



# cf TODAY

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Living with Cystic Fibrosis: CF Week 2011

Talking transition

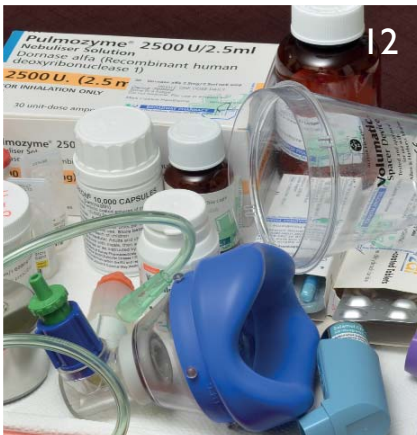
Clinical trial networks

Scottish elections

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# Contents

News	4
Cystic Fibrosis Week 2011	6
Ask the expert	8
Talking transition	9
Clinical trial networks	13
Scottish elections	14



The primary purpose of *CF Today* is to provide for its readers a reliable source of medical, research and other information relevant to Cystic Fibrosis and to play a supportive role for CF families. Opinions expressed in articles do not necessarily express the official policy of the Cystic Fibrosis Trust. The editor reserves the right to edit and otherwise alter articles or letters submitted to the magazine for publication.

Some pictures used in this publication may be posed by models or taken from library images.

*Medical information included in this publication is not intended to replace any advice you may receive from your doctor or CF multidisciplinary team and it is important that you seek medical advice whenever considering a change of treatment regimen.*

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Cover photo: Iain and Sophie Davison with their children James and George  
Photographer: Carrie Bugg

Welcome to this edition of *CF Today*.

The Cystic Fibrosis Trust has a tenacious vision of a world where the quality and length of life is not limited by Cystic Fibrosis. Since taking over from Rosie Barnes as Chief Executive last September I have thoroughly enjoyed meeting a number of the truly remarkable and inspiring people living with Cystic Fibrosis and their families.

I have also much valued visiting a number of Cystic Fibrosis services in the UK and meeting clinicians and other members of the multidisciplinary teams who are making the huge advances in CF care possible through personal drive and dedication.

Over the past 50 years there have been enormous advances in the quality and length of life for people living with Cystic Fibrosis but we are all very aware that there is a huge amount still to be done. Getting on with the research and advancing clinical care still further is urgent and will require a huge effort from us all.

I intend that the Cystic Fibrosis Trust will continue to be a driving force in the UK of advocating for all this to happen, assisting as best we can to deliver it, and ensuring that impact is happening on Cystic Fibrosis.

In a number of areas we are currently reviewing the best way of doing this. I have received a lot of advice and input already as to what the Trust should be focusing on for the next ten years but will welcome other contributions as well. I want the Trust to be addressing the key strategic issues in Cystic Fibrosis and working with others to create impact. We will be focused, agile, transparent and business-like in the allocation of our limited resources.

Some of you are helping us reshape the peer review programme and we are also looking at further developing the CF Registry to be a leading clinical and research tool. We are making closer links with

other European Cystic Fibrosis organisations and working with colleagues in the US. It is in the direct interest of people living with Cystic Fibrosis in the UK for there to be a more focused international effort on research, clinical trials and driving up clinical outcomes. The Cystic Fibrosis Trust will play a full role in that.

We are also reviewing our research programme and will increasingly focus our efforts on where we can achieve the greatest impact and seek leverage through co-financing. We are all very encouraged by the small molecule developments in VX-770 for those with the G551D mutation. News of game-changing results like these urges us all on to seek the next generation of therapies and interventions that will bring step changes in both quality and length of life.

All of this means that we are about to redouble our efforts to raise the money that is required to make all these advances happen. In May Cystic Fibrosis Week will be an excellent opportunity for each one of us to do whatever we can to raise the money that buys the next wave of developments in Cystic Fibrosis.

I am kick starting my own personal fundraising by running the London Marathon this year. If you would like to sponsor me then please visit [www.virginmoneygiving.com/matthewreed](http://www.virginmoneygiving.com/matthewreed).

Thank you.

Best wishes,



Matthew Reed  
Chief Executive



Matthew Reed

## Victory as Cherry Tree remains open

### Northern Ireland

We were delighted to hear in January that Cherry Tree House Adolescent Unit, part of Allen Ward at the Royal Belfast Hospital for Sick Children, will continue to be used for young people with Cystic Fibrosis.

Along with Allen Ward, Cherry Tree House is part of the paediatric CF service. It provides accommodation for three teenage Cystic Fibrosis in-patients at a time along with a parent's room, a gymnasium, a shared bathroom and a treatment room and was built over 20 years ago with funds raised by parents of children with Cystic Fibrosis. The Cystic Fibrosis Trust learned last year that hospital management planned to turn the existing in-patient rooms into general accommodation for parents whose children were being treated in Allen Ward. After lengthy negotiations and consultation with parents and the CF Trust, the Belfast Health and Social Care Trust agreed to keep two in-patient rooms in Cherry Tree House on a priority basis for teenagers with Cystic Fibrosis.

The Paediatric Cystic Fibrosis Centre is the only service of its kind in Northern Ireland and looks after 190 children with CF, including 55 teenagers. We are very pleased that the combined efforts of the Cystic Fibrosis Trust, Belfast Hospital for Sick Children and parents of children attending the Cherry Tree Unit persuaded the hospital trust to halt the closure of this vital resource.

## Brompton paediatric CF services under threat

Following the recommendations of England's Joint Committee of Primary Care Trusts to cease children's cardiac care at the Royal Brompton Hospital (RBH) in London, Royal Brompton & Harefield NHS Foundation Trust is mounting a strong campaign to challenge the recommendation. Should the recommendations go ahead, the hospital could not sustain its children's intensive care or anaesthesia services – making provision for paediatric Cystic Fibrosis patients inadequate.

The Cystic Fibrosis Trust considers the paediatric CF service at the Royal Brompton Hospital to be vitally important in terms of the standard of care provided, its strong research base which has both national and international recognition, and also for the development of guidelines for the care of children with Cystic Fibrosis which are used throughout the UK and referenced on many of the CF Trust documents, including the recently launched CF Clinical Care Pathway. In addition it is unfeasible that the 300+ patients currently cared for at RBH could be easily or readily accommodated by any of the other London-based CF specialist services without significant investment and expansion, neither of which seem to be an option in the current climate.

