

cf TODAY

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New research into lung transplants

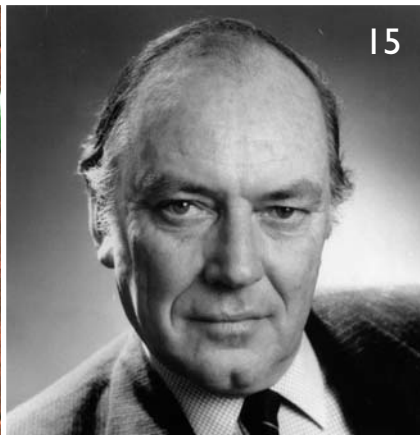
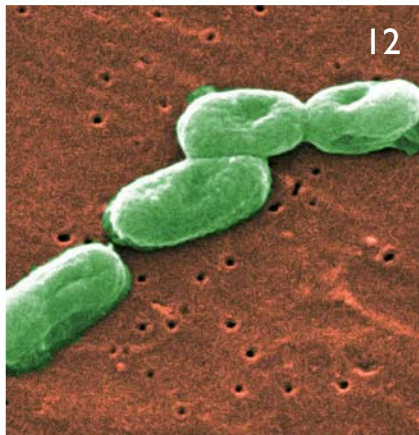
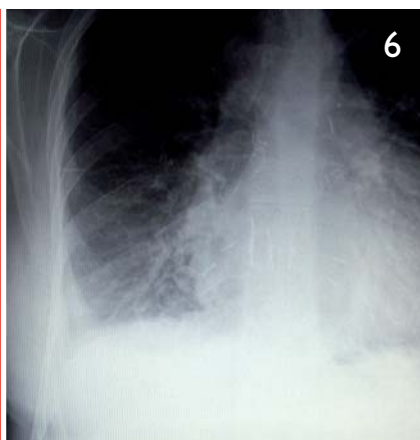
Leaving a legacy

Ask the expert

CF funding update

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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 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: Professor Andrew Fisher, Freeman Hospital, Newcastle.
Photograph: ©Graham Shirley

View from the Trust

Welcome to the new and we hope improved *CF Today*. In the spring, we carried out a survey to find out what our readers thought of the magazine, and how we could make it even more relevant and useful for the CF community. The feedback helped inform the new design, but we have not compromised on content. You will still find articles, news stories and features such as Ask the Expert covering hot topics and emerging issues in CF, from research and clinical care to treatment and everyday aspects of living with Cystic Fibrosis.

Your feedback is very important to us, so if you would like to comment on anything you've read in the magazine, or make any suggestions as to what you'd like to see in future issues, please email cftoday@cftrust.org.uk. Alternatively you can write to *CF Today*, Cystic Fibrosis Trust, 11 London Road, Bromley, Kent BR1 1BY. Your letter may even appear in the magazine (please state when writing if you wish to remain anonymous).

As well as the new season, autumn heralds an era of change at the Cystic Fibrosis Trust. In August we bid a very fond farewell to Rosie Barnes, who was Chief Executive of the Trust for 14 years. During this time she did much to raise not only the profile of CF but the CF Trust's income as well. Under Rosie's leadership, the world-leading UK CF Gene Therapy Consortium was also formed – you can read more about this in our tribute written by our Chairman, Dr Jim Littlewood, on page ten.

Also in this issue, you can read about Rosie's Lasting Legacy (page 11), our new campaign to encourage people to think about leaving a legacy to the CF Trust in their wills. Understandably, it can be difficult to talk openly about will-making, but feedback from focus group research we've carried out suggests many of our supporters would like more information on this form of giving.

In our feature on page six, you can read about a research study at the Freeman Hospital in Newcastle, funded by the CF Trust, which could transform the way lung transplants are carried out in Britain. The UK still has one of the poorest rates of



lung transplantation in Europe, but nationwide efforts are being made to address this situation, and the Newcastle research has the potential to massively boost these efforts.

Our Ask the Expert section can still be found in the centre pages. Our reader survey indicated that the majority of our readers find this a useful and interesting feature. If you'd like to pose a CF-related question to our panel of experts, email askthexpert@cftrust.org.uk, or you can write to Ask the Expert at the address given on the left.

“Autumn heralds an era of change at the Cystic Fibrosis Trust.”

By the time you read this, our new Chief Executive Matthew Reed will be in post. We're all very much looking forward to working with Matthew; he joins us from the charity Christian Aid, where he was Marketing and Supporter Care Director. We're sure you'll join us in making him very welcome.

We hope you enjoy this issue.

Jacqueline Ali
Editor, *CF Today*

Building relations with business

A warm welcome to our new Corporate Fundraising Team, who will be helping to develop the CF Trust's relationships with companies across the UK. One of the key ways in which the CF Trust raises funds is through its relationships with companies, large and small. Increasingly, companies are recognising the benefits of establishing a charity link, and you may be able to help us make those connections.

Does the company you work for have a Charity of the Year? If so, please let us know and we can apply the next time it is reviewed. Perhaps your company has a charity committee which organises fundraising events. Why not propose the CF Trust as the beneficiary or ask if we can come and talk to the committee about our work?

Some companies will match funds raised by staff for their favourite charities – you could encourage colleagues to form a 'CF Team' and join in one of our challenge events. We can give you posters and leaflets advertising our challenge events, which you could distribute to your colleagues and workmates.

What does your company do with its old printer cartridges and mobile phones? Recycling these will raise funds for us – we can provide you with freepost envelopes or for larger quantities arrange for them to be collected free of charge. Or perhaps your company makes regular charitable donations – please make sure the Cystic Fibrosis Trust is on its list if so.

We'd be grateful if you, your friends and family could help us make contact with companies. If you have any queries or suggestions, please don't hesitate to contact a member of the Corporate Team on company@cftrust.org.uk or 020 8290 8046.

The new Team looks forward to hearing from you.

