



# How patients have helped us with our research

UCLH/UCL NIHR Comprehensive Biomedical Research Centre

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# Welcome

This booklet describes some of the many ways that patients have got involved in biomedical research at University College London Hospitals (UCLH) and University College London (UCL).

By patient involvement in research we mean an active partnership between patients and researchers where patients help design and carry out research studies.

We hope the following pages will inspire people to find out more about our research and perhaps even to get involved.



## Foreword by Professor Deenan Pillay

Here at University College London Hospitals (UCLH) and University College London (UCL) we have formed a partnership called the Comprehensive Biomedical Research Centre to make sure that exciting advances in science are applied as quickly as possible in healthcare.

Doctors, nurses and researchers work closely with scientists to make sure advances in medical research have a direct impact on patient care and quality of life, as well as saving many lives.

But what people often don't realise is that patients and members of the community play a key role in our success. Patients and the public are our partners.

We work closely with patients and the public to make sure that what we are doing is relevant to their needs, appropriate and effective. We need to know we are asking the right questions and using the right processes. We need to know we are using the right language and reaching the right people.

Here are just a few examples of the many ways patients and community members have worked with us to make sure our research leads to real improvements in healthcare.

*Professor Deenan Pillay,  
Director, UCLH/UCL National  
Institute for Health Research  
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## Patients help design research studies

Patients and community groups have been working so successfully with reproductive health researchers at the University College London (UCL) Institute of Women's Health based at the Margaret Pyke Centre, that they now actually help shape and design research studies.

What began as a patient/public participation group to improve things like communication of the centre's research findings has now become a Research and Innovation Forum, with 40 lay members including patients, local residents and representatives of local community groups.

Patients now have a much greater input into research on contraception and reproductive sexual health. They don't just comment on research proposals. In some cases they are actually a 'co-applicant' with researchers.

### How the research forum works

The Research and Innovation Forum, which was set up with funding from a UCL Public Engagement Beacon Bursary, meets regularly to help develop research proposals and funding applications.

Researchers tell forum members about their work and ideas for the future. In turn the members will comment and offer suggestions and constructive criticism on research studies. This is an important way of making sure research is relevant to the needs of patients and the community. Members review information such as patient information sheets and consent forms to make sure that information is clear and easy to understand.

The group also looks at procedures in clinical trials and advises researchers on whether these will be acceptable to patients. Members

help disseminate research study findings and improve communication with different audiences.

Importantly, the forum helps researchers link with groups of people who are normally hard to reach. Members of the forum are from a wide range of backgrounds, including age, ethnicity and income.

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### Patients now have much greater input into research on contraception and reproductive sexual health

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Members are already partnering up with researchers to make research proposals together – in this case members are known as co-applicants. And members are sitting on steering groups for individual research studies.



Emma Sydenham is a forum member and advises on patient information

be clearer about things like the side effects or symptoms patients might expect with certain treatments.”

Emma is also a patient adviser on a project to consolidate pre-conception health information for patients. She says: “I had noticed this information was slightly different on the NHS website, the Food Standards Agency website and other government websites patients were likely to search.”

She adds: “It has been a heartening experience to see the staff be so welcoming of patients and our ideas for change.”

### A two-way conversation

Lay member Emma Sydenham first found out about the UCL Institute of Women’s Health Research and Innovation Forum from an advert in a GP surgery. She was keen to get involved because she thought more people should know about the Margaret Pyke Centre.

“What I like about the forum is that it is a two-way conversation,” says Emma, who spends about three hours a month in her role as forum member. “We can suggest ideas for research or changes at the centre. For example, I offered to help improve the patient information leaflets because I thought they should

A lay member is taking over the role of forum chair and together with the other members will be deciding on future agendas.

The Research and Innovation Forum project forms part of a larger initiative within the Women’s Health programme of UCL Partners to engage

more effectively with women in co-designing research and co-developing service improvements.

## Joining forces to pioneer gene therapy

A unique partnership between patients, a charity and UCL researchers is helping doctors look for a way of ending the misery of haemophilia B.

Haemophilia is a clotting disorder which causes unpredictable and spontaneous internal bleeding. A person with haemophilia B has a genetic defect and their liver is unable to manufacture a protein called 'factor 9'. Factor 9 is essential for clotting.

Excitingly, a team from the Haematology Department at the UCL Cancer Institute has been pioneering a technique to correct the genetic defect. But clinical researchers could not have got this far without funding from a charity, volunteers and extensive patient consultation.

