



newsletter

PKD Charity PO Box 141 Bishop Auckland County Durham DL14 6ZD

www.pkdcharity.org.uk

Registered Charity No. 1085662

Message from the Trustees



The PKD Charity has gone from strength to strength in the past year,

thanks to the fundraising efforts of volunteers which have enabled us to hold our first PKD Information Day and support the growing numbers of people who contact us for information and advice. Now we are developing a 3 year strategy aimed at raising substantial funds, especially through grants and government aid, to invest in enhanced support for individuals and families affected by PKD, generate national awareness campaigns and finance research initiatives.

We are confident that the PKD Charity can play a valuable role in informing people with PKD about how the disease progresses and what can be done to manage the symptoms. We want to make sure that everyone knows about and understands the therapeutic and dietary choices open to them at every stage of PKD.

In particular, we want to build a nationwide network of volunteers to help us deliver at a local level, where possible in collaboration with the NHS and established kidney charities. A number of you have already volunteered your services and we will be in touch with you as soon as we have developed our volunteer infrastructure.

We also plan to hold more PKD Information Days, providing you with the opportunity to learn more about PKD from a panel of experts, hear about the latest research into potential drug

treatments and share experiences with others affected by the disease. The next Information Day will be in Manchester on Saturday October 1st, from 10am to 4pm. **See The programme below**

On behalf of the Trustees

Dr Anand Saggar
Chairman



First PKD research conference
see page 2

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Join us at the 2nd National PKD Information Day

Saturday 1st October 2005, 10.00am - 4.00pm

at The Nowgen Centre 29 Grafton Street, Manchester, M13 8WU

Programme

09.55 Welcome and Introduction

Ms Tess Harris, PKD Charity Trustee

10.00 Facts and Fiction - An Introduction to Clinical Genetics

Dr Anand Saggar, Consultant in Clinical Genetics and PKD Charity Chairman

10.20 Discussion

10.30 More than a Kidney Disease - An overview of PKD

Dr Anand Saggar, Consultant in Clinical Genetics and PKD Charity Chairman

10.50 Discussion

11.00 Case study Presentation

Ms Jane Pugh, PKD patient and Freelance Journalist

11.15 Discussion

11.25 Brain aneurysms in PKD - Investigation and New Treatments

Mr Nihal Gurusinghe, Consultant Neurosurgeon, Royal Preston, Hospital, Preston

11.55 Discussion

12.00 LUNCH

13.30 Together the Answer - The work of the PKD Charity

Ms Rebecca Murphy, PKD Charity Trustee and Fundraising Coordinator

13.40 Discussion

13.45 The Genes of ADPKD and New Breaking Research

Dr Richard Sandford, Consultant in Clinical Genetics, Addenbrookes Hospital, Cambridge

14.05 Discussion

14.15 Diet and PKD

Ms Tess Harris, PKD Charity Trustee

14.35 Discussion

14.45 Ask the Experts

Panel Questions and Discussion

15.15 TEA BREAK AND FREE DISCUSSION

16.00 Summing up and close of meeting

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Research & Medical Update

Worldwide experts meet to discuss PKD

Bringing leading scientists from across the globe to discuss a possible breakthrough on drug therapy for PKD was the first financial undertaking by The PKD Charity to promote research into the disease.

The charity co-funded a 2-day conference with the PKD Foundation (USA) and The Novartis Foundation. Scientists with diverse backgrounds in PKD, calcium, cAMP and cell proliferation discussed the molecular basis of PKD and the potential for therapeutic intervention, based on latest research.

Autosomal Dominant Polycystic Kidney Disease (ADPKD) results from loss of function mutations in either the PKD2 (polycystin-

2) or PKD1 (polycystin-1) genes. PKD1 is a 'receptor protein' that spans the outer membrane of the cell. It communicates signals from outside, such as from a hormone, to the nuclear DNA to regulate the expression of certain genes that can, for instance,

"To understand why PKD develops we need detailed knowledge of how PKD1 and PKD2 work"

influence the cell to divide or not. Loss of PKD1 function causes abnormal cells and ultimately cysts. PKD 2 is a 'protein channel' that permits the flow of certain ions, such as calcium, across cell membranes. Calcium is a 'second messenger', a signal used by the cell to

communicate information to genes in the cell nucleus, again influencing the cell's future such as deciding to divide and grow. Loss of PKD2 function also causes abnormal cells in the kidney and generation of cysts.

To understand why PKD develops we need detailed knowledge of how PKD1 and PKD2 work. This is remarkably complicated. Recently, it was discovered that a class of drug called vasopressin V2 receptor (VPV2R) antagonists lowered renal cAMP (like calcium, cAMP is a 'second messenger'), prevented renal enlargement, inhibited cystogenesis and protected renal function in animal models of PKD.