Sue Piper – Senior Corporate and Trust Fundraising Manager

Hayley Richardson – Corporate Fundraiser

Keerti Patel – Events Co-ordinator

Jenni Hearmon – Trust Fundraiser

James Dawson-Davey – Fundraising Assistant

Prescription charges – plans put on hold

Despite the publication of Professor Ian Gilmore's report on prescription charges in which he recommends that people with long-term conditions should receive free prescriptions, these proposals for people in England with long-term conditions have been put on hold due to financial pressures on the NHS. Health Minister Simon Burns

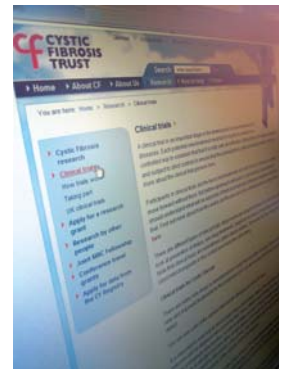


It is unfair that many people with CF have to pay for essential medication

said a decision on prescription charges and exemptions cannot be made before the spending review due this autumn. An early day motion has been put down once again this year by Bob Russell MP – please ask your MP to show their support and sign it. You can download the CF Trust factsheet on prescription charges at www.cftrust.org.uk/aboutcf/publications.

Clinical trials information now on web

Information about clinical trials in Cystic Fibrosis taking place in the UK has been added to the research section of the CF Trust website.



Clinical trials are a vital part of the development of new and improved treatments for a range of conditions, including Cystic Fibrosis. On our website, you can read about how the clinical trials process works, things to consider if taking part, risks and benefits and what to do if you'd like to participate in a trial. There is also a comprehensive database of all CF clinical trials, current and planned, with information such as type of trial (e.g. new drug treatment, physiotherapy device), participation criteria and contact details for each.

We hope that the information on our website will assist those of you considering taking part in a clinical trial. Please note that all of the trials listed will have different participation criteria; some are only open to certain age groups, or only taking place at certain CF Centres. If you are interested in taking part in any of the trials listed, and fit the criteria, you should discuss this with your CF team in the first instance. By taking part in a trial, you may benefit by receiving a new treatment earlier than it would become available to others. However the decision should not be taken lightly and you should always feel completely comfortable that all aspects of the trial and what it will involve have been clearly explained to you.

Visit www.cftrust.org.uk/research/clinicaltrials to find out more.

Share your experiences

The Health Experiences Research Group (previously DIPEX) in the Department of Primary Care, University of Oxford is developing a new area of the www.youthhealthtalk.org website, looking at the experiences of children, young people and their parents of clinical trials. The Group is looking to recruit young people aged 12-25 years and parents of children of all ages, to talk about their experiences of being invited to take part in a clinical trial, even if they declined, withdrew part way through, are currently participating in or have completed a clinical trial. They are recruiting now until Spring 2011. For further information please contact Lesley Powell, Senior Qualitative Researcher, University of Oxford: lesley.powell@dphpc.ox.ac.uk or call 01865 617768.

Keeping CF on the agenda

Since the last issue of *CF Today*, we have been busy responding to various initiatives from the new Government.

You may be aware that a White Paper on the future of the NHS has been published called *Equity and excellence: Liberating the NHS*. We have already submitted a document to the Department of Health with our views on the proposed restructuring of the commissioning of health care in England. Our view – informed by the expert opinion of CF specialists, people with CF and their families – remains that the care for people with CF should be commissioned at a national level and should be of a fair and high standard irrespective of where someone lives. On page 14 you can read how we are working to improve the funding of CF care.

Welfare reform

You may also have seen our statement on welfare reform and benefits at www.cftrust.org.uk/aboutus/what_we_do/campaigns. We will be consulting with people with CF and their families before making further representations to the Department for Work and Pensions.

CF Registry

The CF Registry report is due out later this year and for the first time, data for each Centre and clinic will be identifiable. Individual patient data is still anonymous of course, but it will allow people to examine the outcomes for their CF Centre or clinic. Reports from previous years are available at www.cftrust.org.uk/aboutcf/publications. If you would like to be informed when this year's report is produced, please email your full name and postcode to website@cftrust.org.uk with the subject line: CF Registry.

Expert Patient Advisers now on Facebook

At last we have a Facebook page!

The CF Trust has had its own page for a while but we Expert Patient Advisers wanted one just for ourselves.

The idea of this page is that we can engage more with you, the CF community, informing you of events that you can get involved in or meetings that we may be attending where your vital feedback will be needed.

We have several different tabs for you to get information from or contribute to. Apart from the 'Wall' and our 'Discussions' tabs we want to develop the page further to provide different threads for you to tell us your experiences.



We hope that you will enjoy this page and that it will be a good resource for all the CF community. Visit facebook.com/epacftrust to get involved.

*Sophie Lewis,
Expert Patient Adviser,
South West*



DVD for children with CF

The CF Trust has been producing a new animated DVD for children with CF, to explain what Cystic Fibrosis is and help answer some of the key questions they may have.

The DVD features a young boy, Oli, who has Cystic Fibrosis. His friend Nush plays an inquisitive ant eater who asks lots of questions about Oli's CF and what it means. We worked with a focus group of parents and children and sought advice from clinicians to ensure the DVD is both helpful and appropriate. Issues such as physiotherapy, nutrition, medicines and questions



Oli and Nush from the new animated DVD for children with CF

younger children. The DVDs will be available for loan; to request one, please email publications@cftrust.org.uk or telephone us on 020 8464 7211.

such as 'how did I get CF?' are explained in a light-hearted but informative way. We hope the DVD will help parents explain CF to their children, and that it could also be shown in schools to classmates of children with CF, to help children with CF in the school environment.

While the DVD is aimed at children aged four to eight, we hope it may also appeal to slightly older, or

A new era for lung transplants?

You may have heard on the news in March this year of a young woman with Cystic Fibrosis who sadly died shortly after receiving a lung transplant from a donor who was later found to be a smoker.

Although there is no evidence that the smoking history of the donor was responsible for her death, it does demonstrate that transplantation of lungs that are in less than perfect condition is not uncommon. In using these types of donor lungs, the transplant centres are following guidelines set out by the NHS Blood and Transplant Service. However the news brought to the fore the issue of whether borderline or 'marginal' lungs should be used for transplantation and whether recipients should be made aware of the history of the lungs.

