CJD SUPPORT NETWORK

Newsletter

Issue 30
Spring 2022

CREUTZFELDT-JAKOB DISEASE SUPPORT NETWORK NEWSLETTER

Greetings from the Coordinator

Hello everyone,

Welcome to the 30th issue of the CJD Support Network Newsletter.

Over recent months we have continued to build our membership, offering listening, support, information, and caring grants to individuals and families affected by CJD here in the UK.

Alongside this, myself and the management committee have been busy planning the details of the upcoming Family Support Meeting (FSM) on 9th & 10th September 2022. We look forward to welcoming all who are able to attend. Information on the FSM, including how to register, alongside other network news can be found on **pages 2-3**.

In this issue you will also find an interview with Phil Parker, Lead Nurse at the National Prion Clinic (**Page 4**). Phil is moving on from his role and we would like to extend huge thanks to him on behalf of the many families to whom we know his support and care have meant so much. We wish Phil all the best in the future.

Our Personal Story section (**page 8**) includes a moving account from Charlotte, whose mum Jill passed away with sporadic CJD in 2019. This section of our newsletter provides an opportunity for members to write and read about shared experiences. As we are mindful this may include sensitive content, a gentle reminder that if you need us, we are here for you at support@cjdsupport.net and on **0800 774 7317**.

The research section (**page 10**) features an article summarising the recently published outcome of a study in which CJD patients were treated with a monoclonal antibody called PRN100, and an article from Dr Nihat, recipient of one of our research grants.

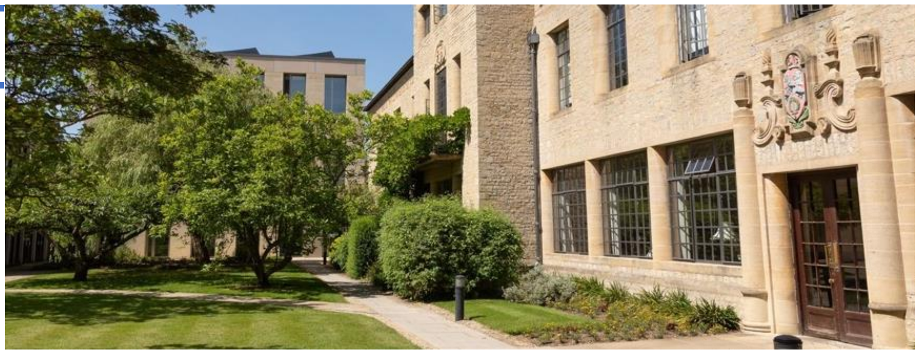
As well as supporting people to connect with others who have had a common experience, we understand the importance of raising awareness of CJD. We now stock a number of products (**right**), which you can order to raise awareness about CJD at work, school, home and in your local community. Please feel free to get in touch for information on these products, as well as the associated suggested donations.

We hope that this issue is interesting and informative, if you have any ideas for future articles please do get in touch.

Warmest wishes,
Beth

In this issue	Page

Network News



Family Support Meeting 2022

The CJD Support Network Management Committee would like to invite you to our next Annual Family Support Meeting (FSM) which will be held on 9th & 10th September 2022 at St Anne's College, Oxford.

The main meeting will take place on Saturday 10th September and there is no cost to attend. Registration and arrival refreshments will be available from 08:45, and the meeting will begin at 09:30. Lunch will be provided. We will aim to close the meeting at 16:00, with the option of staying behind for further informal and supportive discussions.

> ***"There is never a better opportunity to raise the unanswered questions and talk to people in the same position, in a warm, friendly environment."***
>
> Previous family support meeting attendee

Many previous attendees have told us that this is the only opportunity they have had to talk in person with others who really understand their experiences.

In addition to the main meeting, we have arranged an optional meal on the Friday 9th. We request a £20 per person contribution to attend the meal. Following registration, you will receive information on how to access the meeting by road and rail. The venue offers single room accommodation, subject to availability, at £85 per night (B&B). Please indicate on the registration form if you wish to receive details of how to book accommodation.

We hope you are able to join us. Please complete and return the below form to **PO Box 3936, Chester, CH1 9NG.** To request a digital registration form, call **0800 774 7317** or email **support@cjdsupport.net**

Our 2021 Family Support Day will include talks on:

- Overview of prion disease
- Treatment update
- Research presentation(s)

The day will also include previously popular features including 'round table' sharing/discussions and a Q&A session with the option of submitting questions anonymously.

Time will also be made through the day for meeting and talking with other attendees more informally.

✂ -

I would like to attend the above event and require:

___ place(s) for the full meeting (Friday evening dinner, Saturday meeting)*[1]

___ place(s) for the Saturday meeting only

Name(s): ____

Address: ____

Contact number: ____

Email address: ____

Please provide details of any dietary requirements: ______________________

I would like to be contacted about the event via **email/post.** (delete as applicable)

I would like details of how to book accommodation at the venue on the **Friday/Saturday** (delete as applicable)*

* Accommodation is subject to availability.

Network News

New Clinical Nurse Specialist joins CJDRSU

The National CJD Research & Surveillance Unit in Edinburgh has welcomed Clinical Nurse Specialist Terri Hughes to the CJD National Care Team. The National CJD Care Team is based within the NCJDRSU and was formed in order to optimise the care of patients suffering from all forms of CJD.

