Public Health England

Protecting and improving the nation's health

Familial Hypercholesterolaemia: London Paediatric Services Needs Assessment

V0.2

About Public Health England

Public Health England exists to protect and improve the nation's health and wellbeing, and reduce health inequalities. We do this through world-leading science, research, knowledge and intelligence, advocacy, partnerships and the delivery of specialist public health services. We are an executive agency of the Department of Health and Social Care, and a distinct delivery organisation with operational autonomy. We provide government, local government, the NHS, Parliament, industry and the public with evidence-based professional, scientific and delivery expertise and support.

Public Health England
Wellington House
133-155 Waterloo Road
London SE1 8UG
Tel: 020 7654 8000

OGL

You may re-use this information (excluding logos) free of charge in any format or medium, under the terms of the Open Government Licence v3.0. To view this licence, visit OGL. Where we have identified any third party copyright information you will need to obtain permission from the copyright holders concerned.

Corporate member of Plain English Campaign
Committed to clearer communication
339

PHE supports the UN Sustainable Development Goals

SUSTAINABLE DEVELOPMENT GOALS

Contents

Foreword

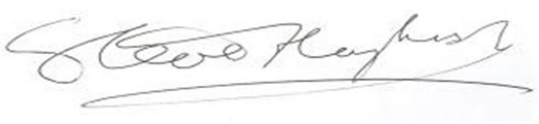
Thirty four million people globally are estimated to be affected by familial hypercholesterolaemia (FH) a common inherited genetic condition that affects the body's ability to manage cholesterol, causing low-density lipoprotein (LDL) or 'bad cholesterol' levels in the bloodstream to be raised from birth. Children with FH have around twice the normal level of LDL-C cholesterol in their bloodstream and NICE guidelines recommend that they should be considered for lipid-lowering therapy by the age of 10 years. The vast majority of individuals with FH are currently undiagnosed and unaware they have the condition. Concerted efforts to address this include the NHS Long Term Plan ambition to improve cardiovascular health outcomes for those with FH by increasing the level of FH cases detected to 25% by 2023.

Information gaps currently exist regarding what proportion of children and young people with FH are accessing services, the needs of this group, and the models of care being delivered. The 2010 national FH audit (1) provided a useful baseline, indicating that 59% (n=72) of lipid clinics in England were delivering an FH service to children under 16 years of age. At that time, 26% of lipid clinics were identifed as delivering a tailored child-focussed service for the management and treatment of FH.

This needs assessment, conducted during autumn 2019 - spring 2021, and led by PHE in partnership with the London Strategic Cardiac Clinical Network, the Institute of Cardiovascular Science at University College London, and the Pan-London FH Steering Group, identified 13 NHS led services providing paediatric FH care within the M25. Data from the project suggests that at best, around 7% of the estimated population of children and young people with FH in London are currently under the care of specialist lipid services, indicating a considerable level of potential unmet need. This project also provides valuable insights on the consideration and prescribing of statins, and the different child-focussed models of care that can be used to inform the commissioning of future service provision.

We would like to take this opportunity to thank members of the Pan-London Familial Hypercholesterolaemia Steering Group for their input to this work during an exceptionally busy time, and the clinical service leads who participated in the data collection between autumn 2019 - spring 2021 during a period of unprecedented impact for health and care services from the COVID-19 pandemic.

I welcome the publication of this report and hope you find the insights useful.



Professor Steve Humphries
Chair, Pan-London Familial Hypercholesterolaemia Steering Group

Key findings

- This needs assessment has established that London currently has 14 paediatric FH services, 13 of which are NHS led. These services were found to be providing care for ~562 children and young people.
- Over 80% of services reported considering the use of a statin by the age of 10 years, to help lower lipid levels.
- All participating services had met some core paediatric quality markers from national guidance, including that 78% were able to provide access to a paediatric professional with lipids experience.
- Less than half of participating clinics (40%) were paediatrician led, with the remainder under lipidologist or metabolic clinical leadership. For clinics not paediatrician led, arrangements were in place to enable paediatric partnership and input.
- 100% of services provided a structured annual review for children under their care.
- Less than half of London paediatric FH services (44%) currently provide transition support to children and young people within their care.
- This study identified that approximately 7% of London's estimated paediatric FH population are known to services. Taking this as a baseline, the NHS Long Term Plan ambition to identify 25% of cases by 2023 could result in a three-fold increase in demand for paediatric FH services within the M25.
- A number of commissioning and capacity factors were identified during this work that would benefit further consideration.

1. Introduction

Familial hypercholesterolaemia (FH) is common, affecting an estimated 1 in 250 people (2) (3) (4). FH requires only one parent to be a carrier of the disease-associated mutation, with on average, 50% of their children then inheriting the disease. There is a significant life course health toll for individuals who have FH, since they experience elevated concentrations of LDL-cholesterol from birth which results in the premature development of arterial atherosclerosis (arteries becoming clogged with fatty substances). This can be documented in the carotid arteries of those with FH by the age of 11 years. The risk of death from cardiovascular disease is up to 80 times higher for people with FH between the ages of 20-39 years, than for those in the general population (5). In the UK it is estimated that 56,000 children and young people under 18 years of age are likely to have FH, and based on data from the UK Paediatric FH Register, it can be estimated that in 2019 less than 2% of these were known to UK clinic services.