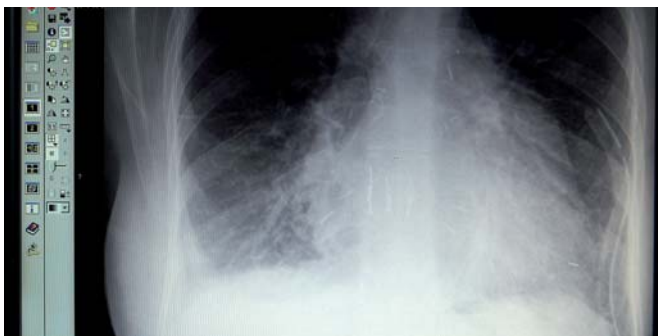
Although it may sound shocking that donor lungs in a less than optimal condition could be transplanted, the reality is that the shortage of lungs available for transplant in the UK means that marginal lungs increasingly have to be considered. The UK has one of the lowest rates of organ donation in Europe, and sadly, many CF patients awaiting lung transplant will not get the new lungs they need in time. This despite people with CF constituting 30% of those awaiting lung transplants, and having the best prognosis post-transplant for all of the conditions for which lung transplant is the last treatment option.

The number of people on the NHS Organ Donor Register is only part of the picture. Legislation and the way donor consent is managed are also significant. Countries such as Spain, which have far higher rates of organ donation than the UK, have implemented stringent procedures for the management of organ donation throughout the whole process, from donor identification to transplant and follow up. The UK is now following suit, and a concerted nationwide effort is underway to address the situation (see box, page seven).

Reconditioned lungs

One such effort is a revolutionary research project taking place at the Freeman Hospital in Newcastle upon Tyne, using a novel technique called Ex-Vivo Lung Perfusion (EVLV) to recondition lungs deemed unusable for transplant.

The study, funded by the CF Trust, has been underway for less than a year and is already yielding very promising results. At the time of writing, one CF patient at the Freeman Hospital and



New lung transplant research is yielding promising results

three CF patients across other transplant centres elsewhere in England had received transplants using reconditioned lungs. In addition, it is speculated that the procedure of 'reconditioning' the lungs could make them even more effective than lungs deemed to be already suitable.

We caught up with Professor Andrew Fisher, the study's principal investigator and Professor John Dark, a leading transplant surgeon and President of the International Society for Heart and Lung Transplantation, to find out more about Ex-Vivo Lung Perfusion, and what it means for people with Cystic Fibrosis.

Why is this research needed?

AF: Lung transplant is a very effective treatment for CF patients with advanced lung disease. Unfortunately, many patients with CF who would benefit from lung transplant do not survive long enough to get new lungs due to the shortage of donor organs. The main reason for the shortage is that the lung is very fragile and susceptible to damage and only a small percentage of the donated organs can be used. The purpose of this research is to convert lungs that are initially thought unsuitable for transplant into lungs that can be successfully transplanted. This is achieved by 'reconditioning' the lungs using a process called Ex-Vivo Lung Perfusion.

What makes donor lungs marginal or unusable?

JD: Brain death in donors causes damage to the lungs by increasing blood pressure and causing blood vessels in the lungs to release toxins, which damage the lungs. Other factors such as the age of donor and previous illnesses can also make lungs what is termed 'marginal' – they can still be transplanted in some cases, but outcomes may not be as good.

What does reconditioning involve?

AF: Reconditioning involves attaching the damaged lungs to a ventilator to provide oxygen and using a modified heart-lung bypass machine to pass a nutrient solution, called a perfusate, through the lungs where blood would normally flow. This technique is called perfusion, and effectively 'washes out' excess fluid and possibly inflammatory molecules which can cause damage to the lungs. *Ex-Vivo* simply means outside the body in an artificial setting, as opposed to in the human body.

How long does reconditioning take?

JD: We can perfuse the lungs for up to six hours. After this time, there is no further improvement to their condition. Once the team is happy that the lungs are functioning well by measuring their ability to absorb oxygen from the ventilator they can be considered for use in a matched patient waiting for lung transplant.

What have been the results of this research so far?

AF: Since last November, our team in Newcastle have carried out perfusion on nine sets of donor lungs, and four of these were successfully reconditioned and transplanted, which is very



Professors John Dark (r) and Andrew Fisher

encouraging. We don't know as yet why the technique didn't work on some of the lungs, but a larger study which we hope to start next year should help tell us this.

How do you decide which patients receive the reconditioned lungs?

JD: All of the patients who are on our transplant list are made aware of this research, and the vast majority have consented to receive reconditioned lungs should they become available. However the decision of which lungs go to which patient is down to which patient is the best match for the donor lungs that become available. This is based on factors such as size and blood group – it is not randomised in any way. Some lungs are suitable for some patients and not others.

Is there an upper or lower age limit of those who could receive the lungs?

JD: We have not as yet attempted to transplant reconditioned lungs into children. There are more ethical difficulties when it comes to carrying out medical research in this more vulnerable group.

AF: The option at this stage is available to any adult patient of any age as long as they are already on our waiting list. The provision of EVLP will not however affect the way CF patients are selected to go on the waiting list.

Has the technique previously been used anywhere else?

JD: Professor Stig Steen in Sweden first pioneered the technique of Ex-Vivo Lung Perfusion. At present, it is still considered an experimental technique, although experience of EVLP has grown hugely in the last three years. The largest centre carrying out this technique is Toronto in Canada and there are now several centres in Europe performing it regularly. In addition, four of the five lung transplant centres in the UK have now gained experience with EVLP.

Why is the number of people with CF who receive a lung transplant so disproportionate to the number on the list? (A third of those on the waiting list have CF but only a fifth are transplant recipients).

JD: This is because CF patients will always require two lungs. Often, only one lung from the donor is suitable for transplant, in which case the lung will go to someone on the waiting list that only requires one lung. CF patients always require two lungs, hence the discrepancy.