Before joining the Edinburgh team, Terri trained and worked in London for 10 years across acute and community settings, focused around red cell disease, as a Clinical Nurse Specialist and most recently as a Senior Charge nurse with the Scottish National Blood Transfusion Service. Terri says: *"Seeing how devastating and life changing CJD can be motivates me to ensure patients receive the best possible care. I am passionate about being a nurse and an advocate for my patients' needs. I will never stop fighting for quality in care".*

Terri describes how most of her professional roles have involved contributing to quality improvements over large geographical areas, and says *"I am keen to work closely with all stakeholders involved in the management of CJD, to ensure we continue to evolve and deliver the very best support at care."*

We look forward to welcoming Terri to our FSM in September, she can be contacted directly at: terri.hughes2@nhslothian.scot.nhs.uk

CJD Support Network Research Funding 2022

Professor Richard Knight

The Support Network's main aims are to provide information and support to those affected by all forms of CJD. However, we recognise that research into better diagnosis, care and the possible development of treatments is a vital way of helping individuals and families.

We are extremely grateful for the funding we receive and this must, in the first instance, meet the necessary running costs for our activities, we feel financially able, from time to time, to offer small grants to researchers.

Given the cost of much research, our grants are able to support only small projects, either as focused, stand-alone projects, or as important parts of larger research activities.

Any researcher who receives funding from us agrees to acknowledge the CJDSN in any discussion or publication of their findings. In addition, they agree to present details of their funded research at the CJDSN Annual Family Support Day. We funded two researchers two years ago and they gave talks to those attending our Family Support Meetings.

We are now in the position of being able to offer funding again and have asked for submissions from researchers for either two grants of up to £12,500, or one grant of up to £25,000. Any submissions will be reviewed by the CJDSN Management Committee with independent advice from two internationally renowned CJD researchers (from the USA and Australia). We are expecting to have made a decision by around the end of June 2022.

We hope this enables families to feel they are helping, even if in small ways, to support research into this very distressing disease. You will have the opportunity to hear 'your' researchers discussing their work at forthcoming Family Support Days.

Update: Regional meetings

In our last Newsletter, we requested that people get in touch if they would be interested in attending regional, informal support meetings.

Having received some expressions of interest, we will begin by advertising online meetings in the coming months. These meetings will be intended to bring people together virtually and act as a first step towards meeting up in person.

Look out for further information on our website and social media. If you would like to get in touch to discuss then please don't hesitate to call **0800 774 7317** or email **support@cjdsupport.net**

Featured Interview: Phil Parker

For this issue we invited Phil Parker, who is moving on from his role as Lead Nurse at the National Prion Clinic, UCL, to reflect on his experiences and hopes for the future of prion disease care and research.

Please could you introduce yourself, your background and your philosophy of care?

I've worked for the NHS since 2009, and decided very early on in my career that I wanted to specialise in Neurodegenerative Diseases, securing my first job as a nurse in this field in 2012. I feel a strong sense of service towards this patient cohort, and believe that the way in which vulnerable people are cared for is a critical test of any civilisation. Good quality dementia care requires kindness, compassion, empathy and a creative approach – all things that I've sought to nurture in myself and those that I've led.

What was your motivation for getting involved in the field of prion disease?

Becoming Lead Nurse at the National Prion Clinic in October 2018 was a great honour for me. To join a world-renowned team of clinicians and researchers, to learn from my colleagues and to offer my experience and hard work to them has been a real privilege. Working alongside people who had contributed so much the empirical evidence base and to learn from people who have dedicated their lives to understanding these diseases and how best to care for those affected by them was strong motivation and a powerful ongoing inspiration once in the role.

What have been the most rewarding aspects of the role for you?

Like so many other nurses, my primary focus is the patient and their family, and I derive the greatest satisfaction from striving to ensure that they get the best care possible whether that's from me or from the wonderful nurses that I lead - helping people to articulate their wishes and preferences, and then working hard to help these come to fruition. It has been truly humbling to witness the displays of bravery and dignity many patients and families have shown in the face of these terrible diseases, and being present to contribute to this brings its own rewards.

What have been some of the challenges you have faced in the role?

Prion disease and its impact poses the greatest challenge. The misfolding and aggregation of prion protein causes a cluster of diseases with a myriad of symptoms which pushes the nurse to the brink of their capabilities. Witnessing the relentless, and often rapid decline in our patients, and the effect this has on those around them, poses its own challenge. Knowing that you are doing your job to the best of your ability helps to keep you focused and mitigates the threat of any negative psychological impact to oneself. The rapidity of the illness requires an intensity of input not seen in other dementias, and requires clinicians to be ruthlessly effective and efficient in ensuring all needs are met as they develop.

Are there any particular highlights or stand out achievements from your time in the role?

Playing a part in the delivery of PRN100 – the first in human experimental treatment for prion disease and the first ever purpose made drug for prion disease – was a major highlight of my time in the National Prion Clinic. The culmination of many years of research and development within the MRC Prion Unit at UCL, PRN100 represents a significant step towards the availability of efficacious treatments for prion disease. A perfect example of a seamless transition from lab-bench to bedside, this drug highlights the profound strength in the marriage of research and clinical work made possible within the Institute of Prion Diseases. So much has been learned about the bioavailability and pharmacokinetics of this treatment at this early stage

and I want to thank again the patients and families who participated. I maintain great hopes for a full randomised control trial in the not-too-distant future.

