Dec 2015 Issue 5

FH PAEDIATRIC REGISTER NEWSLETTER

THE NEWSLETTER FOR CLINICIANS WHO ARE REGISTERED WITH THE FAMILIAL HYPERCHOLESTEROLAEMIA PAEDIATRIC REGISTER WEBSITE

Interactive map — coming soon!

We are developing an interactive UK map which will display the **names of clinicians treating children with FH, along with their hospital contact details.** The map will display on the register homepage, available for view by interested clinicians clicking on the website. If you wish to **opt-out of having your name/ hospital contact details** displayed on the map, please contact us by email: fh@rcplondon.ac.uk **by 31st Dec 2015.**

Publication of Register data

Preliminary data from the FH Paediatric Register has been submitted as a manuscript to the Archives of Disease in Childhood. It is currently undergoing a second round of statistical revisions and we hope that it will be accepted for publication in early 2016.

Genetic Testing—important news

Unfortunately, the free genetic testing facility for FH is no longer available at UCL. From now on, samples should be sent to one of the accredited diagnostic laboratories using next generation sequencing. Two such labs are in Bristol and GOSH:

Contact at Bristol: Maggie Williams
Maggie.Williams@nbt.nhs.uk

Contact at Great Ormond Street: Alison Taylor-Beadling
Alison.Taylor-Beadling@gosh.nhs.uk

LATEST FIGURES

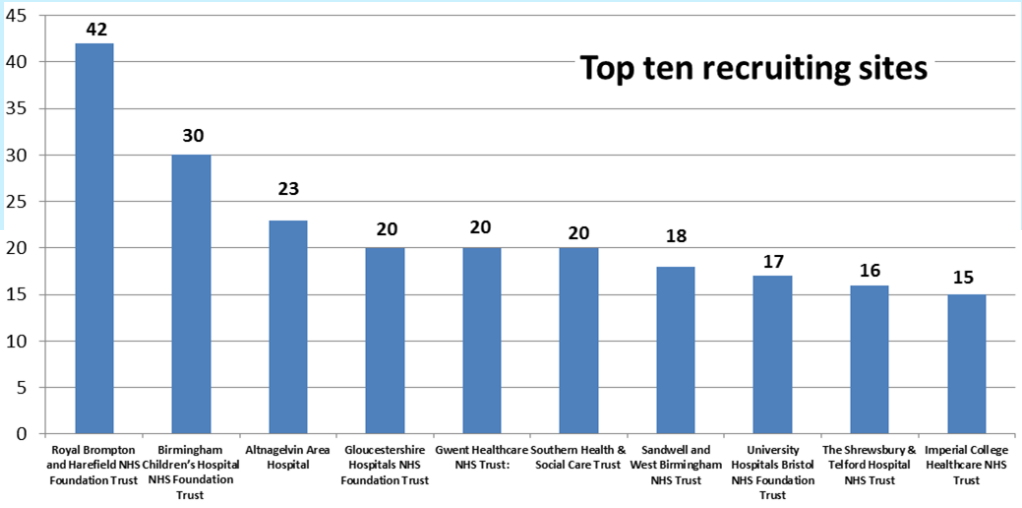
There are currently 326 children enrolled, compared with 250 at the start of 2015.

To date: 61 NHS Trusts and 86 clinicians **have registered on the system.** Four new sites have registered this year: Royal Bournemouth, Norfolk & Norwich University Hospital, Countess of Chester Hospital and Doncaster Royal Infirmary.

In total, 31 Hospitals **have entered patient data into the system**, with the Royal Brompton registering the highest number (42 patients).

Since the new introduction of the automated annual update reminder, we have seen an increase in follow-up data entered. We now have **248 annual updates in the system,** great news!

Remember, clinicians can continue to enter patient data even once the patient turns 18 years old, so long as they remain under that clinician's care.



See page 2 of the newsletter for some recent articles of interest...

Royal College of Physicians

Please click here for more information about registering: FH Paediatric Register

RCPCH Royal College of Paediatrics and Child Health

Articles of Interest.....

Luirink IK[1], Hutten BA, Wiegman A. **Optimizing Treatment of Familial Hypercholesterolemia in Children and Adolescents.** Curr Cardiol Rep. 2015 Sep;17(9):629. doi: 10.1007/s11886-015-0629-1.

