pkd charity newsletter
Polycystic Kidney Disease

Registered Charity No. 1085662

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Tolvaptan trials enter final phase

Tolvaptan is being evaluated as a potential treatment for PKD after its efficacy was demonstrated in studies using rats with polycystic kidneys.

The PKD Charity has heard informally that the drug has been well-tolerated by PKD patients in recent trials but the impact of the drug on disease management remains confidential. We anticipate initial results will not be published for 12 to 24 months.

Human trials were due to start in the US last year, so trustee Dr Peter Lockyer contacted the company that developed the drug–Otsuka–to find out what progress had been made.

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Otsuka is aware that many people with PKD eagerly await word on the Tolvaptan trial and knows that it is frustrating that the details regarding eligibility are not available. However, they are keen to avoid falsely raising people's hopes by issuing information before the protocol is final.

Otsuka explained that it had discussed their trial protocol with the regulatory agencies from the USA, EU and Japan and consulted their global Scientific Advisory Board.
As a result of these discussions, Otsuka had revised its protocol and has begun sharing it with potential clinical investigators. A global contract research organisation will implement the trial.

Otsuka has already contacted some renal clinics in the UK, so you may wish to ask your consultant if your clinic is a possible trial unit.

Check our website for latest research news: www.pkdcharity.org.uk

PKD Information Weekend

Bristol – July 1st and 2nd

Join us at the PKD Information Weekend in Bristol on July 1st and 2nd.

The focus of the event is a programme of information sessions covering the genetics of PKD, an update on treatments and research, advice on diet, pregancy and PKD, and coping with the condition.

The programme starts at 12 pm on Saturday and ends at 4 pm on Sunday. And there is no charge for attending.

The event will be held at the Southmead Centre for Medical Education in Westbury-on-Trym, Bristol.

To register for a place, please email: infoday@pkdcharity.org.uk or call Justina Wilkinson on 01246 823468.

Funding has been provided by Awards for All.

Trustees wanted

The PKD Charity needs committed individuals to join our Trustee Board.

Help us achieve our aim to make a real difference to the 60,000 people with PKD in the UK and their families.

The charity's trustees are all volunteers, supported by a part-time project manager and a grants' consultant.

We are keen to attract people from all backgrounds, with experience of living with PKD as well as a range of communications, fundraising and organisational skills.

If you would like to register your interest email info@pkdcharity.org.uk.

The PKD Charity has changed its logo - we hope you like our eyecatching new look!

Blood pressure study reveals benefits of drug combining

Major international research has examined medicines used for lowering blood pressure. The Ascot study's results are of great importance to people with PKD, two thirds of whom are managing high blood pressure.

The study found that lowering blood pressure will reduce risk of heart disease and stroke.

Taking a combination of a calcium-channel blocker with an ACE inhibitor significantly reduces the risk of stroke, heart attack and diabetes compared with a beta-blocker and a diuretic.

People reduced their risk of heart attack and stroke by more than half when they took a combination of these newer medicines–calcium-channel blockers and ACE inhibitors–together with a statin (a cholesterol-lowering medicine).

Given these results, those people who take beta-blockers without also taking a cholesterol-lowering medicine should discuss treatment with their doctor or nurse.

Do not stop taking medications without consulting your doctor.

For more on the Ascot study and to download an information sheet, visit the Blood Pressure Association website: www.bpassoc.org.uk.

Google plants idea

A team of PKD researchers has identified a bizarre substance in the enlarged kidneys of patients with PKD. The team, which includes PKD Foundation founder Dr Jared Grantham, had been trying to identify the potent, fatty substance that stimulates cyst growth in kidneys for 12 years. When they searched Google for the molecular formula they had created, up popped a picture of a plant!

"We learned that the chemical is forskolin, the same as the chemically-active ingredient in the roots of a herb grown in India" Grantham said. Forskolin is used in health food supplements taken to treat a number of conditions, including high blood pressure.

This new knowledge will hopefully lead to treatments, therapies and preventions

"This new knowledge will help us better understand the process of cyst growth and hopefully lead to treatments, therapies and preventions," Grantham added.

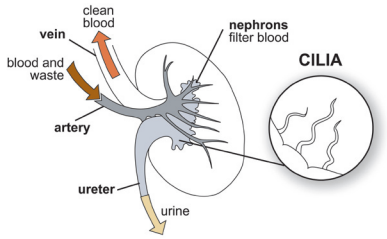
We shall follow this discovery closely.

Given that it is a substance that stimulates cyst growth, Dr Grantham strongly recommended that people with PKD do not take any products that contain forskolin.