What advice would you give to those who are involved in caring for and supporting individuals and families affected by prion disease?

Many carers and family members of patients under our care thank us in the clinic for the work do, but I'm always quick to point out that in many cases it is those very people who are doing the really hard work day in, day out. Prion disease has devastating effects on the patient and can disrupt families and turn lives upside down. It is essential that carers and families do not suffer in silence but reach out and accept the support being offered to them. I encourage families to strive to preserve the patient's identity in the face of an illness that is trying to strip them of it, and to try to maintain all that is dear to them throughout the illness. We explain the random nature of these diseases and give assurances that they do not come about as a result of something that somebody should, or indeed should not, have done. In the absence of effective therapeutics, it is essential that we fight these diseases with the most powerful weapons at our disposal – kindness and acceptance in the face of the unacceptable.

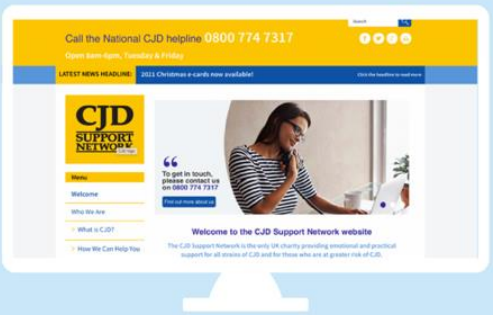
What are your hopes for the future of prion disease care and research?

I look forward to seeing the impact of the Empowering Better End-of-Life Dementia Care (EMBED-Care) study which Nation Prion Clinic nurses are conducting in collaboration with the UCL lead study team. This study promises to have traction on future Government policy in dementia, helping us to understand current and future need, develop effective care innovations and promote collaborations to help people live well, and die well, with all forms of dementia, including prion disease.

Obviously, the ultimate dream is to stop the prion protein from misfolding in the first place and so arrest prion disease before it starts – prevention is always better than cure. For the time being, we need to continue to better understand the prion protein in both its healthy and disease state, develop new diagnostic tests for early identification, and of course develop effective therapeutics – all things that fundamentally underpin all research at the Institute of Prion Diseases.

To imagine a future where prion diseases can be detected before they've had a deleterious effect on brain function, and then to be able to treat them and return the person to a disease-free state seems an elusive dream, but I'm reassured that we have some of the finest minds in the world working with dogged determination to that end. Given that proteinopathy underpins other neurodegenerative diseases like Alzheimer's and Parkinson's Disease, any advancements in prion disease may be applicable to other intractable conditions and offer hope to millions across the globe.

Once again, the CJD Support Network would like to thank Phil for his contribution to this newsletter and in his role as lead nurse at the National Prion Clinic. We wish him all the very best in his new role.



Visit our website for information about CJD and the CJD Support Network

www.cjdsupport.net

Join our mailing list to receive newsletters via email

www.cjdsupport.net/how-you-can-help-us/joining

Find us on social media

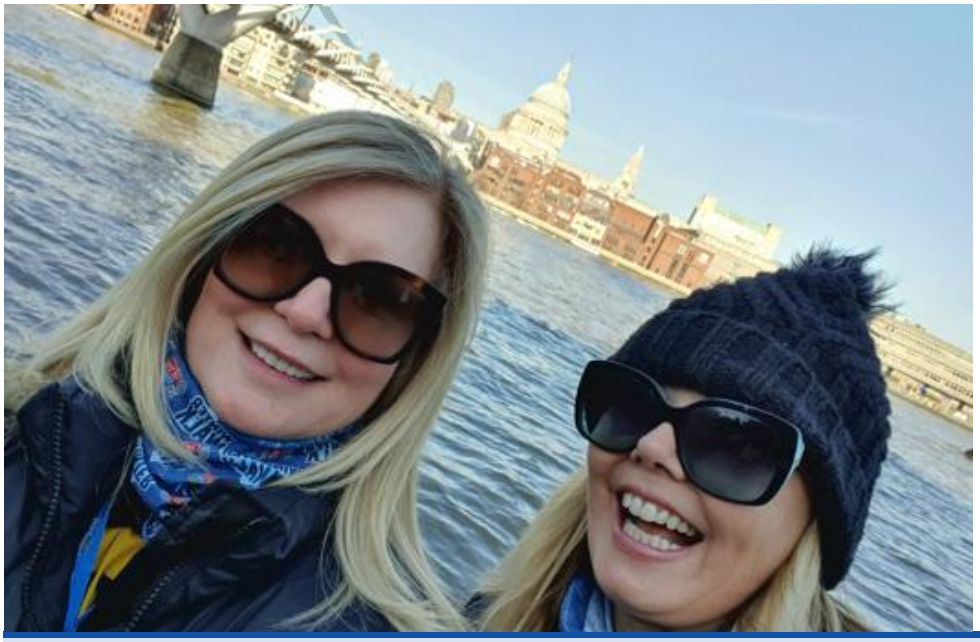
facebook.com/cjdsupport
@supportCJD

Join the discussion in our UK closed group

www.facebook.com/groups/CJDSupport

Fundraising stories

In this section of the Newsletter, we showcase some of the fantastic fundraisers who support us to continue our work. A huge **thank you** to all who have supported us in this way.



London Winter Walk

January 2022

In January Linda Lovett (left) and friend Sue (right) took part in the 10k London Winter Walk.

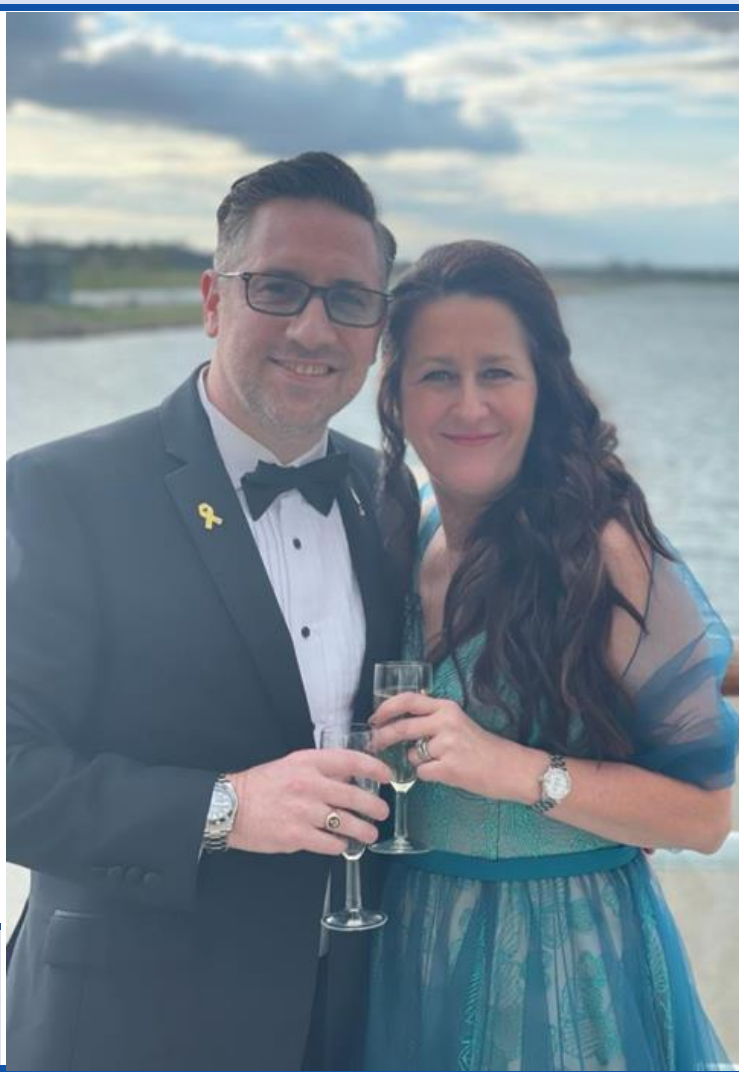
They walked in memory of Carmelina Tomaso, who passed away with genetic CJD. Linda and Sue raised a fantastic £1445 for the CJD Support Network, with the goal of enabling invaluable support to families whose lives are devastated when this rare disease strikes.

