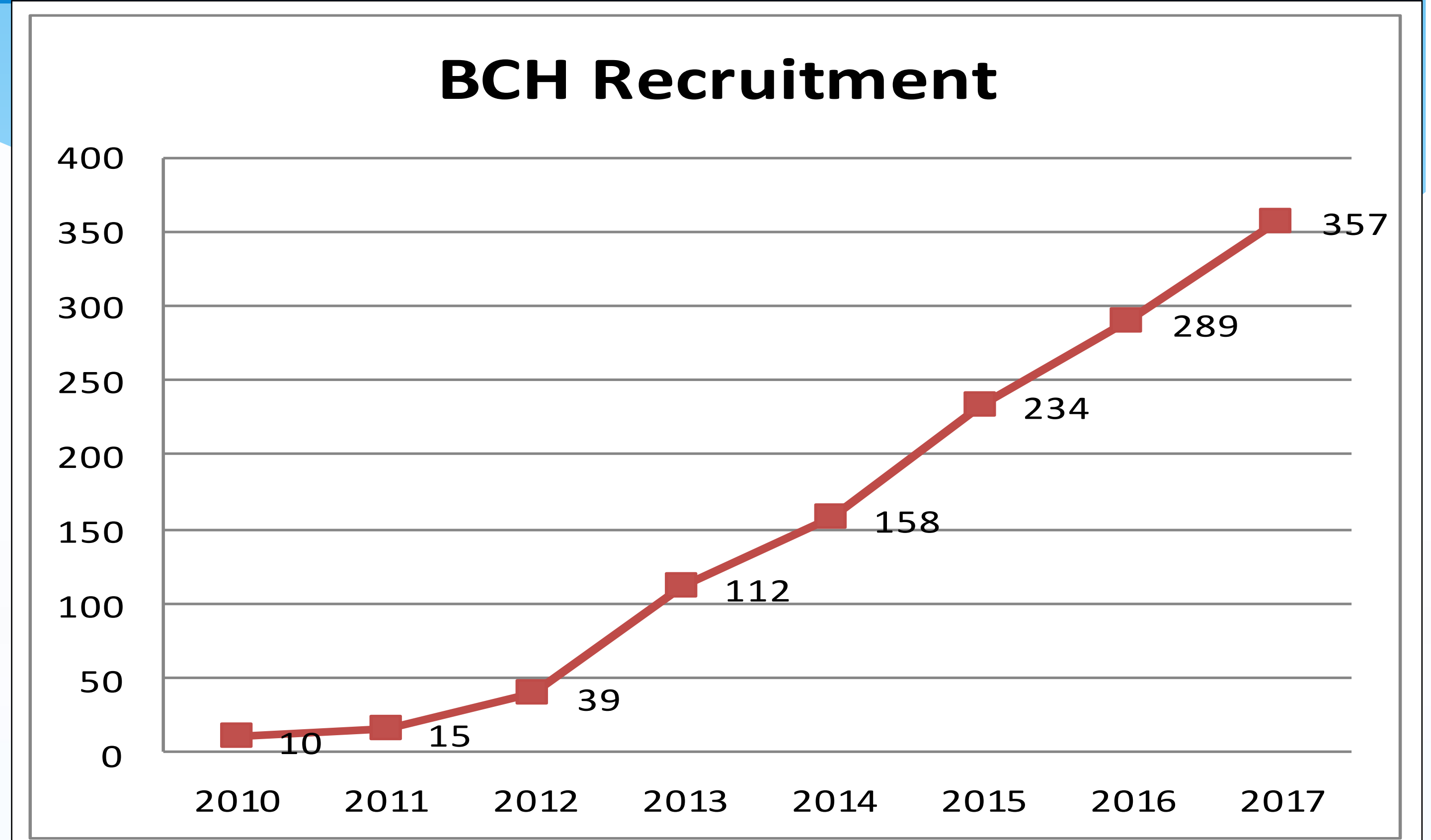


# Overview of Rare Renal Diseases at a UK Paediatric Renal Centre through the National Registry of Rare Kidney Diseases (RaDaR)

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## Background

Rare kidney disease can affect children in various ways, ranging from treatable disorders without long-term consequences to life-threatening conditions. Some kidney diseases are so rare that very little is known about them. Therefore, understanding and improving management of renal rare diseases is vital with starting point in collection of informative clinical data. However, individual hospitals don't see enough patients with these conditions to develop deep understanding of particular diseases. For this reason our centre is participating in RaDaR which is a UK Renal Association initiative designed to gather information from patients with rare kidney diseases. RaDaR recruitment began in 2010 and now covers more than 50 conditions. There are now over 12,500 recruits from 85 adult & paediatric hospitals across the UK. Birmingham Children's Hospital (BCH) is the leading paediatric recruiter in the UK.



## Objective

To describe the range of conditions and patient numbers recruited to RaDaR at BCH, which is a national tertiary renal referral hospital and the only national centre for combined liver kidney transplants in the UK.