Abstract, Cardiovascular disease (CVD) is still the most prominent cause of death and morbidity in the world, and one of the major risk factors for developing CVD is hypercholesterolemia. Familial hypercholesterolemia (FH) is a dominantly inherited disorder characterized by markedly elevated plasma low-density lipoprotein cholesterol and premature coronary heart disease. Currently, several treatment options are available for children with FH. Lifestyle adjustments are the first step in treatment. If this is not sufficient, statins are the preferred initial pharmacological therapy and they have been proven effective and safe. However, treatment goals are often not achieved and, hence, there is a need for novel treatment options. Currently, several options are being studied in adults and first results are promising. However, studies in children are still to be awaited.

Braamskamp MJ[1], Kusters DM, Avis HJ, Smets EM, Wijburg FA, Kastelein JJ, Wiegman A, Hutten BA. **Long-term statin treatment in children with familial hypercholesterolemia: more insight into tolerability and adherence.** Paediatr Drugs. 2015 Apr;17(2):159-66. doi: 10.1007/s40272-014-0116-y.

Abstract, BACKGROUND: Statins are currently the preferred pharmacological therapy in individuals with familial hypercholesterolemia (FH) with the aim to prevent premature atherosclerosis. In adults, these agents have been proven to be safe and well tolerated; however, non-adherence is a significant clinical issue.

OBJECTIVES: In this study, we evaluated tolerability and adherence to statin therapy in young adult FH patients 10 years after this was initiated in their childhood. **METHODS:** A questionnaire including items on medical history, adherence and reasons for discontinuation was sent to 214 young adult FH patients that initiated statin therapy at least 10 years ago. Tolerability was defined as 100% minus the percentage of patients that discontinued statin therapy due to side effects. Adherence was defined as the extent to which patients took their medication as prescribed by their physician. We labelled patients adherent if they took 80% or more of their pills in the month preceding our assessment. **RESULTS:** Follow-up was successful in 205 (95.8%) subjects (age 18-30 years). A history of side effects was reported by 40 (19.5%) of the patients, and mainly consisted of muscle complaints and gastrointestinal symptoms. Three patients (1.5%) discontinued statin therapy because of side effects. Rhadbomyolysis or other serious adverse events were not reported. In fact, 169 (82.4%) of 205 patients remained on statin treatment and 78.7% (148 out of 188) were adherent. None of the patient characteristics were significantly associated with adherence. **CONCLUSIONS:** Individuals with FH who started statin therapy in childhood demonstrated good adherence during ten years of treatment. Furthermore, statin therapy was well tolerated; only a small minority discontinued therapy because of side effects and the side effects that were reported were mild in nature.

Gelissen IC[1], Nguyen HL, Tiao DK, Ayoub R, Aslani P, Moles R. **Statin use in Australian children: a retrospective audit of four pediatric hospitals.** Paediatr Drugs. 2014 Oct;16(5):417-23. doi: 10.1007/s40272-014-0087-z.

Abstract AIM: The aim of this study was to perform an audit of the use of statins in Australian pediatric hospitals. **METHODS:** A retrospective audit of patients prescribed statins during a visit to a pediatric hospital, as in- or outpatients, was performed in four majorchildren's hospitals in three Australian states. Patients were identified through hospital pharmacy dispensing records. Statin use (dose, type) as well as medical history was recorded. **RESULTS:** A total of 157 patients under the age of 18 were included in the audit. The most common reasons for being prescribed a statin included history of organ transplantation, renal disease and familial hypercholesterolemia (FH). Four statins were prescribed: atorvastatin (n = 77), pravastatin (n = 45), simvastatin (n = 25) and rosuvastatin (n = 10). All statins, apart from rosuvastatin, were used in very young children (1-7 years old). Polypharmacy was common in these patients, including combinations with calcineurin inhibitors and diltiazem, which can increase systemic statin exposure. A small number of very young children were prescribed high doses of statin, based on mg/kg dosing. **CONCLUSIONS:** Statins were prescribed to children younger than suggested by current Australian guidelines, with atorvastatin being the preferred statin of choice. Long-term safety studies on the use of statins in children have only included FH patients so far, who are generally healthy besides their raised lipid levels. Further long-term safety studies are needed to include the more vulnerable transplant and renal patients, identified in this audit as being prescribed statins. This can help formulate guidelines for the safest possible use of this class of drugs in the pediatric setting, including the possibility of weight-based recommendations for younger children