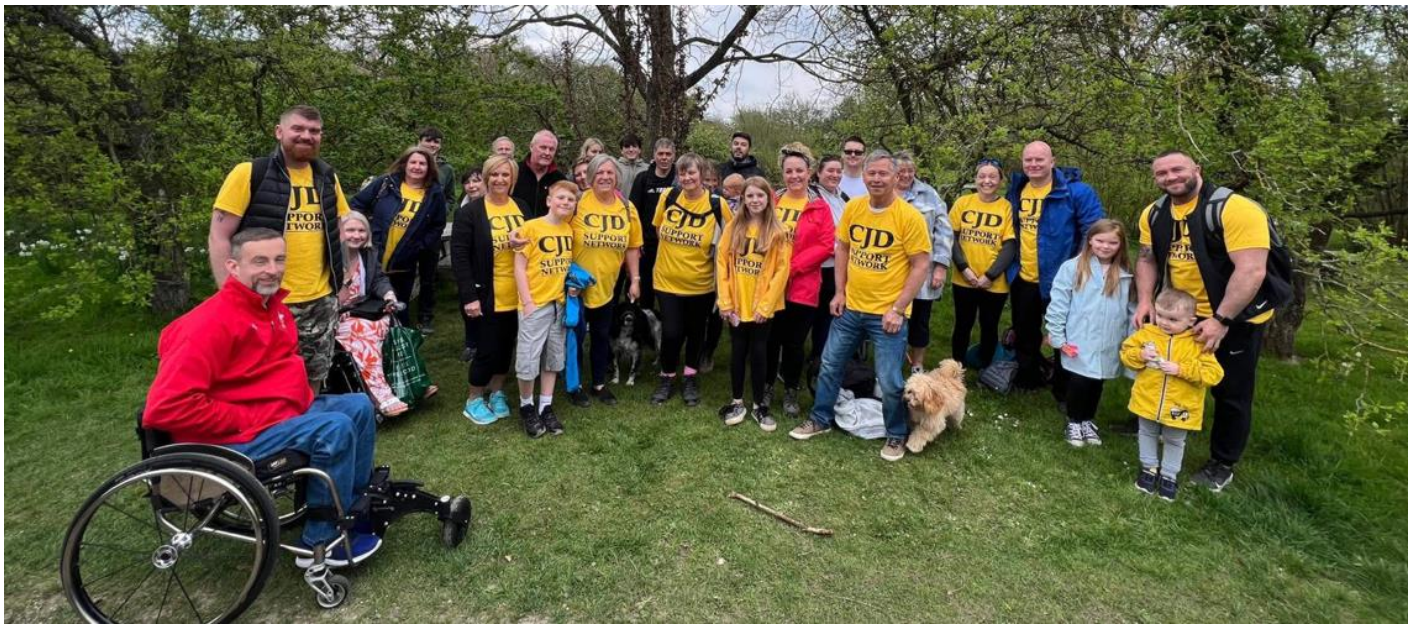
Ladies Festival

April 2022

Dan Hirst and his wife Amelia (right) hosted a Masonic Ladies Festival, as the "President and his Lady" at the Theodore White Temperance Lodge based in Windsor. The event was held at the stunning venue of Downey Lake which is where the Olympic rowing was held when the UK hosted the Games in 2012. Funds were raised through a mix of raffle, auction and a special "Golden Ticket" prize where participants entered a special raffle to win a pair of Diamond and Sapphire earrings, generously donated by a local Jeweller (Flaxman Fine Jewellery).

Dan says: "*Having lost my father to sporadic CJD I know only too well the support that is required in losing a loved one to this truly horrible and incurable disease. Amelia and I are pleased to offer our support in any way that we can to anyone who might be going through what we did back in 2011, which is when my late Dad (Bill Hirst) passed*."





Are you interested in fundraising for the CJD Support Network?

We can help with ideas, planning and merchandise including posters and t-shirts

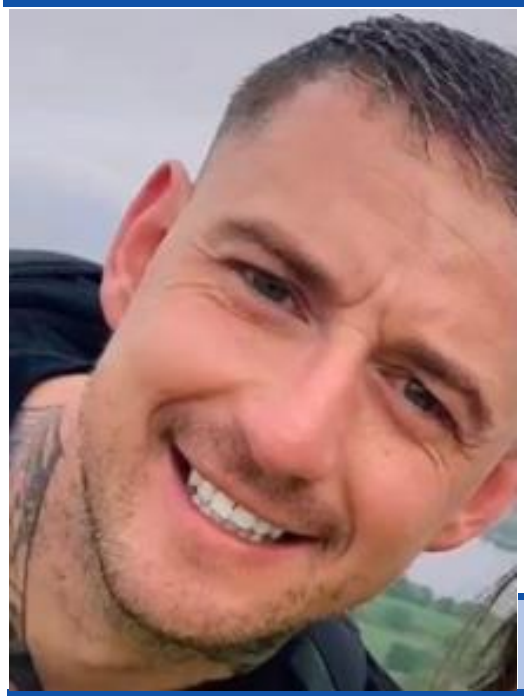
For more info email:
support@cjdsupport.net

Cookie's Erddig Walk

April 2022

Rachel's dad, John Cook, was a kind-hearted, loving and straight-talking man who loved spending time with his family and his dog Marley, walking everywhere. John passed away with sporadic CJD in February 2022, with Rachel and his beloved wife Karen by his side.

Rachel, Karen and grandchildren Lily and Tyler were joined by family and friends (above) on a sponsored walk around the beautiful Erddig estate on 23rd April. Alongside the walk, Lily and Tyler have been busy selling handmade bracelets and the group have so far raised around an amazing £1,000 for the CJD Support Network, in loving memory of John.



Baked with love

August 21-March 22

On 25th August 2021, Jackie lost her beautiful son Darren Quigley (left), one week after diagnosis with sporadic CJD. Darren had celebrated his 33rd birthday just three weeks earlier. A fit and healthy person who loved life, Darren was a devoted father to two beautiful girls and loving brother to his sister, Jennifer.

In a wonderful tribute to Darren, who adored her baking, Jackie has raised a fantastic £1,250 through cake sales which she has donated to the CJD Support Network in his memory.

The **Personal Story** section of the Newsletter provides an opportunity for network members to write and read about shared experiences. Please be mindful that this may include sensitive content. If you require further support with any of the themes raised, we are here for you at **support@cjdsupport.net** and on **0800 774 7317**.

Personal Story: Charlotte Robinson

A photo of Jill enjoying an afternoon tea.

There's an age old saying that tomorrow is never promised. You see, you expect things to stay constant forever, and you expect the people who you love to be just as constant. You never imagine a world where they are no longer here. Unfortunately for me and my family, that nightmare became a reality in 2019 when my amazing Mother, Jill, passed away with sporadic CJD.

I am one of four children, who grew up in a family home surrounded by love (though my siblings will tell you – there were cross words from time to time between us!). We had a mum and dad who were always there for us, throughout our very blessed childhood and right into adulthood, with us all now having families of our own. We were the picture-perfect family and a big emphasis on family events. People wondered how such a large family managed Christmas, but we did, albeit very chaotically! Christmas is still just as chaotic now, but there's an important person missing from the dinner table.

It all started for us around February 2019. Mum had become forgetful, something of great concern. She would forget simple things such as the day of the week, or how to put the television on. At around the same time, she started to lose her balance and became unsteady on her feet. With my dad becoming more and more concerned that something wasn't right, the doctor diagnosed mum with vertigo. We were hopeful that things would soon improve. Our hope was very short lived, with no noticeable progress. In fact, the opposite happened. Mum was becoming more forgetful and losing her balance more. Upon another doctor's visit, she was referred for an MRI. Having not heard anything, on 17th April, dad took mum to the doctors again and she was admitted to hospital.