Familial Hypercholesterolaemia: London Paediatric Services Needs Assessment

There is growing evidence that interventions delivered during early childhood such as increasing physical activity, advice on healthy eating and a low cholesterol diet and considering the prescribing of a statin by 10 years of age, can help prevent early onset of FH related cardiovascular disease and premature death. Due to this, the 2017 update of NICE FH guidance CG71 recommended that all children with FH should have a DNA test to confirm their diagnosis and be considered for statin treatment by 10 years of age, and for this to take place in a child-friendly setting. The NHS Long Term Plan includes an ambition for services to identify 25% of the population with FH within England by 2023. A 2018 survey of the 24 London adult FH clinics delivered insights for the commissioning and planning for services. Building on this, during 2019-21 a paediatric-focussed needs assessment was conducted, to estimate services' capacity and capability for meeting the needs of children and young people with heterozygous FH (those where the FH gene has been inherited from one parent) in London. Based on a prevalance of FH of 1 in 250, and with current population estimates, it is predicted there are approximately 8,500 young people aged from 0-18 years with FH living in London.

Led by Public Health England, working with the London Strategic Cardiac Clinical Network, Professor Steve Humphries from the Institute of Cardiovascular Science, University College London, and the Pan-London Familial Hypercholesterolaemia (FH) Steering Group, this needs assessment has gathered intelligence on current and future service capacity within London, and estimates of future population needs, to aid planning and support for London services. Although this is a London based study, the findings may be of interest for leads throughout England who are supporting the roll out of cascade testing to help meet the 25% FH detection ambition in the NHS Long Term Plan. And findings may also be of interest to those considering paediatric FH service review and planning across the UK.

2. Project aims

Aims and objectives

This 2019 - 2021 needs assessment aimed to establish a baseline of current paediatric FH provision for heterozygous cases, and the contribution that these services could help make to meeting the health needs of children and young people with heterozygous FH within London. The project also aimed to evaluate the impact of NICE guidance and the Heart UK Statement of Care on paediatric care pathways and treatment models within with M25 area.

Project objectives

- Establish a baseline of the number of children and young people with FH who are known to lipid services in London
- Identify current London paediatric FH services, including care and treatment models
- Gather intelligence on key issues for services on meeting the health needs of children and young people with FH in London
- Use a health impact assessment approach to estimate the impact of both the NICE FH guideline CG71 and the Heart UK Statement of Care on pathways and treatment for children and young people with FH within London

Project scope

- Services working with children and young people aged 18 and under
- Care of those with a diagnosis of heterozygous FH.
- Estimate the numbers of clinical staff providing care and support to children and young people with FH
- The geographical remit was services within the M25

Outside of scope

The project omitted paediatric homozygous FH cases (where two faulty genes are inherited - from both parents - rather than one) from the scope of this needs assessment, due to there being a lower population prevalence for this than for heterozygous FH cases in the UK, and also the complexity of needs and care pathways involved in the treatment for this group.

Further details on guidance for homozygous FH care - including for children and young people - can be found on the Heart UK website

3. Policy context

NICE FH guidance CG71 recommends that all children with familial hypercholesterolaemia (FH) should be identified before the age of 10 years, to enable delivery of lifestyle advice and interventions in early childhood including the consideration of statin treatment, to help reduce the substantial cardiovascular risk for those with FH. The NHS Long-Term Plan identified cardiovascular disease as the single greatest area where the NHS can save lives over the next 10 years. By expanding genetic testing for FH the 10-year ambition was to improve the identification of those living with FH to at least 25% by 2023.
In 2017, high levels of LDL cholesterol were responsible for 50.3% of deaths from ischemic heart disease (IHD) and 11.7% of deaths from stroke across both genders and all ages in London. In addition, 50.2% and 20.7% of the years lived with disability following IHD or a stroke were attributed to high LDL cholesterol. The Pan-London FH Steering Group was established to address these high levels of morbidity and mortality, in harmony with the NHS LTP ambition to improve the identification of all those living with FH to at least 25% by 2023.
In 2019 the Pan-London FH Steering Group completed an adult FH health needs assessment, revealing a challenge for London lipid clinics in submitting genetic samples to regional Genomic Laboratory Hubs to confirm a diagnosis of FH. This was due todelays in centralised funding for FH genetic testing. Pan-London genetic counselling services were also found to be limited due to a lack of trained health care professionals including specialist FH nurses to carry out this work. Additionally, the assessment identified a lack of access to dieticians and lifestyle modification education resources, which impacted lipid clinic capacity.