The importance of cilia

by Dr Cathy Boucher of Addenbrooke's Hospital, Cambridge

Tiny antenna-like structures inside the kidney, called cilia, may be important in understanding cyst growth in people with Autosomal Dominant and Recessive PKD. The two genes that are altered in people with these diseases–PKD1 and PKD2–are known to control the function of cilia, among other things.



Primary cilia are located inside each of the millions of nephrons within the kidney. Each of these nephrons consists of a glomerulus, which filters blood, and a tubule, which concentrates urine. Kidney cysts are, in fact, parts of the tubule that have become very dilated.

Lining the tubule are epithelial cells. And on top of each of these cells is a primary cilium, protruding like a small antenna into the middle of the tubule.

The role of cilia is not fully understood, though it is thought that they detect urine flow and send signals to the epithelial cells. It is also believed that cilia help to control the width of the tubule. When the cilium malfunction it is possible that they cause the tubule to dilate enormously to form large cysts.

Renal genetics clinic

A specialist clinic for people with genetic kidney disease was opened in 2005, at Addenbrooke's Hospital, Cambridge.

The Renal Genetics and Tubular Disorders Clinic brings together medical and nursing specialists from nephrology, medical genetics, urology and clinical biochemistry. Genetic counselling and genetic testing are also offered.

The clinic's range of services allows the team to evaluate fully and care for the patient and their family, which should mean better, more efficient care.

Both GPs and hospital doctors can make referrals to the clinic's services.

The clinic was set up following the Government's initiative, *Bringing Genetics into Mainstream Medicine*. It has two years' funding from the Department of Health to see if care for people with genetic kidney diseases and their families could be improved.

More information about the clinic can be found at: www.addenbrookes.org.uk/serv/clin/med/renalgenetics_clinic.html

Donors - use Gift Aid

Please fill in a Gift Aid form when donating–the Government adds 28p to each £1 you give.

Have you considered payroll giving?

Does your employer run a payroll giving scheme? You can give any sum direct from your payroll and it's tax free. Ask your personnel manager and if your company doesn't run a scheme, suggest they contact Give As You Earn, the UK's largest scheme. Plus, until December 2006, the government gives up to £500 to small firms that set up Payroll Giving and will match the first £10 of your monthly donations for six months. That's an extra £60 going to charity!

Give As You Earn
Tel: 01732 520 055
www.allaboutgiving.org

A night to remember Geraldine

Haydock Park played host to the fourth Geraldine Murphy Black Tie Irish Dinner Dance in aid of the PKD Charity in March this year.

This event was set up to remember Geraldine Murphy in the only way her family knew – lively entertainment, fine food and hearty laughter. Geraldine was first diagnosed with PKD at an early age and lived a full and active life until her reduced kidney function required a change of lifestyle, which included regular dialysis. After several years Geraldine received a successful kidney transplant and her life was transformed until she sadly passed away in 2002.

The annual event attracts around 400 friends, family members and business associates who have helped to raise a total of more than £50,000.

The fifth Irish Dinner Dance will take place in March 2007. To find out more, or to donate prizes, call Rebecca Murphy on 07712 773 298.

Revellers at the fourth Murphy Dinner

All change for trustees

by Tess Harris

Dr Anand Saggar has stepped down as chairman of the charity's Trustee Board and was appointed chairman of the newly-established PKD Scientific and Research Advisory Board. News of the new Board will be published in future issues of this newsletter.

Anand is a leading genetics consultant specialising in inherited kidney disorders. He was a founder of the PKD Charity and remains our medical advisor. I know that all of you who have been helped and comforted by Anand will join the trustees in thanking him for his tireless support of the charity.

I was honoured to be asked to chair the Trustee Board as Anand's successor. I have PKD, work in business and live in London. Please feel free to contact me at tess@pkdcharity.org.uk.

On behalf of the trustees, I would like to thank Dr Peter Lockyer, Professor Steve Jeffery and Gloria Fox, who have resigned from the Board, for their huge commitment and dedication over the years.

Help raise funds online

We have teamed up with JustGiving who provide online services to enable people to support their favourite charities online. Donations can now be made easily to the Charity using a credit or debit card.

Supporters can also start their own web page to promote events and collect sponsorship funds. The government adds 28p for each £1 donated. So, email the address to everyone you know –

www.justgiving.com/pkd/raisemoney

Thanks to our donors for their generous support

Thank you to everyone who helped us raise over £40,000 in donations last year. In particular, we would like to thank the Murphy family and friends, St George's Hospital Student Rag, M J Davies, Cameron Johnston family and friends, Mick Perry, Mrs M Wilkinson, Chris Cottrell, Parkstone Golf Club, C Gundle, A Witkin. We'd also like to thank The D'Oyly Carte Charitable Trust, Camelot and the Thomson Corporation.

