MANCHESTER 1824

The University of Manchester

wellcome trust centre for Cell-Matrix Research

# Alport Syndrome Information Day

21st November 2015

alport uk

a brighter future for people living with alport syndrome

Royal Manchester Children's Hospital

shaping futures

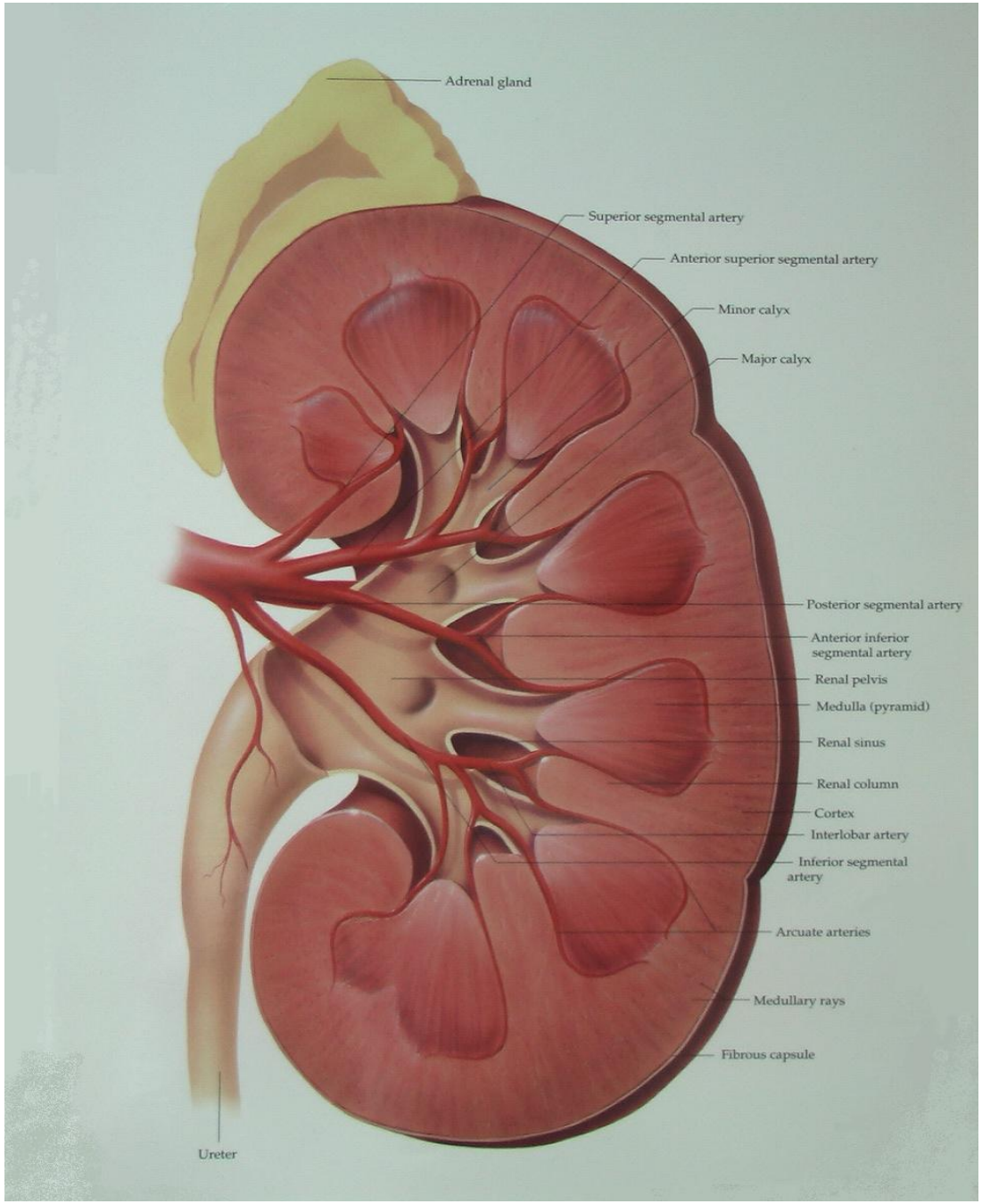

University of Manchester

How do kidneys:

Develop?

Stay healthy?