The 2017 update of the NICE FH guideline CG71 recommended that children between the ages of 0-10 years who are at risk because of one affected parent with FH, should be offered a DNA test at the earliest opportunity. All children and young people diagnosed withor being investigated for FH should be offered a referral to a specialist with expertise in the treatment of FH in children and young people. This should be delivered in a young person-focused setting.
The Pan-London FH Steering Group partnered with Public Health England (PHE) on this current health needs assessment to establish a baseline understanding of current service capacity within London, in light of an expected increase in referrals anticipated from the NHS Long Term Plan FH ambition to improve the identification, care and health outcomes of those with FH.
A two year pilot of a child-parent screening programme was underway at the time of writing this report, taking place across five Academic Health Science Networks (AHSNs) to support delivery of the NHS Long Term Plan FH identification ambition. The pilot will complete in 2023, with an aim to roll out a subsequent child-parent screening service across all 15 AHSNs nationally.

4. Epidemiology

Population overview

Approximately 56,000 children in the UK are likely to have FH, but prior to this needs assessment only 650 (2%) of these individuals were known to be under the care of an NHS service, based on data from the UK Paediatric FH Register (6) . For those with FH, the risk of death from CVD is up to 80 times higher between the ages of 20-39 years, compared to the general population (7). If FH is identified early and interventions to manage risk factors begin in early childhood, for example dietary advice and assessment for statin therapy, the CVD risk for individuals with FH can be reduced to a comparable level to the general population. In 2010 the National Audit of FH services identified 147 paediatric cases that were under the care of services at that time.

Needs assessment 2021: Children and young people receiving care in London

This 2021 needs assessment identified that approximately 562 children with FH were currently under the care of NHS-delivered paediatric FH services in London. Up to 340 of these cases are likely to have not been included in previous service uptake estimates.

At the time of conducting this project, there were an estimated 2,127,524 children and young people in London aged between 0 -18 years (8). Table 1 illustrates that when taking a population prevalence for FH of approximately 1 in 250 people, it is estimated to be around 8,500 children and young people with FH in London.
During 2019 - 2021, this project was able to identify that London services provided care for approximately 7% of the estimated London-based paediatric FH population.

Table 1: Children and young people with FH in London

	General population aged 0-18 years*	Estimated FH population 0-18 years
National UK	14,856,441	56,000
London	2,127,524	8,510

*From Office for National Statistics mid-2019 population estimates

Impact of paediatric FH care - UK data

The increasing levels of childhood obesity in the UK and the morbidity risks that accompany this are recognised as a national priority for action. A previous review of patient data from the UK Paediatric FH Register (9) found that children with FH who were under the care of lipid services had a significantly lower prevalence of obesity at 11%, compared to 22.1% within the general UK population of children and young people. Paediatric FH services include the delivery of advice and support on healthy diet and physical activity from early childhood. Despite children with FH having a genetic propensity for up-to-double the level of blood cholesterol compared to their non-FH siblings, findings from the previous UK Paediatric FH Register analysis identified that paediatric FH patients under the care of lipid clinics had a significantly lower prevalence of obesity than their peers within the general UK population.

Building on the earlier analysis, this needs assessment examined an anonymised UK data extract from the PASS database, an IT system that is used within the NHS in Wales, Northern Ireland and parts of England as a register of patients and families with FH under the care of services, to help the coordination of cascade testing.
Table 2 below illustrates the results of this analysis that was undertaken on an age-stratified UK cohort of children with FH aged 4-5 years and aged 10-11 years, who were receiving care from lipid services in either Wales, Northern Ireland or England.
This UK data was compared with age-stratified UK population data for children collected during 2019-20 via the National Child Measurement Programme (10).

This analysis of UK level data illustrates that although the prevalence of being overweight or obese was comparable for the FH and general population groups at school reception stage (4-5 years of age), there was a marked difference between these groups at school year 6 (age 10-11 years). UK data for children with FH within school year 6 indicated the prevalence for being overweight or obese was over 50% lower than for age-stratified peers from the general UK school population.

This difference could indicate a benefit from delivery of healthy lifestyle and diet advice. It could also indicate - if a lower BMI were to be considered as a proxy measure for a reduced CVD risk - that for children and young people with FH, a preventative health impact can be realised from the delivery of effective care via a paediatric FH service. For reference, the PASS database has varying degrees of uptake by NHS services across Wales, Northern Ireland and England.

Table 2: UK obesity prevalence in children with FH under the care of paediatric FH services, compared to the general population (age stratified comparison)

	General population (NCMP)	Paediatric FH population (PASS)
Reception (aged 4-5 years)		
Combined prevalence overweight and obese (male and female)	23.0	30.8
Year 6 (aged 10-11 years)		
Combined prevalence overweight and obese (male and female)	36.2	16

National Child Measurement Programme data collection includes children in Reception (aged 4-5 years) and Year 6 (aged 10-11 years) within mainstream state-maintained schools in England. Data analysed from 2019-20 school year

Paediatric FH Register

While it is clear that statin treatment in adults has a good safety record, there are no long-term studies of safety in children, with the longest studies usually not extending past two years. To date these have all suggested statins are safe in children with regard to growth rates, progression through puberty, and lack of any major short-term side effects. In 2008 NICE CG71 strongly recommended that a register be established to follow treated FH children as they become adults, and to confirm the long-term safety of these agents. The UK FH Children's Register was established in 2012.
It aims to:

- Monitor safety of current and new treatments by collecting data on growth rate, puberty, liver function, muscle pain and occurrence of cancer, plus unforeseen risks or abnormalities
- Provide comparative audit data on care to the UK centres where children with FH are seen.