The public consultation began on 1 March and we would urge people to make their voice on this issue heard. Visit [www.specialisedservices.nhs.uk/safe\\_sustainable/public-consultation-2011](http://www.specialisedservices.nhs.uk/safe_sustainable/public-consultation-2011) for more information about consultation events and how to have your say. You can also show your support for the Royal Brompton & Harefield NHS Trust on Facebook:

[www.facebook.com/savebromptonchildrenscardiaccservices](http://www.facebook.com/savebromptonchildrenscardiaccservices)

## Big discounts with Big Yellow Storage



Scott Mackenzie from Big Yellow Storage (left) is pictured with Malcolm Moore, Director of Operations at the Cystic Fibrosis Trust

The Cystic Fibrosis Trust is delighted to be working with Big Yellow in Bromley, who are supplying us with storage space to keep important materials close to hand.

Big Yellow are offering readers of *CF Today* an extra 10% off their storage charges plus 15% off any packaging materials upon production of this article. For more information please call Scott at Bromley Big Yellow on 020 8313 0854 for details of your local store.

## CF Week 2011

Cystic Fibrosis Week 2011 will take place from 8–14 May. We're hoping this year will be the biggest and best

**CYSTIC FIBROSIS WEEK  
8-14 MAY 2011**

CF Week yet – and you can help make it happen! Turn to our feature on page six to find out more, or check out the latest edition of our fundraising magazine *Inspired!* If you don't receive *Inspired!* together with *CF Today* you can view it on our website [www.cftrust.org.uk](http://www.cftrust.org.uk) in the publications section or to be added to the mailing list please email [publications@cftrust.org.uk](mailto:publications@cftrust.org.uk) with your name and address.

## Prescription charges abolished in Scotland

Prescription charges have been completely abolished in Scotland as of 1 April following approval by Holyrood's health committee.

In England, the cost of individual prescriptions has risen by 20p to £7.40 per item, though people with Cystic Fibrosis who have to pay for their prescriptions can purchase pre-payment certificates (PPCs) to cover the cost of all their medication for three or 12 months. The cost of the three-month PPC has risen to £29.10 and the cost of the 12-month PPC has been frozen at £104.

For further information see our factsheet on Prescription Charges: [www.cftrust.org.uk/aboutcf/publications](http://www.cftrust.org.uk/aboutcf/publications)

## Counting down to 40

In his late 30s Tim Wooton, who has Cystic Fibrosis, kept a blog of his thoughts and observations as the big 4-0 approached. The blog was featured in the *Guardian* in February and has garnered lots of interest from the CF community – you can view and comment on Tim's posts at <http://timwootton.wordpress.com>.

## Postponed parents conference

The parents conference mentioned in the last edition of *CF Today* has been postponed, however we do plan to hold an event later in the year. Keep an eye on our website for details; we'll also be sending invites in the post when the details have been finalised.

## MBE for Rob

Huge congratulations to designer Rob Law, entrepreneur and creator of the famous 'Trunki' suitcases for children, who has been awarded an MBE in the New Year's Honours List.

Rob, who has Cystic Fibrosis, had the idea for Trunki whilst studying Industrial Design at university, after seeing how bored children could get whilst travelling – particularly in airports. Despite the initial concept being rejected by the panel on the BBC's *Dragons' Den*, Rob didn't let anything stand in the way of his vision – and rightly so! Today, Trunki is a hit in over 60 countries worldwide and Rob's company Magmatic now has an entire range of luggage accessories for kids and an annual turnover of £4.5 million.

"Many of my peers are entrepreneurs and one thing we all share is overcoming a significant obstacle in life," said Rob. "People with Cystic Fibrosis and their carers find it a daily challenge fighting off the next infection, but in doing so it gives us drive and determination not to be beaten.

"My advice to any budding entrepreneurs with CF is that you've already got the grit and determination to succeed at whatever you put your mind to. The trickiest bit is trying to find time for your treatments while being pulled in all directions that running a business demands."

Visit [www.trunki.co.uk](http://www.trunki.co.uk) to find out more about Rob's journey and view his top tips for budding inventors. You can also follow Rob on Twitter @rob4law.



Rob Law, who received an MBE in the New Year's Honours List

## Have you met Oli and Nush?



DVD copies of our new film for children with Cystic Fibrosis – *Getting nosey about CF with Oli and Nush* – are now available for loan.

The cartoon is proving very popular with children and adults alike, and had almost 10,000 hits during its first month on YouTube! If you'd like us to send you a copy, please email [enquiries@cftrust.org.uk](mailto:enquiries@cftrust.org.uk) with your name and address or telephone the CF Trust on 020 8464 7211.

Please note we have a limited number of DVDs and parents or carers of children with CF will be given priority. There is no charge to loan the DVDs and you can keep them for as long as you like, however if you'd like to make a donation to Oli and Nush's fundraising page to contribute towards the costs of producing the DVD, you can do so at [www.virginmoneygiving.com/oliandnush](http://www.virginmoneygiving.com/oliandnush).

## Marathon effort

Good luck to all 220 Team CF runners taking part in the London Marathon this month – including six runners with Cystic Fibrosis and our Chief Executive Matthew Reed!



Photo: Matt Grum

CF Trust Expert Patient Adviser Lynsey Morton, pictured here with her dog Diesel, is one of six runners with CF taking part in this year's London Marathon

## Treatment for G551D mutation shows promising results

Clinical trial results from Vertex Pharmaceuticals have shown that a new Cystic Fibrosis drug, called VX-770, has improved lung function, reduced exacerbations and encouraged weight gain in people with CF who have the G551D mutation. See our research section on page 12 to find out more.

# Cystic Fibrosis Week 2011

During this year's Cystic Fibrosis Week (8–14 May) we will be asking the public to help the Cystic Fibrosis Trust continue its vital work.