### Patients say what they want

To correct the genetic defect, a normal copy of the factor 9 gene needs to be delivered safely to the liver of the patient with haemophilia B. It took the

UCL team 10 years to develop a suitable vehicle – known as a vector – for carrying the genetic material.

### Researchers could not have got this far without patient input

But they couldn't have done this without funding from organisations like the Katharine Dormandy Trust. With gene therapy the next big thing in haemophilia research, the charity funded the original work that enabled the trial to start.

Patient feedback was then crucial in helping researchers develop the gene therapy. "Initially we were going to inject the vector into the blood vessels serving the liver, but this required a small operation, and patients told us they didn't want that. They already treat themselves by injecting factor 9 into a peripheral vein, so we used that route instead," says lead researcher, Dr Amit Nathwani, who is from UCL and

works at the haemophilia and haemostasis unit at the Royal Free Hampstead NHS Trust.

Similarly the vehicle initially used by researchers was a harmless adeno associated virus (AAV) that is so common most people are immune to it and the body's immune defences would normally attack it. To avoid this, there were two options: either use suppressant drugs or modify the vector. Patients didn't want to take the drugs, so the AAV type was changed to a less common one and its DNA altered.

### Other hurdles

But even with the biological hurdles overcome, it was a challenge to get a trial off the ground. Previous problems with haemophilia treatment, including HIV-contaminated blood products, meant patients and doctors were wary of new treatments.

The Katharine Dormandy Trust played a key role by running focus groups for doctors to explain the trial's objectives and



### The first patient to try the new therapy

The first patient to take the plunge was 31-year old Sebastian Misztal, whose haemophilia has damaged his

ankle joints. He was in constant pain, regularly having to stay in hospital.

“Once injected with the vector, you become immune so can’t be injected a second time if it doesn’t work. But I was willing to

Sebastian Misztal was the first patient to try the new therapy for haemophilia

take that risk. Nothing could be worse than what I have now. It might be too late for my ankles, but it could help others,” he says.

“It’s amazing. My factor 9 levels have increased and have stayed constant. I’ve had no side effects and I don’t have to inject myself twice a week, which was not pleasant,” he enthuses.

*While early signs look promising, this study is still ongoing and full results will have to be analysed to determine the success and safety of the treatment.*

supporting the researchers on how best to approach patients.

The research was carried out in collaboration with St Jude’s Children’s Research Hospital, Memphis, who produced the vector, and received support from NHS Blood and Transplant, MRC and Wellcome Trust in the UK and NIH in the United States.

#### Haemophilia facts

- One in every 30,000 men has the genetic defect which causes haemophilia.
- People with haemophilia suffer bleeds that can be disabling if these are into the joints, or life threatening if blood leaks into the brain.
- Current treatment consists of regular injections of factor 9 protein.

## Community representatives ensure success of HIV database

Community representatives are working with UCL researchers to help create a unique database on HIV infection and treatment. The database will be a powerful new research tool, and patients and members of the community are playing a key role in ensuring its success.

Patient representatives are members of the steering group that shapes how the new research database is developed. But they also have a central role in disseminating information about the database and helping to dispel myths about research and treatments. Unusually, the project includes funding for a one-year community post to provide an important link between researchers and the community.

### Why the new database is so important

Backed by a grant from the Wellcome Trust, Professor Deenan Pillay, Director of the Comprehensive Biomedical Research Centre, is working to merge the various sources of HIV data in a bid to track disease trends more effectively and improve treatment based on the latest evidence.

Professor Pillay explains: "There's already been a fair bit of progress on developing large datasets for research purposes, but they are run in parallel by different organisations. They are not combined."

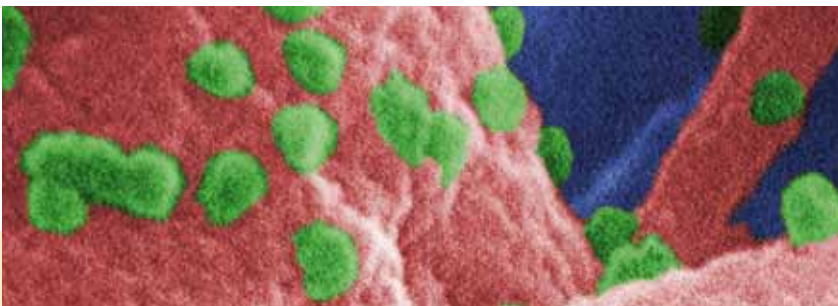
Current resources include the UK Collaborative HIV Cohort Study database containing details of around half of

the UK's 70,000 known HIV positive population; the Health Protection Agency databases on new diagnoses of HIV and on people in treatment; and a database on HIV drug resistance.