A lead from the UK FH Children's Register was part of the steering group for this London-based needs assessment. This study identified that 30% of participants were currently taking part in the Register. Feedback from those not currently participating in the Register highlighted the administrative challenge for busy clinicians - many without nurse or administration support - to enable them to participate and load both initial patient data plus subsequent updates to the Register.

5. Service delivery

The needs assessment identified 13 NHS delivered services within the M25 that provide care to children and young people with FH. These 13 services are included in analysis within this report and are listed in table 3 below. Their locations can be viewed via this map. Prior to this needs assessment project, it was estimated there were up to 5 paediatric FH services working with children and young people within London.

Table 3: London paediatric familial hypercholesterolaemia service locations

London paediatric FH services	ICS
Family Lipid Clinic, Chelsea & Westminster Hospital	NW London ICS
Hammersmith Hospital	
Croydon Health Services NHS Trust	SW London ICS
Kingston Hospital NHS Trust and Lipid Clinic	
Lipid Clinic, Queen Mary's Hospital (part of St Georges' Hospital NHS Trust)	
St Helier Hospital Lipid Clinic	
Epsom Hospital Lipid Clinic	
Paediatric lipid clinic at St George's Hospital	
Evelina London Children's Hospital Lipid Clinic; Guy's & St Thomas' Hospitals	SE London ICS
Harefield Hospital	
Great Ormond Street Hospital, London	North Central London ICS
The Royal Free London Paediatric FH clinic	
London Medical (Private)* *This service did not participate in the 2019-2021 surveys, but did confirm that they provide a paediatric service only to a very small number of patients	

Figure 1: Integrated Care Systems (ICS) map London

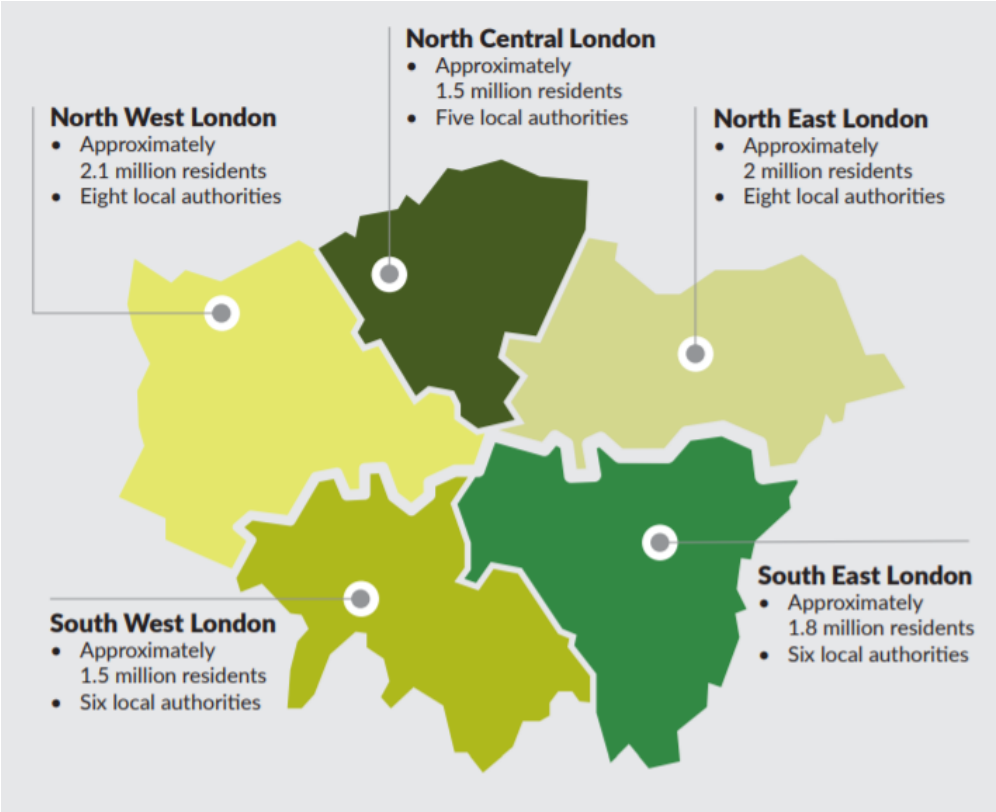


Image reproduced from The King's Fund

At the time of data collection, the 13 London based NHS services in table 3 above delivered 48 paediatric clinic sessions per quarter and provided care for ~562 children and young people with FH. The number of sessions provided varied between services ranging from 1 session per quarter, up to 14 sessions quarterly.
This project attempted to quantify the number of staff hours per month involved in delivery of London paediatric FH services, but it was found challenging to collate as staff were frequently part of a broader team. For example, a paediatric clinic jointly provided with an adult lipid service, utilising the same workforce.

