NATIONAL HAEMOGLOBINOPATHY PANEL

Annual Report 2023/2024

Chair: Professor Baba Inusa

(2020-2024)

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TABLE OF CONTENTS

EXECUTIVE SUMMARY

The National Haemoglobinopathy Panel (NHP), established since January 2020, provides strategic operational and clinical direction, leadership and support of Haemoglobinopathy care via its symbiotic relationship with the Haemoglobinopathies Co-ordinating centres (HCCs). The NHP multidisciplinary meeting (MDT), with membership drawn from the HCCs, provides timely advice on complex cases that need access to a wider range of expertise or opinion that may not be available at the SHT/HCC. The year 1 April 2023 to 31 March 2024 witnessed increased engagement with the MDT activities with the resultant fortunate dilemma of now being at full capacity to manage the demands per session. The NHP provides the platform for data collection/collation between NHS England/the CRG and the HCC/SHT/LHT network, particularly for patient and staff feedback. Said feedback has fed into national sickle cell service improvement endeavours.

Framework & Governance

The NHP reports to the Haemoglobinopathies Clinical Reference Group (CRG). There were some key changes in the NHP structure during this period. Foremost is that the founding Chair of the NHP, Professor Baba Inusa, demitted from his role as Chair, effective 31 March 2024 as he completed his NHS career. The recommissioning of the panel in December 2023 was an opportunity to review the Panel's Terms of Reference (ToR) as well as offer an invitation to renew and reaffirm the panel's leadership structure. The former exercise (ToR) is still ongoing. The NHP operational team (Chair, Deputy Chair, Operational Support officer) has, for some time, felt the strain of the required substantial manpower/time to maintain the work including managing databases, case themes indexing, member and contacts changes, training requirements, and to meet data requests associated with the MDT and surveys. This workload has increased as engagement has increased.

The biannual Business Operations/Governance meetings have held, as per requirements of the Commission and as set out in their Responsibilities and Governance document *(RefD1)*, and have been an effective avenue for sharing strategic updates, concerns, Key Performance Indicators (KPIs)/milestone checks, and other governance issues. This, runs alongside regular updates and meetings between the NHP and subgroups, HCCs and working groups within and connected to the NHP. Some such groups include the Bone Marrow Transplant and Cellular Therapy subgroup, the National Sickle Pain Group, National

Haemoglobinopathy Register (NHR) and the Transcranial Doppler QA programme leads, to name a few. All quarterly reports for this financial year, were completed and submitted to the accountable CRG/NHSE Commissioning personnel.

NHP MDT

The monthly national MDTs, which are a core function of the NHP, have continued with healthy engagement of all HCCs, and covers cases in haemoglobinopathies and rare inherited anaemias. It offers opportunity for learning and knowledge exchange, while ensuring the provision of equitable expert access for complex patient cases. A total of 67 cases were discussed in 2023/2024, a slight increase from the 62 total in 2022/2023. These referrals, as in the previous year, were dominated by stem cell transplant cases. Other overarching clinical, professional and operational matters of national import were also discussed at these meetings.

Education & Training

A key area identified for addressing inequalities and the provision of quality service is in Training and Education. Amidst the hard work and increased awareness following the *No One's Listening* (2021) report, there is still need for better understanding, approaches and skill set for non-haemoglobinopathy staff supporting haemoglobinopathy patients. A rolling schedule of training has been developed by the HCCs/SHTs. There is a need for the various Trust leadership to encourage staff attendance for the available training, including mandating some. There is also some work to engage Academy of Medical Royal Colleges to improve teaching curricula. The NHP endeavours to provide learning via the MDT as well as curating training events such as the *TCD in Sickle Cell Disorder* webinar held on 14th June 2023, and publicising events from HCCs and partner organisations to members. The Annual ASCAT conference was also a highlight in which various members of the NHP, including the founder, Prof Inusa, featured on this global scientific, academic and catalytic stage.

Policy & Guidelines

There is continued work towards standardisation of investigations for Liver Iron Concentration (LIC) with a survey taking place March to September 2023. The survey aim

was to assess the current range of methods (MRI T2*, FerriScan etc.) used to evaluate liver iron concentration for patients.

NHP reports the uptake of immunomodulatory treatment- Eculizumab, Rituximab and increasing Tocilizumab use, particularly encouraging clinicians to present their cases to the panel as well as fill out SHOT (Serious Hazards of Transfusion) reports to increase evidence base for the potential future policies on treatments for transfusion reactions.

No One's Listening

The NHP continue to engage with HCCs on the responses from SHTs, LHTs and stakeholders to the NHSE letter on Trust compliance status and plans as outlined in recommendations made in the *No One's Listening* report, 2021 by the All-Party Parliamentary Group for Sickle Cell and Thalassaemia (SCTAPPG). While LHT engagement has been a general challenge for most HCCs, there was reported an overall agreeable level of responses from SHTs and LHT.

Our Network/Partnerships

The NHP is strengthened by partnership with key organisations comprising patient representative groups, and complementary scientific bodies, which are listed in the next section. These allow for a broad and unique reach in our trend identification, information dissemination, and expert input.

1. NHP FRAMEWORK

The NHP carries out its duties in line with the commissioning precepts and aims a laid by the revised Responsibilities and Governance 2021/2022 <*RefD 1*> by Commissioners as well as the Terms of Reference which is currently being updated. This is accomplished via the National MDT, HCC bilateral engagement, the designation of subgroups, and the strategic partnership with various other organisations, as previously noted. The NHP also acknowledges the vital role played by the Integrated Care Boards in their role of driving the HCC/SHT initiatives forward, particularly with community services.

At the national MDT (both monthly scheduled and emergency email cases), the NHP is able to provide expert input and advice on complex clinical cases for all national regions, which are represented by HCCs. These meetings also serve as a rich learning experience, which the NHP is regularly harnessing in various ways. This forum is also an avenue to identify challenges and trends, as well as spotlighting, and/or agreeing consensus in approach and best practice.

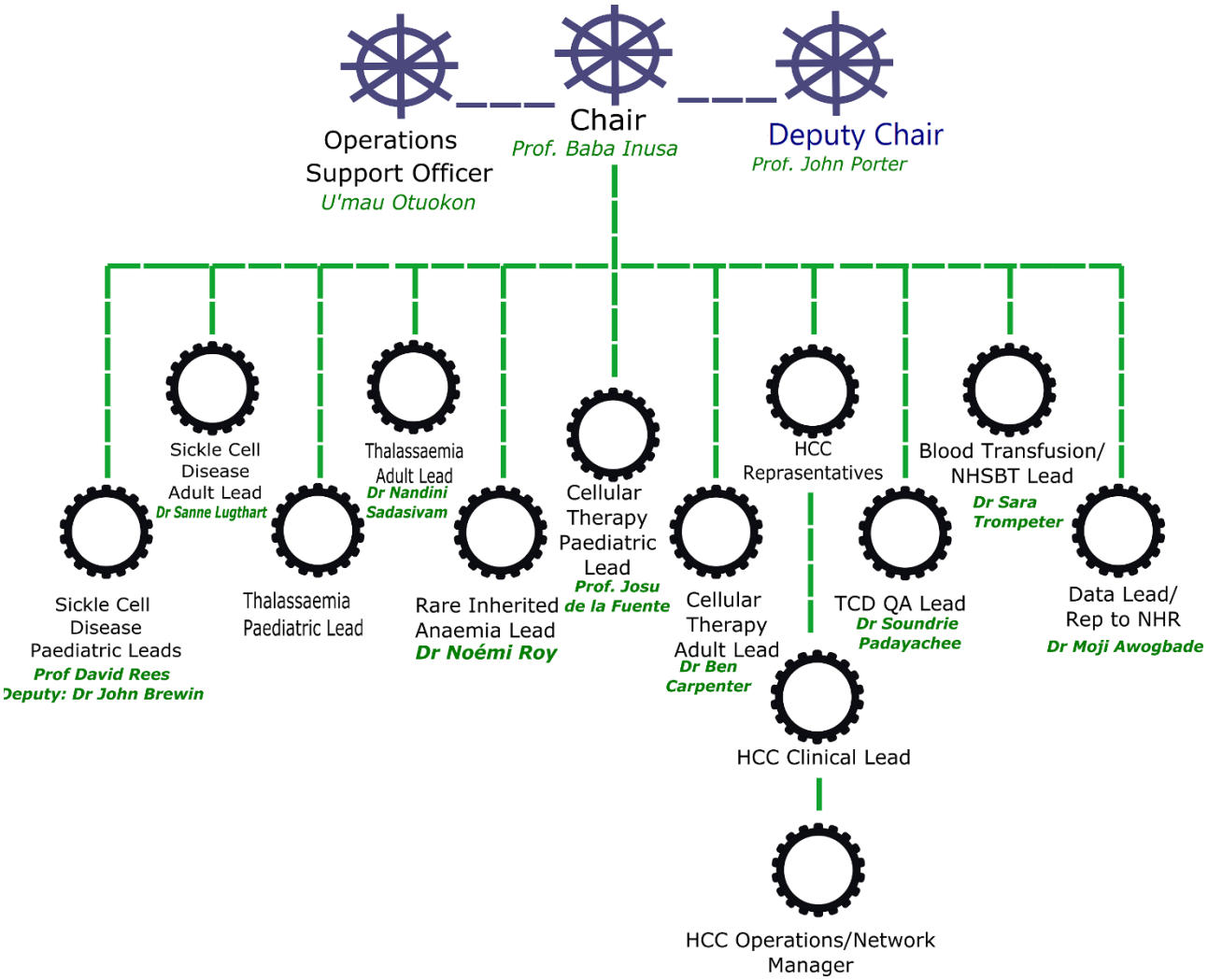
NHP currently has a representative at the national HCC Managers meetings and aims to increase its engagement with individual HCC meetings in the future. Further HCC engagement occurs via the biannual Business Operations/Governance meetings. Dissemination of information from the NHP to these regional hubs, and Vice Versa, allows for a good flow of information, contact and oversight with HCCs and organisations within the network.