The theme of the Week is *Living with Cystic Fibrosis*, and we'll be illustrating how the Cystic Fibrosis Trust supports people with Cystic Fibrosis in a variety of ways, with help from four fantastic case studies. Introduced below, our faces of CF Week will be sharing their stories of living with Cystic Fibrosis. We hope that by showing the determination of those affected by CF to live their lives to the full, despite the obstacles they sometimes have to face and the intensive daily treatment regimen needed to stay as well as possible, this will inspire people to help make a lasting difference to the lives of all those living with Cystic Fibrosis in the UK.

## Get involved

There are many ways you can get involved this CF Week. To get started, visit our special CF Week microsite [www.cfweek.org.uk](http://www.cfweek.org.uk). You can find out about different ways to fundraise and events taking place across the UK, order a CF Week pack with lots of great ideas and information, find out more about our faces of CF Week and view our live twitter feed to see what's happening throughout the week. We'll be updating the microsite with lots more great news and activities as CF Week gets closer so do check back regularly to see what's new.

Join us online

[www.cfweek.org.uk](http://www.cfweek.org.uk)

[www.twitter.com/cftrust](https://twitter.com/cftrust)

[www.facebook.com/cftrust](https://www.facebook.com/cftrust)

## Introducing the faces of CF Week 2011

### Cassie Hawthorne, 21



Cassie Hawthorne

Cassie lives in Birmingham and was diagnosed with Cystic Fibrosis at just six weeks. At 16, Cassie was asked to be in the annual report for the Birmingham Children's Hospital where she was a patient at the time. This led to Cassie being approached by a modelling agency, kicking off a successful career working on campaigns for big brands, as well as appearing in a fashion show in Liverpool for the Cystic Fibrosis Trust.

Last April Cassie had a bad chest infection and was admitted to hospital for a three-week course of IVs. Yet just a few weeks later, to celebrate her 21st birthday, Cassie took part in a skydive with 25 friends and raised over £5,000 – she and her family are regular fundraisers for the Cystic Fibrosis Trust.

### Aaron Brown, 25

Aaron was born in Leeds and went straight from his A-levels to studying medicine, refusing to let Cystic Fibrosis stand in the way of his dream to become a doctor.

Aaron admits it can be difficult fitting in a gruelling treatment regimen, especially when he is working long hours on-call and studying. Despite this, Aaron's determination saw him through and he has recently qualified as a surgeon, citing the medical staff who have treated him over the years as his inspiration.



Aaron Brown

### James and George Davison, four and two

James and George were diagnosed with Cystic Fibrosis at the same time. When George's CF was picked up through newborn screening, James was already in hospital battling pneumonia. Since then mum

Sophie and dad

Iain have

thrown



George and James Davison with mum and dad  
Sophie and Iain

themselves into fundraising for the Cystic Fibrosis Trust – Sophie has completed the Hadrian's Wall trek and Iain took part in the Ben Nevis Challenge, and other family members are joining Team CF in the London Marathon this year.

James and George are active little boys – George greatly enjoys swimming and James is a very keen reader.

### Sam Roonan, 12

Sam lives with his mum Lisa, dad Mark and sister Josie, and has been on the waiting list for a lung transplant for three and a half years; however both he and his family remain positive and are determined to make the most of every day.



Sam Roonan

Sam loves football, and also enjoys cricket, tennis, music and playing the guitar. He started secondary school last year and so far has managed to spend more time at school than he has for a few years. If he is not able to attend classes he completes lessons using a webcam and is taught at home.

*You can find out more about our faces of CF Week 2011 on our CF Week microsite [www.cfweek.org.uk](http://www.cfweek.org.uk).*

## How the Cystic Fibrosis Trust helps

Money raised during Cystic Fibrosis Week will help the Cystic Fibrosis Trust continue to support families and individuals affected by Cystic Fibrosis. Our aim is to help people with Cystic Fibrosis like Cassie, Aaron, James, George and Sam to live their lives as fully as possible. These are just some of the ways in which the CF Trust helps people living with Cystic Fibrosis.

### Research

The Cystic Fibrosis Trust is one of the leading investors in CF research in the world. Last year we spent £3.1 million on medical research. We fund work to control the symptoms and treat the cause of Cystic Fibrosis.

The CF Trust funds research into gene therapy to add a healthy copy of the gene to the lungs of those with Cystic Fibrosis. It is hoped that a major clinical trial will begin later this year involving up to 100 young people with Cystic Fibrosis, which would be the first time in the world CF gene therapy has been studied this way.

Last year, the Trust also funded ten projects into areas of concern such as controlling infection and inflammation in the lungs, detecting early signs of lung disease, ways of using drugs to treat CF and transforming unusable lungs into viable lungs for transplantation.

### Care

The Cystic Fibrosis Trust funds the training of specialist CF doctors to care for the growing numbers of adults with Cystic Fibrosis. Our peer review programme helps ensure that the care of people with CF in Specialist CF Centres and Clinics across the UK is safe and of a high quality. It has so far secured £18 million of extra NHS funding for CF services. The Trust funds posts in the multidisciplinary team, such as dietitians and physiotherapists, needed for the appropriate care of people with Cystic Fibrosis. It also funds the national registry of CF patients in the UK which helps us tailor our clinical care programme. Last year, the Trust spent £1.3 million on work to improve clinical care.

### Support

The Cystic Fibrosis Trust runs three national helplines for people with Cystic Fibrosis and their families. It also awards around £100,000 of grants each year to help pay for respite holidays, prescription charges and funeral grants. Last year, the Trust spent over £900,000 supporting people with CF and their families.

**You can find out more about our activities and how we spend our money in our 2010 Annual Review – visit [www.cftrust.org.uk/aboutcf/publications](http://www.cftrust.org.uk/aboutcf/publications).**

# Ask the expert

Ask the Expert is the Cystic Fibrosis Trust's expert advice service for people with Cystic Fibrosis and their families. Questions are answered by a panel of clinicians who specialise in different areas. Although your query and the response may appear on the Cystic Fibrosis Trust website or in this magazine, your name and personal details will always remain confidential and will not be published.

## Glandular fever risk

**Q** I am a male with Cystic Fibrosis and my partner's child may have glandular fever (we're waiting for blood test results). Would it be in my interest to avoid contact with the child? I don't know if I have ever had glandular fever before.