For referrals, two thirds of participating services reported receiving their paediatric cases via cascade testing, and 75% of children and young people had waited less than 3 months to access a paediatric FH clinic, with 100% waiting less than 6 months.

Participating clinics confirmed that 40% were paediatrician led, with leadership for the remainder from an adult lipidologist or metabolic lead. For clinics not paediatrician led, most had made steps to address this via joint delivery or other arrangements with paediatric specialists in their organisation. An example included a clinic jointly run between a lipidologist and a paediatrician. Overall, 78% of clinics provided access to a paediatric healthcare professional with expertise in FH and lipids, and 58% of clinics were delivered in a paediatric outpatient setting.

With 22% of services reporting facing issues accessing paediatric FH workforce, further pan-London consideration of how best to address these workforce issues would be beneficial.
Services were asked how many additional children and young people they would be able to see once the roll out of cascade testing increases. For the nine services that responded to this question, an estimated 26–70 additional patients could be seen per month across these services. This would equate to an additional 60-210 children and young people with FH who could access care in London per quarter.
It should be noted these service capacity projections may subsequently be impacted by COVID-19 health service recovery.

Multi-disciplinary team

Whilst all participating FH services confirmed access to paediatric phlebotomy, limitations were identified for other aspects of a multi-disciplinary team. For example, only one third of participating services had access to a paediatric FH nurse and a third of services did not have direct access to a genetic counsellor. Of concern was the report from one service that they did not have direct access to dietetic expertise.
A gap was also identified for physical activity expertise, with only 44% of services having access, although 100% of services did report providing dietary and physical activity advice from early childhood. With strong evidence of the beneficial contribution that physical activity expertise can make to help reduce risk factors for cardiovascular disease, this warrants further consideration.

6. Care models

Statin treatment

Previous analysis in 2017 of data from the Paediatric FH Register (10) indicated that 53% of children at that time were prescribed statins. This varied significantly though by age group:

- 0% in those under 5 years
- 17% in those between 5 and 10 years
- 57% in those between 10 and 15 years
- 73% in those over 15 years.

In the 2017 analysis, for children over 10 years of age who were not on statin treatment, 36% were found to have had a family history of early coronary heart disease (CHD), and 89.9% had LDL-C over 3.5mmol/l, suggesting that in 2017 there were still a significant proportion of children over age 10 years who were at high risk of early CHD, and where statin therapy should have been actively considered.

Familial Hypercholesterolaemia: London Paediatric Services Needs Assessment

In this London 2019-21 needs assessment, 88% of services were consistent with NICE guidance and the Heart UK statement of care recommendations, for considering a statin by 10 years of age for patients under their care. This is a 35% improvement on the previous 2017 UK Paediatric FH Register data.
In 2019-21 not all London FH services had a protocol for statin treatment, but where this was absent, participants confirmed the approach was based on the individualised needs of the child, and agreed in discussion with the family. Some services reported considering a statin after 8 years of age, dependent on the needs of the child, based on the updated NICE CG71 recommendation to consider a statin by age 10 years.

Annual review

The HEART UK Statement of Care details the importance of an annual review for children and young people under the care of FH services. This needs assessment identified that 100% of participating London services were providing an annual review, in line with guidance. Annual reviews were reported to include key components from the national guidance, including height and weight measurements, checks on medication adherence and side effects, the provision of dietary advice, and liver function test and lipid profile.

This needs assessment identified that London-based services had exceeded the previous 2010 national FH audit baseline for the delivery of annual reviews. In 2010 87.8% (n=129) of children and young people had received an annual review, with 1.4% (n=2) not being offered a review. For London in 2019-21, 100% of participating services provided an annual review to children and young people under their care.

Child friendly service

NICE CG71 recommends a child friendly approach for services working with children and young people with FH that is consistent with the previous National Strategic Framework for Children and Young People; Core Standard 3. This includes:
listening and respecting children, young people and parents; information regarding support and treatment; seeking consent; improving access to services that are integrated around children and young people's needs; quality and safety of care; and planning, commissioning and staff development
The Heart UK Statement of Care details key components of a 'child friendly approach' for the delivery of FH care. Clinics that participated in this needs assessment reported meeting core aspects for a child friendly approach such as a named lead for children and young people's cases, and the provision of paediatric phlebotomy. For further consideration was the fact that only two thirds (67%) of services had appropriate training and/or a dedicated protocol in place for a child focussed approach.