The NHP is also responsible for policy development, advice and input, as well as initiatives to attain equity across the national landscape of haemoglobinopathy and rare inherited anaemia. Through MDTs and other online/digital discussion, the Panel is able to agree consensus on many subjects and lends its collective voice on matters such as NICE technology assessment consultations and, specifically for this period, appeals regarding the Voxelotor drug and gene therapy for Sickle Cell Disorder and Thalassaemia.

The organisation’s comprehensive framework continues to see that leadership is brought to the various focus areas and disciplines that make up the panel’s jurisdiction, such as

Thalassaemia, Paediatric and Adult Sickle Cell, Rare Inherited Anaemias, Newborn Screening, Transcranial Doppler Quality Assurance (TCD QA), Nursing communities, as well as Adult and Paediatric Sickle Cell Transplant/Cellular Therapy. Various clinical leads were appointed to coordinate these developments, working with representatives across the regions. With the recommissioning of the NHP, Prof Inusa, with support of the CRG National Specialty Advisor (Dr Subarna Chakravorty) and NHS England Commissioner (Zoe Hamilton), carried out an NHP cohort leadership re-confirmation, with invitations for expressions of interest to fill specific lead roles. The below structure was the result of this exercise (Fig 1.1).

Fig 1.1 Core NHP structure



The external network of partner organisations such as the National Sickle Pain Group, the National Haemoglobinopathy Register (NHR), Sickle Cell Society, UK Thalassaemia Society (UKTS), UK Forum for Haemoglobin Disorders (UKFHD), and Sickle Cell & Thalassaemia Association of Nurses, Midwives and Allied Professionals (STANMAP), continues to be an invaluable source of knowledge, reach and diverse perspective and strengthens our ability to learn of, amplify, and empower the patient voice and experience. This is also key in clinical and service development. The central positioning of the NHP with the HCCs, enables it to offer and relay key perspectives when interacting with bodies such as The All-Party Parliamentary Group on Sickle Cell and Thalassaemia (SCTAPPG) which is an effective means of getting the Haemoglobinopathies and Rare Inherited Anaemia voice heard in Parliament.

Fig 1.2 NHP Wider Network

2. THE NATIONAL MDT MEETING

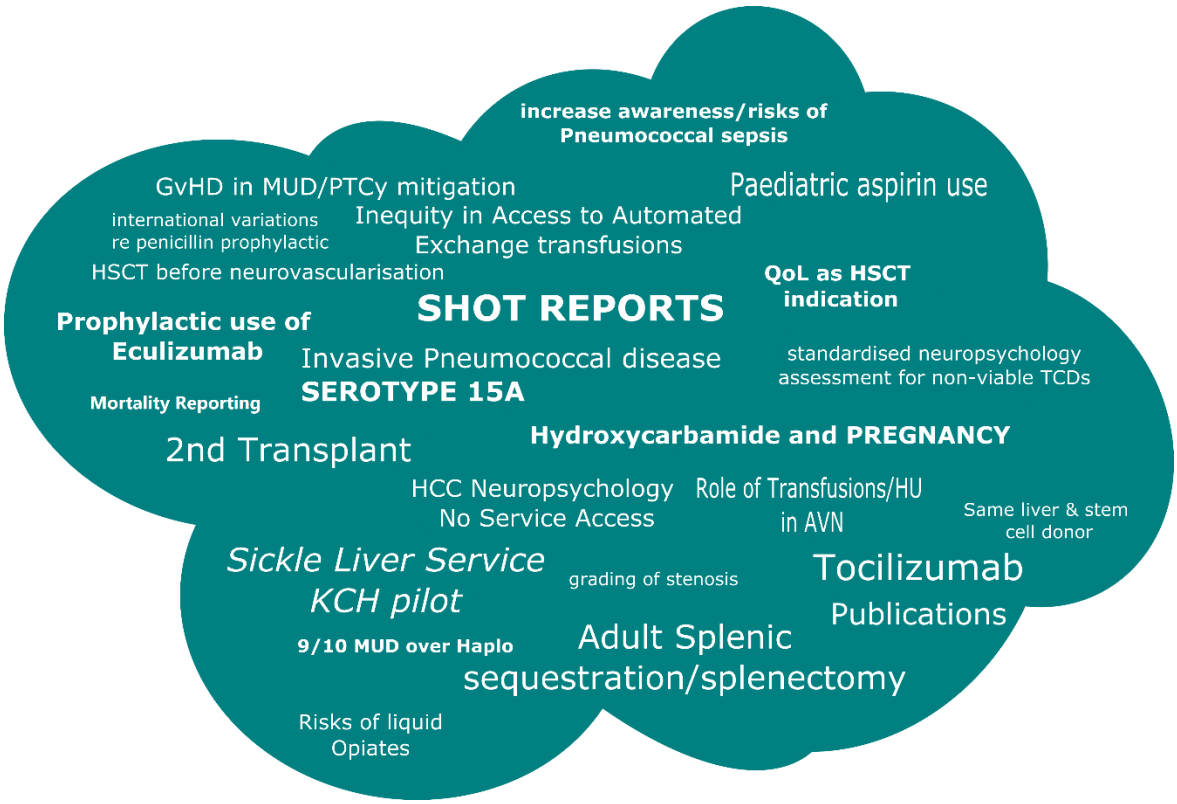
2.1 OVERVIEW

All 12 scheduled MDT meetings held in 2023/2024. There were 67 cases discussed – slight increase on the 2022/2023 total of 62, but much more than 46 and 41 recorded in the first years. There was a sequential increase in cases per quarter with the highest number of cases in Quarter 4 (N=21). Also noted in the Q4 2023/2024 activity report, was that meetings are operating at capacity and some innovation and expansion is necessary to meet the growing need that this forum satisfies. This is more so in light of the fact that there are still areas and subjects that require more engagement than is currently the status in this forum, such as death reporting and more Thalassaemia and Rare Inherited Anaemia (RIA) cases.

The presence of varied clinical specialists such as psychologists, neurologists, nurses and clinicians from the paediatric and adult subspecialties makes for a robust perspectives brought to all cases. The NHP acknowledges and appreciates the regular input by Dr Sara Trompeter and the NHS Blood and Transplant (NHSBT) team, with regards to complex blood transfusion issues brought to the panel. The Stem Cell Transplantation/Cellular Therapy clinicians have provided vital support for decision-making as evidenced by the large number of referrals for curative therapies; the NHP remains indebted for their expertise.

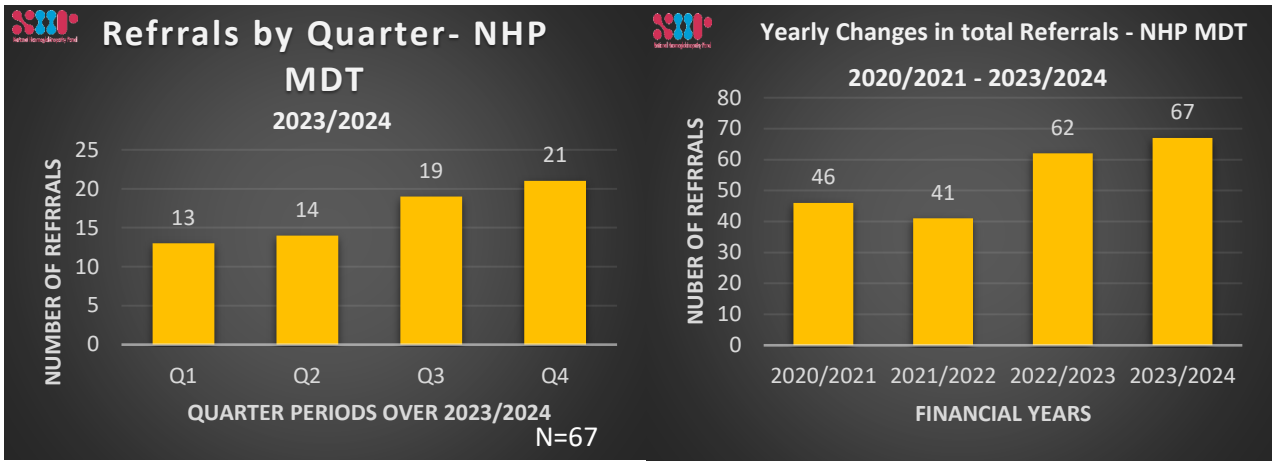
A national MDT Survey was carried out in March 2024, regarding participants' experience of the MDT over the financial year. While engagement in the exercise was low, it was an insightful and worthwhile exercise that offers learning for the NHP. Among other insights, it highlighted the relevance and added value of the MDT to attendees' learning and practice, but also highlighted the impression of overwhelm with Transplant/Cellular Therapy matters. *<RefD 2>*

Over this period, the NHP MDT included discussions on issues such as mitigating for high GvHD risk in MUD in light of PTCy (post-transplant cyclophosphamide), inequities of patient access to automated exchange blood transfusions, Tocilizumab use and the need for SHOT reports and publishing outcomes for evidence building for that drug use, difficulties in neuropsychology access for some Trusts/HCCs, the necessity of HSCT before cerebral revascularisation, the disparity between a haematological approach and a neurosurgery approach in adults and children regarding the use of aspirin, a possible growing occurrence of pneumococcal serotype 15A, and the limited data for DBA transplant and role of transfusions vs Hydroxycarbamide with Avascular Necrosis. These are just a few amongst many pertinent emerging issues beyond the case details which were processed by the panel.

