

Pears

In association with



Building

Issue 5 February 2018



Pears project gets
the green light

IIT research into inherited
metabolic conditions

Q&A with patient
Tony Haymes

We're delighted that Camden Council has given the green light to start construction work on the Pears Building, home of the UCL Institute of Immunity and Transplantation.

The UCL Institute of Immunity and Transplantation (IIT) will be one of five leading centres of its kind across the globe, bringing scientists and clinicians together to research revolutionary new treatments.

The unique co-location of NHS care and university research means that patients, nurses, clinicians and scientists can easily work together to accelerate the development of new therapies for patients.

Now that Camden Council has approved the 'detailed basement construction plan' for the Pears Building, the work on this pioneering and exciting project can start. See overleaf for more information.

In this newsletter we also highlight research into rare inherited metabolic conditions such as Fabry disease, and how a new treatment developed at the IIT is enabling a patient with Fabry to enormously improve his quality of life.

Update on the building project

At the end of January, Camden Council approved the final elements of the section 106 agreement which controls how we can construct the Pears Building. This now means the Royal Free Charity, will be starting work in the next few weeks on securing the site and starting work.

Camden appointed an independent assessor to judge the plans we have set out. Their unanimous recommendation is that construction can begin, as we have proven that we will not bring harm to the hospital or other local buildings. The safety measures put in place include:

- over 150 monitors of water levels and vibration
- an acoustic barrier to prevent noise transfer
- plans to monitor road congestion and ensure construction vehicles stick to agreed routes
- a robust monitoring action plan to safeguard local buildings.

With work to secure the site likely to begin shortly, the first noticeable activity will be the closure of the link road adjacent to the decommissioned car park. Traffic personnel will be on site to help drivers and pedestrians. Over the spring and summer, the car park will be demolished and the new Pears Building structure will start to take shape. We have established a construction working group to allow us to respond quickly to any issues raised by the local community.

We welcome an open dialogue with residents.

To read earlier issues, visit <https://www.royalfree.nhs.uk/about-us/investing-in-our-future/pears-building/>

Do you have any feedback on this newsletter?

Please contact the communications team at: rf.communications@nhs.net, we would love to hear from you.



If you have any questions or comments on the construction process, please contact: Pearsbuilding.community@willmott Dixon.co.uk



The Institute in focus

Focus on IIT research into lysosomal storage disorders

In this issue we hear about the work of Dr Derralynn Hughes, senior lecturer in haematology at UCL and clinical director of haematology, oncology and palliative care at the Royal Free Hospital.

Derralynn's IIT research work at the Lysosomal Storage Disorders Unit has changed the lives of hundreds of people with rare inherited metabolic conditions, such as Fabry disease. Derralynn examines the efficacy of enzyme replacement therapy and other new therapies in the treatment of these disorders.

What is Fabry disease?

Fabry is caused by the deficiency of a particular enzyme. Symptoms typically first appear in early childhood, but they can be so variable and difficult to understand that sometimes doctors fail to make the right diagnosis. Children with Fabry disease can have intermittent pain in their hands and feet during an illness and in hot or cold weather, and have reduced sweating. They may also have diarrhoea alternating with constipation, vomiting and bloating.

In adulthood it can lead to life-limiting effects such as kidney complications, strokes, bowel problems, an enlarged heart, hearing impairment and an inability to regulate sweating.

The road to success

The unit is a national designated specialist centre. People from across the UK come here for diagnosis and treatment, and Derralynn's team has brought about some fantastic outcomes for patients.