**A** Over 60% of adults will have antibodies to Epstein-Barr virus (EBV) – the virus that causes glandular fever – either from clinical or subclinical infection as a child or adolescent. Contact is therefore unlikely to be a problem. Repeat infection does not occur.

The only time EBV can be a problem is after transplant, which does not apply here. So there is no evidence that the infection is any different in those with Cystic Fibrosis to those without – no need to take precautions.

## Bone scans in children

**Q** Our CF team has recommended that my eight-year-old daughter has a DXA scan at her next annual review, to check her bone density.

I have looked this up and it seems to be another – admittedly low dose – X-ray. Even if she did have low bone density, from what I understand there is not much you can do about it other than continue taking vitamin K, increase calcium intake and do more weight-bearing exercise (she does all of this at present).

Please could you advise whether this is necessary and whether there is any risk?

**A** We have discussed your concerns with an expert on osteoporosis in Cystic Fibrosis. He confirms that the DXA scan gives a very very low dose of radiation – no more than you would get from a sunny day – and is of no danger to organs at all.

Eight years may be rather earlier than is routine in many centres where ten or 11 is

the age for the first scan. However, there is no absolute rule and clinicians may vary slightly, but most would advise a scan around this age. If the scan is normal it need only be repeated every three years. If by some chance the result is abnormal, it is important to monitor with more frequent scans and to investigate for cause e.g. low vitamin D or calcium and if older the pubertal status. In summary – sensible to do, safe, and interventions are available if needed.



**DXA scans are needed as people with CF are at risk of early thinning of the bones**

For further information see the CF Trust factsheet *Bone Disease in CF*, available from [www.cftrust.org.uk/aboutcf/publications](http://www.cftrust.org.uk/aboutcf/publications). You may also find the Report of the CF Trust Bone Mineralisation Working Group's *Bone Mineralisation in Cystic Fibrosis* of interest. It is the consensus view of a number of national and international experts on the subject, and also available on the CF Trust's website or as a hard copy by contacting the CF Trust switchboard.

## Vitamins and nerve damage

**Q** In the last *CF Today* I read the article about vitamins in which it mentioned that a very low vitamin E level

can lead to nerve damage. My son is one of these cases; he developed bilateral facial nerve damage and now is unable to smile. I was wondering if you could tell me what other nerves have been reported as damaged, and what happened to them – is recovery possible?

**A** As was stated in the article, severe vitamin E deficiency is very rare in people with Cystic Fibrosis. There are some early reports of severe vitamin E deficiency in patients with CF who were not receiving appropriate pancreatic enzyme replacement therapy and appropriate vitamin supplementation, but those reporting neurological problems are over 20 years old. With early diagnosis of Cystic Fibrosis and early introduction of pancreatic enzyme replacement therapy and vitamin supplementation severe vitamin E deficiency has been virtually eliminated.

There may be other potential causes of nerve damage in addition to vitamin E deficiency. Vitamin levels are usually monitored as part of the annual review so it would be worth discussing this with your CF team.

## Our experts for this issue were:

**Dr Steve Conway, Consultant Paediatrician and Physician, St James's University Hospital, Leeds**

**Dr Jim Littlewood, Chairman, Cystic Fibrosis Trust**

**Alison Morton, Specialist CF Dietitian, St James's University Hospital, Leeds**

You can view an archive of past Ask the Expert questions at [www.cftrust.org.uk/aboutcf/asktheexpert](http://www.cftrust.org.uk/aboutcf/asktheexpert).

Please note if you have any queries or concerns about any aspect of Cystic Fibrosis you should contact your CF team in the first instance.

Submit your question to [asktheexpert@cftrust.org.uk](mailto:asktheexpert@cftrust.org.uk)

# Talking transition

The process of transition from paediatric to adult care can be a difficult time, not only for young people with Cystic Fibrosis but also for those who have looked after them. However with an open and honest approach, good preparation and the support of both the paediatric and adult clinical teams, transition need not always be turbulent. In this article, a young person with CF, a parent and two CF nurse specialists describe their experiences.

Zanib Nasim, 19, has recently undergone transition from the paediatric to the adult clinic, and now receives her care at Wythenshawe Hospital in Manchester. As Zanib explains, she asked to transfer a little later than her team suggested, so that she could focus on studying for her A-levels and applying for university places without disruption.

My name is Zanib, and I've just undergone transition from the paediatric unit to adult services. Overall my experience has been good, however my move was slightly later than normal. I asked my consultant if he could postpone transition until I had taken my A-level exams and started applying for university places, as it was a rather stressful time and the added complication of transition was the last thing I wanted on my mind. Thankfully the CF team agreed to this, and I was transferred over to the adult CF Centre when I was 19, by which time I had received my A-level results and had started studying for my degree in pharmacy.

During the process of transition, I first of all met the CF nurse who was going to take over my care. After this I had the opportunity to have a look around the Centre, which was a great opportunity to see some of the nurses who were going to look after me, and the actual unit. However even after this, I was still slightly apprehensive about moving, mainly because I knew all the CF team at paediatrics. I didn't want to go from somewhere I knew everyone to somewhere I didn't know anyone.

Later I got my first official appointment from the adult Centre. It was a bit scary and I wasn't really looking forward to it, but when I met the friendly consultant he immediately put me at ease. At the same appointment I met a physiotherapist, dietitian, social worker and the CF nurse – it was a long appointment but I found that after meeting the people who were going to look after me, the worries I had about my transfer were far less. I've since had my first admission, which was also better than I expected.

I feel that transitioning after my A-levels gave me a bit of time to prepare myself mentally for the transition process, and I was relieved to have less on my plate during a stressful time. However I would tell those about to



Zanib Nasim

*“I didn't want to go from somewhere I knew everyone to somewhere I didn't know anyone.”*

undergo transition not to worry too much. Once you meet your new team and spend a bit of time at the adult unit, you will probably find you feel a lot more comfortable with the whole idea. And as the adult Centre will be treating loads of other adults with CF, you can be confident that they will know what they are doing!

The focus during transition is rightly the young person who is making the move to adult services, but for some parents the issue of letting go can be difficult. Clare Thomas, mum to 19-year-old Jordan, explains how the transition process left her feeling 'redundant'.

