it once your month is up.

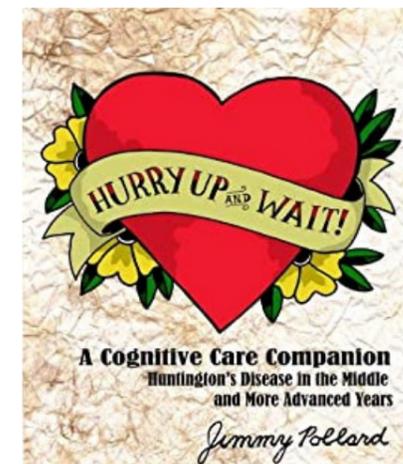
\*It is your responsibility to keep the prepaid postage safe, if the prepaid postage is lost, you will have to cover the cost of returning the book. If this happens, please contact Ashley\*.

[Ashley@hdani.org.uk](mailto:Ashley@hdani.org.uk)  
[07810330949](tel:07810330949)

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### Books available

- Mapping Fate - Alice Wexler
- Life Interrupted - Sharon McCellan Thompson
- You Me Everything - Catherine Isaac
- Rules for 50/50 Chances - Kate McGovern
- I Fight For...Understanding - Ginnievive Patch
- Can you help me - Thomas Bird, MD
- If you were here - Alice Peterson
- Someone else's life - Kate Dale
- How to quieten your mind - Anna Barnes
- A guide to Happiness
- The little book of meditations
- Cards against anxiety
- In between Years - Steven Beatty
- How to be Resilient - Anna Barnes
- Learning to live with Huntington's Disease - Sandy Sulaiman
- Hurry up and wait - Jimmy Pollard



## HDANI Youth

### HDANI Youth Easter Baking

Ashley and Zelig were joined by service users and HDANI Youth for a fun afternoon of baking Easter treats! HDANI youth enjoyed baking treats which included cupcakes, cookies and gingerbread men. Everything looks fit for the Bake Off!!



### Social Media

Did you know you can find HDANI Youth on Facebook and Instagram?

Simply search Facebook for HDANI Youth and you can join our private Facebook Group for youth members aged 13 – 35 (as per Facebook rules and to align with HDYO of which we are a member) and relevant youth workers. It is a place to share experiences, chat and ask questions and will until further notice be moderated by Sorcha McPhillips, CEO of HDANI. This group should be treated in confidence unless there is reason to believe that a person is in danger from themselves or someone else, or they themselves are considered a danger to others.

Don't worry parents, you can join the HDANI Member Chat Group, it is open to carers, patients and family members including those who are at risk of the disease. This is a private group, which means only members of the group

can see the information. It is a safe space to connect, share and receive information. please do not be offended but this space is just for the young people so requests to join will be denied.

You will find HDANI Youth on Instagram by searching [@hdani\\_youth](#)

### HDYO

HDYO have been busy working on their rebrand and new programmes including the first global registry for patients and carers impacted by Juvenile onset Huntington's Disease – JOIN-HD.

The HDYO Congress took place on Saturday 13th March and Sunday 14th March, shortly after this Ashley sent out an email with links to the sessions which are available to watch on the HDYO YouTube channel. Simply search YouTube for HDYO and visit the playlist titled 'HDYO International Young Adult Virtual Congress March 21' where you will find all 35 videos available to watch for free.

### Research updates with HDYO

HDYO held a session with Matt, Hayley, Lauren and Heather who talk about the news that both Roche and Wave's trials have stopped due to not working well enough. You can watch this video on the [HDYO channel](#) or searching YouTube for HD Research Update session after Roche and Wave bad news.



### Monthly Emails

Each month Ashley sends out an email to our Youth members/ parents, there is a different theme or topic and it can range from Huntington's Disease, HDYO, other services available in Northern Ireland such as tutoring, Action for Children or AWARE, social media, helpful videos or HDANI events. If you do not receive these emails and would like to, please contact Ashley on [07810330949](tel:07810330949) or email [ashley@hdani.org.uk](mailto:ashley@hdani.org.uk). This is a great way to keep in touch and stay up to date with helpful information and HDANI events.

### Growing Mindful

HDANI would like to thank the ALPKIT Foundation for the generosity, because of this HDANI were able to send 30 pollinator – friendly bulbs and seeds to members of the Youth Group for them to enjoy with their families in their gardens and local area.

