

PKD research – becoming a treatable disease?

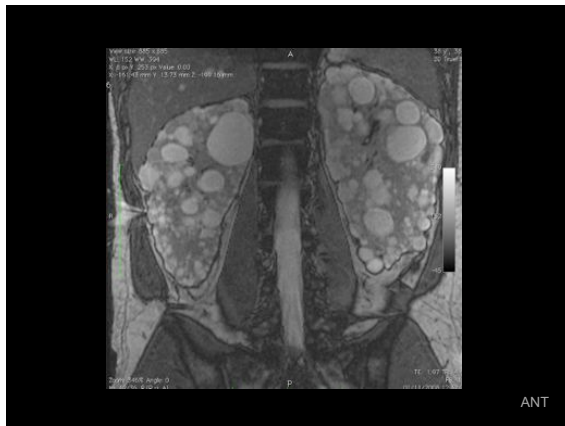
Neil Turner

October 2014



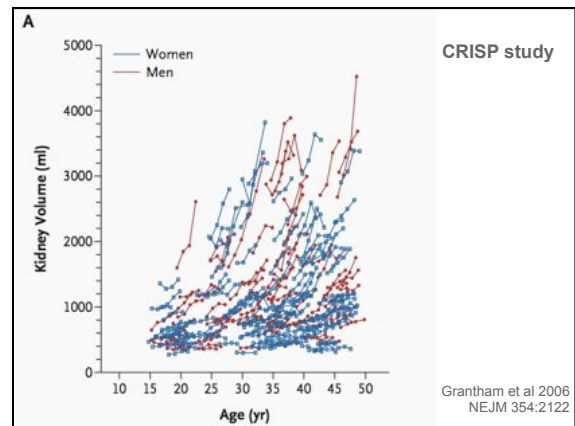
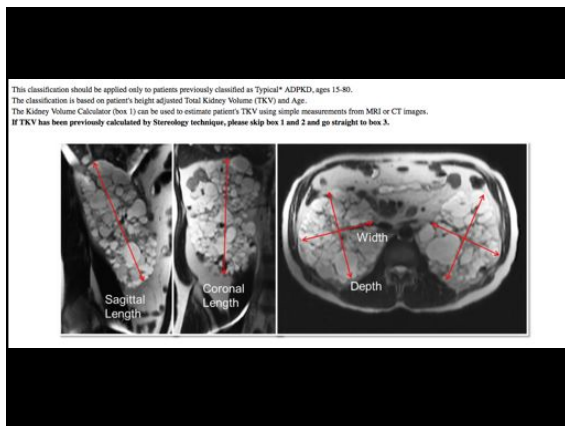
What works in CKD and PKD

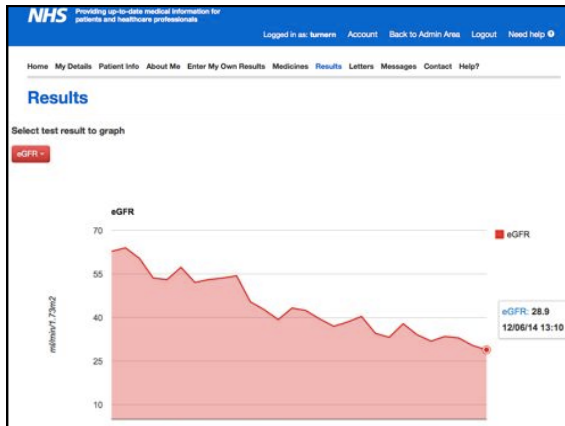
Diet	Not major
Blood pressure	Yes CKD, <i>No PKD</i>
ACEi	Yes in many, <i>No PKD</i>
Herbal treatments	No – <i>PKD, No, No, No!</i>
Fish oil	No
Gene therapy	No
Stem cell therapy	No
Lots of water	No CKD, <i>??PKD</i>



The tolvaptan (TEMPO) trial

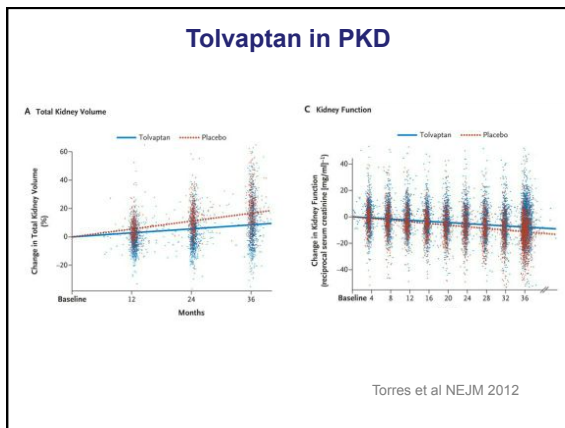
- 3y treatment with drug or placebo
- Age 18-50
- Good kidney function (eGFR>60)
- Big kidneys
- MRI
- Pregnancy not allowed





Cyst growth inhibitors

- Sirolimus
- Vasopressin (ADH) antagonists
- Somatostatin
- A number of others ...