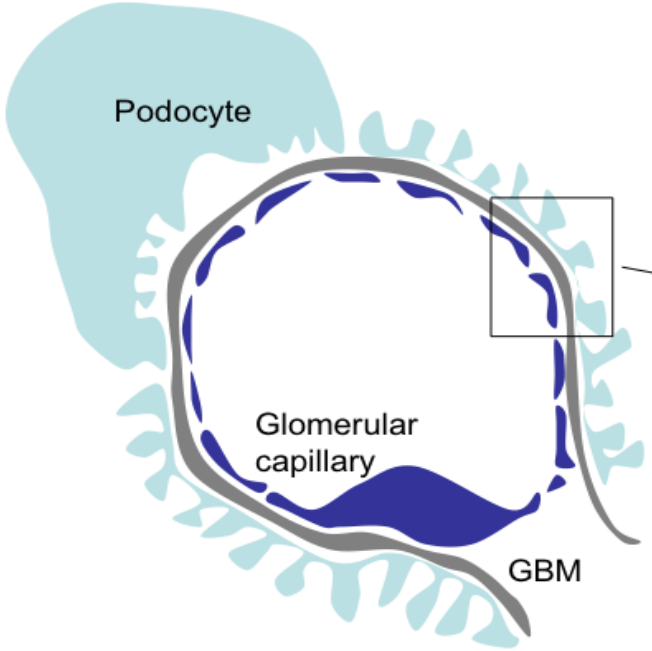
Change in disease?

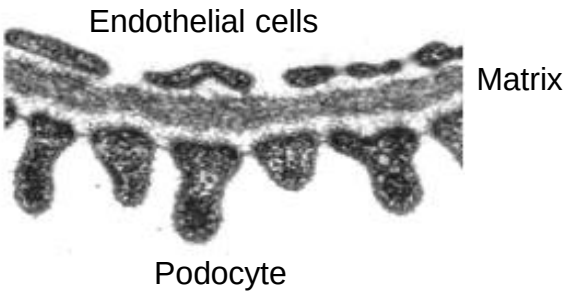



**What do kidneys do?**

1. Remove waste
2. Keep bones healthy
3. Help to control blood pressure
4. Help to make red blood cells

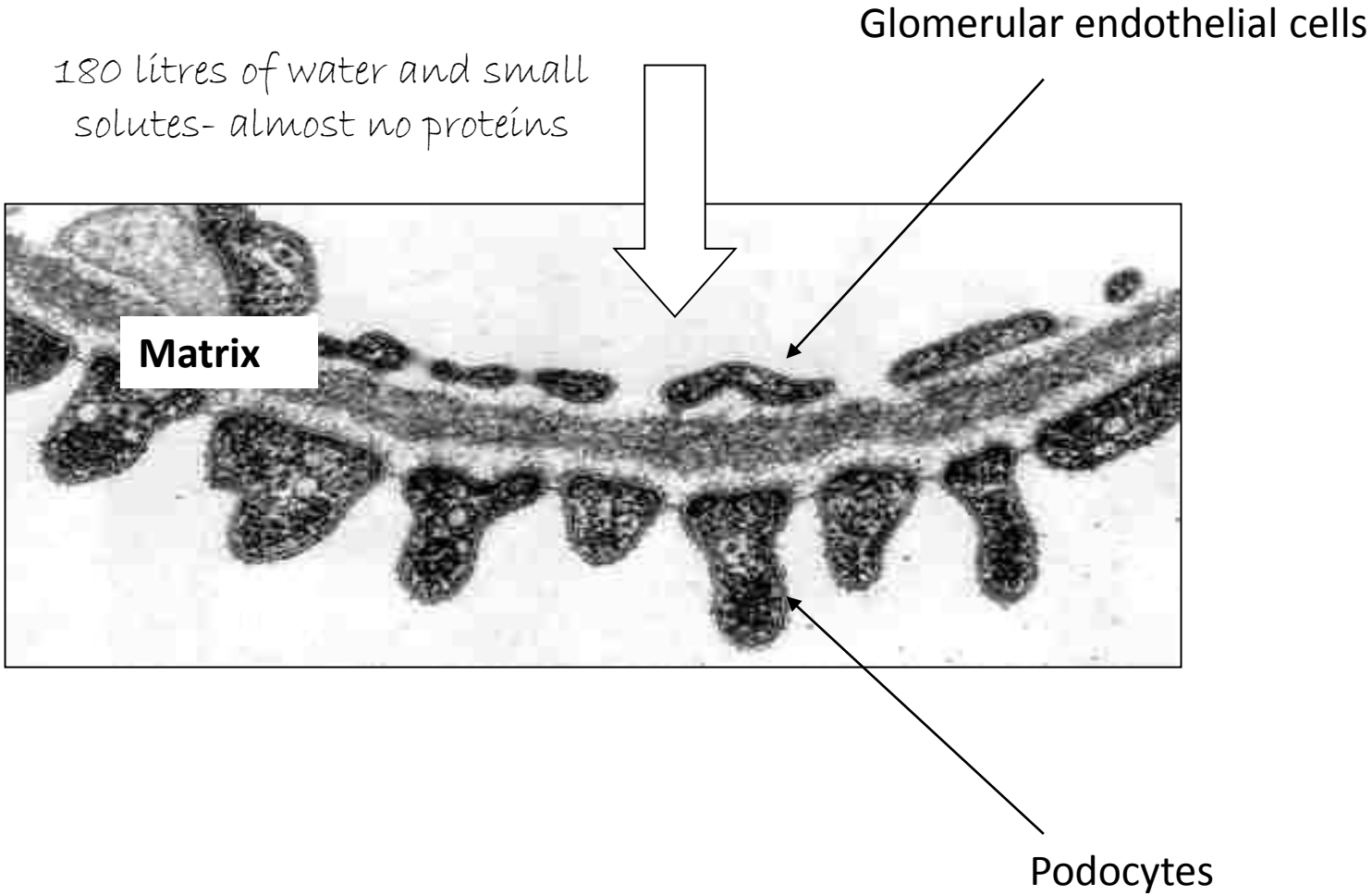
**Kidney disease affects up to 1 in 10 people**