The quality markers (for paediatric heterozygous FH care) from the HEART UK Statement of Care examined in this project included:

- Children are managed by a paediatric health professional with expertise in FH
- A statin is considered by the age of 10 years
- An age appropriate target LDL-C level is used for children with FH
- Dietary and lifestyle advice is begun in early childhood
- A structured review is offered at least annually
- Joint review with adult lipidologist from age 14, to prepare for transition to adult service

Table 4: Child-friendly service markers present in London FH services

Paediatric quality marker	% London services
Access to paediatric nurse	89%
Child friendly setting e.g. toys/books	89%
Access to children and young people's FH resources	89%
Dedicated transition clinic for young people	44%
Staff trained in paediatric safeguarding	100%
Access to paediatric emergency equipment	100%
Training/protocol on child focussed approach	67%
Named lead for children and young people's cases	100%
Paediatric phlebotomy	100%
Paediatric outpatient service	89%
Feedback loop for families and children on their experiences within a services' care	44%

N=9 Paediatric FH services participated in this section of the needs assessment

The transition for children and young people from a paediatric to an adult service can be a significant step that risks, according to a Cochrane review a 'deterioration in health.' To help mitigate this risk, the HEART UK Statement of Care recommends that services incorporate:
'Joint review with adult lipidologist from age 14, to prepare for transition to adult service.'

Less than half (44%) of London paediatric FH services were currently providing transition support to those under their care. This area would benefit from further

examination to help identify how children and young people can best be supported to prepare for transition to an adult-based FH service. NHS England has developed a Transition Policy that could be shared with providers and considered for inclusion in contracts for paediatric FH services going forward. Services highlighted the existing challenges of trying to integrate child and adult processes without sufficient resources - including limited staff capacity to enable this. Links with primary care are also key, and the importance of good communication between a service and a child's GP as part of the transition planning process, was also raised.
Another quality marker explored was whether a feedback mechanism was in place services to capture insights from families and children on their experiences of care. Currently only 44% of services within the M25 have this in place. There is likely to be useful learning where this is currently provided and it could be useful to consider what levers are available to help support this in practice, for example the inclusion of a KPI in service level contracts.

The project also asked services about appointment scheduling, due to the impact that attending a clinic - including during school time - can have for a child or young person. It should be noted that the approach for all participating services shifted during the needs assessment work, in response to the COVID-19 pandemic, with more virtual and telephone appointments provided.
Services were also asked how follow-up appointments were managed for children and young people under their care, and 100% of participating services confirmed they offer a flexible approach, based on the needs of their paediatric patients.

Children and young people's needs

Feedback from families living with FH has highlighted how daunting clinic appointments can be for children and young people, once they are aware that the visit may include a blood test.

'As far as a child friendly approach is concerned the biggest issue for any child is the bloods, and from personal experience and that of my children the whole visit to a clinic is overshadowed by the fact that you will be having a blood test.'
Patient Representative, Pan-London FH Steering Group.

This feedback reinforces the importance of access to paediatric phlebotomy being a core part of a paediatric FH service. In this needs assessment, 100% of the participating London services confirmed they provided this. In light of the importance of paediatric phlebotomy from a child and family perspective, this could be a useful KPI to consider in service delivery agreements.

7. Commissioning and planning

Commissioning issues

The participating services also provided information on a number of service planning issues, including a lack of funding and several capacity challenges. Common issues rasied by services included:

- A lack of paediatric FH services in many district general hospitals, despite adult clinics being run at several sites, with currently no commissioning strategy in place to help remedy this.
- At least one paediatric FH service highlighted their delivery is currently recorded under general paediatrics due to a lack of specific commissioning.
- Limited paediatric FH services clinic space and a lack of appropriate child-focussed clinic rooms was highlighted as a significant issue for a majority of hospitals. Services felt that London paediatric FH capacity could significantly increase if more clinic rooms were made available. An increase in clinic room space would also be required if discussions regarding attempts to increase the number of FH nurses were to be realised.
- Limited access to workforce expertise was also raised including dietetic advice, FH nurses for specialist cascade screening and lifestyle advice/support, and access to a dedicated genetic counsellor
- Demand for paediatric FH services is already increasing, and participants highlighted that support to help meet future capacity demands will be required
- Staff training (for both medical and nursing workforce) is reported as currently not being supported by some paediatric services
- Linking IT system to primary care to access local blood results, to help reduce time currently spent chasing up results
- Imminent local service pressures were also flagged. These are anticipated from the current child-parent FH screening pilots and the eventual national roll-out.

Practice-based experience that was shared included a clinic where the workforce capacity for delivering the paediatric FH service was included in broader service plans and staffing commitments.

Service planning

A previous report from the British Heart Foundation identified:
'There is a need to develop a consistent approach to deal with paediatric cases in the context of budgets, access points into a service, treatment and management plans, consolidating information on the paediatric register and alignment with the family clinics.'

London services are currently providing care for ~562 children and young people. Using ONS population estimates for London, it is predicted that there may be up to a further ~8500 children and young people with FH within London who are currently not known to services, and as a result are not accessing care. The NHS Long Term Plan includes the ambition to identify at least 25% of individuals with FH by 2023. For London this could amount to up to 2,000 children and young people being identified from cascade screening and other routes, and a potential three-fold increase in demand for paediatric FH services within the M25 region.