It is hinted at in the run up to the transition from paediatrics to the adult clinic. We know its coming; we start to hear about it way in advance of the actual day when we leave the paediatrician behind. The plans to meet the new team, the questions addressed to the patient

instead of the parent, the pregnant pauses when realise that we must keep schtum rather than expand upon the monosyllabic answers provided by our offspring... We're placated by assurances that we too can attend the adult clinic, that of course parents are welcome, of course we must come along and meet the team, visit the ward etc. And so we go. But as the colourful, cartooned walls of paediatrics are left behind forever, it slowly dawns on us that we are no longer needed.

I am a redundant CF parent. On the surface this is good news; I am the mother of a healthy (CF-notwithstanding) 19-year-old young man who enjoys a full, happy life and is popular, funny and obnoxious (in equal doses) – he basically has the life that 19 years ago I feared wouldn't be possible. We are very lucky and I am only too aware of all of this.

But this 'redundancy' comes at a price. It is not something that's easy to talk about; there is no professional on standby to counsel us. We've played our part; seen our job through to its conclusion. We're dismissed. And whilst this is a good and positive thing, it is as I have discovered also a time of discomfort and disquiet for the parent of the almost-grown-up-person-with-Cystic-Fibrosis.

*“Suddenly the adult clinic is the norm and our presence is not.”*

From the moment Jordan was diagnosed we were immersed, reluctantly, in a strange new world of physiotherapy, unusual dietary advice, more drugs than we have ever seen, nebulisers, clinic appointments, cross infection, genetic counselling... the list goes on. It becomes our life, whether we want it to or not. We juggle 'ordinary parenting', marriage, work and housekeeping with specialised nursing, and get used to the fact that we know more than our GP about our unchosen specialist subject. We become experts, hoping that if we do our very best we can retain some control over this disease.

And then the 16th birthday dawns. Or maybe the 17th or 18th. There is no hard or fast rule we're told; transition can happen at our own pace, to suit our individual needs. But suddenly the adult clinic is the norm and our presence



Jordan and Clare Thomas

is not. We must grit our teeth while our slightly bored teen does a vague (and incorrect!) calculation of how many Creon he takes a day, almost daring us to contradict him.

Of course it is only right that our children grow up and out of our care. All we've ever wanted is for them to be well enough to be able to be independent. But I for one did not subscribe this agonising interim stage where our responsibility has been relinquished, and our children have had it made clear to them that they are now in charge of their own care. The unintended message seems to be "we can no longer make you comply... and nor can your parents!"

The point is, even at 18, they are not fully adult yet – full maturity comes much later. Meanwhile we must merely watch and listen as the very religion that we have lived by for as long as we can remember is ignored or given cursory, fleeting attention. How am I supposed to calmly sit back and watch my careful regime of 16 years be blown to the wind?

I don't know what the answer is. My son is not 'off the rails'; he is articulate, intelligent and knows all about Cystic Fibrosis including the grim facts. Yet whilst he knows how important he is to me, as far as he's concerned he isn't hurting anyone because he feels 'great' and his lung function is 'tops'. But how long can it stay like that?

So whilst I recognise the requirement of adult clinics to allow young people with Cystic Fibrosis to be responsible for their own health, please spare a thought for us 'redundant parents' – only too aware that our days are

numbered, that our presence won't be tolerated much longer by our son or our daughter even if the clinic staff are prepared to continue putting up with us. If we look as if we're sucking wasps as you consult with our teenage offspring during the transition process, well, perhaps you now have a deeper insight as to why. We are biting our tongues. Hard.

At the Norfolk and Norwich University Hospital (NNUH) both the children and adult CF Centres are on the same site, with multidisciplinary teams attending joint monthly clinics for young people with Cystic Fibrosis between 14 and 16 years of age. Mary Ann Boyfield and Alison Betteridge, CF Nurse Specialists at the hospital outline their three key principles of transition, which is treated not as a single event but a process that takes place over time.

Whilst many young people view careful, planned transition as 'a right of passage' a recent survey by the NNUH adult team revealed that some individuals and their families remain anxious. To this end we must acknowledge that learning to take control of one's own care often continues well into an individual's adult clinic years, and transition is an opportunity to start helping young people to make choices that are beneficial to their health and well-being.

#### Early, open communication about moving

Discussions with our young people with Cystic Fibrosis Trust start around the age of 13. They are a three-way process between the young person, their family and the children's CF care team at first, and develop over time with the adult team. At the NNUH we have a folder of written contributions from young people who have already transferred to adult services, in which they give a glimpse of their lives as established independent adults. As well as being available for young people attending the transition clinic, we also introduce this to parents attending the children's clinic when their children are newly diagnosed – it gives a real sense of hope for the future and sets the seed of expectation of moving to an adult clinic as a normal progression.

#### An agreed structure and plan for transition

Based on continued discussion, the young person and their family agree a timescale for attending the 'teenage' clinic. This usually starts at age 14 and transfer to the adult service is at a planned, mutually agreed point some time after GCSEs. For those individuals with additional complications such as diabetes, the co-ordination of subspecialty transition is planned to be simultaneous with CF transition, i.e. attending the adolescent diabetes clinic.



Members of the NNUH team

During transition the young person learns to gradually increase responsibility for their own health needs and take on a more pro-active role during consultations. At some point during the process the young person may feel ready to be seen on their own for all or part of the clinic. To make informed choices, a young person requires sufficient information, and we are introducing a checklist of skills with which we can work together with an individual and their family.

A tour of the adult facilities and an information brochure is also provided prior to transfer.

*“Learning to take control of one's own care often continues well into adult years.”*

#### Addressing the concerns of young people and their parents

The shift in responsibility towards self-care can be a stressful time for families. An assertion of independence can be at odds with parental concern together with fearing the loss of trusted support from the children's team. Careful and patient listening to those concerned is crucial in easing anxieties. Building a relationship with a new team requires ample time and this will continue well after transfer has occurred.

#### Further information

The Cystic Fibrosis Trust publishes three factsheets on transition for young people, parents and clinical teams, which can be downloaded from: [www.cftrust.org.uk/aboutcf/publications/factsheets](http://www.cftrust.org.uk/aboutcf/publications/factsheets)

## Treatment for G551D mutation shows promising results

Clinical trial results have shown that Vertex Pharmaceuticals' new Cystic Fibrosis treatment VX-770 has improved lung function, reduced exacerbations and encouraged weight gain in people with CF who have the G551D mutation – around 4–5% of the UK CF population.

The oral medication belongs to a class of drugs known as 'potentiators' designed to improve the function of the CFTR protein, which controls the movement of sodium and chloride into and out of cells. In Cystic Fibrosis, CFTR is either absent or doesn't work properly, therefore causing the build up of thick sticky mucus. Correcting the action of CFTR would allow sodium and chloride to move properly in and out of cells lining the lungs and other organs.

In the phase III trial, those who received VX-770 over a period of 48 weeks showed a 17% improvement in lung function compared to those receiving the placebo, a 55% reduction in pulmonary exacerbations (worsening lung symptoms requiring antibiotic treatment), significant

reductions in sweat chloride (considered to be a marker of improved CFTR function) and also gained an average of 7lb.

Vertex will now apply to regulatory bodies to make VX-770 available in the clinic and hopes to have this approved in the second half of 2011.

The Cystic Fibrosis Trust is encouraged to hear the results of this research and will be monitoring progress with interest. Dr Diana Bilton, chair of the Cystic Fibrosis Trust Medical Advisory Committee and a Consultant Physician at the Royal Brompton Hospital in London, said that whilst this drug will benefit a small, specific group of people with Cystic Fibrosis, it represents a real breakthrough in the principle of treatments for Cystic Fibrosis and opens the way for exploring such therapies for people with more common mutations. "We were very excited by the results that show a product that alters the function of the dysfunctional CFTR protein in patients with the G551D mutation really benefits

the person in terms of improved lung function and reduced exacerbations," Dr Bilton said.

### Other mutations

Vertex is also conducting a phase II clinical trial of VX-770 in combination with another treatment, VX-809, which it hopes will prove effective for those who have the much more common F508del mutation. VX-809 belongs to the class of drugs known as 'correctors' which means it targets the production of CFTR as opposed to the function, delivering it to the correct location in the cell. The results from this trial are expected later this year.

### Further information

Vertex Pharmaceuticals [www.vrtx.com](http://www.vrtx.com).

You can also visit the CF Trust's YouTube channel [www.youtube.com/cftrust](http://www.youtube.com/cftrust) to see Dr David Sheppard of the Cystic Fibrosis Trust's Research Advisory Committee talking about VX-770 and the action of CFTR potentiators.

## Denufosol turnaround

In December last year, Inspire Pharmaceuticals released data from a clinical trial suggesting that their inhaled treatment Denufosol could benefit people with Cystic Fibrosis with normal to mildly impaired lung function.

At the time, the news sounded very positive. However in January Inspire announced that the drug, which was

intended to help clear mucus from the airways, had failed to show statistically significant improvement in lung function in a later trial. This was unexpected due to the promising data shown previously.

Although disappointing, this turnaround does demonstrate the uncertain nature of scientific research and particularly clinical trials (see box).



All new medicines must undergo clinical trials before reaching patients

### Interpreting clinical trial results

For a new treatment to reach patients, it must undergo clinical trials – a process which can take several years. This lengthy, thorough procedure is essential to ensure that the product being developed is both safe and effective. Although a treatment may look promising initially, during early trials conducted in a relatively small number of people, positive results cannot always be replicated when a trial is scaled up.

Whilst negative results in a Cystic Fibrosis trial are disappointing, it is important to remember that it is a good thing that the research has been carried out, even if it is shown that a treatment is not effective. Even in a trial with negative results, scientists learn things that they and their colleagues can put to use in future trials and in other areas of research, ultimately benefiting patients.

So whilst it is good to take an interest in scientific research in Cystic Fibrosis, it is important to be realistic about treatments in the pipeline, some of which will be successful and will significantly help those with CF, and others which will unfortunately – but for good reason – not make it through the strict regulatory processes.

### Further information

The organization Sense about Science produces a useful leaflet entitled 'I don't know what to believe', to aid understanding of scientific research in the news: [www.senseaboutscience.org.uk](http://www.senseaboutscience.org.uk)

Clinical trials explained. *CF Today* Summer 2009. Download from [www.cftrust.org.uk/aboutcf/publications](http://www.cftrust.org.uk/aboutcf/publications).

See also [www.cftrust.org.uk/research/clinicaltrials](http://www.cftrust.org.uk/research/clinicaltrials).

# Clinical trial networks: Improving treatments for Cystic Fibrosis

In this article **Dr Tim Lee**, CF Consultant at Leeds Regional Paediatric CF Centre and **Professor Alan Smyth**, CF Consultant at Nottingham Children and Young Person's CF Centre explain how the development of clinical trial networks is helping speed the journey of new Cystic Fibrosis treatments to the clinic.

Treatments for people with Cystic Fibrosis have improved dramatically over the last three decades, largely due to research funded either by the Cystic Fibrosis Trust, other charities, government grants or pharmaceutical companies. But before new medications are made widely available to use in routine CF care it is vital to prove that they are safe and effective, and this means carrying out clinical trials involving large numbers of volunteers.

New medications are tightly regulated by both UK and European legislation and new Cystic Fibrosis treatments have to first pass stringent laboratory tests, followed by trials in healthy adults, to ensure the medication is safe. The next stage of clinical testing, to show drugs are effective, can only be carried out in fully informed volunteers with Cystic Fibrosis. Some of you reading this article may have already participated as volunteers in such studies, or consented on behalf of your son or daughter with Cystic Fibrosis. Drugs that are now part of routine treatment for CF, such as dornase alfa, nebulised antibiotics and hypertonic saline, are only available because people with CF have agreed to take part in clinical trials. People with CF who take part in trials today will be helping a future generation of patients.

In the past, small studies in just one or two CF clinics were enough to show dramatic benefits and appropriate safety data. However, because people with Cystic Fibrosis are now so much healthier and are living longer, clinical trials need to include many more volunteers to prove additional benefits over and above the treatments we have now, as well as ensuring safety in a healthier CF population. The only way to achieve these larger studies, to help continue to improve treatments for CF, is to run large multi-centre studies, either across all CF clinics in the UK, or often, across Europe or worldwide. Consequently, clinical trial networks are now extremely important.