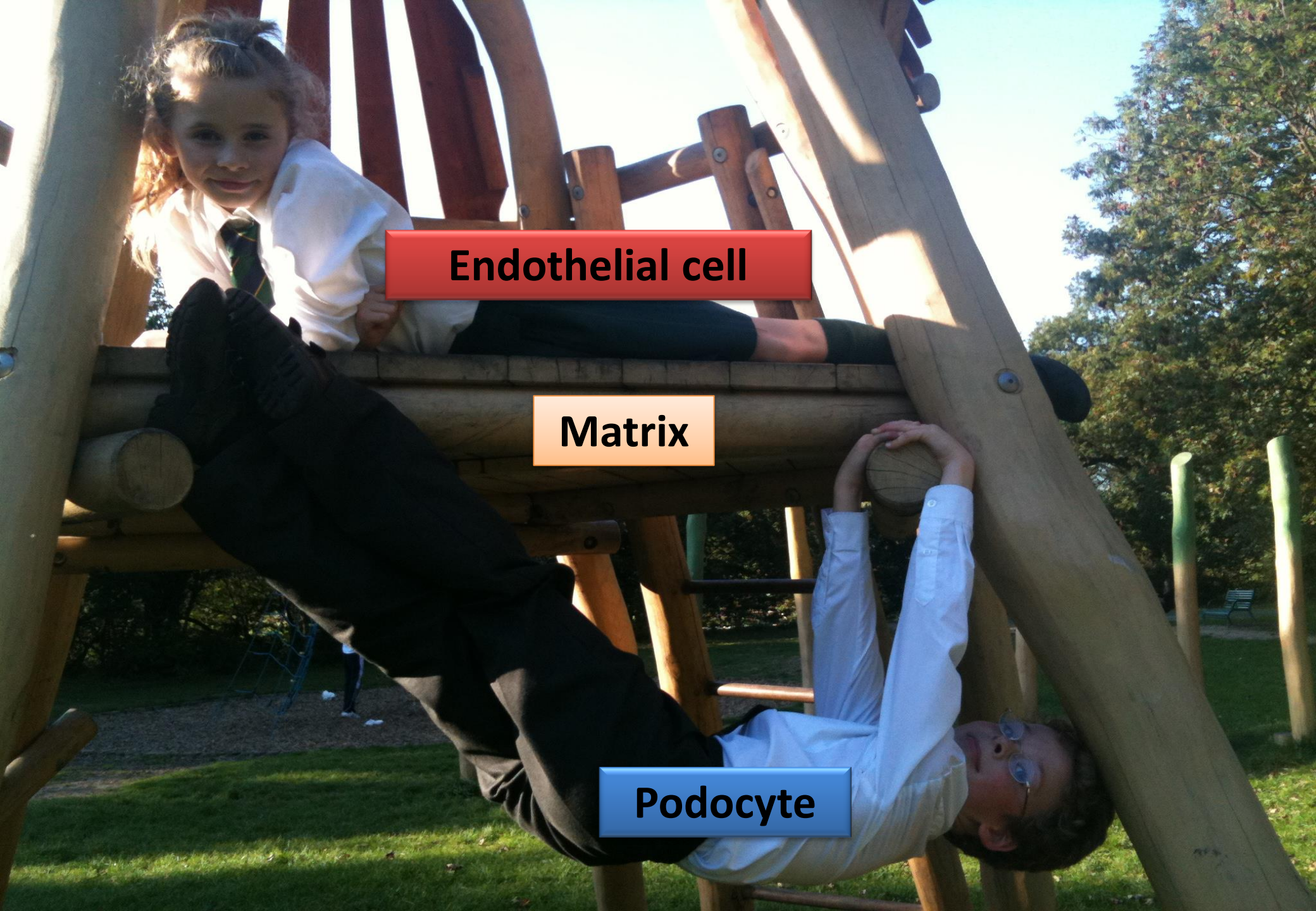


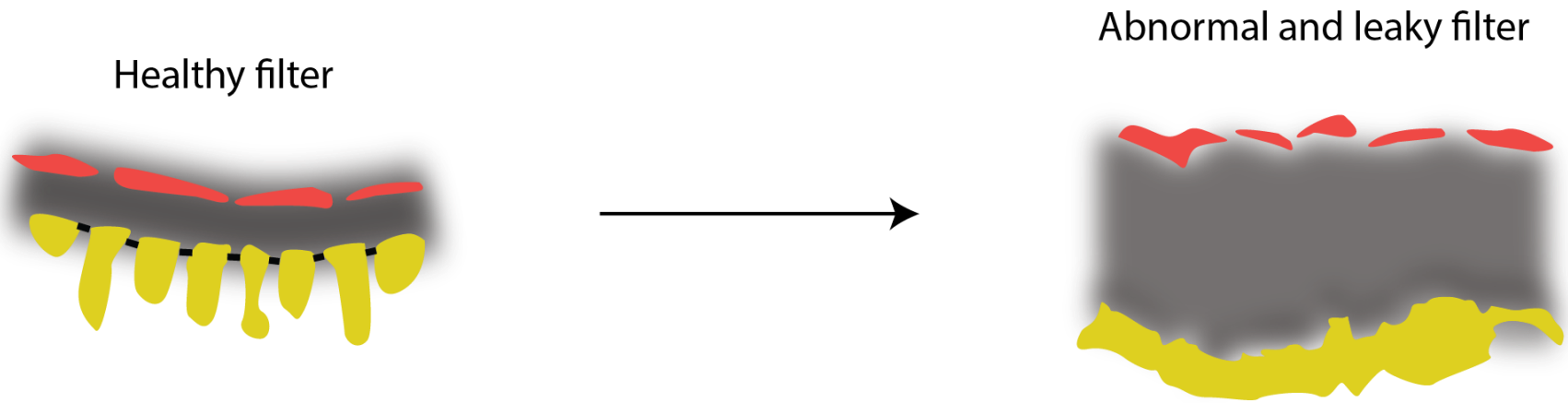

1 million filters in each human kidney…

# The glomerular filter




Endothelial cell