Is the post-transplant prognosis improving for transplant recipients who have CF?

JD: Many CF patients do extremely well after transplant. At the Freeman, we are now seeing average survival of over ten years

and in some individuals survival can be over 20 years. CF patients are in a sense 'self-selected'; they are already used to living with the treatment demands of CF, so they are well-placed to deal with the considerable treatment burden that having a transplant entails.

AF: CF patients actually have the best outcomes of the three commonest conditions for which lung transplant may be necessary. Every death of someone on the waiting list is a tragedy. Our ultimate aim is to make waiting list deaths a thing of the past. Seeing the instant benefits that EVLP can have in enabling transplants in CF patients who might otherwise not have received new lungs makes us think this might be possible one day.

What is the next stage of your research?

AF: Thanks to the continuing support of the Cystic Fibrosis Trust, we are working together with our colleagues in all five UK Adult Lung Transplant Centres (Freeman Hospital, Papworth Hospital, Wythenshawe Hospital, Birmingham Heartlands Hospital and Harefield Hospital) to set up a national EVLP study called 'DEVELOP-UK'. It will require considerable funding from the National Institute of Health Research to conduct the study which we are currently applying for. This study is very important as it will provide us with the evidence we need to make a case to the NHS commissioning bodies to fund this procedure, so that it can become widely available to CF patients and others awaiting lung transplant.

Interview by Jacqueline Ali, Communications Department, CF Trust

Further information

The CF Trust produces a factsheet on transplantation in Cystic Fibrosis. To download, visit www.cftrust.org.uk/aboutcf/publications/factsheets

Organ donation

Whilst the research in Newcastle is very exciting, the CF Trust recognises the importance of keeping the issues of organ donation very much in the public mind.

We have joined two coalitions to promote organ donation to the public and to raise awareness of donation to politicians.

The Organ Donation Coalition is a group of charities working together to ensure that the government goal of a 50% increase in organ donation by 2013 is achieved. With our partners we will be creating campaigning materials and working with NHS organisations to improve donations both locally and nationally.

Transplant 2013 is a group of patient organisations and industry bodies working to ensure that politicians and policy makers are aware of, and support, the need for increasing donor numbers.

Between these two campaigning and lobbying groups, we will ensure that the issue of organ donation is not forgotten or swept aside during spending reviews.

Ask the expert

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

Treatment for blood poisoning (septicaemia)

Q My son has had septicaemia on more than one occasion. Could you tell me whether this can be treated successfully?

A It is not the policy of Ask the Expert to give advice on individual patients, but we can assure you that septicaemia can be treated successfully in people with Cystic Fibrosis. It is not common except in those patients who have a totally implantable venous access device (TIVAD) for the repeated administration of intravenous antibiotics. These devices sometimes do become infected which often necessitates their removal to clear the infection.

Proportion of children and adults with CF

Q Could you tell me what the proportion of children to adults with CF is in the UK?

A These details and further statistics can be found in the CF Registry (national database) reports on our website www.cftrust.org.uk/aboutcf/publications/cfregistryreports. Each gives a summary at the beginning showing percentages. In 2008, 56.2% of people with CF in the UK were aged 16 years or over.

Safety of farm trip

Q My four-year-old son is due to go on a trip to a farm in a couple of weeks. I would be grateful if you could give me some guidance as to what I should advise the school with regard to potential sources of infection.

A There should be no reason to be concerned about infection; farms that encourage visits from young children are usually very responsible and adhere to Health and Safety. Pseudomonas is everywhere in the environment and you would not be able to fully protect your son at all times. The best advice is always good hygiene practices including plenty of hand washing especially when touching animals; risks would then be minimal.

Your son is at an age when you can explain to him the importance of hand washing and not putting his hands in his mouth if he has touched an animal. Stables where there is damp straw, particularly at times of "mucking out", do contain many Aspergillus spores in the air and are best avoided or only visited briefly.



Photo: © surpasspro - Fotolia.com

The pleasure children can gain from farm visits outweighs the minimal risks

The general consensus of experts seems to be that the risks are minimal and are outweighed by the amount of pleasure children gain from the experience.

Sore throats

Q I am writing because for the past two days my daughter's 'normal' sore throat has become far worse and is lasting all day. She has for several years now always woken up with a sore throat, which a hot cup of tea seems to help. She does have a cold at the moment. In addition she is presently on Itraconazole and 20 mg steroids every other day for Aspergillus. She has Azithromycin once a day and nebulises Colomycin and Gentamicin twice a day. She also nebulises Pulmozyme. She takes four puffs of Bricanyl and three of Symbicort morning and night, as well as Omeprazole and Domperidone. She is 11 and is in some discomfort. She does not have a fever or swollen throat glands and I cannot see signs of infection as with tonsillitis. Your input would be appreciated (we are due to see the CF team in two weeks).

A It is not altogether surprising that your daughter has a sore dry throat in view of all the inhaled treatment she is taking. Presumably things are now worse because she has a cold. Certainly Pulmozyme, the inhaled steroids (in the Symbicort) and the Colomycin are all a potential cause of a sore throat and husky voice.

It is important that you discuss the problem with your daughter's paediatrician who will be able to examine her throat to make sure there is no local cause (Candida or "thrush" is a possibility) and also decide if all the inhaled treatments are still necessary – particularly the three mentioned above.

But do see your paediatrician as soon as you can.



Inhaled treatments for CF can cause a sore throat, but always consult with your CF team

NB With regard to the use of Gentamicin, we draw your attention to the article entitled 'The safe use of antibiotics' in the January 2010 edition of CF Today

What is the life expectancy?

Q I was wondering if you could help me. I am 21 years old and I've finished my university degree. Obviously this means I've been thinking about the future and the way my condition will affect my future career. I don't often linger on these things but of late I have been rather depressed about it as I know that people with CF don't have high life expectancies. Having said this I know that some can live longer than others depending on how serious their condition may be. I know it is not possible to tell a person how long they may live and that the life expectancy changes from person to person, but I was wondering if you could perhaps tell me the average life expectancy of a person suffering with Cystic Fibrosis. I know it changes every couple of years or so but if you can help answer this question and help put my mind at ease, I would be incredibly grateful.