## What are clinical trial networks?

Clinical trial networks help studies to include enough people with Cystic Fibrosis as volunteer participants. They focus on ensuring that the studies are run extremely carefully and smoothly in each participating Centre, and that all staff involved in studies are fully trained in the appropriate technical and ethical aspects of running clinical trials. Clinical trial networks also are involved in assessing clinical trial protocols for new CF treatments at an early stage, to ensure that the study is definitely needed and that the protocol will be practical for people with CF (e.g. not involving too many visits or tests). These networks have allowed more people with CF to take part in clinical trials.

For people with Cystic Fibrosis in the UK there are three clinical trial networks to be aware of:

### 1) European CF Society Clinical Trial Network

This network of large adult and paediatric CF Centres across

Europe has an administrative base in Leuven, Belgium. There is an overseeing committee which includes representation from CF patient organisations such as the Cystic Fibrosis Trust. This network has been running for two years and has already boosted trial participation – in some studies a third of participants worldwide have been from European CF Centres. The network, which is part-funded by the CF Trust, has 18 CF Centres in eight European countries and will expand this year to include more Centres.

### 2) Medicines for Children Research Network

This network, funded by the UK Government, covers the UK and supports and facilitates studies to improve treatments for children, including children with Cystic Fibrosis. The co-ordinating centre is based in Liverpool, and there are a series of regional networks throughout the UK. Currently around 10% of their studies are in CF or other forms of chest disease.

### 3) Respiratory National Specialty Group

This UK Government-funded network supports studies in adults with Cystic Fibrosis.

As we work in our CF clinics, we are aware that whilst treatments are very much better than they were there is still much improvement to be made. Treatments need to be more effective, and easier to take. We see the development of clinical trial networks, in partnership with people with Cystic Fibrosis – many of whom volunteer to be involved in the studies – as a really important contribution to moving treatments forward.

*“People with CF who take part in trials today will be helping a future generation of patients.”*

## Further information

For information about clinical trials and to view a list of trials in which you might be able to take part see: [www.cftrust.org.uk/research/clinicaltrials](http://www.cftrust.org.uk/research/clinicaltrials)

For further information about clinical trials networks see:

ECFS-CTN: [www.ecfs.eu/ctn](http://www.ecfs.eu/ctn)

MCRN: [www.mcrn.org.uk](http://www.mcrn.org.uk)

Respiratory Network:

[www.crncc.nih.ac.uk/about\\_us/ccrn/specialty/resp](http://www.crncc.nih.ac.uk/about_us/ccrn/specialty/resp)



*If you are interested in participating in a clinical trial, you should discuss this with your CF team. There is never any obligation for you to take part in a clinical trial, and whether or not you agree to take part in a clinical trial will have no impact on the care you receive from your CF Centre or Clinic.*

# Welfare reforms latest

The Cystic Fibrosis Trust has recently submitted a response to the Government's consultation on changes to Disability Living Allowance (to be replaced with Personal Independent Payment in 2013/14). The proposals include reducing the benefit to just two rates, and regular re-assessment by a contracted healthcare provider. Both of these proposals have the potential to cause unnecessary stress and anxiety for people already living with a serious health

condition, and as such our opposition to this move has been made known. At the time of going to press, the Government intended to publish its response to the consultation in the spring. The CF Trust will continue to engage with the process where possible, and we will provide further updates on the changes when more information becomes available.

## Scottish elections: Your chance to influence CF care in Scotland

With the elections taking place next month, this is an important time for Scotland and a chance for those affected by Cystic Fibrosis to get their voices heard. **Yvonne Hughes**, CF Trust Expert Patient Adviser for Scotland, Northern Ireland and North East England explains how.



Yvonne Hughes

On Thursday 5 May, whether we see a status quo or a change to the administration system, the elections could have an impact on the care and provision of CF services across Scotland.

In time for the forthcoming election, the Cystic Fibrosis Trust has devised a Manifesto setting out what we expect for people with CF from the new government.

- All monies for paediatric CF services to be ring-fenced the same as adult services. This would prevent health boards from eroding the posts of specialist nurses' time away from direct CF care
- All people with CF to be cared for by an adequately resourced, specialist multidisciplinary team
- All people with CF to have access to the medication and equipment they need irrespective of postcode
- All people with CF to have clean in-patient and out-patient facilities, safe from the risk of cross-infection
- All people with CF, and indeed everyone on the transplant list, to have the best possible chance of receiving a life-saving transplant
- All hospital entrances to be free from smokers and smoking areas

If you want your voice to be heard, there are a number of ways in which you can engage with the Scottish Parliamentary system.

### Use the Manifesto

A handy postcard, flyer and the Manifesto itself can be downloaded from a special page on the CF Trust website [www.cftrust.org.uk/manifesto](http://www.cftrust.org.uk/manifesto), which also contains details of how the CF Trust will be helping in these areas. Please use these tools to raise awareness among MSPs of the importance of fair, safe care for people with CF in Scotland. If you'd like us to post any of these materials to you, please email [enquiries@cftrust.org.uk](mailto:enquiries@cftrust.org.uk) with your name and contact details, or telephone the CF Trust on 020 8464 7211.

### Contact an MSP

Most people engage with political issues through their MSP. On the CF Trust website there is a template letter that you can use to write to your MSP highlighting one or more of the areas in the Manifesto. The Minister for Health and Wellbeing is currently Nicola Sturgeon. Alternatively you could write to the Shadow Cabinet Secretary for Health, Jackie Baillie MSP for Labour and Murdo Fraser MSP for the Conservatives. These MSPs take issues forward on a national level whilst your representative MSP can determine what can be done locally. To find the name of your local MSP, visit: [www.scottish.parliament.uk/apps2/msp/mshome](http://www.scottish.parliament.uk/apps2/msp/mshome)

### Hold an awareness event

If you wish to raise awareness of Cystic Fibrosis, you can request to hold an event

in the Scottish Parliament building. To do this you would need an MSP to sponsor the event and, if you can provide a draft press release and photos, this could be a way of gaining some media attention and highlighting Cystic Fibrosis to the wider public.