To combine these databases would provide a unique source of information for researchers, especially on the population infected and on the spread of drug resistant viruses. Research using databases can answer questions that clinical trials of a drug or treatment can't.

However, there are challenges to overcome including the huge number of patient records.

Also, because HIV infection is now a long-term condition, patients change address and care has to be taken to avoid duplications. Clinical centres can be very protective of their data, especially as they may be competing for patients or be defensive of their research reputations.







Simon Collins, editor of the HIV Treatment Bulletin, sits on the steering group

## Involving the community and patients

This is a new HIV database with a difference. Community representatives will be playing an unusually important role in linking the research to the HIV positive community.

“Patient activism in HIV has a long history,” says Professor Pillay. “This has led to greater demands for high quality care, and availability of effective therapies. In addition, it is only right that the patient voice is

also influential in the way that research is carried out. This is transformational.”

Steering group member Simon Collins, who is editor of the HIV Treatment Bulletin for the online resource i-Base, points out it is very unusual to have funding for a community activist included in academic grant applications.

The role of the activist is not just about health promotion and prevention. It is also about raising the profile of research, reassuring people about how data is used and showing people the value of database research in addressing those scientific questions that remain unanswered by more established methods of research.

“You can track what happens to people in the real world in a way that clinical trials can’t,” he says.

He adds: “HIV is still highly stigmatised. So the idea that people are collecting data on your personal medical history and social contacts has to be dealt with very carefully.”

By disseminating results from the database, community representatives can help provide the evidence which will dispel fear and uncertainty about different treatments and drugs. If the database becomes accepted as an important resource for the HIV community then this may prove a big incentive to take part.

## HIV facts

- An estimated 80,000 people in the UK are HIV positive. About 20% of these people don't know they are infected.
- Every year 7,000 people are diagnosed HIV positive for the first time.
- Thanks to treatment advances, the death rate of people who are HIV positive is now much more comparable with that of the general population.

## Community groups make sure genetic cancer screening study happens

The Genetic Cancer Prediction through Population Screening (GCaPPS) study is looking at the feasibility of offering everybody in the population a genetic test (in the form of a straightforward blood test) to see who is at high risk of developing cancers linked to certain genetic alterations.

Community members have been helping researchers recruit volunteers and deal with some of the difficult sensitivities surrounding genetic testing.

### The GCaPPS study

The study is looking specifically at screening for faults in two genes – BRCA1 and BRCA2. People who have inherited faults in these genes are at high risk of developing breast, ovarian or prostate cancers.

Researchers are comparing the effects of just testing people who have a family history of these particular cancers with the effects of screening people

regardless of family history. They want to know which kind of screening is the best way forward.

“We need to understand how acceptable offering [testing] on a population basis will be, whether it will detect more people at risk, how it will affect quality of life, and how many people will opt for screening and prevention,” says Dr Ranjit Manchanda of the Elizabeth Garrett Anderson Institute for Women’s Health at UCL, which is coordinating the GCaPPS study.

To do this, researchers are working with people of Ashkenazi Jewish origin who are 10 to 20 times more likely to carry a faulty gene than the general population. Usually people of Ashkenazi Jewish origin are only tested on the NHS if they have a known strong family history of the three cancers or if a relative has been diagnosed under the age

of 50. But this can miss a lot of people at risk. What the study wants to do is to recruit 10,000 people of Ashkenazi Jewish origin to be tested regardless of their family history.

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### Groups helped researchers deal with genetic testing sensitivities

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### Partnership working

Given the sensitivities involved and the potential implications for people being tested, it has taken extensive partnership working to get the study off the ground.

Liz Engel, from the Eve Appeal charity, helped with patient information



“We met with a lot of individuals across the board in the community, including rabbis and community leaders, and representatives from charities. We spent a year going out and getting their views,” says Dr Manchanda.

The result was the study was set up in the community with five accessible counselling and testing sites, including one in a north west London branch of Boots the Chemist. Various charities also provided sites in non-clinical settings.

Liz Engel from the Eve Appeal, one of the funding charities involved, helped with leaflets: “We needed to explain a complex health issue.” She continues: “It was a balance between not overloading with information, making sure it was appropriate for the Jewish community and not upsetting anyone.”