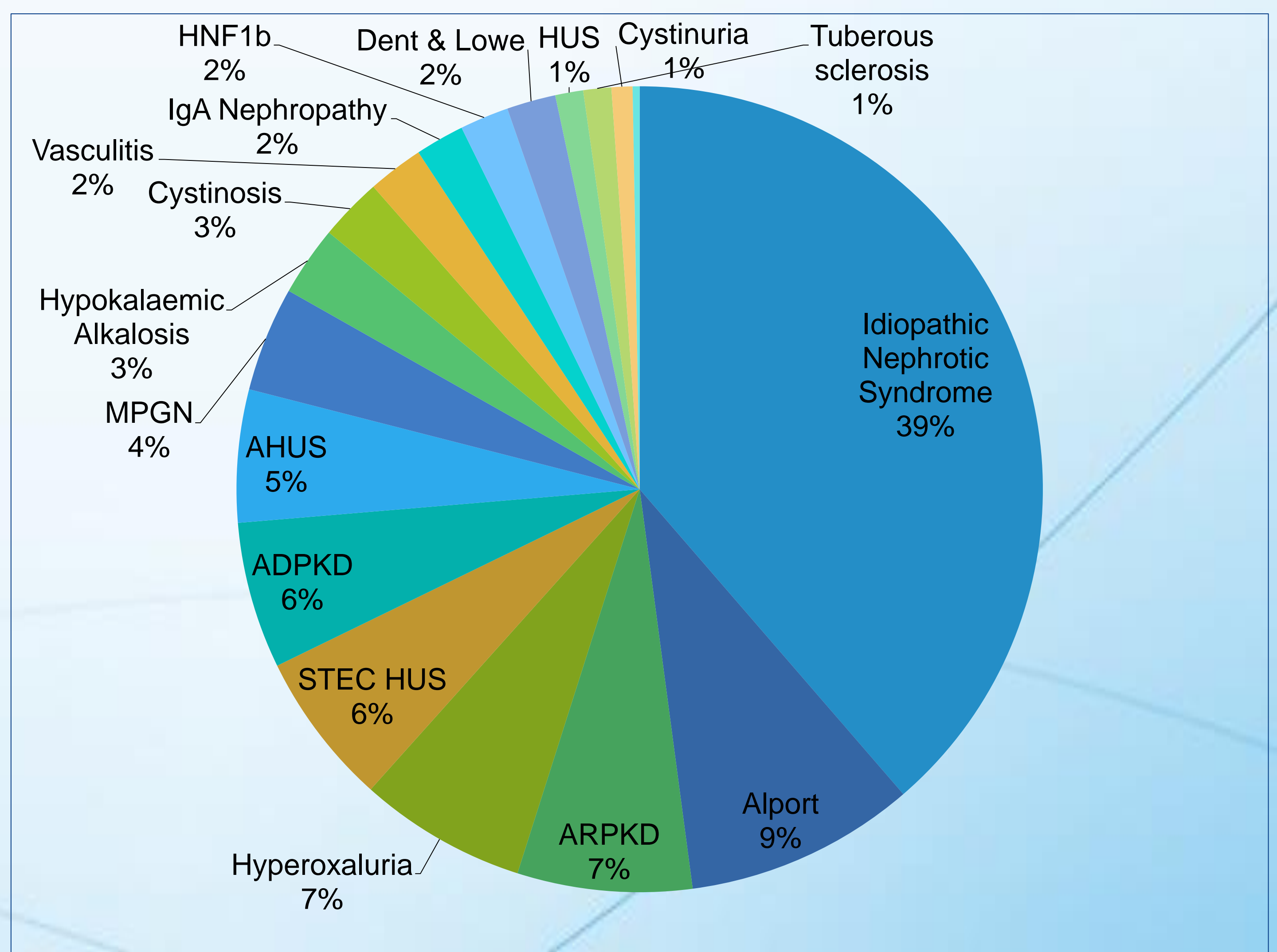
The primary purpose of using RaDaR at BCH is to give us a better understanding of how rare kidney conditions affect children. We also use it to identify eligible patients for family information days, tailor clinical interventions and access genetic testing services. In the future it is intended to be used as a tool in providing meaningful research which will lead to practical benefits, including better diagnosis and new treatments modalities.

## Results

A total of 357 patients have been consented at BCH to date. The age range is from birth to 16 years with mean of 4.9 years with male to female ratio of 55%:45%. The most common condition is Idiopathic Nephrotic Syndrome (n=138), followed by Alport Syndrome (n=33), ARPKD (n=25), Hyperoxaluria (n=24) and STEC HUS (n=22) and ADPKD (n=21). The other conditions with numbers of patients recruited so far include: aHUS (n=19); Cystinosis (n=9); Cystinuria (n=3); Dent & Lowe (n=7); HNF1b (n=7); Hypokalaemic Alkalosis (n=10); MPGN (n=15), Vasculitis (n=8) and Tuberosus sclerosis (n=4).

## Methods

The RaDaR dataset is defined by the UK Renal Registry in association with over 25 Rare Disease Groups, made up of experts in each eligible condition. Data fields include demographics, blood and urine results, medications, transplant and dialysis history, genetics and co-morbidities. Data is entered retrospectively from the patient's medical records following informed consent.



## Conclusions

RaDaR provides important epidemiology data based on the whole country population which is shared amongst the renal team to develop further research into rare kidney diseases and improve the quality of care for these patients. It also gives an opportunity to define the best treatment practices across the country in the future. Plans are in place to develop the infrastructure of the current RaDaR system to apply for all of the over 500 rare diseases treated at BCH. The new Stars Together Registry will be a key feature of BCH's forthcoming Rare Disease Centre, which will be the first of its kind in the UK when it opens in November 2017.

RaDaR is supported by:

