Strategic Plan

Cure CJD Campaign, A fund within University College London Hospital (UCLH) Charity
Registered Charity no: 1165398

June 2019

Campaign Vision

To raise funds to support the work of the Medical Research Council Prion Unit at University College London ('MRC Prion Unit at UCL') to develop a treatment or cure for Creutzfeldt-Jakob Disease (CJD), and to increase awareness and understanding of the disease.

Mission Statement

Our strategy sets out financial targets to support vital research in the development of a treatment or cure for CJD. We will report on progress against these targets and tailor activities to achieve them. We will create a co-ordinated approach to fundraising by developing partnerships to maximise revenue opportunities. We will develop an awareness raising plan and the materials to support its delivery.

Strategic Goal

The first priority and our current fundraising focusses on supporting the first PRN100 treatment project where our support is vital to help cover the cost of staffing, overheads, equipment maintenance and laboratory consumables.

It is estimated the cost to cover the project management of this and necessary bespoke patient laboratory work is approximately £250,000 - £300,000 per annum, aiming to offer PRN100 to a small number of patients who are impacted by CJD.

After this initial phase has been completed additional funds raised will be used to continue to support the therapeutic research at the MRC Prion Unit at UCL.

This could be the development of a successor or alternative to PRN100 or it could help towards a full clinical trial of PRN100 if justified by initial results. Whatever direction this takes will be costly, running into millions of pounds and we desperately want to help raise money to contribute to this vital work. We will:

- Agree with the MRC Prion Unit at UCL a set of targets for the next five years;
- Consider which sectors to approach for support (and why);
- Agree a plan of outreach activity to identify possible partners by sector;
- Agree with the MRC Prion Unit at UCL the information to be communicated;
- Identify the most appropriate channels to raise awareness;
- Create content for and produce awareness raising materials.

We will adopt a dual approach to fundraising:

- We will look to foster and develop corporate relationships to support fundraising;
- We will continue to promote CureCJD to those supporters who can implement local fundraising campaigns through regional activities. This will typically be via people or groups with experience, directly or indirectly, of the disease.

Background to CJD and other prion diseases

Creutzfeldt-Jakob Disease (CJD) is the commonest form of a group of invariably fatal brain conditions known as prion diseases. There are about 150 new patients diagnosed with prion disease in the UK per year, and CJD is responsible for about 1 in 5000 deaths. Prion diseases are also known as Transmissible Spongiform Encephalopathies (TSE's) and they also affect a wide range of animal species – for example causing scrapie in sheep and goats and bovine spongiform encephalopathy (BSE) in cattle.

Prions are rogue forms of a naturally occurring brain protein (called the prion protein). The normal prion protein is thought to play a role in the transport of messages between specific brain cells. However, in prion disease, the normal prion protein becomes misshapen and forms clumps or chains composed of many individual misshapen prion proteins. These chains break up and form more "seeds" which grow in the brain as more and more normal prion proteins attach to them and in turn themselves become misshapen. This process causes brain cells to stop working properly and eventually die, leading to progressive loss of the normal functions of the brain.

The human immune system produces antibodies to fight infection with viruses and germs as the system recognises them as being new and not a normal part of the body's make up. However, because prions are made of one of the body's own proteins the immune system does not recognise them as different in the same way and so does not make antibodies to destroy them.

Prion disease can arise in three different ways leading to *sporadic*, *inherited* and *acquired* forms:

The commonest type of prion disease (about 85% of patients) is **sporadic CJD**. This form occurs at random in the population all over the world. It is thought to occur when clumps of rogue prion proteins form spontaneously in the brain as an unlucky chance event. The lifetime risk of this happening and causing sporadic CJD is about 1 in 5000;

Inherited prion diseases (accounting for about 15% of patients in the UK) are caused by a faulty gene passed down in families from one generation to the next. The faulty version of this gene (which normally instructs the body how to make healthy prion protein) results in the body making a defective prion protein which is predisposed to form prions at some stage during that person's life. There are about 40 different faults (called genetic mutations) known in the gene which can cause prion disease. Other names given to different forms of inherited prion disease are familial CJD, Gerstmann-Sträussler-Scheinker disease (GSS) and Fatal Familial Insomnia (FFI);

Acquired prion diseases occur when the patient has been infected with prions from the outside environment. These forms are rare and now account for only about 1% of patients.

Medical procedures that have inadvertently led to iatrogenic (that is, caused by medical examination or treatment) CJD include injections of human growth hormone given to children with growth problems. These hormones were extracted from large numbers of pituitary glands from bodies after death, some of which were prion-infected. This practice stopped in 1985 when the risk of transmitting CJD infection was realised and patients are now treated with synthetic hormones which do not carry a risk of CJD.

Another past cause of iatrogenic CJD was use of a type of human tissue called dura mater following some types of brain surgery. Again, after the risk of transmitting CJD was appreciated, this practice stopped and synthetic materials are used instead.

Very rare cases of iatrogenic CJD have resulted from contaminated neurosurgical instruments (prions are partly resistant to normal hospital sterilisation methods) and a type of eye surgery called corneal grafting.

As prions are not completely destroyed by conventional sterilisation, Department of Health guidelines are that certain surgical instruments used on medium or high infectivity tissues (mainly brain and spinal cord tissues) on a patient with CJD are quarantined and not reused unless an alternative diagnosis is found.

Variant CJD is caused by dietary exposure to BSE infected food products. Variant CJD has been in decline since 2000 and is now very rare. It is however unclear if the variant CJD epidemic is over given the potential for extremely long incubation periods in human prion diseases. Four cases of possible infection with variant CJD transmitted by blood transfusion or blood products have been reported.