Problems with tolvaptan

- Tolerating polyuria
- Liver test abnormalities
- Cost

Important Qs about tolvaptan

- Earlier diagnosis may be valuable
- When should you start it?
- Who should have it?
 - Should we be measuring kidney size??
- Does it keep on working for ever?
- How much is it worth?

Am I going to need dialysis?

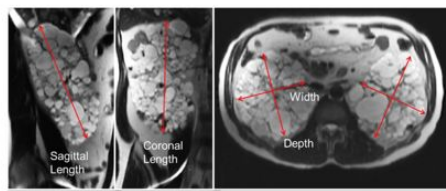
- Look at your family
- (The gene abnormality)
- Your kidney function (Creat, GFR)
- Age when you got trouble
- Size of kidneys

Predicting who'll get into trouble

The CRISP study and others show

- Bigger kidneys – more trouble
- But cysts grow with time
- And it depends how big you are

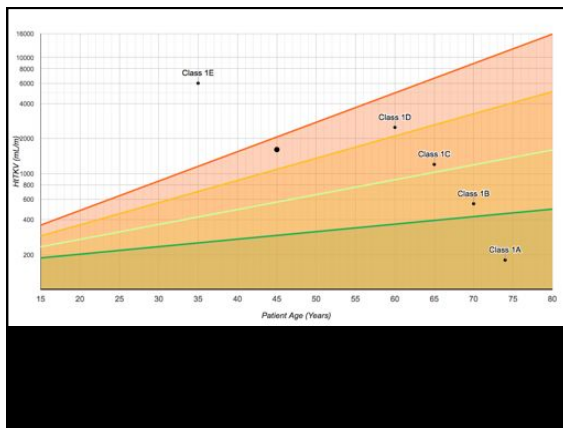
mayocl.in/1mFSfgh



Kidney Volume Calculator based on Ellipsoid equation (WxLxWxL) from MRI or CT image

Required Data Entry			
Right Kidney			Left Kidney
Sagittal Length (mm)	200		Sagittal Length (mm)
Coronal Length (mm)	260		Coronal Length (mm)
Width (mm)	100		Width (mm)
Depth (mm)	95		Depth (mm)
Right Kidney Volume (mL)		Calculated Results	Left Kidney Volume (mL)
1293.3			1446.3
		Total Kidney Volume (mL)	2739.6

Clear All Calculate Volume



Research studies in PKD

Updated: 08/25/2014

Active, Recruiting

Study Name	Observational	Phase I	Phase II	Phase III	Phase IV
ADPKD Pain Study					
Genetic and Molecular Description of PKD1 and PKD2 Mutation Negative Centers in ADPKD (GeneDives)					
Consortium for Radiologic Imaging Studies of Polycystic Kidney Disease (CRISP II)					
Core A: The HepatoRenal Fibrocystic Diseases Translational Resource					
Diet as a Potential Treatment for Autosomal Dominant Polycystic Kidney Disease					
Efficacy and Safety of Tolvaptan in Subjects With Chronic Kidney Disease Between Life Stage 2 to Early Stage 4 Due to ADPKD (REPROBE)					
The Efficacy of Everolimus in Reducing Total Nephric Kidney Volume in Polycystic Kidney Disease (PKD) Transplanted Recipients (E-VERVO-012)					
The European Initiative Building a Network of ADPKD Reference Centers Across Europe					
Evaluation of Gut Bacteria in Patients With Polycystic Kidney Disease					
Everolimus on CKD Progression in ADPKD					
Lamivudine in Polycystic Kidney Disease Study (LIPS)					
Research on Stem Cell Transplantation in Patients with Chronic Renal Failure Due to Polycystic Kidney Disease (PKD)					
Open-Label Tolvaptan Study in Subjects With ADPKD					
Paenololol LAN in Severe Polycystic Liver Disease (SOM203)					
PKD Clinical and Translational Core Study (PKD Core)					
Pulsed Oral Sildenafil in ADPKD - The Vienna RAP Study					
Radiographic Model for ADPKD Study: Prevalence and Disease Progression Control (RAFALE)					
A Safety, Pharmacokinetic and Dose-Extension Study of K0019 in Subjects With ADPKD					
Sildenafil for Massive Polycystic Liver (SILVER)					
Somatostatin in Patients with ADPKD and Moderate to Severe Renal Insufficiency (SLADN-2)					
Study of Lamivudine to Treat Polycystic Kidney Disease (DPRAK)					
Topical-Combining Everolimus as Treatment for Autosomal Dominant Polycystic Kidney Disease (ADPKD)					
Uncontrolled, Open-Label, Pilot and Feasibility Study of Mesenchyme (Vitamin B3) in Polycystic Kidney Disease (NIAC-PKD1)					
Urodeoxycholic Acid as Treatment for Polycystic Liver Disease (CURSOR)					

Treatment

- Lead a healthy and normal life
 - Be active
 - Eat a healthy diet
 - No bad things
- Check your BP
- Keep in touch



Beth Shortt, © Kidney Research UK

More info



patientview.org
edren.org/info
pkdcharity

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