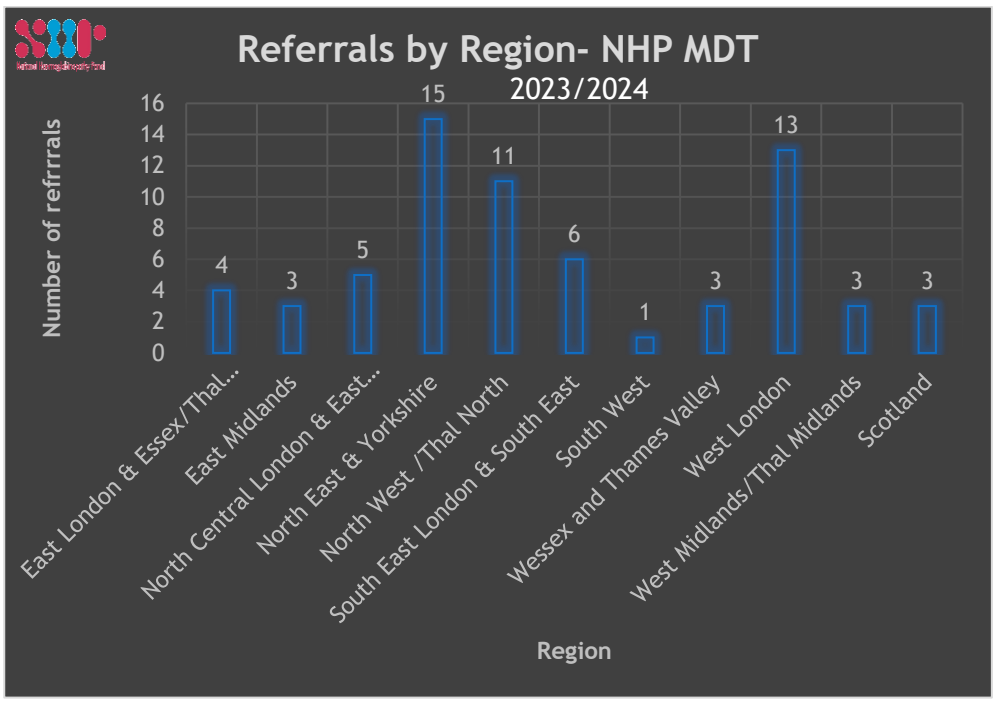


2.2 ANALYSIS OF REVEIWED CASES

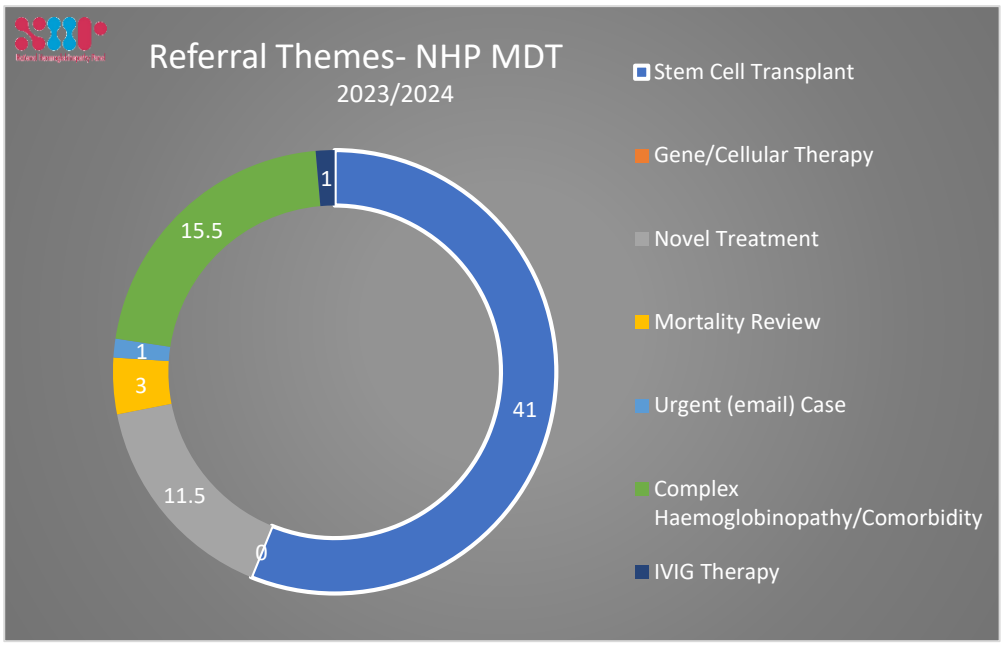
Trending Increase in Referrals: As previously noted, there has been a steady increase in MDT referrals in each quarter. This closely mirrors the increasing activity seen over the past 4 years.



Referrals by region: The highest number of referrals came from the North East and Yorkshire HCC (15), followed by West London HCC (13), the latter status a buck in the trend from previous years. North West HCC presented the 3rd highest referrals with 11 cases. It is unclear if the reduced number of South East London and South East referrals, previously a leading referrer, was due to a change in the population, their treatment requirements or the HCC's management of complex cases.



Referral Themes: Referrals for Haemopoietic Stem Cell Transplantation (HSCT) made up the highest category of referrals (41) followed by Complex Haemoglobinopathy/comorbidity referrals (15.5) and Novel use of drugs/novel treatments (11.5), which include Eculizumab, Rituximab and Voxelotor. There were 3 Mortalities discussed, from North East & Yorkshire (NEY) HCC, South East London & South East (SELSE) HCC, and East Midlands (EM) HCC.



Mortalities:

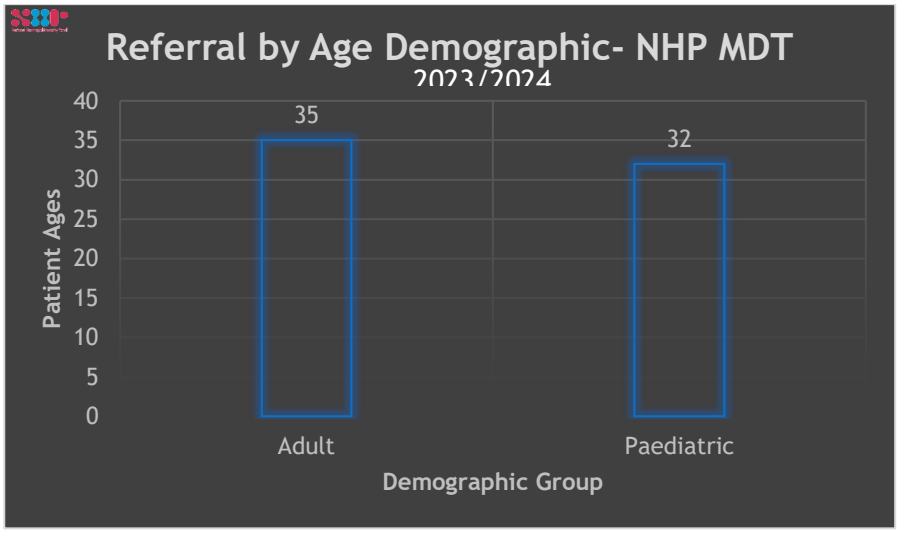
Learning from the NEY HCC case in July 2023 incorporated Trust assessed learning points as well as the NHP discussion. This included the need for partial observations to be taken from patients if a full one is not obtainable, and that cases must be escalated to a senior doctor at certain early stages. NEWS scores may not always indicate observation escalation so this should be borne in mind. The rotational ward staff and loss of continuity in care has an impact on patient care. There needs to be more education amongst ward staff regarding sickle complication management and empowerment to raise concerns where necessary. There is a need for more comprehensive documentation between teams. Advanced care planning and communication with families ahead of adult care/in transition is vital, as well as the capacity for management of complex cases within transitional services. The contribution of complex comorbidities management in this mortality was duly noted. Multidisciplinary TYA support/units was a suggestion. Actions taken in the Trust include a

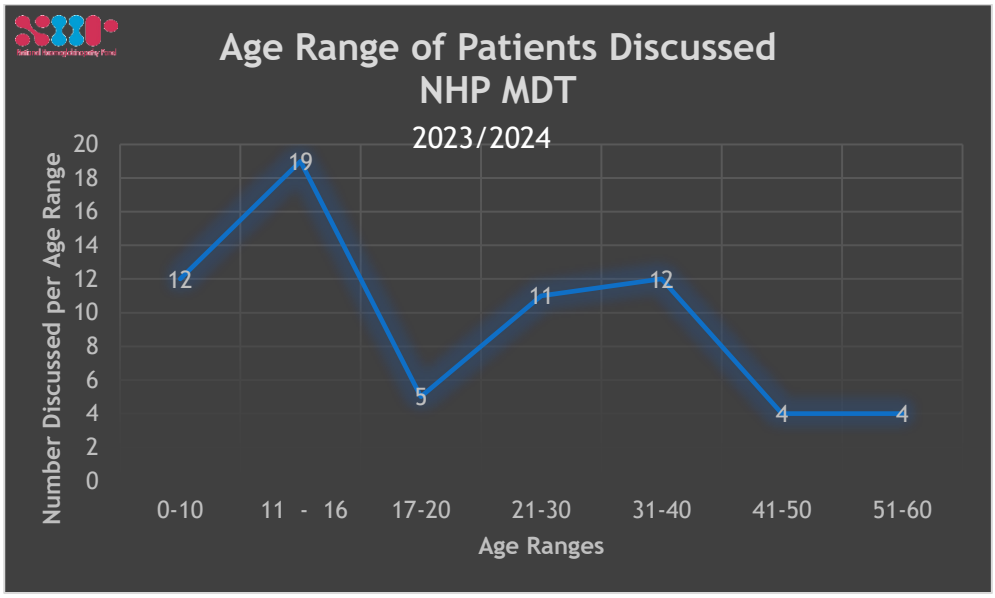
Sickle Cell Steering Group, discussion with complex transition team about passports and when they can be overridden due to clinical need, cross-Trust transition steering group presentation of the case, alternative stats monitoring probes for patients who cannot tolerate standard equipment, a debrief with the ward, medical, and CNS teams led by the clinical psychology team, and a recommendation to downgrade SJR (structured judgement review) outcome which was formerly reported as 'good' care overall.