Human prion diseases are not contagious – you cannot “catch” prion disease by being a carer or partner of a patient. Infection only occurs if someone eats prion-infected material or was accidentally exposed to it during certain medical or surgical procedures (so-called “iatrogenic CJD”).

For more information on the different types of CJD and how they are diagnosed please visit http://www.prion.ucl.ac.uk/clinic-services/.

Research & Potential Treatment

There is currently no treatment to prevent onset or to delay or stop the progression of established disease. Prion diseases are progressive and invariably fatal. Caring for a patient with CJD is therefore palliative and focused on making life as comfortable as possible in the time remaining. Medicines can be used to alleviate some aspects of the disease such as abnormal movements or seizures if necessary although not all patients will need these.

A number of drugs have been tested on patients with prion disease worldwide in the past but no survival benefits have been seen.

Recently researchers at the MRC Prion Unit at UCL have developed a humanised monoclonal antibody treatment called PRN100. This has been designed to bind tightly to normal prion protein in the brain. The aim is to prevent the rogue proteins from attaching to normal prion protein and therefore preventing prions growing and spreading in the brain. This has shown encouraging results in tests on laboratory mice and is now being offered to a small number of patients with CJD at University College London Hospital on a special needs basis, see:

http://www.uclh.nhs.uk/OurServices/ServiceA-Z/Neuro/NPC/Pages/PriondiseasesandCJD.aspx.

This represents cutting edge research by the world leading scientists at the MRC Prion Unit at UCL and it is the only experimental treatment specifically designed for CJD in clinical use anywhere in the world. Funding raised by the Cure CJD Campaign has helped make his possible.

PRN100 was approved to be given as an NHS treatment to the first human patient in October 2018 after a judge at the Court of Protection approved it was both lawful and in the patients best interest to receive the unlicensed treatment. Several further patients have since been treated.

Background to the Campaign

Initially the MRC Prion Unit at UCL had hoped to conduct a full regulated trial with PRN100 but the cost of such a trial would have run into millions and the funds were not available. An alternative approach was identified with the aim of treating a few patients with initial estimates that it would cost in the region of £200,000.

The Cure CJD Campaign was launched in 2016 by a group of individuals personally affected by the disease. They came together to support the work of the researchers and medical team at the MRC Prion Unit at UCL to raise funds to contribute to the development of an experimental treatment for CJD.

An initial target of £100,000 was set to raise funds for the Unit with matched funds provided by the UCLH Biomedical Research Centre. Further support was generously provided by the J P Moulton Foundation. The target was quickly achieved.

The Cure CJD Campaign functions as a standalone body but has administrative support provided by the MRC Prion Unit a UCL. Following the success of the early campaign there is now a structure in place to support its second chapter. The Committee was formed in December 2018; it is committed to supporting the original aims of the Campaign, and the therapeutics work of the MRC Prion Unit at UCL.

Committee Members

Chair - Nicola Carnie

Nicola became involved in the Campaign in June 2017 when her partner Mark Phillips was diagnosed with sporadic CJD. She was Mark's primary carer until he passed away eight weeks and three days after diagnosis. She is therefore only too aware of the rapid development of this devastating illness and what it is like to see the person you love be taken away in the most cruel way. Her experience led to what will be a lifelong commitment to the Campaign and supporting work to find a treatment or cure for this disease. Her commitment to this Campaign is part of her legacy to Mark. Nicola has been a career civil servant since 1995.

Fundraising Director - John Camidge

John has first-hand experience of how devastating prion diseases are. An inherited form of prion disease (Gerstmann-Sträussler-Scheinker - GSS) runs in his family. It has taken the lives of his grandmother, five of her siblings, two uncles, his mother and more recently his sister Diana who passed away in September 2016. With help from family and friends he set up the Diana Camidge Foundation with the specific objective of raising funds and awareness for the Cure CJD campaign. He has worked in the marketing industry for many years including roles in Vodafone, Centrica and currently Activate Learning who own Schools and Colleges in the Thames Valley area.

Treasurer - Danny Goldsbrough

Danny's experience with this disease was when Andy French, his brother in law, started showing some symptoms, but no one could diagnose what the issue was. Previously a fit and healthy 52 year old, he was finally diagnosed in February 2017 but sadly died 4 months later. This experience has driven Danny and his family into fundraising for the CureCJD Campaign. He is determined to do all he can to support work to help find a cure for this unforgiving disease and becoming involved directly with the Campaign is part of his commitment.

Member – Peter Mills

Peter's daughter Holly was diagnosed with variant CJD in 2003. Peter's involvement with the MRC Prion Unit at UCL to support a future diagnostic and novel treatment is a legacy to her memory. Peter has been a businessman all of his working life and has an active interest in farming.

Member – Charlotte Saigne

Charlotte's brother Frédéric was diagnosed with GSS several years ago. They are both French, he lives in Stockholm and she lives in Barcelona. Doctors in several countries all pointed to the MRC Prion Unit at UCL's project as the most promising for a cure for the prion diseases. Charlotte's highest hope for a cure lies in the Cure CJD Campaign. Charlotte has a professional career in the media, entertainment and publishing industries, and has worked as business manager for various European

countries. She is currently Director for France, UK and Germany in a major Spanish publishing company.

Member – Jacqueline Burke

Jacqueline first learnt of the work on the development of a treatment for CJD when her brother was diagnosed with CJD. At that stage it was too early in the research for it to help her brother. She joined the Cure CJD Campaign to help prevent other families having to endure the grief of this devastating illness. Jacqueline is an Assistant Professor in a School of Nursing in a University in Ireland.