8. Conclusions and recommendations

This 2019-21 needs assessment has established that London currently has 13 NHS led paediatric FH services, providing care for ~562 children and young people. Encouragingly, many services are meeting key quality markers from NICE and paediatric guidance, for example 78% of clinics provided access to a paediatric healthcare professional with expertise in FH and lipids, or had arrangements in place to enable paediatric professional partnership and input, and 100% of services reported providing a structured annual review for children under their care. There are areas that warrant further consideration though, such as support for the 22% of services without access to a paediatric healthcare professional with expertise in lipids, to help ensure that children and their families receive the high quality care they should expect. Pressing commissioning issues identified during the needs assessment included limited access to paediatric FH nurses, and the anticipated potential three-fold increase in demand for paediatric FH services, linked to the NHS Long Term Plan identification ambition. The Pan-London FH Steering Group, a multi-disciplinary forum of clinical expertise and patient and family perspectives, could help explore how these issues could be best taken forward within London.

It is recommended that commissioners and providers in London consider how future demand for paediatric FH services could effectively be met by addressing the following nine key actions:

- With the reported lack of paediatric FH services in many district general hospitals despite adult clinics at those sites, it is recommended that a commissioning strategy is urgently developed to help remedy this.

- Given that the FH ambition in the NHS Long Term Plan could result in a potential three-fold increase in demand for paediatric FH services within London by 2023, it is recommended that commissioners review clinic capacity and staffing requirements as a priority, particularly in light of the imminent local pressures anticipated from the child-parent FH screening pilots underway, which include North London sites.

- Addressing services' limited access to specialist FH expertise should be a priority. Including considering an increase of paediatric FH nurse capacity in London, as only one third of participating services currently had access. A third of services were also without direct access to a genetic counsellor.

- Making training on a child-focussed approach available for services is recommended, given that one third of providers were currently unable to access training or did not have a suitable protocol in place. Incorporating this as a KPI into future provider agreements could be considered. And where services are not paediatrician-led, it is recommended that learning is also gathered on the different care pathways on joint

approaches with paediatric teams that have been undertaken.

- Whilst this project did not collect information about the ethnic breakdown of children with FH currently in care in the 13 NHS centres, it is important that this data should be collected in the future to examine equity of access to paediatric FH services across ethnic groups.

- With less than half of London paediatric FH services currently providing support for transition to adult services, it is crucial this is explored further and examples of models where it is taking place are captured to help establish it as a part of standard paediatric care. NHS England's paediatric Transition Policy should also be utilised.

- For services that are not currently meeting the guidance quality markers for a child-friendly service, support should be provided to assist planning on how this can be addressed. It may be useful to explore this across ICS footprints, to help indicate any unwarranted variation in health outcomes between locations. Given the importance of paediatric phlebotomy highlighted by families living with FH, considering a KPI for this in service delivery agreements would be particularly useful.

- Follow up work is recommended to help understand the health outcomes from these services. For example, have services reduced the risk of CVD events in children under their care and if yes, does that vary by age, gender, socioeconomic status,locality or geography.

- Experiences of care from children and families' perspective should be collected. Currently only 44% of participating services in London had a system in place to enable families and children to discuss their experiences of care. It is recommended that the Pan-London FH Steering Group consider how this could be taken forward with services, exploring how practice-based learning where systems are already in place can be shared.

Although outside the scope of this assessment, clinical leads highlighted a pressing need to formalise referral pathways for children and young people with homozygous FH. It is recommended that this is taken forward, as at present paediatric FH services in London are receiving some homozygous FH referrals, but only adult centres can prescribe the required treatment of PCSK9 inhibitors. There is a subset of paediatric FH cases which need further additional specialist input and management.

9. Resources and references

Resources

- Current management of children and young people with heterozygous familial hypercholesterolaemia, Heart Uk Statement of Care
- Heart Uk Children and young people's resources: including living with FH Food chart and Staying active guide hiips://www.heartuk.org.uk/cholesterol/children-and-young-peoples-resources
- Heart Uk What is FH (family) video hiips://youtu.be/R_1x0x-YOxY
- Heart Uk Living Life to the Max (family) video https://youtu.be/hkKXw2UGwuQ
- Heart UK Being a parent with FH webpage hiips://www.heartuk.org.uk/cholesterol/being-a-parent#videos