We had no idea on that day how much things would change. In the hospital they performed the MRI and a Lumbar puncture to determine what was going on. The original diagnosis was Encephalitis, an uncommon and serious, but treatable, condition in which the brain becomes inflamed. Though a frightening diagnosis, we were again hopeful. That hope was also to be short lived. Mum was put on IV antibiotics, but no improvement could be seen. Again, the opposite happened. There was great deterioration in mum's condition and no one understood why. Mum was transferred to a larger hospital, and the reality of the nightmare started to unfold.

Dad was called to the hospital for a meeting on May 1st, 2019. It was a weekday, so off to work I went. It was far from a normal day as I was sat at my desk with concern, but again feeling hopeful that dad would have a positive meeting with the hospital. Dad was always the hardworking family man, doing everything he could to provide for us no matter what. I knew he would never call us out of work unless the situation was serious. And so, when I received the message from him "You all need to come to my house, now!", my heart sank.

I don't remember leaving work, I don't remember driving to my dad's house. I just remember a black cloud over me and wanting to break the speed limit just so I could get there. My siblings and I walked in together and the look on dad's face said it all – the nightmare had become a reality: "Your mum has CJD – it's fatal." Confused and wondering 'What even is CJD? What's going to happen now?' we discovered that it is a rapidly progressing brain disease that affects two in a million people. I was in disbelief – 'this can't be right, can it? Things like this don't happen to my mum!' Before we had time to digest the information, we were told that a doctor and a nurse from the NCJDRSU in Edinburgh were coming down to speak with us as a family.

The doctor and nurse from Edinburgh were nothing short of fantastic. They told us we could take mum home, or find a care home and that all the care would be sorted by them. We discussed our options as a family and decided it was best to find a care home suitable for mum. Mum was moved into a care home and the Edinburgh team were in close contact, advising the staff on what to expect from the disease and what the best course of care would be.

Between the professionals from Edinburgh and the nurses caring for mum, she was made to feel very comfortable and as a family we

could not be more thankful for this. Watching the person who gave me life losing hers, alongside my dad and siblings, over the following weeks is by far the most harrowing experience I have faced – it will stay with me forever. I felt helpless, I just wanted my mum. Talking with her whilst she was still able to, she would mention Christmas. I talked happily of it, but knew she wouldn't be here to see it. In the last week, mum was unable to talk, eat or drink. The day before she passed, a nurse told us 'It's imminent' We all sat with mum through the night and on June 19th 2019 she took her last breath.

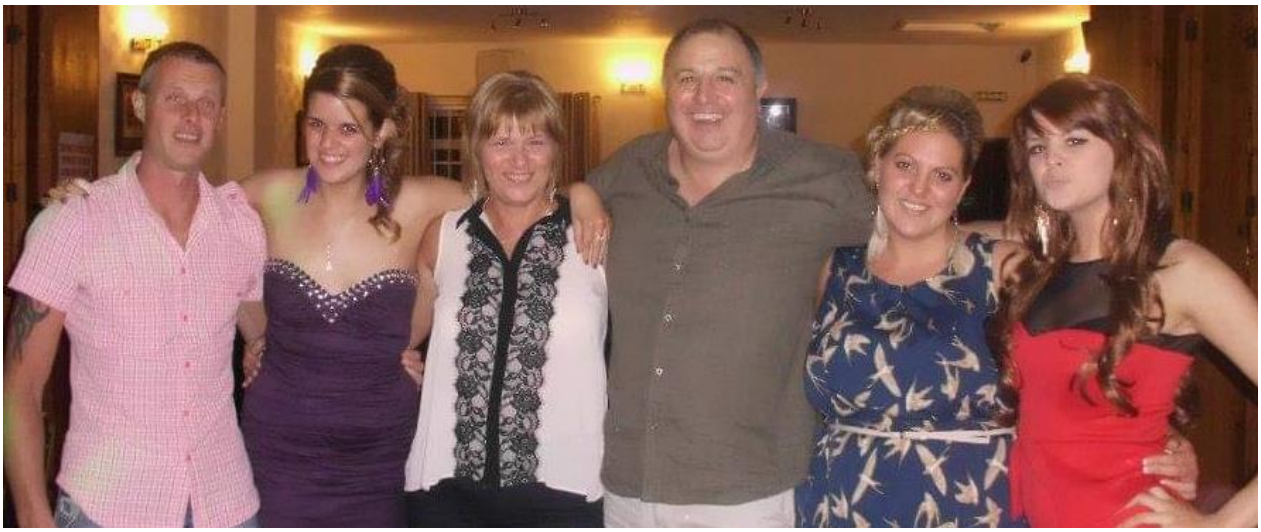
On many occasions I've been asked what it was like towards the end. When you try and explain about the confusion, all too often people will comment such things as "it sounds like dementia" or "it sounds like MND." Even when I try to explain what CJD is, this is often met with "Oh yes I know, mad cows' disease." People mean well and many have supported us during mum's illness and after her passing. However, as many of you reading this will know all too well, unless you have lost a loved one to CJD, you cannot know the true impact it can have and what unfolds in those last few weeks.