Matrix
Podocyte

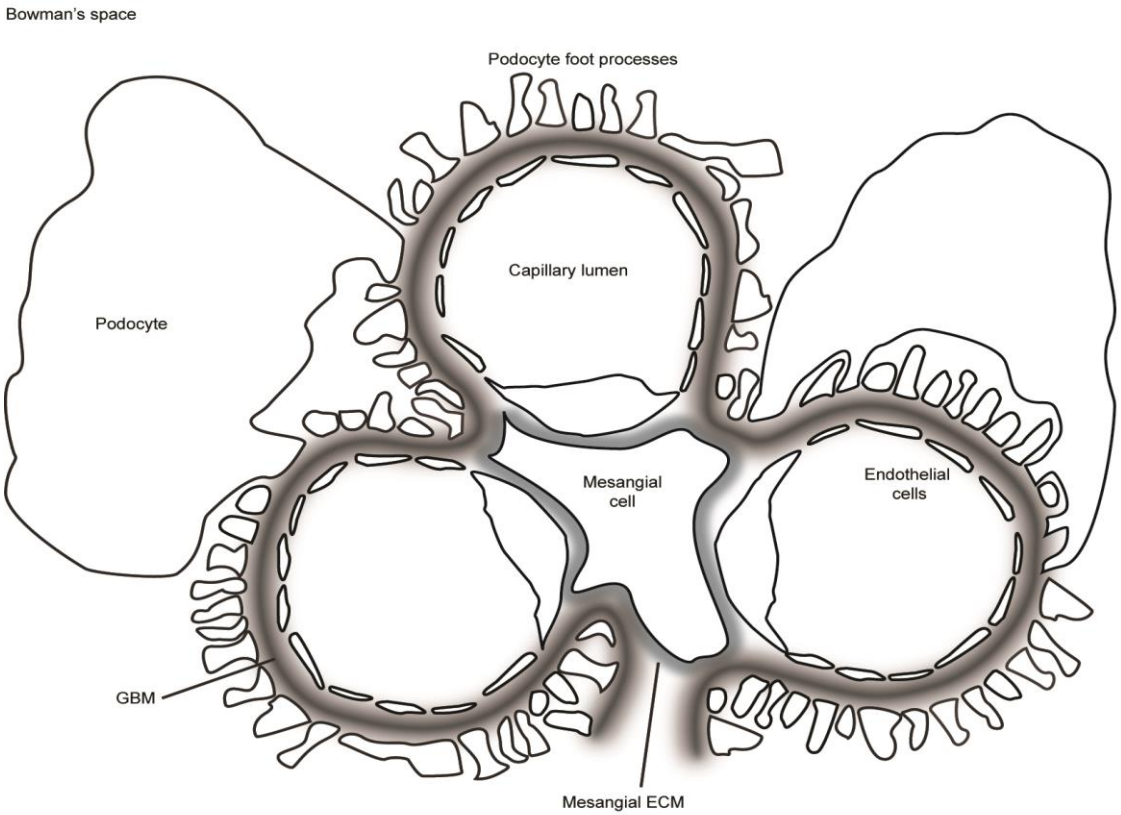
# What happens in Alport Syndrome?




Blood cells and protein leak into the urine

Urine testing sticks- blood cells and protein