Before taking the test all volunteers are counselled on the implications including the possibility of carrying the genetic variants and the impact on job prospects, insurance and relationships.

© UCL Medical Illustration



Laura Miller chose to help by taking part in the study

### To know or not to know?

Head teacher Laura Miller picked up a leaflet at her synagogue and fitted the criteria of being over 18 and having four grandparents of Ashkenazi Jewish origin.

“I thought it was worthwhile because I had never seen a project before that was

specifically about Ashkenazi Jews, and if I could help in any way, I was happy to be part of it,” she says.

“I am one of those people who want to know about my genetic inheritance,” Laura says, but she understands why some people in her community would rather not know for religious and/or personal reasons.

The psychological impact of taking the test is a key aspect of the study. “What the test won’t tell you is whether you will develop cancer, and when,”

says Dr Manchanda. For those with a positive result, healthy lifestyle changes, surgery and even embryo testing are among the preventive options.

## Website helps brain injured patients read

Patients have been helping UCL researchers develop an innovative website for people whose vision has been damaged by brain injury.

Researchers wanted to find out the best way of helping people with a condition called hemianopic alexia (HA) to rebuild their reading skills. HA, which can be caused by a stroke, brain tumour or severe head injury, robs a person of half of their field of vision on the left or right side, sometimes slowing their ability to process written information by more than 50%.

### The challenge was to create an easy to use website

There is a therapy which has been shown to improve patients' eye movements and help them rebuild their reading skills, although it cannot restore their lost vision. The treatment, which until recently was not readily available to patients,

involves reading moving or scrolling text.

The challenge for researchers was to create a website that people could use to practise reading moving text and that they would find convenient and easy to use. So researchers enlisted patients to help them with the Read-Right project.

Dr Alex Leff, a consultant neurologist at UCLH's National Hospital for Neurology and Neurosurgery, is leading the project. He is researching into how language disorders can be

improved by different types of therapy, using brain imaging to track the effects of therapy on the brain.

One patient Dr Leff worked with is Mark Jarvis who had a stroke five years ago at the age of 42.

"I lost my right field of vision and couldn't read at all – not even my own name," explains Mark. "That function in my brain had shut down because there had been so much damage and swelling."

Although he made a rapid

### Patient Mark Jarvis helped develop the Read-Right site



recovery and went back to work, Mark could no longer read as quickly as he needed to. "In my line of work, I have to do a lot of presentations and proof read a lot of material," he says.

## Read-Right

The Read-Right website [www.readright.ucl.ac.uk](http://www.readright.ucl.ac.uk) helps people like Mark by providing text that moves from right to left. Patients can choose from a changing range of texts and can test their visual fields and reading speeds.

"When you look at a newspaper it can be really daunting because there is so much there. After a while you don't read anything because you get worn out," says Mark. "But the screen allows you to change the colour and font and to focus into certain things."

"The site tells you how long you are taking to read a paragraph/page, so it gives you an incentive to carry on," he adds.

Mark helped develop the website by pointing out things

like the need for simpler instruction phrases and changes to the language. His suggestions were taken on board and he thinks the site is very accessible.

"It will make a big difference," he enthuses. "There is nothing else available for people with this condition. Before, you just had to muddle through as best as you could."

## How the website retrains the brain

The moving text "induces a certain type of eye movement rather like watching a train pulling out of a station," says Dr Leff. Evidence suggests this can retrain the brain to produce the sorts of eye movement needed to read static text.

"Patients [with HA] rarely fully recover their original reading speed but after using the

web-based therapy for 15 hours or so, they can improve their reading skills by up to 50%," he says.

Patients need to practise daily. "It's better to do half an hour a day for a few weeks than an hour a week for a few months," he says.

The Read-Right website was produced with support by a grant from the Stoke Foundation and input from UCL's multimedia team.



## Patients find new ways of describing pain

Patients have been working with a visual artist and clinicians in a pioneering project at UCLH's Eastman Dental Hospital – one of the UK's few national centres of excellence for facial pain.

The aim of the project is to boost the quality of facial pain consultations. If a patient can describe clearly how pain feels – for example, whether it's stabbing, dull, or like an electric shock – then it is easier for doctors to identify what type of pain it is and the best way to treat it.

Visual artist Deborah Padfield is working with facial pain specialist Professor Joanna Zakrzewska on a study to find out whether visual images of pain can help communication between doctors and patients.