A Currently the median predicted survival is 38.8 years (so half of all people with CF will reach this age or older). This statistic is based on the information collected by the CF Registry, a national database of UK CF patients. The age is increasing year on year and there are many adults in the 45 to 55 age range.

I suggest you discuss your questions with the team at your Specialist CF Centre especially the social worker and clinical psychologist. You can also speak to someone on the CF Trust Helpline who will be happy to chat to you about this or any other issues, if this would be helpful.



The prognosis for people with CF continues to improve

Vitamin K

Q Could you tell me what the current situation is with regards to supplies of Vitamin K?

A Vitamin K is one of the fat-soluble vitamins that people with CF can have difficulty absorbing from their regular diet. Supplementation with Vitamin K is used to optimise bone health, and in patients with CF liver disease, it can help with the blood clotting process. It is also often given at birth as an injection to help with the blood clotting process.

Until last year separate preparations of oral Vitamin K were available in the UK as 10mg tablets of Phytomenadione and 10mg tablets of Menadiol. Phytomenadione was discontinued last year by the product manufacturers. Menadiol was until last month made by a company called Cambridge laboratories. They have been taken over by Alliance Pharmaceuticals, who have informed us that due to problems with availability of raw materials, they are currently unable to supply Menadiol tablets. The company is aiming to get supplies re-established by the end of 2010, but has not yet been able to specify a date.

If you have been taking Menadiol on a regular basis you will need to discuss this with your local CF medical team at your next appointment. They will assess whether it is necessary to give you further treatment or if a short break from treatment will not be a problem until supplies are re-established.

For those who require further treatment a number of options are available, for example your hospital may decide to obtain an unlicensed form of Vitamin K from abroad, or give you periodic IV infusions of Vitamin K.

If you have any questions or concerns about the availability of Vitamin K, please discuss them with your CF team.

For further information, see the CF Trust Information Sheet on Vitamin K, available at www.cftrust.org.uk

Our experts for this issue were:

Dr Jim Littlewood, Cystic Fibrosis Trust

CF Trust Helpline

You can also view an archive of past Ask the Expert questions at 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

Thank you to an exceptional Chief Executive

In 1996 Rosie Barnes took up her appointment as Chief Executive of the Cystic Fibrosis Trust, and she retired in August 2010. Over the past 14 years Rosie (as she is affectionately known by all) has been undoubtedly a most successful and effective Chief Executive. During her time at the CF Trust major changes occurred. The charity tripled in size as did the income. Rosie was also directly responsible for creating the UK CF Gene Therapy Consortium and led and inspired the very substantial fundraising effort to support it. This initiative is now recognized worldwide as a novel and very important new way for scientists to work together.

Although initially there were some concerns that clinical care would take second place, as it has turned out, nothing could be further from the truth. Rosie initiated and led the Peer Review program of CF Centres and Clinics which has vastly increased the quality and quantum of care delivered to CF patients across the UK. Using her experience in politics, she has led discussions with the NHS of how funding for patient care in the Health Service is to be reformed and has placed CF in the vanguard of this change program. A successful campaign led by Rosie in the Nineties eventually led to the introduction of national neonatal CF screening in 2007, so now every baby with CF born in the UK is diagnosed soon after birth.



Rosie Barnes and Dr Jim Littlewood OBE

An extraordinary ability to communicate and grasp a particular situation has resulted in Rosie becoming very knowledgeable on both the clinical aspects and scientific progress in Cystic Fibrosis. This and her natural empathy with and genuine liking for people with CF and their families has resulted in her being held in the highest regard by the whole CF community, be they patients, parents, professionals or other interested parties.

Rosie has been quite exceptional in her total commitment to effectively improving the lot of people with Cystic Fibrosis – always carried out with determination but with kindness and a sense of humour. It has been a great pleasure and privilege for me to work with her over the past 14 years. We are pleased that Rosie will not lose contact with the CF Trust as she is keen to continue to support the CF Trust in an ambassadorial role. We wish her all health and happiness in the future in her new role as retiree and grandma.

Dr Jim Littlewood, Chairman, Cystic Fibrosis Trust

“Reading through *CF Today* I often wondered if Rosie Barnes had a twin; she managed to be at so many events and meetings, usually at different ends of the country and seemingly so close in time, that it would be impossible for her to cover the territory required, and anyway how could anyone have that much energy? Rosie’s organisational skills are amazing, and she has so much energy she could sell back the surplus to the national grid. But it is her enthusiasm and enjoyment of all she does that has made being in her company such a pleasure. Working with her as a Trustee has been a wonderful experience; she has always guided with insight and inspiration. Rosie has a warm and sympathetic way about her that helped in the most difficult circumstances; and I will miss seeing that very particular twinkle in her eye that made one aware of the fun to be had.”



Jenny Agutter, CF Trust Patron and Trustee



“Although the Trust had become established as a major sponsor of research, progress seemed frustratingly slow, particularly for those of us with CF children. Into this situation came Rosie Barnes, an inspirational administrator and communicator – a born leader. She lifted us into a different league.

“She conceived and drove forward a plan that led to us speaking for the first time of “finding a cure”. Suddenly there was hope. Sadly these developments will be too late for some but the struggle will have been worth it.

“Thank you Rosie.”

Sir Robert and Lady Linda Johnson

“To paraphrase history, so what did Rosie do for us?

“Her key first change was to see that the difficult science underpinning gene therapy needed a large team to solve the issues. The three groups in the UK were working largely independently; with a characteristic mix of charm and firmness of hand, Rosie brought them together to form the UK CF Gene Therapy Consortium.



“Secondly, it is clear that such 'big science' does not come cheaply. Rosie's fundraising energy, enthusiasm and success are legendary, but importantly were coupled with an understanding that rigid bureaucratic vigilance of CF Trust funds would not allow the ambitious aims of the gene therapy programme to be met. Within the framework of rigorous external peer review, the Consortium was given the flexibility to direct funds as changing circumstances dictated, likely saving years in the delivery of a clinically relevant product.