### Other activities

There are other ways to gain recognition in Parliament, such as raising a motion, submitting written questions to the Government to receive an answer to a specific issue or joining a related health debate by providing the MSPs with an outline of your concern and what you would like to see happening. These may be done on your behalf if you contact your MSP to discuss your concerns and then agree what course of action would be appropriate.

Everyone with Cystic Fibrosis should be represented in some way in our devolved Government, but it takes us to speak out and ensure that the needs of people with CF are taken into consideration.

If you would like to email any comments to me please do so on [yhughes@cftrust.org.uk](mailto:yhughes@cftrust.org.uk) or visit the Discussions tab on the CF Trust Expert Patient Adviser Facebook page [www.facebook.com/cftrustepa](http://www.facebook.com/cftrustepa).

Adolescents between the ages of 14–19 who receive treatment at Children's Hospital for Wales in Cardiff have been left wondering where they will inpatient procedures following the closure of the Young Person's Unit (YPU) in November last year.

Hospital management stated that the unit was closed due to "operational constraints". This does not offer any comfort to families of those with Cystic Fibrosis who require regular, ongoing hospitalisation and treatment for exacerbations of chest infections and related conditions. No contingency plans have been drawn up for adolescents with Cystic Fibrosis requiring inpatient services,

who are faced with admission to a general paediatric ward catering for patients aged two upwards. The dedication and competency of nursing staff on this ward is not in question but they do not possess the specialist knowledge required to fully address the needs of CF adolescents.

The Cystic Fibrosis Trust will endeavour to ensure that the hospital includes optimal facilities and services for the delivery of safe and appropriate care for those with Cystic Fibrosis, and that there is an appropriate plan in place during the interim, by campaigning against this situation. If you are affected by the closure of the YPU, we would urge you to write to your local MP, to hospital management and



Kayleigh Old

to the CF Trust to explain how important the service in Cardiff is to you and set out your concerns. Our hope is that with patient voice and power this situation can be resolved.

Please contact Kayleigh Old, Expert Patient Adviser for Wales, if you have any questions or comments – [kold@cftrust.org.uk](mailto:kold@cftrust.org.uk).

## Update on fairer funding for Cystic Fibrosis care

We are very pleased to report that following a three-year project to develop a fairer funding system for provision of Cystic Fibrosis care, the Department of Health has agreed to implement a 'Payment by Results' (PbR) scheme from 1 April 2011. This means that funding will be linked to the level of care and treatment each individual patient requires, based on their annual review for the preceding year. This differs from current arrangements whereby most services are paid 'block' sums of money that do not reflect the number of patients with CF a service looks after or the level of resources needed to provide high quality, safe and appropriate care.

### Banded tariff

The Cystic Fibrosis Trust proposed that payment for CF care should be based on an annual 'banded' tariff, and care for each



Payment by Results will ensure that each person with CF receives care appropriate to their individual needs

patient funded annually, based upon the severity of condition and the level of input / treatment required to look after the patient as prescribed by their clinical team. The Department of Health has agreed that this is the best way forward to ensure that care for people with Cystic Fibrosis is provided at an agreed, equal and appropriate level for all no matter where they live or what CF Centre they go to.

This new way of funding care for people with CF will mean that CF services receive the proper level of funding for each and every patient. Cystic Fibrosis is a very complex condition, affecting every patient differently and PbR recognises that. Each person with CF will be assessed individually and the cost of their annual care worked out. This will mean that every person with CF, wherever they live in the UK, should receive the same level of care.

The new system will be rolled out over the next two years. For 2011–12 the Department of Health has made the 'bands' of severity mandatory and Primary Care Trusts will now fund care on a patient by patient basis. From 2012 the tariff – the actual amount it costs to treat each banded patient – will become mandatory.

## Peer review consultation commences

The Cystic Fibrosis Trust's programme of peer reviews has been running since 2006, supporting improvements in resources and funding of Cystic Fibrosis services with the aim of driving up and maintaining excellent standards of care. Recently, the Trust has launched a project to streamline the review process and ensure it is 'fit for purpose' in the context of the currently proposed NHS changes. It aims to ensure that the reports produced will enable Specialist CF Centres and Clinics to deliver ongoing service improvements. The project will be running over the summer

and includes an update of the Cystic Fibrosis Trust Standards of Care first published in 2001.

We are running a consultation as part of this project to ask members of the CF community for their ideas about peer reviews and how you think we can improve the process.

If you would like to join our consultation please contact Alicia Ridout, Clinical Care and Commissioning Manager at [aridout@cftrust.org.uk](mailto:aridout@cftrust.org.uk).

# Founded in 1964, the Cystic Fibrosis Trust is the UK's only national charity dealing with all aspects of Cystic Fibrosis.

## Our objectives are to:

- Fund medical and scientific research to develop a cure and provide effective treatments for Cystic Fibrosis.
- Ensure appropriate clinical care for those with Cystic Fibrosis.
- Provide information, advice, support and, where appropriate, financial assistance to anyone affected by Cystic Fibrosis.

## Cystic Fibrosis Trust

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[www.cftrust.org.uk](http://www.cftrust.org.uk)

You can view *CF Today* online and download our extensive range of factsheets and booklets providing further information about Cystic Fibrosis at

[www.cftrust.org.uk/aboutcf/publications](http://www.cftrust.org.uk/aboutcf/publications)



## Cystic Fibrosis Trust Helplines

**Our Support Service has three Helplines offering the following services:**

For information and advice about benefits and how to apply for them –



**Benefits Advice**  
0300 373 1010

For a *confidential* service that enables anyone to obtain information, advice and support on any aspect of Cystic Fibrosis –



**CF Helpline**  
0300 373 1000

For information and advice on how to access small grants from the Cystic Fibrosis Trust and other organisations –



**Welfare Grants**  
0300 373 1020

Our Helplines operate from 9am – 5pm weekdays. An answer machine is available during busy periods and outside these hours.

You can also access our website [www.cftrust.org.uk](http://www.cftrust.org.uk) to find out more about CF Trust Helplines and to download various forms and factsheets relating to these services.