The SELSE mortality case was discussed in December 2023 and learning from that was around the presentation of Fat Embolism Syndrome (FES). Discussion also highlighted the unexpected and pervasive impact of SCD on all organs. The panel discussed standard management when FES is suspected, even though it is not likely the outcome would have been different.

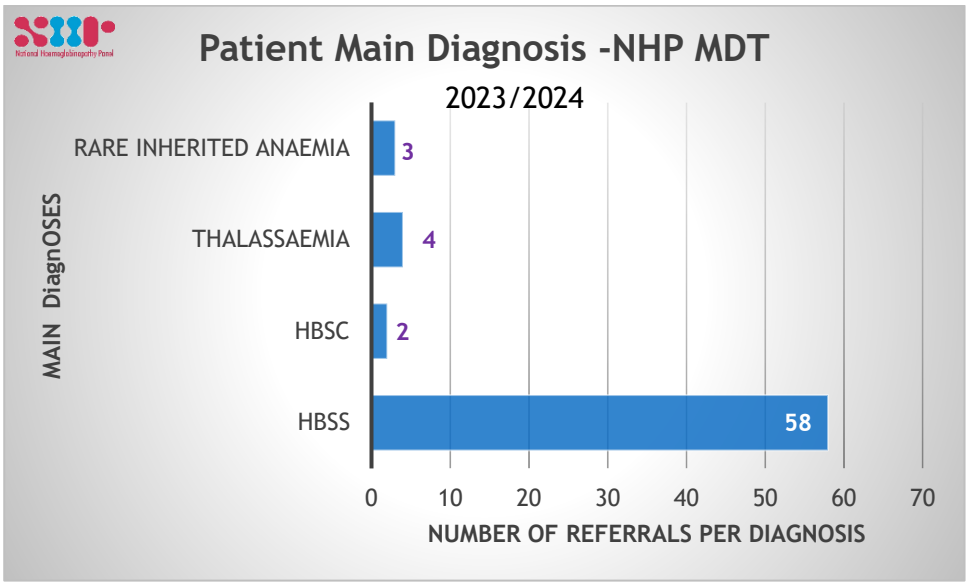
The learning from the East Midlands case discussed in March 2024 noted that more work needs to be done to address the updating of haemoglobinopathy/haematology teams, nationally, when sickle Cell patients are on admission to other departments. The role of earlier transfusion in this scenario was uncertain.

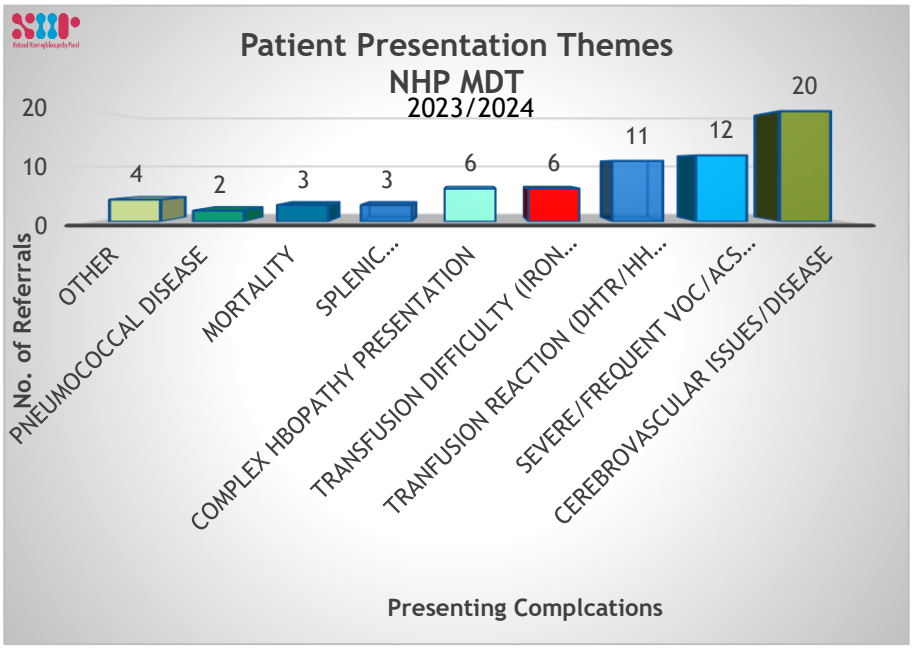
Age Demographics: In another change from previous years, there was an almost equal ratio of Adult to Paediatric referrals (35:32) where previously, adult cases were usually a clear majority. The mean age of patients referred was 22.7, with a median age of 18. The highest age range for referrals was with 11-16 year-olds (19). The most recurring age in the series was 11 - occurring 6 times - then 9, 16 and 31 appearing 4 times each.





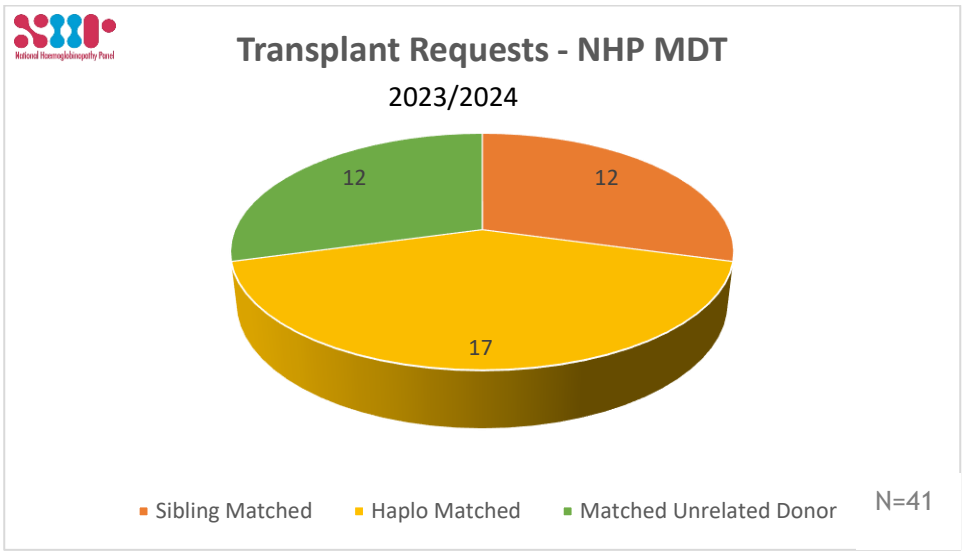
Disease and Presenting Themes: The majority of cases were based on patients with a main diagnosis of homozygous sickle cell disease/HbSS (58) followed by Thalassaemia (4), Rare Inherited Anaemia (3) and Haemoglobin SC (2). Clinicians are regularly encouraged to submit a range of disease group cases for the benefit of learning. Patients presented with a wide range of indications but cerebrovascular issues accounted for the majority of patient main presentations (20) with severe/frequent VOC/ACS despite disease modifying treatment following with 12 referrals. Closely running up were issues with transfusion reactions (11). Other transfusion issues such as iron overload and venous access featured with 6 cases, in equal measure with complex haemoglobinopathy /comorbidity were conditions such as splenic sequestration, Moya Moya and Avascular Necrosis.

