P050

P050 -The ADPKD Rare Diseases Group-2019 Update

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Introduction: The UK National Registry of Rare Kidney Diseases (RaDaR) has recruited over 21,000 patients across 33 disease groups since 2010. For autosomal dominant polycystic kidney disease (ADPKD), registry data is largely confined to those individuals who have developed end-stage renal disease as this diagnosis accounts for ~10% of individuals receiving renal replacement therapy including transplantation. As ADPKD is a multisystem disease and affects all ages, a comprehensive description of the natural history of the disease whilst renal function is preserved, from childhood, is required to guide clinical management, research and the development of novel therapies. This abstract describes the current activity of the ADPKD Clinical Study Group in 2019 now that recruitment in to ADPKD-RaDaR has exceeded 5700 patients.

Objectives: The ADPKD clinical study group is using RaDaR to comprehensively define the natural history of ADPKD and facilitate research and clinical trials recruitment. Using UK wide data collection via RaDaR and strategic interaction with the PKD Charity, a large prospective cohort of patients is being developed that may also be approached for future research studies. The main objectives are to develop best practice guidelines, to provide better evidence-based patient information, assess the impact of new therapies and support research, in collaboration with international groups, into basic and clinical science, disease progression and clinical trials.

Methods: Patients are recruited to RaDaR according to current study protocols employed for all the rare renal diseases (www.RareRenal.org). Inclusion criteria for ADPKD include a clinical or molecular diagnosis according to established criteria. A predefined and comprehensive dataset developed with the UK Renal Registry and international collaborators and disease experts is employed including demographics, routine laboratory measurements, imaging, family history, genetics and co-morbidities.

Results: 5717 ADPKD patients from 86 UK hospitals have been enrolled to January 2019 from the start of recruitment in January 2016. Data collection via Patient View and data linkages established as part of RaDaR to include information from HES and ONS, are being developed along new consenting processes that will allow direct patient contact by RaDaR. Funding for the ANCHOR study to generate an enriched nested cohort has been secured and supports research posts to ensure comprehensive data collection from large ADPKD centers. In addition, results from the 100,000 Genomes Project will also be included to bring the number of patients and families with molecular confirmation to ~1500. Preliminary analyses are in progress and will be presented.

Conclusion: The ADPKD RaDaR registry has the potential to become one of the largest observational studies of ADPKD. It will serve as a platform to guide further epidemiological, clinical, genetic and laboratory research; to facilitate the rapid identification of individuals suitable for interventional studies and enable safety and effectiveness studies for new treatments such as Tolvaptan in a real world setting. Initial priorities will be to improve data capture and the recruitment of a representative cohort of patients at all stages of disease. Longer term goals will include the establishment of a disease specific biobank.