HDANI look forward to watching them grow and see the amazing work our youth can do. If you received a Growing Mindful pack, please remember to send updates to Ashley via email or WhatsApp before May 28th 2021.

[ashley@hdani.org.uk](mailto:ashley@hdani.org.uk)  
[07810330949](tel:07810330949)



## #LightItUp4HD



We are delighted to announce that the following sites are joining us on May 15th to shine a light on Huntington's Disease as part of International Huntington's Disease Awareness Day.

Sites will be lighting up either Blue or Purple as part of the yearly [#LightItUp4HD](#) awareness campaign.

### Northern Ireland Sites:

- Palace Demesne Armagh
- Armagh Gaol
- Clock Tower Banbridge
- Craigavon Civic Centre
- Enniskillen Castle
- Omagh Council buildings
- Lagan Valley Island
- Derry Council Buildings
- Antrim Civic Centre
- Mossely Mill
- Ballyclare Clock tower (town hall)
- McKee Clock Tower
- Conway Square
- Down Leisure Centre
- Newry Town Hall
- Titanic Building Belfast

If you would like to see the sites lighting up around the world or find out more information on the [#LightItUp4HD](#) campaign please visit the Huntington's Disease Society of Canada website and go to events, awareness month and light it up.

## UK & Ireland Wide Huntington's Disease Awareness Campaign

We've been so excited to receive funding from Roche, a pharmaceutical company working on a new drug for HD, to allow us to partner with our friends in the HD charities in England, Wales, Scotland and Ireland to launch a huge awareness campaign this May.



To find out more and get involved, please visit our website.

We have been working behind the scenes on this for the past 6 months and hired a specialist PR company to help us deliver a campaign which includes social media, print and broadcast media as well as developing research and imagery that we can use to help challenge the stigma around HD and improve services. A special website has been set up <https://hdfamilymatters.com/> linking the four charities under the banner of #Family Matters.

It will be a space for info on Huntington's disease but also a place for family members to share how HD impacts them or things that help keep them positive, inspired or precious memories. Some of the contributions from local HD service users are shown here! We are so grateful for everyone who contributed, especially those families who publicly shared their stories to help challenge the stigma and improve awareness of the disease. We will also be reaching out to our service users, health and social care contacts, other service providers and politicians to push for improvements in the provision of HD services across the province. Please visit our social media pages on Twitter, Facebook and Instagram and share our images and stories with your contacts. You will also find an online petition <https://www.change.org/HDNISstrategy21> which you can sign to help us highlight the need for a NI wide strategy and consistent care pathway for people living with Huntington's disease. A copy of the petition will go to relevant politicians and the people in charge of running the health trusts and designing services.

### Awareness Campaign

#### Ashley:

This poem has always stayed with me through my HD journey due to my own close relationship to my brother. When our dad is gone, we have each other and our HD journey may not be over as neither of us are tested. He will be there for me, and I will be there for him and we will start all over again - Ashley Clarke, Northern Ireland

I read this poem in 2014 during the talent portion of the Lady of the Erne pageant I was entered in. It was one of the first public speaking events I done, and one of the first times I spoke so publicly about Huntington's Disease to a large crowd. I received a standing ovation from the judges, reduced most of the crowd to tears and walked away with the crown.

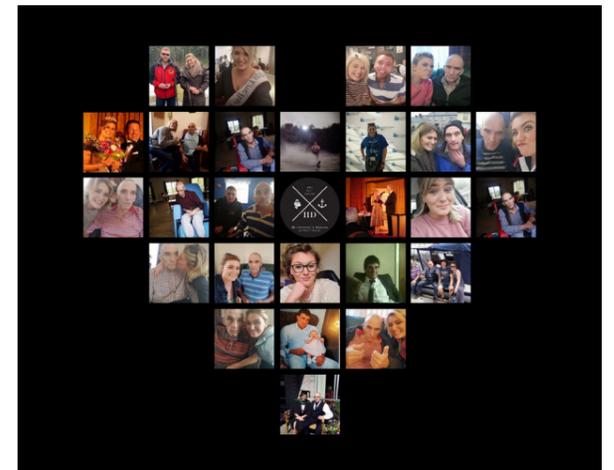
### Siblings by Rebecca Ambrose

Heredity gives us 50% risk of death in prime  
It's hard to know which one of us will be cut  
short in time?