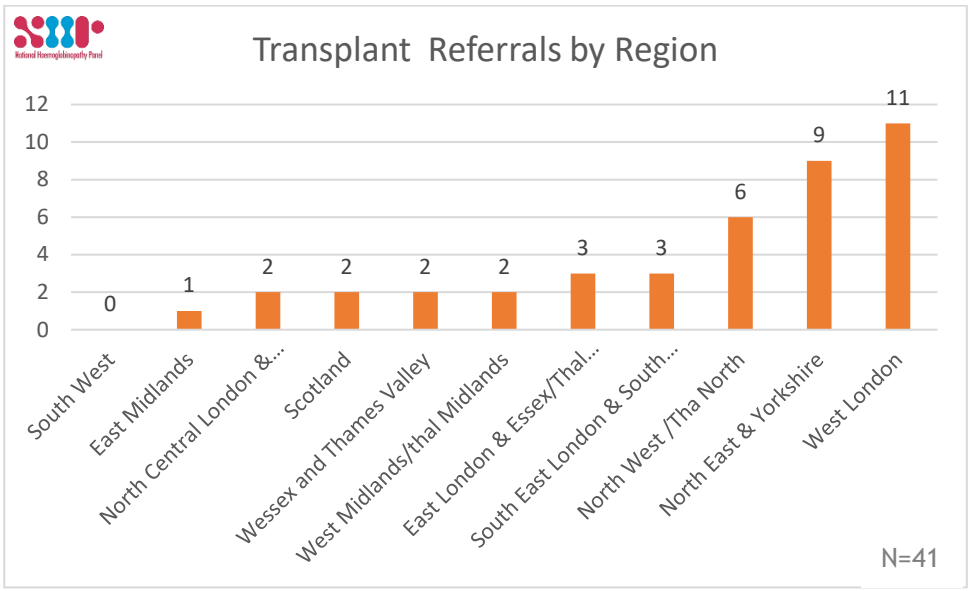




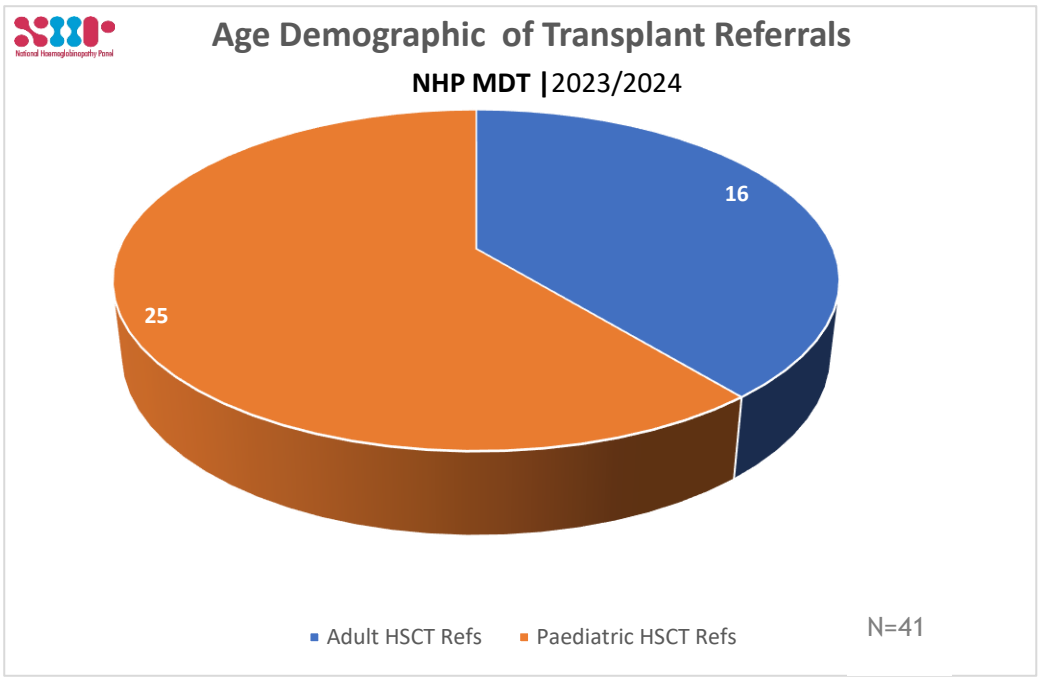
Patient Presentation Themes	
Cerebrovascular Issues/Disease	20
Severe/frequent VOC/ACS despite DMT	12
Transfusion reaction (DHTR/HH etc.)	11
Complex Haemoglobinopathy presentation	6
Transfusion difficulty (iron overload/chelation, venous access)	6
Other	4
Mortality	3
Splenic Sequestration/splenectomy	3
Pneumococcal Disease	2

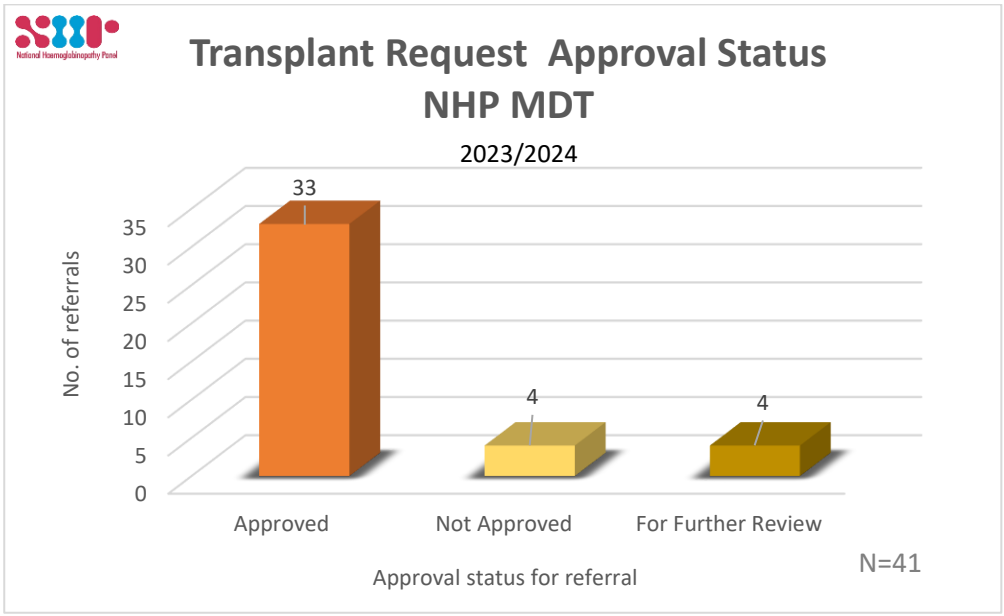
Of the 41 transplant referrals for the year to March 2024, Haploidentical donor HSCT referrals were the highest submitted, at 17; another surprising deviation from past trends. Sibling donor referrals, which are normally the majority, follow as next highest with 12 referrals, and on par with Matched Unrelated donor transplant also with 12 referrals. West London HCC referred the most patients for transplant (11), followed by North East and Yorkshire (9) and North West HCC (6). The first cases from Scotland since February 2021 featured in the 4th Quarter. Due to the REDRESS trial, adult haploidentical donor requests (3) also featured in the 4th Quarter.

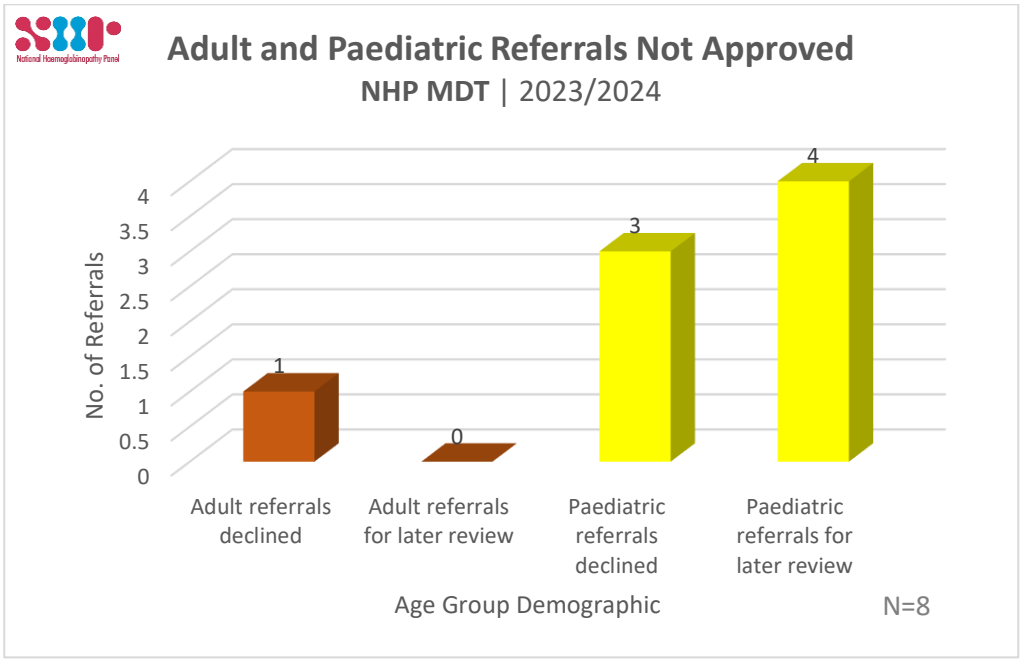




Paediatric referrals (25) accounted for the majority of total HSCT referrals. Unfortunately they also made up 75% of the 4 declined referrals and 100% of those for possible later consideration.

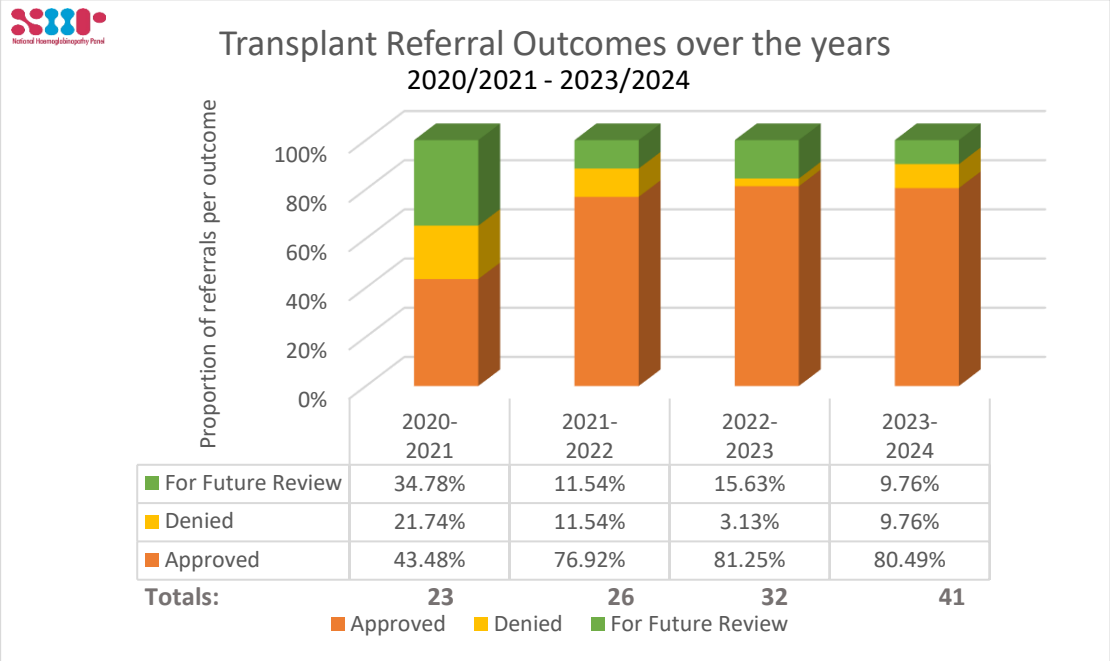



Transplant Request Approval Status
NHP MDT
2023/2024
No. of referrals
35
30
25
20
15
10
5
0
33
4
4
Approved
Not Approved
For Further Review
Approval status for referral
N=41


Adult and Paediatric Referrals Not Approved
NHP MDT | 2023/2024
No. of Referrals
4
3.5
3
2.5
2
1.5
1
0.5
0
1
0
3
4
Adult referrals declined
Adult referrals for later review
Paediatric referrals declined
Paediatric referrals for later review
Age Group Demographic
N=8

Overview of Transplants Referred and Approved over the Years

A steady increase in referrals and rate of approval for HSCT could indicate increased conversance and confidence in referring and approving. However, deliberation at MDTs and <100% approval could indicate a need for continued discussion and the value of the varied and multidisciplinary perspectives of panel members.