Throughout the nightmare that started for my family in 2019, we are so glad we had – and still have - the CJD Network and their support.

The Facebook group has been a great source of information and it's been a comfort to share experiences with others. I have yet to attend one of the family days, but my dad has attended two and found them to give a great sense of support and comfort. I encourage anyone to support the network and fundraise as much as possible. They are such a small charity, but for families like ours and many families affected by CJD they have been a lifeline. I would like to close by thanking everyone at the CJD Network.

Tomorrow is never promised. Tell your loved ones how much they mean to you. Hug them, tell them you love them and make memories.

*The CJD Support Network would like to thank Charlotte for sharing her family's story. If you would like to share your experiences in our newsletter, please do get in touch. If you would benefit from further support, we are here for you at **support@cjdsupport.net** and on **0800 774 7317**.*



A photo of Jill with her husband, Jeff, and their children (L to R: Lee, Charlotte, Jill, Jeff, Amii & Kate)

"It is always good to share with everyone and have a chance to speak with professionals who were involved at a painful time."
2021 Family Support Day attendee

Our 2022 Family Support Meeting will be held on:
9th & 10th September at St. Anne's College Oxford

All are welcome, ***see page 2 for details***. If you have any questions or queries regarding the Family Support Meeting, please don't hesitate to contact:
0800 774 7317 or **support@cjdsupport.net**

Research

Press release from University College London Hospitals NHS Foundation Trust: World-first CJD treatment shows promising early results

A world-first treatment for CJD has shown "very encouraging" early results following its use in six patients at University College London Hospitals (UCLH) NHS Foundation Trust.

Researchers at the Medical Research Council (MRC) Prion Unit at University College London (UCL) have developed a monoclonal antibody, called PRN100, which was given to six UCLH patients with CJD between October 2018 and July 2019.

The results, which were published in Lancet Neurology on 16 March 2022, show the treatment is safe and able to access the brain. In three patients, disease progression appeared to stabilise when dosing levels were in target range.

Given the small number of patients treated, researchers say the findings should be regarded as preliminary and further studies are needed to draw more comprehensive conclusions. None of the six patients experienced side effects while receiving the treatment but all sadly died as a result of their condition.

Professor John Collinge, Director of the MRC Prion Unit at UCL and UCLH consultant neurologist, who led the development of the PRN100 treatment, said: "Drugs used to treat other diseases have been tried experimentally in treating CJD in the past but none has had an impact on disease progression or mortality. This is the first time in the world a drug specifically designed to treat CJD has been used in humans and the results are very encouraging.

"While the number of patients we treated was too small to determine whether the drug altered the course of the disease, this is nevertheless an important step forward in targeting prion infections.

"It has been a huge challenge to reach this milestone and we still have a long way to go but we have learned a great deal and these results now justify developing a formal clinical trial in a larger number of patients."

Looking further into the future, Professor Collinge added: "We hope the drug may also have the potential to prevent the onset of symptoms in people at risk of prion disease due to genetic mutations or accidental prion exposure and may contribute to the development of therapies for more common dementias, such as Alzheimer's disease."

In a comment piece published alongside the results in the Lancet Neurology, Professor Inga Zerr, from the Department of Neurology at Georg-August University of Gottingen, Germany, also called for further studies in this area.

"These outcomes are very encouraging and long awaited but, in light of the limitations, such as the small number of patients included and the use of historical controls, these results must be considered preliminary," she said.

UCLH provided the PRN100 drug to patients under a "Specials" exemption, rather than a regulated clinical trial.

A "Specials" exemption permits a healthcare professional to treat an individual patient with an unlicensed drug when their special clinical needs cannot be met by a licensed product on the market. Three of the six patients were able to consent to receiving the PRN100 antibody themselves. The other three did not have the capacity to consent, so with the support of their families, we sought the opinion of a judge in the Court of Protection in order to proceed.

UCLH created an oversight group, independent of the MRC Prion Unit at UCL and treating clinicians, to consider the numerous and complex clinical, safety, legal and ethical issues arising from the potential use of this unlicensed treatment. The group comprised world-leading experts from a range of disciplines and met regularly with lawyers and patient advocates from the Cure CJD Campaign.

Professor Bryan Williams, director of the National Institute for Health Research (NIHR) UCLH Biomedical Research Centre (BRC), said: "UCLH is a bold healthcare institution which, along with its academic partner UCL, is always seeking to push the frontiers of medicine and science to deliver innovative treatments to patients. "Creutzfeldt-Jakob disease (CJD) is a rare and cruel disease which rapidly destroys the brain and for which there is currently no cure or licensed treatment. It was extremely important to us to find a way through the many challenges arising from the potential use of this novel treatment in order to offer it to a small group of patients.

"We are encouraged by these results which demonstrate the treatment is safe and there is some signal of benefit. The hope is that this could pave the way for new treatments for other neurodegenerative diseases."
*Click **here** to learn more about the MRC Prion Unit, and **here** to access the full text article.*

Research

Predictions of care milestones in CJD, Dr Akin Nihat

Sporadic Creutzfeldt-Jakob Disease (CJD) is a devastating, progressive illness that affects memory, language, balance, co-ordination and behaviour. It's the most common form of prion disease in humans, which are caused by the normal prion protein misfolding and causing damage to the brain and nerve cells. Sporadic CJD is often particularly rapid in its progression, and affected patients can change from being independent and well, to being bedbound and unable to communicate in weeks or months. Consequently, patients and their loved ones need dedicated and responsive support from a range of health and social care professionals and local services, often over a very short period of time.