“Above all, Rosie kept CF patients centre-stage. There were no decisions in favour of 'interesting science'; the gate-keeper has always been, and remains, how will any experiment speed translation to the clinic?

“So what did Rosie do for us? As my teenage children would say – 'durrgh, it's not very difficult.' She made gene therapy a real therapeutic option for patients with Cystic Fibrosis.”

Professor Eric Alton, Chair, UK CF Gene Therapy Consortium

Rosie's Lasting Legacy

We are delighted that Rosie has agreed to become our legacy ambassador, and this autumn she is helping the CF Trust launch a new campaign, encouraging our supporters to leave a legacy to the CF Trust in their wills. You can read about Rosie's decision to leave the Trust a gift in her will in our new legacy leaflet.

Leaving a legacy to the CF Trust is a relatively simple way of supporting the Trust's work in the future, and making sure your family are cared for. Remember, you don't need to be wealthy to leave a gift in your will – every gift, no matter the size, makes a difference.

Increasing our legacy income is important. We need to ensure we have funds to continue to develop and improve the gene therapy products as effective treatment for CF in the lungs (read more about the progress of the gene therapy trial on page 13). To give you an indication of the costs involved in this programme, we have committed nearly £11 million over the next three years to the Gene Therapy Consortium.

We also need to improve the quality of life for those with CF, for example through research into CF-related diabetes and other non-lung complications. The Trust invests £1m on non-gene therapy research each year.

In the leaflet you will find information about why the Trust will continue to need legacies in the future and what they have helped us to achieve so far, why it's important to write or update your will and the benefits of leaving a legacy, and the information you will need to include a gift in your will.

Look out for our new legacy leaflet this autumn. In the meantime, if you would like any information, please contact Sue Whitehead, Legacy Marketing Manager on 020 8290 8051 or swhitehead@cftrust.org.uk.



Rosie with her daughter and grandson

CF Trust awards new research grants

At the last meeting of our Research Advisory Committee, eight projects were reviewed by the expert panel, of which four were recommended to the Trustees for consideration. The Trustees approved funding for the following four projects in keeping with the CF Trust's commitment to fund research that will be of important and/or imminent benefit to those with Cystic Fibrosis.

Tackling new *Burkholderia* bacteria

A grant was awarded to Dr Mahenthalingam of Cardiff University for a 12-month project investigating *Burkholderia* bacteria. A lot of effort has rightly been put into ways to combat *Burkholderia cenocepacia*, but over the last ten years, *Burkholderia multivorans* has also emerged as a threat in Cystic Fibrosis and less is known about how it causes infection and resists treatment. The scientists will be using techniques learnt in research into *B. cenocepacia* to study how *B. multivorans* switches on or off the genes it uses to cause infection, so researchers will know which genes to target with potential treatments. The team has also found an antibiotic, which can kill drug-resistant strains of *B. multivorans*, and will be testing how this may be used to treat infection in people with Cystic Fibrosis.

CFTR activity

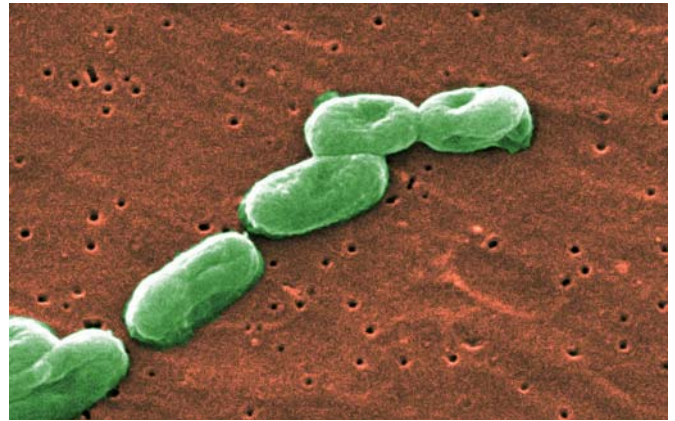
The Trustees approved a three-year grant to Professor Ford at the University of Manchester for a project aimed at studying the activity of the purified CFTR protein. CFTR (Cystic Fibrosis transmembrane conductance regulator) is the protein that controls the movement of chloride through the lining of the cells. It is the faulty CFTR that causes the problems in Cystic Fibrosis by not working at all, or not working enough. The PhD student funded in this project is working as part of an international team of scientists studying the structure of CFTR, how it acts as a channel for chloride through the cell lining and how different drugs interact with it. This information will be important in the development of new drugs and will help them progress from the lab to clinical use.

Enzymes in the CF airway

A grant was awarded to Dr Martin at Queen's University, Belfast for a one-year project, which aims to identify specific enzymes that are responsible for the excessive absorption of sodium in the CF airway. It is believed that the airway becomes very dehydrated in people with CF through a combination of defective CFTR, and an over-activity of channels which control sodium absorption. This is due to the presence of specific enzyme activators which keep these channels open resulting in a reduction in the airway surface liquid in the lung. When the airway is dehydrated like this, the normal clearance of mucus and bacteria is affected, which means thick mucus builds up leading to the cycle of infection and inflammation in the CF lung. Dr Martin and her team will try to identify exactly which enzymes are involved with a view to then finding drugs that can correct the problem.