# How do we make and maintain the filter?




Unique and complex structure

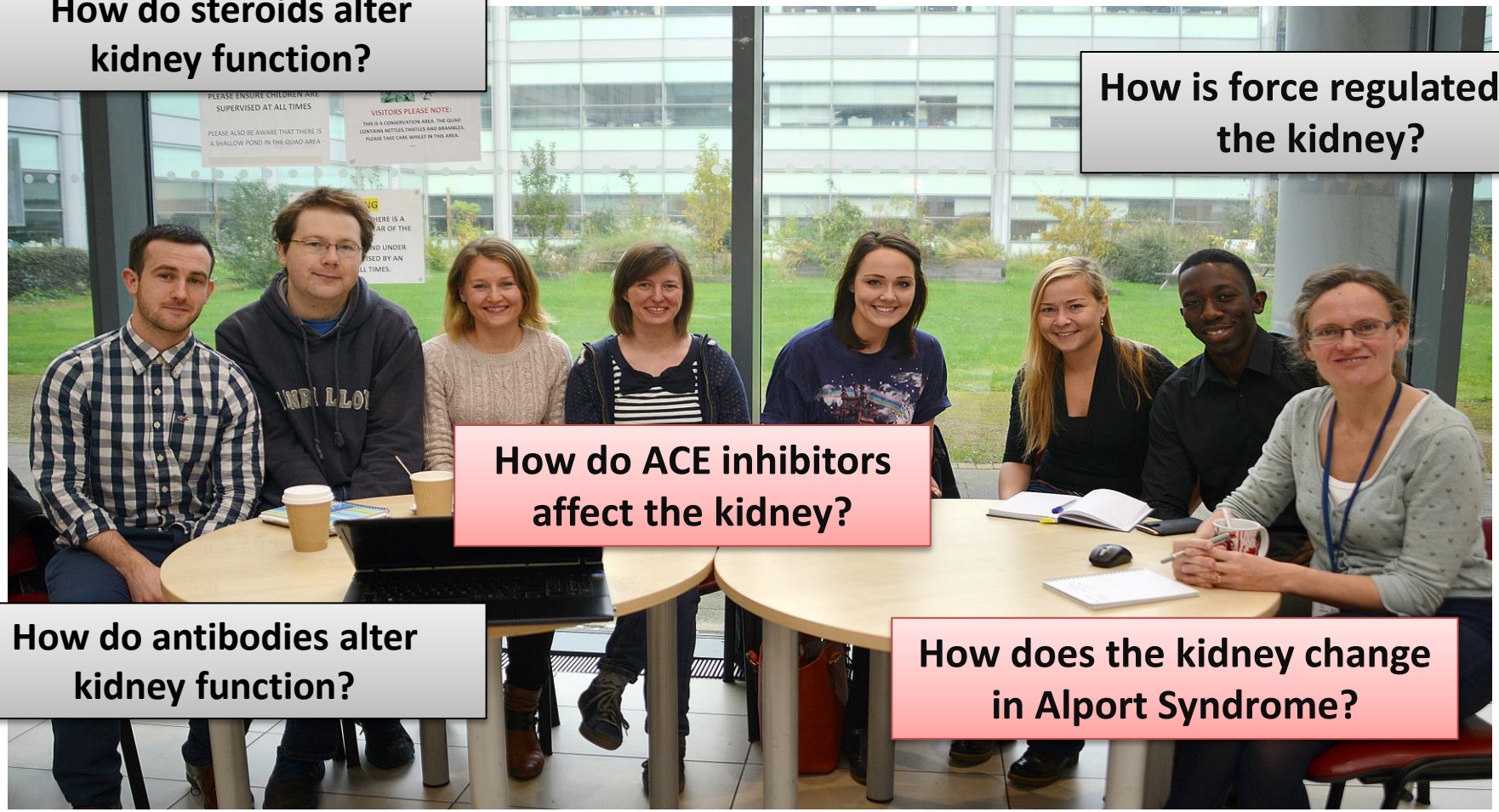
How does the filter stay healthy?