Remember us

When writing a will, please consider the PKD Charity. Contact us to find out more.

Less salt doesn't have to mean less flavour

The food we eat can help lower blood pressure and slow the progression of PKD. Most important, we should reduce the amount of salt in food.

So, what is a safe amount of salt? No more than one level teaspoon–six grams (2.4g sodium)–per day. One gram of salt contains 0.4g (400mg) of sodium.

The Dietary Approaches to Stop Hypertension Eating Plan (DASH) strongly recommends reduced salt along with high levels of fruits and vegetables, low-fat dairy and proteins. To get a copy of DASH, email tess@pkdcharity.org.uk.

PKD Charity chairman Tess Harris suggests adding these ingredients to your food instead of salt: a bay leaf, garlic, mint, pepper, curry powder, ginger, onions, a pinch of sugar, fruit, dry mustard, paprika, parsley, rosemary, tomatoes, lemon. Or try Tess's recipe for no-salt seasoning.

Consistently high BP (140/90) must be treated with medication.

Recipe for no-salt seasoning

Mix together:

- 1 tsp of herbs (dried or fresh) basil, marjoram, thyme, parsley and sage
- 1 tsp of black pepper, onion powder and ground garlic to taste
- If desired add a tsp of cayenne

ADPKD leaflet

The PKD Charity has been busy putting together a leaflet on ADPKD, aimed at people who have recently been diagnosed with the disease.

The leaflet is the first in a series of nine that will each focus on a different aspect of PKD. The next to be published is dedicated to information for people with ARPKD.

Other leaflets will cover managing diet, brain aneurysms, the emotional effects of the disease, blood pressure and urinary tract infections.

To place an order for any of these leaflets, or to offer to circulate them at your local surgery or clinic, contact us at info@pkdcharity.org.uk or call 01388 665004.

Adopt the PKD Charity

We are looking for a business to adopt us. Interested? Email tess@pkdcharity.org.uk

What is ARPKD?

Autosomal Recessive Polycystic Kidney Disease (ARPKD) is a rare genetic disorder affecting approximately 1 in 6,000 to 1 in 40,000 persons in the general population.

ARPKD is a chronic, progressive disease and a major cause of renal and liver-related sickness and mortality in children. The child's kidneys can be greatly enlarged, peaking in size when the child is one to two years old, and stabilising when they reach four to five.

There is no cure and, sadly, many children die shortly after birth, due to problems with lung development. However, those children who do survive have the potential for a high quality of life, with careful medical management of the condition in adolescence.

Almost all cases of ARPKD are caused by abnormalities in a single defective gene called PKHD1. ARPKD is recessive, which means the defective gene must be inherited from both parents for the disorder to occur. When both parents are carriers, there is a 25% chance of ARPKD occurring with each pregnancy, a 50% chance that their children will be carriers but not have ARPKD, and a 25% chance they will neither be carriers nor have ARPKD.

Almost everyone with ARPKD is diagnosed during infancy or childhood, with around 50% of cases diagnosed during pregnancy.

At birth, the infant's enlarged flank areas may complicate delivery. In the most severely affected children, Potter's syndrome may be present, a condition characterised by facial disfigurement and, occasionally, deformed limbs. Babies may be born premature and some will have low serum sodium (salt) levels and water imbalances.

During the first four weeks of life renal function may be affected but failure is rare. Breathing may be impaired by large cystic kidneys and selective kidney removal may allow room for the lungs to expand. If ventilation can be given, chances of survival increase. The prognosis, especially for those who survive the newborn period, is less bleak than once thought. The five-year survival rate is 80–95%. Because of improved renal treatment, and better control of blood pressure, it is now common to survive into adulthood.

Children with ARPKD may urinate frequently and have excessive thirst. Bed-wetting is not uncommon in school-age children. There is a risk of dehydration with prolonged fevers, vomiting or diarrhoea. Water consumption is very important.

High blood pressure occurs in almost 80% of children and is often severe. It can develop soon after birth. High blood pressure puts added pressure on the kidneys and, if severe, can be life-threatening.

Visit our website to find out more about ARPKD:
www.pkdcharity.org.uk

Alternatively, the American organisations ARPKD/CHF Alliance and the PKD Foundation provide more information on their websites:
www.arpkd.org
www.pkdcure.org

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