Diabetes and *Burkholderia*

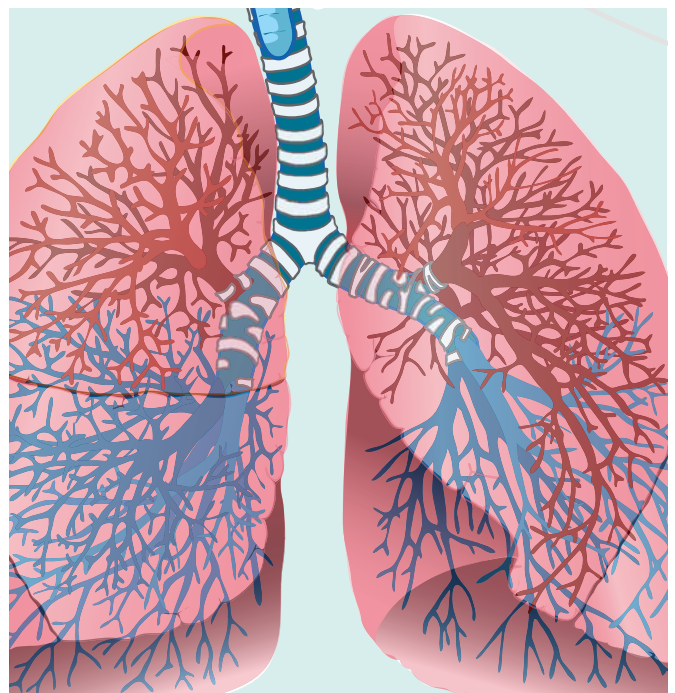
A grant was also awarded for a three-year project under Dr Brown at the University of Exeter investigating the links between CF-related diabetes and *Burkholderia cepacia* complex (Bcc). When people with CF develop diabetes, it not only affects the gut, but also has an effect on the lungs. The team will research how bacteria in Bcc respond to different conditions relevant to diabetes such as heightened sugar concentrations and insulin, and look for evidence of these same responses in samples from people with CF-related diabetes. The results will assess the direct impact on *B. cepacia* and help guide the most effective way of managing CF-related diabetes.



Burkholderia cepacia – under the microscope

Image: Janice Haney Carr

These four new projects will be running alongside the other research currently funded by the CF Trust into areas such as early detection of lung disease in infants, transplantation (see page six), depression in people with CF, inflammation and drug treatments. Details of all of these can be found on our website: www.cftrust.org.uk/research.



Sodium absorption in the airways will be studied in detail

Image: © turhanerbas – Fotolia.com

Gene therapy: Where next?

As the run-in study of our gene therapy trial has now concluded, we thought it would be timely to provide an update on the progress of the trial and what happens next.

Run-in study

The purpose of the run-in study, for which almost 200 people with Cystic Fibrosis were recruited, was to assess a new range of sophisticated and sensitive tests, which will be used to measure the effect of gene therapy in the multi-dose trial. The run-in also helped the Consortium decide which patients are best suited to go through to the multi-dose clinical trial.

Those on the run-in to the clinical trial had to have various measurements to assess lung function, bacterial infection, inflammation, and other tests of clinical severity including quality of life questionnaires. Each patient also had to travel to Southampton for a special scan to test how well their lungs clear mucus. The Consortium and the CF Trust are very grateful to those who have undergone this long and demanding study.

Multi-dose trial

Out of the 192 patients recruited, 153 completed the four visits for the study. Based on the data collected, the Consortium is now in a position to select the participants for the multi-dose clinical trial.

Each patient on the run-in is being contacted by the Consortium to discuss the selection criteria and to be thanked for all their help in the study so far. Around 100 of the run-in participants will be chosen to go through to the trial. The choice is made on two main criteria: the ability to deliver the gene to the lungs, and the ability to measure any improvement following gene therapy.

The people with CF on the run-in have therefore been placed in three categories:

Group 1

Patients asked to take part in the multi-dose trial

Patients in this group have healthy enough lungs to allow efficient deliver of the gene, but also their lung disease will allow measurement of improvement after gene delivery.

Group 2

Patients not suitable to take part in the multi-dose trial

Group 2 is split into two further groups: those who are extremely well and have very healthy lungs, and those who have quite advanced lung disease. The first group is excluded because although it would be very easy to deliver the gene, there would be very little to measure as



A nebuliser will be used to deliver gene therapy to patients

the lungs are already healthy. Those with quite advanced lung disease are excluded as delivery of the gene would be more difficult and therefore any improvement may be harder to measure. Also, the Consortium is concerned that the treatment may be less well-tolerated in this group.

Group 3

Patients for whom a decision has not yet been made

Group 3 is also split into two groups: those who are quite well, and those who have had an average lung function of lower than 50% during the run-in. The run-in data for those who are quite well are still being analysed to see whether inclusion is possible. For those with reduced lung function, the Consortium is concerned that the gene therapy would be less well tolerated, so further work is being carried out to prevent the short-lived reduction in lung function seen in the pilot study. This work will determine whether these patients will be suitable for the multi-dose trial.

The Consortium will be discussing these issues with those who have participated in the run-in and their families, as well as the results of the tests taken during the run-in. All patients, whether included in the multi-dose trial or not, will be offered the chance for a final visit and to discuss any issues. For those selected to go forward into the multi-dose trial, these visits will continue every six months until the start of the trial, which is estimated to begin in the second half of 2011.

Further updates will be provided in *CF Today* and on our website.

John Deplin, Communications Manager, Cystic Fibrosis Trust

Update on fairer funding for Cystic Fibrosis

I am delighted to report that we remain on course for implementation of an improved strategy for funding of CF patient care by 1 April 2011. In July, we held a meeting with CF clinicians, hospital managers, commissioners and the Department of Health at which we discussed and debated the various issues around the proposed new funding arrangements. We now have a clear plan for the way forward. This includes some additional and important work around the costs involved in providing shared care, the first year of diagnosis, treatment of particularly difficult infection, pregnancy in CF and post-transplant care to ensure that the allocated funding will sufficiently resource **all** elements of CF care. A further report will be produced at the end of November 2010, which will provide the Department of Health with the information they require to publish details of the new funding arrangements on their website by mid-December 2010, prior to final implementation in the new financial year.

In spite of the current difficult economic climate and in the face of impending cuts to public service spending, we remain optimistic that the extensive work we have done on behalf of those affected by Cystic Fibrosis and their families will help us understand precisely what costs are incurred in the provision of safe and appropriate care for those with Cystic Fibrosis and ensure that funding will follow to support all services in their efforts to maintain and continue to improve the overall standard of care for all.