It can be very difficult for patients to find the words to describe their experience of pain. "Patients try to explain their pain, but very often just

say that it's very severe or like a toothache," says Professor Zakrzewska.

Deborah works with patients to co-create images which reflect their experience of pain. "People often do have an image in mind," she says. "The challenge is to translate that into a photographic form which is realisable and communicates beyond literal description. I think the images are more powerful because they combine both our strengths."

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### Being heard is a key step in a patient's ability to manage their pain

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One patient brought in a tree branch to be photographed, because it represented shooting pain "like lightning." Another patient described her pain as like "being sandwiched between two slices of bread".

Professor Zakrzewska says:

"So many patients tell us, 'Thank you for listening. It's the first time someone understands, and hasn't just told me that it is all in my mind.'" Being heard is a key step in a patient's ability to manage their pain, she says.

The project will be tracking the images patients create to see how pain is perceived before, during, and after treatment. This can be used to measure how well clinicians have understood and responded to patients' needs.

The project will also enable clinicians to build up a bank of images, in the form of a pack of pain cards, they can use in pain consultations.

## In a strange way it helped me

Elizabeth Aldous is one of hundreds of patients who have been helped by the Eastman Dental Hospital's work on facial pain consultations.

Elizabeth worked with Deborah to co-create images representing the pain she was experiencing. Elizabeth says: "The images were of decay, destruction and despair. I was once a functional, useful person who used to do loads of things and was now in pieces. It was upsetting but in a strange way it helped me manage my pain," she explains.

Initially Elizabeth was told by a dentist that a decaying tooth was the problem. But after the tooth had been removed, the pain actually got worse.

"The pain spread to my neck and my ears. Wind hurt my face, I couldn't eat, swim, sing, work – do all the things I love."

Elizabeth was finally referred to the facial pain clinic. She says: "The different specialists are



© Deborah Padfield

### Elizabeth Aldous is one of the patients who worked on the facial pain project

working together to try to find the right approach for me."

She adds: "Some approaches work, some don't – but they might for someone else! The

pain hasn't gone completely but I am noticing that I have more days when the pain is less severe."

## Patients advise on best way of doing prostate research

Patients have been playing a major part in helping researchers develop new ways of diagnosing and treating prostate cancer.

Researchers at UCLH/UCL have developed promising new ways of diagnosing prostate cancer more accurately and of using less invasive treatments. But it has taken essential input from patients to bring these new options closer to becoming a reality.

One of the key ways patients helped was by encouraging researchers to be clear and honest about the processes involved in their research and about the purpose of treatments. This has meant researchers have been able to enlist men to participate in their study. Researchers have also been able to understand better which treatments patients prefer and why.

### Why new ways of diagnosing prostate cancer are needed

The blood test for Prostate Specific Antigen (PSA) which is normally used can be misleading. Raised PSA levels may be a sign of cancer but may also be caused by other factors like infection or inflammation. The test cannot distinguish between aggressive

cancers needing radical treatment and slow growing tumours that need monitoring only.

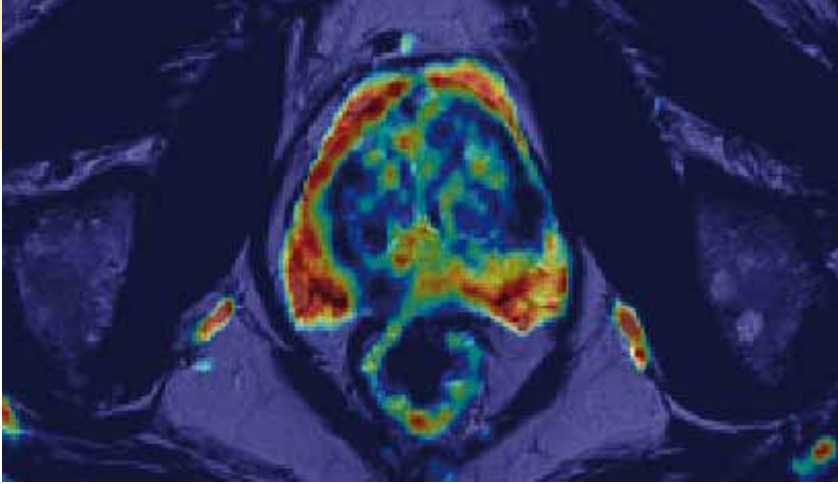
The current biopsy test is also not accurate. The test involves taking 10-12 tissue samples from the prostate via the rectum. But trying to pin point cancer like this is very hit and miss – a bit like trying to hit the pip in an orange using a needle.