Chances are slim that we will be fine  
I see you watching me for signs of decline  
There are many things that I wonder  
I don't want to be left to ponder  
If it's me who's got the gene, will you turn  
away or be mean?  
If food or drink is what I spill, will throwing  
me out be your will?  
If I am afraid to swallow, will you and I  
together wallow?  
If you see me stumble, will frustration be  
what you mumble?  
If I cannot walk or talk, promise me you'll not  
let the public talk

If anyone says I must be drunk, go ahead,  
scream at that ignorant punk!  
If wheelchairs and restraints make their way  
into my fate, understand it's the helplessness  
that I would hate  
If I look at you with a vacant stare, please  
understand that I am still there  
If I call you by some other name, I may recall  
later that it was you who came  
Whenever I should go ballistic, always  
remember my true spirit is altruistic  
And when I feel overwhelmed with despair, Ill  
value knowing that you care  
Whichever of us deteriorate? I'll forever resent  
this genetic fate

Do not ask me to feel blessed, faith is  
impossible given such a tragic mess  
Growing apart I do fear, please don't become  
distant if my time is near  
Self-pity id be guilty of, but If it's me who  
dies, stand by me with love



#### Amy:

Huntington's affects every aspect of a sufferer's life, but they don't understand how their body and brain have changed. There's a person behind the disease - Written by Amy Dalglish, Northern Ireland

#### Please be patient with me

Please be patient with me.  
I have Huntington's Disease.  
I like to have the same routine,  
and I can't do things with ease.

Please be patient with me.  
My words are slow and stumbly.  
Please give me time, wait and listen,  
it's a lot of effort for me.

Please be patient with me.  
Emotions to me are abstract.  
I do not mean to be so stubborn,  
but I can't control how I act.

Please be patient with me.  
My thoughts aren't always complete.  
I don't understand why things have to change,  
and my brain gets stuck on repeat.

Please be patient with me.  
I remember how life used to be.  
I used to do everything all by myself,  
but now it gets done for me.

**Paul:**

I used to be able to run 8 miles with a rucksack on my back but now Huntington's Disease has taken all my independence - Paul Baggaley, Northern Ireland



**Huntington**

There was something about her, something.  
I could not recognise,  
Not then, I was way too young.  
But I saw the delicate difference  
That is making forty-year-olds older  
Even before the body is worn-out.

There was something about her.  
While her steps danced softly  
In slow-motion,  
As her arms followed uninvited.  
She lost direction chaotically  
The grip on her movements loosened.

There was something about her.  
With the hopeless intense process  
Still to be who she once was:  
Bright, caring, clear-headed  
Until her mouth didn't follow her words  
And sentences chattered into distorted sounds

There was something about her.  
With the years going by  
Braincells disappeared, one by one.  
Destroyed her body, more and more.  
A default of the genes, an unequal  
Battle, however brave it is struggled against.

**Richard:**

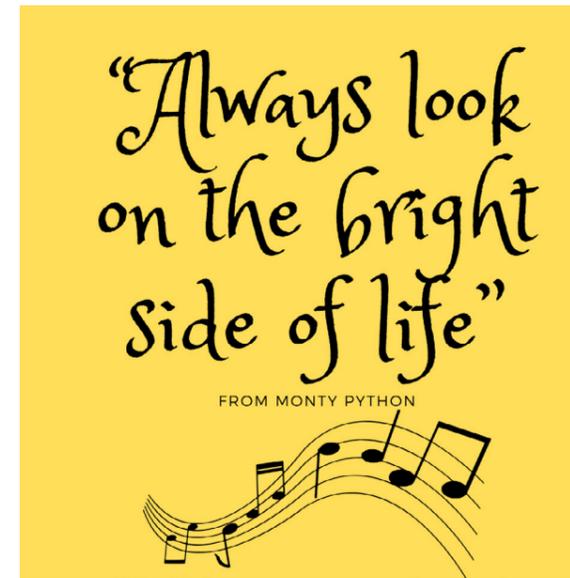
(Huntington Poem):  
Huntington - A Lifelong Journey  
- Richard, Northern Ireland

The following poem was written and read by Magdalena Van Renterghem at the evening presentation following the EHDN Blankenberg conference in Belgium. It is a very fitting message as it was an emotional and poignant tribute to all those who suffer or have suffered as a result of Huntington's Disease. It touched and inspired all of us who were privileged to experience it.