	2020-2021	2021-2022	2022-2023	2023-2024
For Future Review	34.78%	11.54%	15.63%	9.76%
Denied	21.74%	11.54%	3.13%	9.76%
Approved	43.48%	76.92%	81.25%	80.49%
Totals:	**23**	**26**	**32**	**41**

More data regarding the MDT can be found in the MDT Metrics Table in the *Appendix*.

3. MANPOWER AND STAFFING

As stated earlier, Professor Baba Inusa stepped down as NHP Chair, having been in the role since the inception of the NHP, and leading with expertise and vigour. Professor John Porter, who has been the Deputy Chair for just as long, kindly agreed to assume care of the role while the recruitment process was underway.

The NHP continues to keep/manage a substantial collection of data, primarily based on the MDT activities. The organisation also provides data for a wealth of processes and stakeholders, in line with Information Governance guidelines. With the increase in robustness of processes, data requests, and monthly cases, there has been a resultant pressure on manpower, a challenge that is shared amongst its network partners. Record-keeping, minute producing, website updates, case summaries (for audits and indexing for use in educational settings), creative ideas and design are a few tasks which factor into the regular and time-consuming undertakings of the very small operational team.

Following months of meetings, planning, and a subsequent application, the NHP, with support from Dr Subarna Chakravorty (National Specialty Advisor, Haemoglobinopathies CRG) have secured funding for an Education Fellow for 1 year, via Health Education England (HEE). The role is hoped to start in early 2024 and it is hoped that the candidate will help create innovative solutions to promote ongoing learning for haemoglobinopathy and rare inherited anaemia cases as well as, on a broader scale, create an opportunity for awareness and interest in the haemoglobinopathies for succession planning and addressing the well-documented lack of sufficient staff and trainees in this sector. This should also alleviate some of the task pressures that make up the regular and ongoing NHP processes.

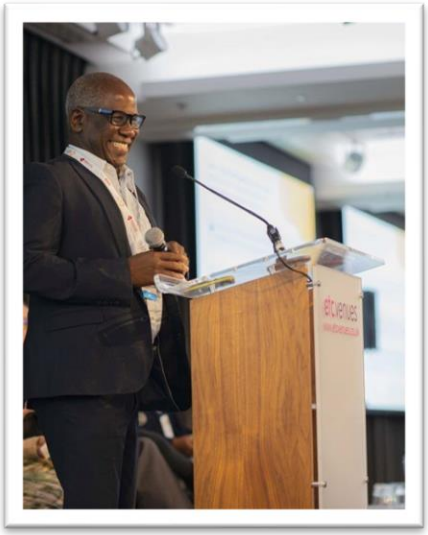
As noted in a previous section, the Panel's leadership structure was updated at the end of this period.

4. PROFESSOR BABA INUSA- A CHAPTER CLOSES

Professor Baba Inusa was the Chair of the National Haemoglobinopathy Panel (NHP) from its commissioning in 2019 and becoming operational in 2020, until March 2024 when he left the NHS and United Kingdom to take up a role in Denmark.

Professor of Paediatric Haematology, King's College London and lead clinician at the Paediatric Sickle Cell service of Evelina Guy's & St Thomas' Hospital, Professor Inusa's experience in Sickle Cell Disorder, Thalassaemia and Rare Inherited Anaemias (RIA) is extensive and has positively impacted the Haemoglobinopathy landscape nationally and internationally.

Of note, amongst many other valuable efforts and accomplishments, are his enduring clinical practice, his overseeing of national and international research projects, founding the internationally renowned Academy for Sickle Cell and Thalassaemia (ASCAT), and leading on the African Research and Innovation Initiative for Sickle Cell Education (ARISE). He was the Steering Committee Chair of the London Sickle Improvement Project (2022-2024), Associate Editor of the Journal of Sickle Cell Disease and a member of the World Health Organisation (WHO) expert group on centre of excellence for Sickle Cell Disease in Africa. He has co-authored over 130 papers and edited 2 books on Sickle Cell Disease.



While NHP Chair, Prof Inusa dedicated not only his clinical and professional expertise, but also personal resources and time to build up the NHP and its network, which (network) is the bedrock of NHP success and efficacy to date. He had the drive and capacity to galvanise colleagues and foster engagement and familiarity amongst members, shaping these into a well-connected, functioning, relevant and value-giving network, with a vision that exceeded the written mandate. During the MDTs, a major function of the NHP, Professor Inusa ensured detail and reasoning for actions was robust, and that knowledge and experience of members

was optimised for deep learning and evidence-based decision-making. His vision included extending the learnings and data from these meetings to other educational platforms as well as being an aid to informing on the progress and gaps that form the haemoglobinopathy landscape. While tackling issues of national import or collaborating with network partners, Prof Inusa was always able to keep in perspective the network's key objectives, for the benefit of the patient and community's best interests, and above any self-interest or digressions.

Wonderfully underpinning all this is Prof Inusa's capacity to be human in all circumstances, extensively acting from empathy and understanding, and drawing out the best in people. His patients have nothing but confidence in, and warm admiration for him. His colleagues and friends, of which latter he managed to collect almost everywhere he went, had utmost respect and fondness for him.

Professor Inusa will be genuinely missed in the NHP team, network and his wider NHS associations. However, his great efforts and vision have been and will continue to be of benefit to many in the haemoglobinopathy community, with immense appreciation.

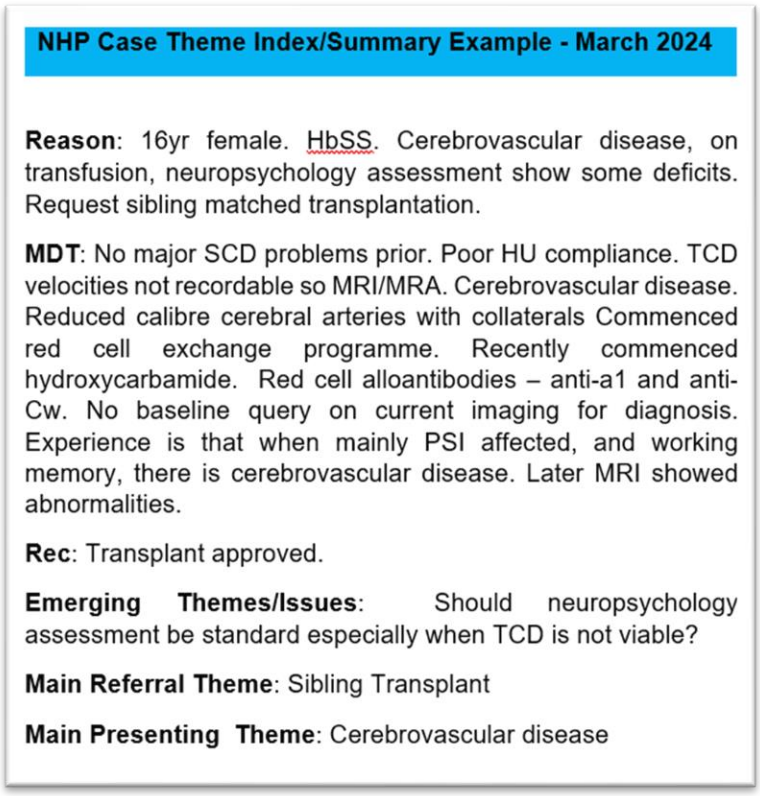
5. EDUCATION AND TRAINING

The NHP engages in, and facilitates, training and education in a number of ways.

5.1 NHP MDT

The monthly NHP MDT continues to be a platform for learning on multiple levels. Expert opinion is shared on the day, exploratory questions asked to delve deeper into scenarios, premises and options. Best practice and policy/protocols are highlighted and/or reiterated, consensus agreed, problematic trends noted, and detailed minutes are produced to provide a reference for further learning, guidance and discussion.

There is also a robust database with searchable themes, which is a great resource in extracting and focusing on specific themes for learning. It is hoped that these anonymised sessions will be published on the NHP website once updated. Since 2022 all cases have been furnished with a brief summary of key buzzwords, and summaries, in addition to the detailed minutes, to aid better identification, searchability and data extraction. In this financial year, this indexing/theme summary process has further evolved <RefD 3>. Furthermore, during talks with HEE regarding an NHP Fellow post, it was proposed that NHP publish MDT and other learning on the RCPath learning hub.



NHP Case Theme Index/Summary Example - March 2024

Reason: 16yr female. HbSS. Cerebrovascular disease, on transfusion, neuropsychology assessment show some deficits. Request sibling matched transplantation.