As part of our work in the National Prion Clinic, we have seen that one of the most difficult challenges, particularly in sporadic CJD, is the pace of change in a patient's symptoms and function. Whilst we do our best to offer guidance and advice to local services, it has been unfortunately common for the rate of change in a patient's condition to outstrip the speed at which services can be provided, or were anticipated by the local team. One of the major unmet needs that emerged regularly in our patient consultations was finding a way to predict certain key changes in someone's condition – for example, progressing to a point when they will require full-time nursing care, a care placement, or how long their disease will last before its final stage.

To try to address this, we aimed to use the incredible resource of the National Prion Monitoring Cohort (NPMC).

This is an observational study of all forms of prion disease that has been active for over 10 years, collecting anonymised data on symptoms, clinical course, investigations and bedside assessments from over 1000 patients and carers.

With the kind support of a CJD Support Network research grant, we collaborated with colleagues at the University of Exeter, who are experts in using a powerful statistical computing technique called machine learning. This allows you to look for patterns in large amounts of data from different sources, and make predictions from this. The main aim was to try to develop tools that could take information available when a patient with sporadic CJD is assessed, and use it to make meaningful predictions about some of these important questions, and provide actionable information for patients and their loved ones.

In total, we used data from over 500 patients with sporadic CJD, and looked at some of the key pieces of information we thought would be useful to tell how quickly the condition would progress. This included some tests of thinking, speaking, movement, and how well the patient can perform day-to-day activities like climbing stairs or use the toilet. It also included results of tests used to make the diagnosis, such as the MRI scan of the brain, and duration of symptoms. Ultimately, we found that combining 9 pieces of information about each patient allowed us to build computer models that could make accurate, useful predictions about when someone may reach certain milestones.

In particular, we could predict with at least 85% accuracy whether a patient would have passed away at 10, 30 and 100 days after the assessment. We could also predict whether a patient would have increased care needs within these time points – for example, progressing from needing informal care by a family member, to full-time professional carers. With these two predictions, we could start to make objective judgements about how quickly certain local services might be required, and what the most appropriate care setting would be – and also provide some more information about the likely disease duration. Importantly, all of the information used is either collected routinely for patients to determine the diagnosis, or can be obtained quickly and simply, so could be used in the community or general hospitals.

This represents a major advance, and the first time these kinds of predictions have been made with the simple data we used. However, there are still some things we can't do. Our predictions so far are quite broad – we can estimate the likelihood of these events at 10, 30 and 100 days, but can't give a more specific individual prognosis, for example in weeks or months. Part of the difficulty is that, although we probably have the largest single set of collected data on CJD patients in the world, these statistical models always perform better when they have more data. We would also like to make these predictions even more accurate, and test them on other populations and

areas, to make sure they still hold true. We would also like to use newer blood and spinal fluid tests that we and others have developed and collected, which may improve accuracy. In order to do this, we hope to establish international collaborations with units in other countries, to pool data and test the models in a larger number of patients. We have submitted the research for publication in a scientific journal, to disseminate our results and receive feedback from the scientific community.

We also recognise that the information provided may not be welcomed by everyone. We need to think carefully about who should have access to it, and how the information should be interpreted and delivered.

We presented some of our findings at the CJD Support Network Annual Meeting in November 2021, and were overwhelmed by the positive responses and useful feedback.

Going forwards, we hope to work together with interested members of the CJD community and the Support Network to establish how best to use and disseminate these results.

This research grant from the CJD Support Network has enabled us to develop new statistical models that for the first time can make predictions about important milestones for patients with sporadic CJD, including when they might require more formal care arrangements, and how long their disease course might last overall.

At the moment, these models are only being used for research purposes, as we continue to develop, test and refine them.

We want to establish international collaborations with other research groups to improve the models further and bring them into daily use, and also importantly work with interested parties from the UK CJD community to determine how best to use deliver the information they provide.

The CJD Support Network would like to thank Dr Akin Nihat for his presentation at our 2021 family support meeting, which was very well received, and for contributing to our newsletter. We are pleased to have been able to support his work.

Management Committee 2021-22

The CJD Support Network Committee is made up of individuals with a personal or professional interest in CJD. The Management Committee works to advance the aims of the CJD Support Network, in the interest of our members.

Prof Richard Knight, Chair, is a Clinical Neurologist at the National CJD Research & Surveillance Unit (NCJDRSU) Edinburgh.

Prof Simon Mead is a Neurologist working at the National Prion Clinic, London.

Brian Marsden joined the committee after losing his wife to sCJD in 2017.

Anita Tipping, Secretary, is a registered nurse, RSCN, whose son David died of iCJD through growth hormone injections.

Annette Beal works in a care home; she lost her husband to Sporadic CJD in May 2017

Margaret Leitch is a National Care Co-ordinator and Senior Nurse at the NCJDRSU in Edinburgh.

Andy Tomaso, Treasurer, lost his mother Carmelina to genetic CJD in 2007.

Dr Kate Dahill works as a junior doctor. She lost her aunt to sCJD in 2012

Beth Marsh, National Coordinator, lost her father to sCJD in 2016.