Until recently men at risk of prostate cancer have undergone tests such as blood tests and biopsies that are deeply flawed. So a team of researchers at UCLH led by Professor Mark Emberton decided to see whether MRI imaging or scanning could be used to diagnose prostate cancer more accurately.

Professor Mark Emberton leads the team researching the use of scanning to diagnose prostate cancer







MRI scan of prostate tumour

The challenge for the research team was to enlist patients willing to undergo several demanding and unpleasant procedures to give them the information they needed.

Imaging has long been used to pinpoint cancer in other parts of the body, but before it could be used for diagnosing prostate cancer, researchers needed to find out what cancer looks like in a scan of the prostate.

To find this out they needed to sample the whole prostate of each study participant by taking biopsies every 5mm. For this the men needed to be under general anaesthetic. They also needed to have an MRI scan and biopsies via the rectum.

Two patient representatives, who are part of Professor

Emberton's study group, were concerned whether men would be willing to take part in such a demanding study. It was clear the men would benefit personally from greater accuracy offered by the surgical biopsy of the whole prostate. However, the men were unlikely to benefit from the biopsies taken via the rectum. The patient representatives advised the team simply to be very open and explicit about this.

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I advised them to tell [prospective recruits] like it is and not to dress it up

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Robert Oldroyd, one of the patient representatives who is familiar with research in

the field, said: "The team did recognise the burden we were placing on patients, but it's one of those trials where you can't do much else. So I advised them to tell [prospective recruits] like it is and not to dress it up... I look at it from a practical and logistical point of view. How will patients deal with it? Will they be happy to go through all the procedures? And will it actually improve their subsequent treatment?"

### Finding less invasive treatments for prostate cancer

Patients are also working with Professor Emberton's team to develop new treatments for prostate cancer. Professor Emberton says patients have had a major role in guiding the direction of research at UCLH into new treatments: "They are very good at pushing us to be clear about the therapeutic objectives and the benefits."

Finding new treatments is important because standard surgery and radiotherapy for prostate cancer can damage

surrounding nerves and muscles, with unpleasant and permanent side effects such as erectile dysfunction, incontinence or bowel problems.

The new minimally invasive approaches being trialled at UCLH/UCL (see box) include the use of heat (high intensity focused ultrasound), light (photodynamic therapy) or ice (cryotherapy). These therapies are called focal therapies as they only treat the cancerous tissue rather than the whole prostate gland and so minimise damage to healthy tissue.

But Professor Emberton's team needed to find out which of these different treatment options patients prefer and why. To do this they borrowed a technique used in business called 'discrete choice'.

Patients were asked to put a value on different aspects of treatment or diagnosis such as length of hospital stay, number of procedures required, likelihood and type of side effects, complications of surgery/treatment, and

### Focal therapies for prostate cancer

**Photodynamic therapy** takes place in the dark to avoid the risk of skin burns and is carried out under general anaesthetic. Fine laser fibres are inserted under the skin into the diseased parts of the prostate. The patient is then injected with a drug that reacts with the light from the lasers to destroy the blood vessels feeding the cancer.

**High intensity focused ultrasound (HIFU)** involves the insertion of a lubricated probe into the rectum, which gives out a beam of ultrasound and generates heat to destroy abnormal cells. HIFU is a day case procedure carried out either under general anaesthetic or after an epidural injection, which blocks feeling from the waist down.

**Cryotherapy** involves the insertion of several metal probes containing liquid nitrogen or argon into the affected part of the prostate under local or general anaesthetic. This freezes the abnormal cells and kills them.

Sources: UCLH, BUPA, Cancer Research UK

toxicity related to radiotherapy. The values patients place on particular aspects of treatment give researchers a much more detailed and deeper understanding of patient preferences.

Professor Emberton says: "In healthcare, we often do straight trade-offs between 'either' and 'or'. But this is more complex in that we present several different attributes at the same

time. This allows for more sophisticated judgement about what the tipping point is that makes a patient choose one option over another."

The research received funding from Medical Research Council (UK), Pelican Cancer Foundation, Prostate Action, Prostate Cancer Research Centre and St Peter's Research Trust.



University College London Hospitals   
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*National Institute for  
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The UCLH/UCL National Institute for Health Research Comprehensive Biomedical Research Centre is a partnership between University College London Hospitals (UCLH) and University College London (UCL) and is part of the National Institute for Health Research.

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