There was something about her.  
Until one day she became seventy.  
Still little sparks in her eyes,  
A burst of sound  
Who will ever know, who shall ever measure  
The dying before one actually died.

There was something about her, something  
That I will recognise now, in the  
Endless suffering, in the  
Unmasked forced introversion.  
There was something about her, something  
That now has got a name.

Auteur: Van Renterghem Magdalena  
Waarschoot België  
Vertaling: Hannelore Michiels  
Alle rechten voorbehouden aan de auteur.  
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by copyright.



**Heather:**

Song lyric which was instrumental for me staying positive & keeping me sane when getting the diagnosis - Heather Hooisma, HDANI



**Simon:**

I drew this picture to raise awareness for mental health, it is the Foyle Bridge bridge in Derry where I live in NI. - Simon, Northern Ireland



**Mark:**

I made this wooden Land Rover, my Dad had one just like it, which we grew up with, and brings back lots of camping holiday memories for me - Mark, Northern Ireland



# Huntington's disease clinical trial round up

By Dr Rachel Harding

April 26, 2021

Edited by Dr Leora Fox

& further edited by HDANI

## Tackling the root of the problem huntingtin-lowering therapies

We've been talking about huntingtin-lowering therapies for a long time now at HDBuzz including the advanced clinical trials which have been taking place. The basic premise of huntingtin-lowering therapies is that they aim to reduce the amount of the harmful form of the huntingtin protein which is made in the bodies and brains of people who have Huntington's disease. Scientists working on these approaches hope that by reducing the amount of the harmful protein, they might slow or even reverse the course of Huntington's disease. Huntingtin is an essential gene, especially during brain development, which means we must be wary of entirely eliminating it from cells. Therefore, huntingtin-lowering is a careful balancing act of reducing the protein enough to hopefully improve symptoms while leaving enough functional protein present to do its normal jobs in the cell. But what are the different strategies companies are taking to make huntingtin-lowering therapies?

### ASOs to lower huntingtin

Researchers have developed molecules called anti-sense oligonucleotides or ASOs which target the genetic message of huntingtin, called mRNA. This message gives our cells the recipe to make the huntingtin protein so if we make the recipe unreadable using an ASO, the levels of the huntingtin protein will go down. One downside of ASOs is that because they are big and bulky molecules that require repeated dosing, they must be given as a spinal injection.

The **Roche** clinical candidate ASO, called tominersen, targeted the recipe for both the normal and the harmful forms of the huntingtin protein. **Wave** Life Sciences also uses an ASO approach but their strategy is a little bit different to Roche. The ASOs that Wave designed will only target the genetic message of the harmful form of huntingtin because they are engineered to recognise tiny genetic signatures only found

in the harmful form of the huntingtin gene. This strategy would leave healthy huntingtin intact, but not everyone has the same genetic signatures, so this means that such drugs, if successful, could not treat all people with HD.

Unfortunately, recent Roche and Wave trials of HD ASOs were not successful. In the case of **Wave's PRECISION-HD trials**, the ASOs just weren't performing as scientists had hoped: the treatment was safe but simply did not lower huntingtin. However, Wave is developing a third ASO with an improved chemical structure that will be tested in clinical trials in the near future. In the case of Roche, **dosing in the GENERATION-HD1 trial was stopped** because of a recommendation by an independent committee who could see that the drug wasn't effective.

It is important to remember that trials are not treatments. Trials are some of the biggest and most complex experiments scientists can run and even if they don't pan out as we might hope, they provide a wealth of information and data which can help inform future decisions and design of treatments. Both Wave and Roche have stated that they are committed to developing treatments for Huntington's disease.

### Genetic therapies to lower huntingtin

The company **uniQure** has developed a therapy called AMT-130 which they hope might be a one shot treatment for Huntington's disease. AMT-130 is delivered by a brain surgery and uses a virus to spread the treatment throughout the brain. The treatment targets the genetic messages of both the harmful and normal huntingtin protein, lowering both.

All of the news so far from UniQure is very positive for the technical aspects of the AMT-130 approach but we are still waiting for long-term safety data as well as whether AMT-130 therapy helps treat the symptoms of Huntington's disease.

### A pill to lower huntingtin

There is a lot of interest in making small molecules which could be taken as a pill with the aim to lower huntingtin throughout the whole body.