MDT: No major SCD problems prior. Poor HU compliance. TCD velocities not recordable so MRI/MRA. Cerebrovascular disease. Reduced calibre cerebral arteries with collaterals Commenced red cell exchange programme. Recently commenced hydroxycarbamide. Red cell alloantibodies – anti-a1 and anti-Cw. No baseline query on current imaging for diagnosis. Experience is that when mainly PSI affected, and working memory, there is cerebrovascular disease. Later MRI showed abnormalities.

Rec: Transplant approved.

Emerging Themes/Issues: Should neuropsychology assessment be standard especially when TCD is not viable?

Main Referral Theme: Sibling Transplant

Main Presenting Theme: Cerebrovascular disease

Figure 5.1: Current state of NHP MDT Case Theme Summary/Indexing

5.2 ASCAT 2023

The Academy for Sickle Cell and Thalassaemia (ASCAT) was founded by Professor Baba Inusa. The annual ASCAT conference, in conjunction with European Haematology Association (EHA) and British Society of Haematology (BSH), took place in London on 25th – 28th October 2023. The theme for 2023 was '*Therapeutic Strategies for Sickle Cell Disease and Thalassaemia: Time to Consider a Multi-Modal & Personalised Approach'*. The conference was a truly international event with 547 attendees from 46 countries ranging from clinicians,

nurses, scientists, pharmacists, haemoglobinopathy charities, service coordinators, pharmaceutical companies, patient representatives, service coordinators and more. Faculty and speakers featured prominent clinicians, healthcare professionals, scientists and patient representatives from all over the world - a good number of whom were members of the NHP network. The NHP exhibition stand received substantial visitors and there was a common thread of interest and great appreciation for the unique concept of what the NHP and its wider network stands for, and the vast benefits it actively and potentially holds for harmonisation and advancement of the haemoglobinopathy community.

5.3 HCC NATIONAL ROTATIONAL TEACHING

The NHP continues to support the dissemination of information on regional learning events for national audiences, as well as other learning and development opportunities.

5.4 OTHER LEARNING AVENUES

The NHP continues to disseminate other opportunities and materials for learning, development and calls to action within the haemoglobinopathy community.

5.5 NHP LEARNING

The NHP carried out a webinar on *TCD in Sickle Cell Disorder* on 14th June 2024. Further details on this are noted in the TCD QA section of this report.

5.6 NHP/HCC Education Steering Committee

In October 2023, the NHP initiated the NHP/HCC Education Steering Committee, looking to further develop the collaborative aspect of the education and training effort and also reduce duplication of time, resources and subjects. Part of this entailed a plan to set up the NHP Website as a central repository for various teaching materials. There have been calls for a representative from each HCC as well as requests to prepare materials to be shared for digital publishing/nesting on the site.

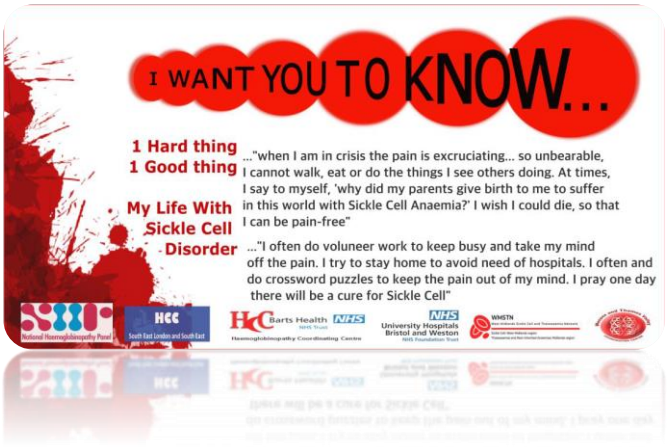
5.7 SICKLE CELL & THALASSAEMIA AWARENES DAYS 2023

In 2023, the NHP were fully engaged in building knowledge and awareness, as well as sharing other network partner activities and resources for the Global and National awareness days for Sickle Cell Disease and Thalassaemia. The International Thalassaemia day featured bespoke

materials based on the Theme of ‘Be Aware, Share, Care’ borrowed from TIF’s (Thalassaemia International Foundation) theme for the year, an organisation which the NHP also engages with on the global stage <*REfD4a, RefD4b* >.

The 2023 World Sickle day was particularly rewarding due to the collaborative aspects working with a few HCCs (South East London and South East HCC, East London & Essex HCC, South West HCC, West Midlands HCC, Wessex & Thames Valley HCC) in design feedback and dissemination of the survey that undergirded the *I Want You to Know* campaign, and sharing the resultant awareness material <*RefD 5a, RefD5b*>.

Particularly rewarding was being able help with signposting patients to local psychology services, due to the prevalent theme of psychological impact/difficulty reported by participants



6. CLINICAL REFERENCE GROUP (CRG) FOR HAEMOGLOBINOPATHIES/NHS ENGLAND SPECIALISED COMMISSIONING

The CRG for Haemoglobinopathies and the national Commissioner (Zoe Hamilton) continue to oversee the NHP via guidance, governance and support. The NHP quarterly and annual reports, as well as members who are officers of the CRG, provide the CRG and Commissioners with accounts of the progress and issues within the organisation and the Haemoglobinopathy community.

The CRG and NHSE Commissioning commit their presence at each of the biannual NHP Business Operations and Governance meetings, during which they share updates on national matters relevant to the haemoglobinopathy community and give invaluable guidance for many issues raised during the meetings.

Following the compliance exercise undertaken by the HCCs and NHP in December 2022, recurrent funding has been secured for HCC networks to spend as they required based on reported gaps. The analysis of the compliance exercise is still ongoing and will be published at a future date. Some issues raised will be reflected in the subsequent Service Specification review. There was also acknowledgment of the various policies in development including the approved allogeneic HSCT for Transfusion-dependent Thalassaemia, Sickle under 5 transplant, MRI FerriScan, Cord blood collection and more. Other updates included a Newborn Genomes programme involving a whole genome approach for many conditions including haemoglobinopathies and rare inherited anaemias, blood group genotyping for mothers giving birth to siblings of affected children and many more.

The London NHSE Commissioner also provided inspiring insights into the designation of Sickle Cell Disorder as a priority pathway. Initiatives of note include bids for the emergency bypass pathways, the community-based peer-to-peer programme developed by the Sickle Cell Society, piloting of the ACT NOW Acronym noted in the *No One's Listening* section of this report, developing the universal care plan, and introducing the role of Sickle Cell Champions/Advocates in hospitals.

7. GOVERNANCE AND OTHER RESPONSIBILITIES

As noted above, the NHP sends quarterly reports to the CRG and NHS England Commissioner, as well an annual report to the above and all its members. Annual Reports are published on the website for transparency.

Business Operations & Governance meetings: The biannual Business Operations and Governance meetings are attended by a specific group comprising HCC Clinical leads, subgroup and project leads, health care practitioners, such as psychologists and neurologist, and network partners which includes the patient representation groups (UKTS and SCS). HCC operational managers have been invited as observers since 2022 and their perspective and input has been valuable to the quality and breadths of data shared. The Business Operations/Governance meeting has taken on increasing benefit, particularly since discussion topics have been harmonised to ensure key issues are covered and evenly represented, but with freedom for other matters to be discussed. The gathering serves as a forum the NHP and network members to share updates, challenges and achievements, to learn from each other, and to receive guidance from NHSE/CRG representatives while enabling transparency, multifaceted perspectives, and potential collaboration between various members of the network.

Policy Development: The NHP continues to support policy development and the building of evidence for future policies. The efforts to standardise Liver Iron Concentration (LIC) continues and during this period, Dr Nandini Sadasivam submitted a policy proposal to the CRG. Over the period of March – September 2023 data was requested/collected from national sites to assess the MRI methods used to evaluate LIC to further support a policy proposal. The results show 20 sites responded with a picture of very mixed approaches but just 55% using FerriScan alone and some sites having multiple measurement methods *<RefD 6>*. The NHP regularly encourage reporting of Eculizumab, Rituximab and Tocilizumab use, and record those presented to the NHP, for the establishment of evidence for use in transfusion reactions.

Learning from Mortalities: The NHP Chair has been very vocal in his encouragement of HCCs to present more mortality cases at the MDT for learning. This has been echoed by the Sickle Cell Society who note that there are deaths in the community that are seem to the patient community as not being openly discussed and of lessons not being learned or implemented.

The 3 mortality discussions were from North East & Yorkshire HCC, South East London & South East HCC, and East Midlands HCC. Details of which were noted in MDT case Review (pg. 13).

Advocacy: The NHP has also been active in responding to consultations and making appeals to NICE regarding Voxelotor and Exagamglogene Autotemcel (Exa-Cel) for treatment of Sickle Cell Disorder and Thalassaemia.

Evidence Base and Key Matters Arising: The NHP reinforces good governance by highlighting precedent and policies to undergird decisions during the MDTs. The mortality sessions of the MDT are good learning and supportive experiences that can only add to clinicians' carrying out of their duty of candour in many facets.

Good Financial Stewardship: The NHP continues efforts to gain clarity on, and access to, its finances in order to be able to rightly steward allocated funds, and respond to development needs and reimbursements pending.

8. TRANSCRANIAL DOPPLER NATIONAL QUALITY ASSURANCE (TCD QA)PROGRAMME

The TCD QA programme, led by Dr Soundrie Padayachee, continues to progress as Dr Padayachee and the team of regional TCD QA Leads continue to meet regularly and develop the project nationally. This work is in conjunction with MDSAS and the NHR team. As part of oversight of this project, the NHP operational team also attend and contribute to the periodic TCD QA team meetings.

During this period, the first 6-monthy TCD practitioner report was to be published which, along with other benefits, would help identify compliance with scanning quotas, as well as practitioners who may need additional training or more scanning opportunities. By the end of this financial year, there was no longer a training back-log, which had built up from the covid-19 period. There is an ongoing training programme for new and existing TCD practitioners both online and in-person.