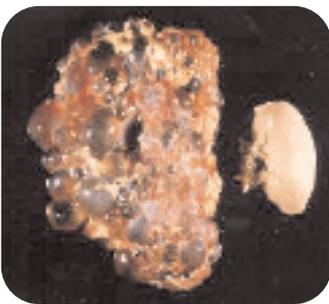
Participants discussed progress in recent drug results to halt PKD in mice

models of the disease. Tolvaptan is one such drug, now being tested in early phase clinical trials in the USA (see article on this page). Discussions centred on the molecular targets of the drug and site of action, as well as highlighting other avenues of research relevant for drug intervention.

Feedback from the conference was excellent and we expect that new research developments will result from the meeting. The conference was an excellent example of how we can promote research in the UK, since it included some of our leading scientists and physicians, as well as major research figures in the USA and Europe.

OPC 31260 Tolvaptan clinical trials are underway

Peter Lockyer



The drug Tolvaptan (OPC31260) has recently been shown to block the growth of kidney cysts in rodent models of PKD. In some cases, the drug has even led to the regression of existing cysts in some animals (reported in *Nature Medicine*, 2004).

Tolvaptan is just one of a family of similar drugs now

being investigated as potential treatments for PKD. The drug works by blocking a 'protein receptor' at the cell surface called the vasopressin receptor. Similar types of drugs are already in use for water retention diseases and chronic obstructive pulmonary disease. So far patients tolerate these treatments very well, with minimal side-effects. The treatment is potentially useful for ADPKD and ARPKD, the Autosomal Recessive version, according to the research performed on rodents.

Phase II clinical trials and American FDA approval is

completed in the United States. This phase determined the side effects and the dose of Tolvaptan. Phase III clinical trials take place later this year.

The drug trials in the US have arisen from research by scientists funded by the PKD Foundation in the US. Around 1000 to 3000 volunteers will be needed to complete FDA approval for Tolvaptan. US centres are the natural focus for this research as several PKD clinics have extremely large patient cohorts, far in excess of numbers available in the UK.

At the moment The PKD Charity is unaware of plans

for OPC research in the UK, although we have raised the issue with leading UK researchers at the recent PKD Charity/Novartis Foundation/PKD Foundation (US) held in London in January 2005 (see article on this page).

Nevertheless, we are monitoring results from the trials as they become publicly available and will keep you informed of progress. We must point out that the results in rodents, although exciting and hopeful, should be tempered by the knowledge that many drugs fail at this stage of research.

Fundraising

Black Ties & Mountain Highs



Rebecca Murphy presents a cheque of £11,905.75 money raised at the Geraldine Murphy Irish Black Tie Dinner and Dance to Dr Anand Saggar. The dinner at Haydock Park Racecourse is an annual event in memory of Geraldine, Rebecca's mother

Dancing the night away at Haydock Park Racecourse and slogging up through ice and snow on Mount Everest has generated over £13,000 of much needed funds for the PKD Charity.

The Geraldine Murphy Black Tie Irish Dinner Dance was held again this year at Haydock Racecourse with all the guests enjoying a great evening and raising over £11,000 through their auction and raffle. Dinner guests keenly bid for tickets to the sold out Ricky Hatton boxing match, a McFly engraved gold disc, an autographed Robbie Williams' Autobiography and, the star lot was a custom-made wheelbarrow bicycle which went for £1200. Then the night was spent dancing to jigs of live Ceilli band 'Amran'.



High in the Himalayas, the PKD flag flies on Mount Everest. Nick Reddington, friend of the Murphy family, climbed to Base Camp and planted the flag simultaneously raising £2000.

Sporting heroes



Five brave runners for the PKD Charity made a staggering £4 to £5 a minute as they ran the 26-mile Marathon from Greenwich Park to the Mall. The fabulous five brought home over £7000 for the charity. Martin Bootman had to withdraw through illness but still donated all the funds he had raised.

Friends of Cammeron (Cammy) Johnston, who died sadly young of PKD while playing rugby for London Scottish Club, organised a 'Memorial Rugby Match' in May 2005. Cammy is survived by Meredith and their two children, Meghan (4) and baby Callum - Meredith was 8 weeks pregnant when the terrible tragedy happened.

Over 70 players donned their boots for the Match and approximately 300 people came to watch. After the rugby, they had a hog roast and barbeque followed by a ceilidh (Scottish Country Dancing). The day was a huge success as it brought together friends from across the world with Meredith, her children, and many members of Cammy's family.

During the day, as well as the rugby and partying they organised a raffle and hosted an auction of autographed rugby shirts, helicopter rides,

weekends away, tickets to rugby matches, a VIP day at Silverstone on an FI testing day to name but a few.