References

(1) Pedersen KMV, Humphries SE, et al. (2010) National Clinical Audit of the Management of Familial Hypercholesterolaemia 2010: Full Report. Clinical Standards Department, Royal College of Physicians, December 2010
(2) Walter K, Min JL, et al. (2015) The UK10K project identifies rare variants in health and disease. Nature. 2015;526(7571):82–90.
(3) Wald DS, Bestwick JP, et al. (2017) Child-parent familial hypercholesterolemia screening in primary care. N Engl J Med. 2017;376(5):499–500.
(4) Benn M, Watts GF, et al. (2016) Mutations causative of familial hypercholesterolaemia: screening of 98 098 individuals from the Copenhagen General Population Study esti.prevalence of 1 in 217. Eur Heart J. 2016;37(17):1384–1394.
(5) (7) Humphries SE, et al. (2018), Coronary heart disease mortality in treated familial hypercholesterolaemia: Update of the UK Simon Broome FH register. Atherosclerosis. 2018 Jul;274:41-46.
(6) (11) Ramaswami U, Cooper J, Humphries SE; FH Paediatric Register Steering Group. The UK Paediatric Familial Hypercholesterolaemia Register: preliminary data. Arch Dis Child. 2017;102(3):255-260
(8) ONS (2020) Mid-2019 Population Estimates for Census Output Areas in the London region of England, Office for National Statistics under the Open Government Licence.
(9) Humphries SE, Cooper JA, et al, (2017) The UK Paediatric Familial Hypercholesterolaemia (FH) Register: Statin-related safety and one-year Growth data. J Clin Lipidol 2017 Nov 22. pii: S1933-2874(17)30514-7.
(10) NHS Digital (2020) National Child Measurement Programme England, 2019/20 school year, Data tables

Appendix: Methodology and policy impact

Methods and Governance

With the project taking place during the COVID-19 pandemic, the approach was tailored with online surveys in place of the original planned mix-methods of online survey and interviews, to help maximise input from clinical service leads.
In addition to input from services, this needs assessment used desktop research to identify, assess and describe the health needs of children and young people with FH at a population level, for example through calculating estimated prevalence rates for London, and to provide a snapshot of the policy and guidance landscape that services are operating in. Combining this analysis with insights from services the project has anticipated future population needs and how this may impact on capacity demands for paediatric FH services in London, in particular anticipated impact from the NHS Long Term Plan ambition on cascade testing.
Findings from the online survey, desktop analysis and expert input from the Pan-London FH Steering Group and PHE London have been distilled into this report to help inform future service planning and support within London. Insights from this needs assessment may also be useful for those supporting the design and development of national policy and commissioning to help meet the health needs of children and young people with familial hypercholesterolaemia.

Governance

From a governance perspective, no patient identifiable data was involved in this study, and the storage of the contact details for service leads who participated complied with requirements of the Data Protection Act and GDPR. The project reported into the Pan-London Familial Hypercholesterolaemia Steering Group, chaired by Professor Steve Humphries, to provide governance and oversight.

Policy impact

An impact assessment of NICE guidance and HEART UK Statement of Care was also investigated as part of this service needs assessment.
For Heart UK Care Statement of Care: over half of the services that participated in the needs assessment (n=5) had begun to implement this within their service, but four services indicated that at that point in time, they had not adopted this guidance. This was though deemed as a positive signal, considering that the guidance was only published a few months before the data collection, in autumn 2020.

For the NICE updated CG71 guidance, participants were asked if the update of the guideline and accompanying implementation guide had led to any changes in the

services' care models for either paediatric or adult FH care. Two thirds of participants stated the NICE guidance update had not impacted for their service, but a third (n=3) did provide detail on some of the effects for their delivery, including: a positive impact on the implementation of cascade testing, with improved access to genetic testing for the children of index cases, and that diagnostic testing was now being offered by age 10. Two services reported that the age for considering treatment in children and young people had been positively impacted by the NICE guidance, and that the age for considering starting statin treatment had now decreased.

As this project was focussed on paediatric FH services, it is unsurprising to see a larger number of services had actively implemented the Heart UK Statement of Care (as this is tailored paediatric guidance) rather than the NICE guideline. In contrast, the NICE guideline appeared to have a more overarching role for paediatric services, spanning the identification of adults and children with FH. Detailed interventions for the management and care of children and young people with FH were largely outside the scope of the NICE guideline.

Acknowledgements

- Dr Uma Ramaswami, Consultant in Inherited Metabolic Disorders and Clinical Lead, Lysosomal Disorders Unit, Royal Free London NHS Foundation Trust
- Gregory Heeger, Clinical Project Manager – London Cardiac & Stroke Clinical Networks, NHS England and NHS Improvement
- Members of the Pan-London FH Steering Group
- Kate Haralambos, and the PASS Database team
- Dr Marilena Korkodilos, Deputy Director, PHE London
- Dr Jacqueline Lindo, PH Consultant, Specialised Commissioning (Children and Young People lead) NHS England and NHS Improvement, London
- Leah De Souza-Thomas, Health Improvement Manager, PHE

www.gov.uk/phe
Twitter: @PHE_uk
www.facebook.com/PublicHealthEngland

© Crown copyright 2021

Prepared by: Mandy Harling, National Healthcare Public Health team, PHE, and
Professor Steve Humphries, University College London
For queries relating to this document, please contact: england.cardiac-strokecnldn@nhs.net
September 2021