*Jo Osmond,
Director of Clinical Care,
Cystic Fibrosis Trust*



Safeguarding Specialist CF Nurse posts

Some of you may have become aware of changes to the role of the CF nurse at your Centre and I would like to take this opportunity to explain what is happening. The CF Clinical Nurse Specialist (CF-CNS) is a key member of the multidisciplinary team with responsibility to patients, their families and to staff involved in CF care. Unfortunately the variability of the role is often misunderstood by people working outside CF as it can be difficult to describe, define and measure.

With the current economic climate we are all being asked to tighten our belts and this includes the NHS. Now more than ever the NHS is trying to reduce its costs and this has implications for everyone, including nurse specialists who are an expensive resource for the NHS.

Over the last few years some NHS Trusts have been asking CF-CNSs to leave their work for one day or more each month to work on the wards. This was originally explained as a way to reduce nursing costs by 'filling in' with the CF-CNS, however many managers are now saying that this initiative provides further support and education to ward nurses. Increasingly, we are finding that this is being reported in many more CF Centres throughout the UK.

It is important that you are aware of this as your CF-CNS may be struggling to maintain the service they offer, while trying to fit their usual workload into a shorter

working week. The CF Trust understands this situation and is trying to support us in any way they can.

*Milly Dack
Chair, UK Cystic Fibrosis Nursing Association*

The CF Trust says:

The CF nurse is often the backbone of the CF service. Along with expert clinical care, they provide a huge service to families and adults with CF, helping with benefits, being a listening ear and often are the first port of call for any help or advice.

Whilst we appreciate the ongoing need for efficiency and cost savings, this should never be at the expense of the health of people with Cystic Fibrosis. CF nurses and teams are often already overstretched and reducing nursing time further is a grave concern, particularly as we understand that this has led to a reduction in the number of home visits that some CF nurses are now able to undertake.

The UK CF Nursing Association is part of the Royal College of Nursing's campaign Front Line First to ensure that cuts do not impact on services. The Cystic Fibrosis Trust is supporting this campaign and will bring you news on further developments. Find out more at <http://frontlinefirst.rcn.org.uk>.

Duncan Bluck CBE:

A boon to the CF community

In February, Mr Duncan Bluck retired both as President and as a Trustee of the Cystic Fibrosis Trust.

After a distinguished business career, including a major role in the development of the Cathay Pacific airline whose chairman he became in 1984, Duncan has recently ended a 22-year involvement with the CF Trust. We have indeed been fortunate to have had Duncan's close involvement for so long, first as a Trustee, then in 1995 as Vice Chairman prior to becoming Chairman in 1996, an office which he held until 2003 when he became President of the CF Trust.



Duncan Bluck helped shaped the CF Trust into the organisation it is today

It has been a period during which there have been many changes and many challenges on the way as the charity has grown. It is no exaggeration to say that, during Duncan's involvement, both the size and income of the charity have more than tripled and the CF Trust has changed from a relatively small charity to a medium-to-large one, with a very high international reputation.

Duncan was responsible for appointing Rosie Barnes as Chief Executive and during his time as Chairman, Duncan led the Trustees in supporting Rosie Barnes's initiative in introducing a radical change in our research policy to focus on gene therapy and alter our ways of funding scientists and clinicians in London, Oxford and Edinburgh (the Gene Therapy Consortium) provided they worked as a team. This initiative has been widely admired by the international clinical and scientific CF community and, during the past year has led to the first pilot clinical trial of the gene therapy product developed as a direct result of this research.

In 2005 Duncan was awarded the John Panchaud Memorial Medallion of the CF Trust to mark his major achievements on behalf of people with Cystic Fibrosis. He retires with sincere thanks and good wishes from us all.

Dr Jim Littlewood OBE, Chairman, Cystic Fibrosis Trust

Further your education with the Joseph Levy Memorial Fund

The Joseph Levy Memorial Fund provides financial assistance to young people who are proposing to further their education via university, college or vocational training.

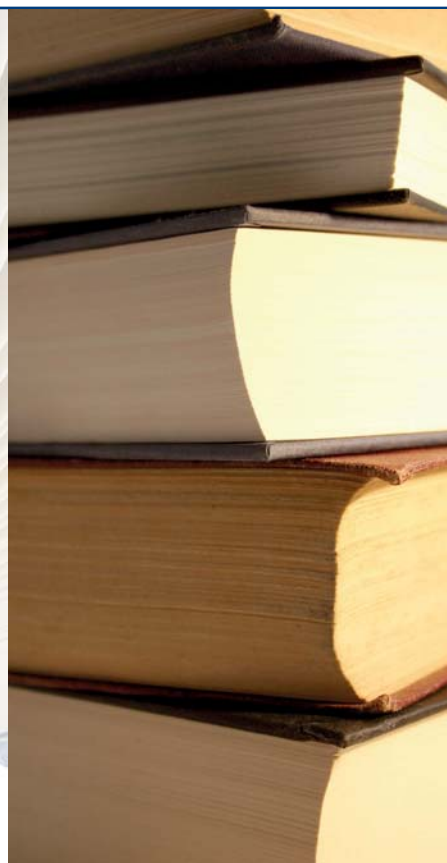
Over the past few years we have been delighted to support students studying many subjects ranging from those training to be doctors, lawyers, teachers, chefs, beauticians and many more who will be moving on to successful careers in their chosen profession.

Applications for the academic year commencing September 2011 will need to be received by **31 March 2011** for consideration at a Trustee meeting in June. If you would like to receive an application form, please contact Elizabeth Neville at the address below.

Please note: do not wait for A-Level results before applying.

Joseph Levy Memorial Fund
SHAFTESBURY PLC, Pegasus House
37/43 Sackville Street, London W1S 3DL

Elizabeth.Neville@Shaftesbury.co.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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Tel: 020 8464 7211 Fax: 020 8313 0472

www.cftrust.org.uk

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

www.cftrust.org.uk/aboutcf/publications



Cystic Fibrosis Trust Helplines

Our Support Service has three Helplines offering the following services:

For information and advice on Disability Living Allowance (DLA), how to apply and advice on completing the DLA form –



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 to find out more about CF Trust Helplines and to download various forms and factsheets relating to these services.