Including Gift Aid, Cammy's friends raised more than £6,500.



Make your donation go further with gift aid



Money donated to the PKD Charity can be boosted by nearly a third under the UK Gift Aid scheme. Provided you are a UK taxpayer, we can reclaim the basic rate tax paid on your donation, worth an additional 28% of the amount.

So, a gift of £10 from you is worth £12.80 to the PKD Charity. At no extra cost to you!

Subject to a few simple rules, you can give any amount of money, large or small, regular or one-off, and we can reclaim the tax. All we need from you is a simple declaration saying that you want to use Gift Aid.

A declaration can cover one or more donations, and it can be made in writing, by e-mail or orally. It can also be backdated to cover all donations since 6 April 2000.

To Gift Aid your donation, please email info@pkdcharity.org.uk or phone 01388 665004 and request a form. Or visit our website www.pkdcharity.org.uk and download a form from the site.

Patient support

Insurance and PKD



If, like me, you have had difficulties finding life insurance cover at affordable rates then this article should be of interest.

I should stress this is not an advertising ploy but a serious investigation of the insurance industry, thanks to Simon Fisher, MD of Coleman Financial Services, who kindly researched the state of the industry in relation to life-threatening conditions such as PKD. Coleman Financial Services is part of one of the largest and most successful independent insurance brokers in the south.

I suggested to Simon Fisher that insurers could obtain more business if they look more carefully at the risks associated with specific genetic conditions, which could result in cheaper premiums, or even obtainable cover in some instances. After carrying out some research Simon found that this view did not match that of the industry. He said, 'Unfortunately, whilst this appears a perfectly reasonable argument, this is not necessarily a view shared by insurance companies. I have spoken at a high level with several insurers and they have explained that, generally speaking, insurance companies are in the business of spreading risk rather than concentrating it'.

There is the obvious commercial risk that if they undertake a lot of research on a particular medical condition and only a small amount of business is written, then this is clearly not cost effective. However, if the insurance company does become a specialist in this area and attracts lots of clients with the same condition, then they are concentrating risk, which by their very nature they are inclined against. It was explained to me that within the industry there was an attempt to test an initiative for a

higher risk medical group some years ago that did not work out well for the insurers, so they are not keen to repeat the experience.

A useful point that did come out of my discussions is that the industry acknowledges that those with a particular medical and/or genetic condition find it frustrating, time consuming and often very difficult to obtain terms. As a result of this, a small number of specialist underwriting agencies have recently been established. These have negotiated with leading insurance companies so that clients need only complete one application form, undertake one medical examination etc., and that this information will then be supplied to a number of different companies for a quicker underwriting decision.'

If those affected by PKD and are interested in obtaining life insurance and critical illness cover then Simon Fisher and colleagues would be happy to act as an intermediary in this regard. Please email the PKD Charity if you would like to obtain their address and telephone number.

*Dr Peter Lockyer, Trustee,
The PKD Charity*

Email: info@pkdcharity.org.uk

The National Kidney Federation has a list of companies that will assist with obtaining holiday insurance. Visit <http://www.kidney.org.uk/holidays/insurance.html> or call their helpline on 0845 601 0209.

Note that The PKD Charity is not endorsing any particular insurer or broker.

We welcome your views and your questions. The Helpline - 01388 665004 - is open to all. Visit our website - www.pkdcharity.org.uk or email us on info@pkdcharity.org.uk.



Recipe for keeping kidneys healthy

This recipe was developed by Jamie Wiesenberg, who is the Renal Dietitian at Burbank Dialysis Partnership in Burbank, California.

Gingered Chicken

Makes 4 servings

Ingredients

4 Boneless, skinless, chicken breasts (about 4 oz. each 113g)
2 Tablespoon ground ginger
1 Teaspoon pepper
2 medium onions, thinly sliced
1/4 cup finely chopped parsley
1 Tablespoon lemon juice
1 Tablespoon olive oil
1/2 Cup water

Instructions

Mix together ginger and pepper on plate or piece of wax paper. Rinse chicken breasts and pat dry. Roll each piece in ginger mixture, shaking off excess seasonings.

Brown chicken breasts in oil, about 3 minutes on each side. Reduce heat to low and pour water over chicken. Add lemon juice and spread sliced onions over the chicken. Cover and simmer for 5-6 minutes until breasts are cooked through. Place chicken pieces on serving plate. Spoon onion slices over chicken and sprinkle with parsley. Serve immediately.

Nutrient Information

Approximate analysis per 1/4 chicken:

Protein:	28 grams
Sodium:	82 mg
Fat:	5 grams
Potassium:	455 mg
Carbohydrate:	7 grams
Calcium:	35 mg
Calories:	194 kcal
Phosphorus:	257 mg