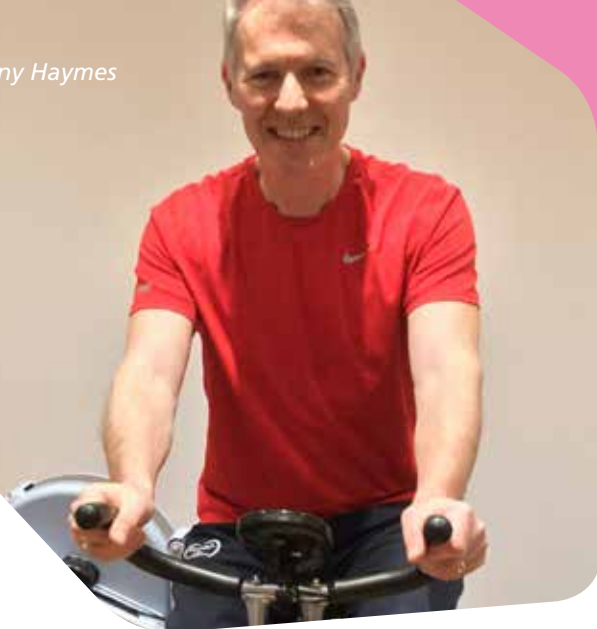
Dr Derralynn Hughes

Original clinical trials at the IIT, approved in Europe in 2000, meant that patients could have enzyme replacement therapy for Fabry disease, which replaces the missing enzyme with intravenous infusions every two weeks. However, these injections take time and sometimes there can be difficulty with access to veins, so there has been a real desire for an oral treatment.

Determined to give patients a better quality of life, Derralynn and her group took up the challenge and started to research a better option. They did the original laboratory screening work for phase 2 studies here and, working closely with the sponsors and trial pharmacy teams, got approval for use of an oral treatment in the UK in April 2017. This treatment is really life-changing for patients because no injections are necessary and they are not dependent on being in a certain place at certain times to have them.

Patient involvement

With its outstanding team of nurses and researchers, the unit looks after 400–500 patients with rare disorders, and has an exceptional international reputation with both patients and drug companies. Everyone attending the unit is offered the opportunity to participate in clinical trials or new treatments where appropriate, or join in basic science and observational studies that contribute to our understanding of the mechanisms and clinical features of these rare disorders. It also hosts and participates in numerous national patient meetings.



Q&A



with **Tony Haymes,**
an IIT patient with Fabry disease

Tell us a little about yourself

I'm 55 years old and I'm married with two grown-up sons. We've lived in Bedfordshire for 25 years and I work as a company director selling pumping equipment. I really enjoy keeping fit, especially running. Over the years I've entered many races, including quite a few marathons.

Why do you attend the IIT?

It all started about three years ago, when out of the blue I had a serious heart complaint. I was rushed to the Lister Hospital in Stevenage, where the doctors suspected I had blocked arteries. At the Royal Brompton Hospital I was fitted with an implantable cardioverter defibrillator (ICD), a type of pacemaker. They believed I had hypertrophic cardiomyopathy, when the heart muscle cells enlarge and blood flow is blocked. Then after two years and incredible genetic testing, they discovered I had Fabry disease. I was lucky enough to be referred to the IIT at the Royal Free Hospital, where I met Dr Derralynn Hughes. She started me on enzyme replacement therapy in September 2016 and since then my energy levels have increased.

What effect did this have on your life?

It was obviously a big shock to learn that I had a heart problem and then discover I had such a rare condition. I'd always been very fit and had no symptoms – even when running. Having the ICD meant that I could have a near-normal lifestyle, but I had to be careful in some situations and near certain machines or equipment.

When I then got the Fabry diagnosis and started receiving enzyme replacement therapy, it certainly gave me a better outlook on life in terms of the illness. But the treatment affected my whole routine. Luckily I was able to have the enzyme replacement therapy at home, but this meant that

every second Wednesday I had to be in a certain place at a certain time for 90 minutes, while a specialist nurse gave me the infusion. I had to plan my work, family life and any holidays around this fortnightly routine.

What does 2018 hold for you?

Dr Hughes and the IIT team are about to start me on Migalastat, a new oral treatment that is better suited to my condition as it increases levels of my own enzyme. Best of all, it comes in a pill format. So I will simply have to take one pill every other day, rather than lengthy fortnightly infusions. This is a real game-changer!

My life will be so much easier to plan – I now know I can travel when I want, work when I have to, and just have a normal routine without having to worry. It's also really boosted my enthusiasm and general wellbeing.

What have your experiences been of the NHS and the IIT?

I really am very, very thankful to the NHS for the treatment I've had and the care I've received. Everyone I've encountered at the Lister, Royal Brompton and Royal Free hospitals has been so professional, kind and caring – and my treatment at the IIT has made a complete change to my life!

Tony and his family