**Novartis** is one company interested in this

approach. A drug called branaplam, developed to treat the neurological disorder spinal muscular atrophy (SMA), could be repurposed to treat Huntington's disease. **Novartis has received orphan drug status for branaplam** to be used in a clinical trial to treat people with Huntington's disease, which should launch this year.

## New genetic technologies to treat Huntington's disease

There are companies working on new genetic technologies to treat Huntington's disease.

**Atalanta Therapeutics** is working on RNAi therapies for different neurodegenerative diseases, including Huntington's disease. RNAi therapies work in a similar way to ASOs by interfering with a specific genetic message to lower the levels of a specific protein. Atalanta makes a special form of RNAi which has a branched structure which is able to spread well throughout the brain so they think this will be good for treating brain related diseases.

**Locanabio** is another company which has also developed new genetic technology which aims to target genetic messages which contain the instructions for cells to make disease causing proteins, such as the harmful form of huntingtin.

## Other approaches to treat HD

**Annexon Biosciences** has a Phase II trial underway for Huntington's patients with their drug called ANX005. This therapy targets part of the immune system called the complement system. People with Huntington's disease seem to have overactive complement systems, leading to nerve cell damage and changes to the connections between brain cells. This therapy aims to correct that by stopping the complement system from switching on too much.

**Prilenia** is conducting the PROOF-HD trial, a Phase III study of a drug called pridopidine. Recently, scientists have made advances in understanding the potentially protective effect of pridopidine in the brain, through its action on a type of nerve cell called the sigma 1 receptor. Although earlier trials of pridopidine had disappointing results in people with HD, this new trial will treat early manifest patients for much longer in hopes that they may see

better outcomes for patients.

## Stopping CAG repeat expansion

Although longer CAG numbers are generally associated with earlier signs of disease symptoms, some people who have the exact same CAG number will get sick with HD at very different ages. It turns out that one of the reasons for this variation is found in the DNA code of genes involved in the process of **DNA damage repair**.

**Triplet Therapeutics** and **LoQus23 Therapeutics** are two companies which are targeting these DNA damage repair processes, with the aim of slowing or halting the progression of HD.

Triplet is currently conducting a study called SHIELD-HD, which does not involve a drug, but aims to track HD progression over time and further explore CAG repeat expansion alongside the development of symptoms. The overarching goal is to determine the best time to treat with the types of therapies that Triplet is developing.

## Trials targeting the symptoms of Huntington's disease

Whereas genetic therapies are aimed at altering the course of HD, another important approach is to develop treatments to target symptoms and improve quality of life for people with HD.

**Sage Therapeutics** is working on tackling some of the cognitive changes that happen to people with Huntington's disease. They are developing and validating tools to measure thinking and planning abilities, specifically reported from the perspective of individuals with HD. This type of evaluation is known as a patient-reported outcome (PRO) and it involves questions answered directly by patients, rather than measurements made by doctors. Sage is also in the process of gearing up to begin a Phase I/II clinical trial to see if their drug, SAGE-718, is beneficial for Huntington's patients in improving their cognitive symptoms.

**Neurocrine Biosciences** is working with the Huntington Study Group to conduct a Phase III clinical trial of a drug called valbenazine. The trial is called KINECT-HD and will study valbenazine's effects on the movement symptoms of HD (chorea).



HDANI have created a new space for those who wish to share photos and memories of those they have lost through HD on Facebook, album, "In Loving Memory." You can also include birthdays/anniversary dates you would like us to remember.

We have been contacting all service users on our current database and been adding HD family members who have been mentioned in our newsletters over the years, but wanted to ensure everyone was given the opportunity to share their own memories.

If you would like to share memories, please get in touch via email [info@hdani.org.uk](mailto:info@hdani.org.uk) or via Facebook [@HDANorthernIreland](https://www.facebook.com/HDANorthernIreland) with photos, messages, dates and we will add to the album but it won't be shared as a public post on the newsfeed.

If you require any support from HDANI please do not hesitate to get in touch.

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HDANI was sorry to hear of the passing of Lynn (Mary) Dodds and Philip Doherty, we all send our sympathies to all families and friends affected by their loss.

## In Memory of recently lost loved ones

- Lynn (Mary) Dodds aged 64 sadly passed away with HD in February, "No more suffering" - Olive Campbell



- Phillip 'Doc' Doherty: Former band frontman lived for music and Cliftonville FC "He was a loyal friend, you couldn't have asked for a nicer guy"- Lifelong friend John McAuley. Phillip Doherty died on December 28 2020, the day before his 54th birthday. He is survived by his father Liam, brother Barry and son Caoimhin.