# My research team

How do the kidney filters stay healthy?

How do steroids alter kidney function?

How is force regulated in the kidney?




How do ACE inhibitors affect the kidney?

How do antibodies alter kidney function?

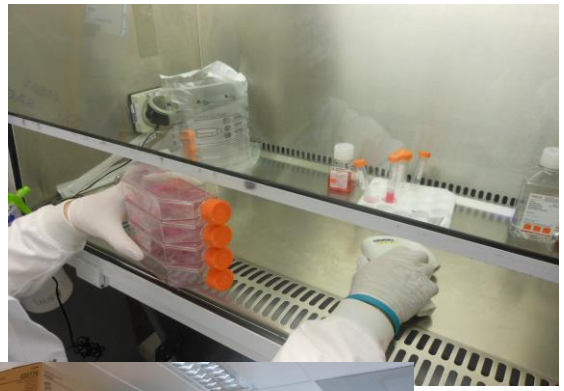
How does the kidney change in Alport Syndrome?

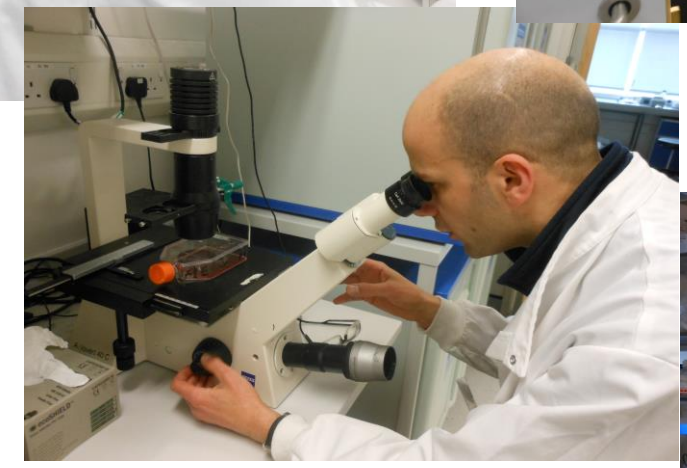
MRC Medical Research Council

Kids Kidney Research

Kidney Research UK
Funding research to save lives

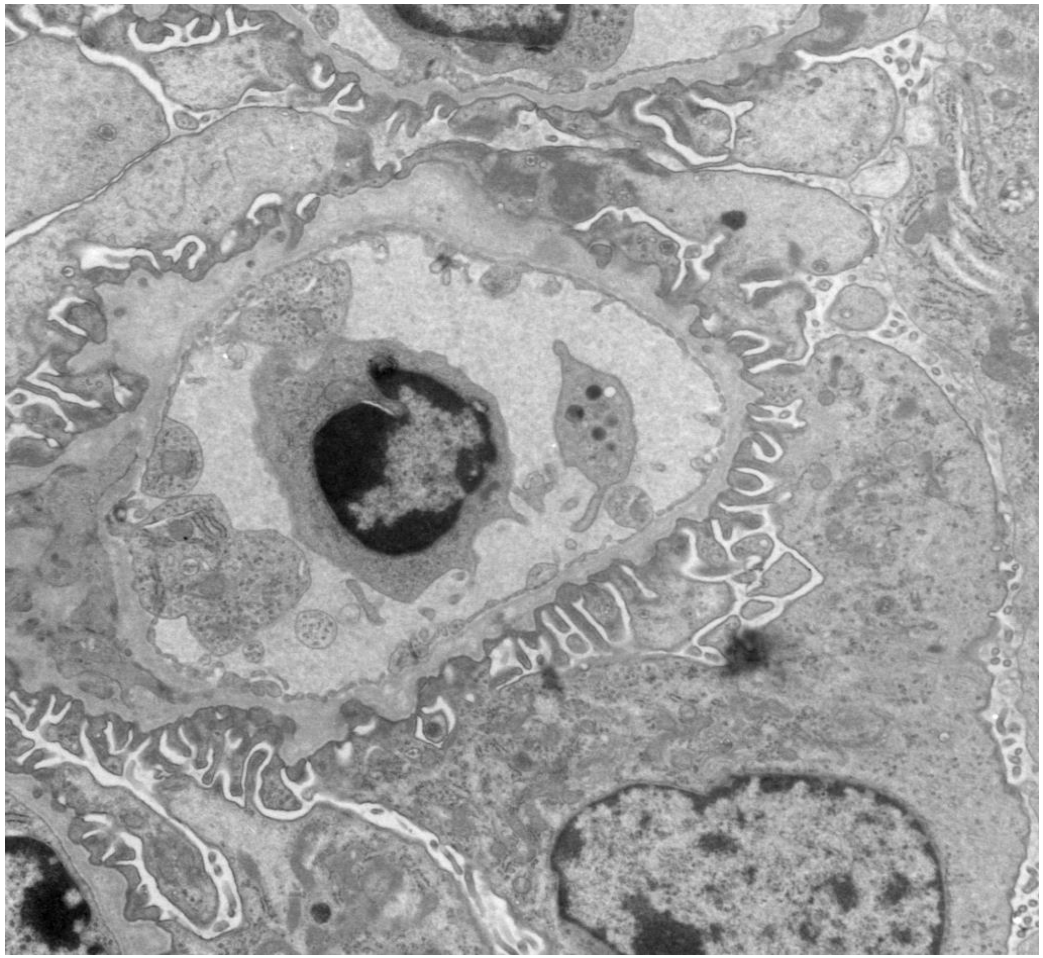
wellcome trust





# How do the Kidneys change in Alport syndrome?

## *Using the newest technology*



*Electron microscopy- human and mouse kidneys*

*Michael Randles*

*Jeff Miner*

*Washington University St Louis*

Kidney Research UK
Funding research to save lives

http://www.kidney-international.org meeting report


# The 2014 International Workshop on Alport Syndrome

Jeffrey H. Miner[1], Colin Baigent[2], Frances Flinter[3], Oliver Gross[4], Parminder Judge[2], Clifford E. Kashtan[5], Sharon Lagas[6], Judith Savige[7], Dave Blatt[8], Jie Ding[9], Daniel P. Gale[10], Julian P. Midgley[11], Sue Povey[12], Marco Prunotto[13], Daniel Renault[14], Jules Skelding[15], A. Neil Turner[16] and Susie Gear[15]

**Research tools**

- ✓ Research toolbox
- ✓ Developing additional animal models to better model human mutations
- ✓ Deriving cell lines from Alport mice and/or patients to gain a better understanding of how changes in the GBM cause changes to the overlying podocytes

**New therapies**

- ✓ Gene repair, gene replacement, protein replacement, or podocyte replacement therapies

# Why is this important?

- *Exciting times for medical research*
- We need to understand more….

- Can we repair the filters?
- Can we protect kidneys?

- *New treatments for kidney disease*

# Focus on research

- Breakout session
  - Why research is important for Alport Syndrome?
  - What types of research?
  - What more needs to be done?
  - How patients can get involved?
- Feedback this afternoon