Cruse Bereavement Care is here to support you after the death of someone close. If someone you know has died and you need to talk, please call their helpline on [0844 477 9400](tel:08444779400) or send an email to [helpline@cruse.org.uk](mailto:helpline@cruse.org.uk) Face-to-face and group support is delivered by trained bereavement support volunteers across Northern Ireland. All support is free of charge and confidential. Cruse Bereavement Care also offer information, publications, and support for children.

### Donations in lieu of flowers

When a loved one passes away we are honoured that the family think of requesting donations to HDANI.

We are honoured to receive

- £50 from Mrs Greene and family in lieu of flowers for Kelly Gribben.
- £260 from Thomas Wilson donation in memory of Kelly Gribben.
- £40 donation from Wallace High School in respect of Brian Murphy who was the brother of our colleague Sid.
- £40 from Eilisha McMullan in memory of the late Ivan James McMullan.



# HDANI Fundraising

## Birthdays

Turning your birthday into a gift for local HD Families is a great way to fundraise for HDANI.

If this is something you would like to do, you can create a Local Giving page and share it on your social media and with friends and family.

Ashley can help you set up your Local Giving page, please contact her on **07810330949** she can set the page up for you, answer questions or send you our helpful how to guide for setting the page up yourself.



## Collection's

Shop collection boxes are a great way to fundraise for HDANI, simply contact Ashley and let her know you are interested in looking after a shop collection box.

When you look after a shop collection you will provide your local shop with one of our collection boxes. When it is full, you then take the donations, count them out and lodge them to the HDANI bank account.

If this is something you are interested in, please contact Ashley and she can talk you through the process and provide you with all the information needed.

If this is something you are already doing, please contact Ashley and let her know as we are currently working on updating details.

Email: [ashley@hdani.org.uk](mailto:ashley@hdani.org.uk)

Call/ text: **07810330949**



HDANI are grateful for the continued support we receive from shop collections,

Thank you to Moira & Mickey McNulty for their donation of £52.50

Valeria Crompton donated £10

Thank you to the Oliver family and Killylea Spar shop for their kind donation of £95.46 through their shop collection Box

Thank you Jeff and Orangefield shop for their continued support through their shop collection box which has raised £258.80

Des Farry looks after collection boxes in his local area and made an amazing donation of £1,200 to HDANI from over the 2020 year.

## The Wilson Group

The Wilson Group selected Huntington's Disease Association Northern Ireland as their charity of the year in 2020. We would like to thank them for their kind donation of £200 and were delighted to find that due to the pandemic they have decided to continue with HDANI as their designated charity this year.



## Skipway

Instead of sending Christmas cards the Heatrick family – Skipway made a generous donation of £1,00 to HDANI

## TBF Thompson

TBF Thompson continue to support HDANI, they have made a donation made to cover the registration of the Belfast City Marathon relay team and walkers to the a sum of £200 and TBF Thompson kindly rounded up to £1,000 to kickstart the fundraising.



## Julieann Spence

Julieann has raised an amazing £556.25 by completing 300,000 steps in March.

Julieann had this about her fundraiser,

Even though dad isn't with us anymore, HD will always be a part of the family's lives because of the genetic factor to the disease. I do not carry the gene but other members of the family are untested. HD will always be a part of the family as it is something we went through. I will continue to raise funds and hope for a



cure for whoever can benefit from it, as it is such a heart-breaking illness and watching a family member go through it is soul destroying.

Julieann would like to thank all of her supporters who donated and she continues to walk as she finds it was a great motivation!

## Chez Marie Thrift Shop

A presentation was made to the McCann family by Mary Gorman and the team at Chez Marie Thrift Shop in Fintona. HDANI would like to thank Mary and everyone at Chez Marie for their kind donation of £500



## Sullivan

Ian Sullivan made a kind donation in the name of Erinn Sullivan in honour of Richard Byrne. A fantastic man who fights this disease with courage and humility every day. Ian shared this photo of Richard with his grandchildren.

## One Off Donations

HDANI are grateful for one off donations, we have received recent donations from Billy Caldwell and Haydn Blair.



HDANI would like to congratulate Richard and Christine on their wedding and thank both Richard and Christine and their friends for choosing HDANI as their chosen charity in lieu of wedding gift.



### Contact Us:

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