An NHP TCD webinar took place on 14th June 2023, hosting 80 attendees. The focus of the meeting was to discuss aspects of the TCD screening protocol that required clarification, particularly regarding low or asymmetric velocities in relation to stroke risk classification. These were not used in the original STOP trials. The diverse audience was particularly valuable for the collective knowledge enhancement. Attendee feedback indicated that the meeting was highly beneficial and achieved its aims. A high proportion were pleased to be contacted for similar events <*RefD 7*>.

In March 2024 the TCD QA Leads meeting took place. There was a recap on outcomes and learning points from the Webinar on 14.06.2023 and how to act on it. Considerations for edits, clarifications and definitions in the SOP were discussed as said document was due for review. MDSAS shared exciting forthcoming features of interactivity on the NHR platform, with interactive Power BI, which would enable more specific and customised analysis of data.

9. NATIONAL HAEMOGLOBINOPATHY REGISTRY (NHR)

During this period, Dr Farrah Shah demitted from her role as Chair of the NHR, a role which she had carried out with much dedication and tenacity, despite her very heavy roster of other responsibilities and leadership roles. Dr Noémy Roy took over as acting Chair. The invaluable support by MDSAS continued as the platform continues to grow in data integrity and multifaceted capacity.

Close engagement and communication with the NHP and HCCs means that vital patient data is available to the relevant HCC and Trust staff across the nation and to provide a map of the national haemoglobinopathy landscape from consolidated data. Conversely, in the NHP, NHR have a conduit through which they can update or make requests to, HCCs and other practitioners, for various actions and requirements in order to ensure data is accurate and robust.

During the period in question, the NHR reported having 18,000 patients register and announced its first published report. Other matters on the radar for the group included:

- Addressing issues with patients being registered in more than one place
- Identifying centres responsible for annual review
- Earmarking specific annual review fields for RIA and Thalassaemia patients
- Regarding the NHSE contract, it was reported that a revised contract would be sent out in November 2023, with only mutually agreeable elements included. It was hoped that this would be back-dated to cover the period from March 2023.

10. NATIONAL SICKLE PAIN GROUP (NSPG)

The NSPG, led by Dr Sanne Lugthart, is a subgroup of the Haemoglobinopathies CRG. It aims to 'Improve quality of care for acute and chronic pain in children, adolescents and adults and across different health care settings.' The main objectives are to gain improvements in initial analgesia, staff education, patient information, pain management of VOC in hospitals, and chronic paing management. Outcomes hoped for include creating access to staff and patient education material, protocols with clear recommendations that are auditable, access to chronic pain programmes, research outcomes.

During this period, the research subsection of the group, led by Professor P. Telford, reports securing funding to deliver a trial protocol for optimisation management of acute pain in SCD. A literary view of supportive treatments and opioid analgesia as well as the impact of social determinants of health was also being carried out, led by Dr K. Anie. This work is currently funded by the NHS Race and Health Observatory.

The team were instrumental in the 19.05.2023 *National Sickle Cell Disease Workshop: 'How Can We Improve Care Pathways for Acute Sickle Cell Pain?'* which looked at current practices, improving ED pathways, ambulatory care as an alternative to Emergency Department (ED) and future pilot projects, such as digital care plans.

In July 2023 the NHP Collaborated with the NSPG to carry out the Acute Care Pathway in Sickle Cell Disease exercise. This was a short national survery for all cenres in England (including District General Hospitals, as well as the usual SHT/LHT network via HCCs) to aid analysis of what extra care is currently available in the Emergency Department. It was hoped that this would provide evidence relevant to a project proposal to the Health Inequalities and NHS Race and Health Observatory, to support ED enhanced care for Sickle Cell patients presenting with acute pain.

11. NEWBORN OUTCOME SCREENING

The National Screening Programme for Sickle Cell Disorder (SCD) and Thalassaemia comprise Antenatal and Newborn Screening facets. This project is led Amanda Hogan and is now under NHS England, having previously been overseen by Public Health England.

Screening is a very important aspect identification, support, prevention and education for current and potential Haemoglobinopathy patients and families. The Newborn screening programme works closely with many members of the NHP network, such as with the rollout of the Newborn Outcomes (NBO) digital processing system which refers babies to clinical services, following a screen positive result for SCD and Thalassaemia. This system has been created by MDSAS, who built the NHR platform, and this system also links in with the NHR.

In April 2023 the team, in collaboration with UK Thalassaemia Society (UKTS) and Sickle Cell Society, published *It's In Our Genes: Service User Experiences and Feedback on the Communication of Screening Results for Sickle Cell and Thalassaemia*. This publication brings together key guidance and support strategies for patients and families with haemoglobinopathies. UKTS and Sickle Cell Society engaged with communities less likely to access health information through usual NHS channels. The feedback from a host of interviews and lessons learned from the communities went into producing this comprehensive and directed publication which can be found on both the societies' websites.

12. NOVEL AND NEW THERAPIES

Voxelotor for treating haemolytic anaemia caused by sickle cell disease NICE Single Technology Appraisal [ID1403];

The NHP participated in an appeal against the unfavourable decision by NICE concerning Voxelotor, a drug for treating haemolytic anaemia caused by Sickle Cell Disorder. The NHP appeal was unfortunately dismissed on a technicality, but the appeals of Pfizer and Sickle Cell Society were upheld, resulting in a hearing taking place on 13th October 2023. In December 2023, the panel were informed that the NICE committee would carry out further discussions on 8th February 2024 around identifying a plausible ICER (incremental cost-effectiveness ratios) or ICER range.

Gene Therapy

The NHP made submissions to the single technology appraisal and additional data exercises regarding the Vertex Exagamglogene Autotemcel product for treatment of Sickle Cell Disorder and Thalassaemia. The Transplant and Cellular Therapy subgroup have been working on SOPs for both therapies ahead of anticipated approvals.

Eculizumab and Rituximab

The NHP records reported cases of Eculizumab and Rituximab use in the transfusion reaction setting. There were 4 Cases of Eculizumab use presented at the NHP. The one emergency email MDT was a transfusion reaction case approved for Eculizumab use, with positive outcomes. Towards the end of the period in question, there emerged a debate on policy amendment to allow prophylactic use of Eculizumab in mitigating circumstances.

Tocilizumab

The NHP continue to encourage clinicians to present to the Panel all cases of Tocilizumab use in the acute setting of transfusion reactions. Clinicians are encouraged to publish these cases and their outcomes. The NHP also urge clinicians to complete SHOT (Serious Hazards of Transfusion) reports for all use of this drug as well.

13. NHP CELLULAR THERAPY OPERATIONAL GROUP (CTG)

The NHP Cellular Therapy Operational Group (CTG), led by Dr Ben Carpenter, Dr Victoria Potter, and Professor Josu de la Fuente, continue to support the direction, approval, implementation and audit processes surrounding NHP Haematopoietic Stem Cell Transplantation (HSCT) and Cellular Therapies. During the period, the CTG commenced a review of their Terms of Reference (ToR) in light of the group's structure and mandate.

The group reviewed details and outcomes of the 14 Adult Sibling Transplantations to date. Amongst much learning from this exercise, it was also noted that the uptake for this was substantially lower than hoped and encouraged HCCs/SHTs, via the NHP Business meetings, to consider HSCT for their patients more consistently and at earlier stages of their treatment journey, offering further advice and support with the patient discussions. The monthly CTG meetings encompassed clinical discussions on patient progress and outcomes, managing patient expectations for HSCT and Gene Therapy, patient access to limited Thalassaemia transplant centres, and managing mixed chimerism,

The group were key in coordinating and supporting the REDRESS Trial which offers potential opportunity to adults to undergo haploidentical HSCT. The group also commenced SOPs (Standard Operation Protocol) for the Vertex Exagamglogene Autotemcel (Exa-Cel) treatment for Sickle Cell Disorder and Thalassaemia. There was also some insightful review of the NICE draft guidance on Exa-Cel for Sickle Cell Disorder, with a view to formulating a response by 11th April 2024. NHP operations carried out an exercise to ascertain the level of actual therapy uptake of transplants following NHP approval, and also provided the CTG with data on the degree of various indications that precipitated transplant referrals. These were in aid of demonstrating that there would not be an unfettered deluge of requests, due to the approval controls and drop-offs before therapy stage, as well as showing the severity of conditions indicated in referral, respectively *<RefD 8 >*

There was also an update shared by Professor Josu de la Fuente regarding EBMT guidelines on Diamond-Blackfan Anaemia positing that, for best outcomes, transplant should take place early in the life of the patient, as with haemoglobinopathies. This should be discussed for patients between 10 and 18 years of age. These findings were due for subsequent publishing in the

Lancet. The NHP network have also benefited from updates on various trials for haemoglobinopathies, from the CTG.

In January 2024, the group delivered 2 perspectives at the NHP MDT.

a) Dr Beki James shared national Paediatric Transplant outcome data from 2017 to 2022. The Panel members and observers found the data insightful and reassuring, particularly noting Overall Survival (OS) at 9 months at 96%. Sibling donor procedures had the highest (OS) at 98%, haploidentical HSCT at 92%, and Matched Unrelated Donor HSCT at 90%. The discussions also opened up considerations as to whether the process of NHP approval hindered the paediatric transplant process. However, it was acknowledged that the approval process allowed for discussions that create awareness and exposure for centres which previously would not have considered, or had access to, transplantation for their patients.
b) Professor Josu de la Fuente shared intimations that the 1507 study showed positive outcomes, which could impact the role of randomisation in the REDRESS trial. However, it was acknowledged that NICE would require NHS data to make any changes to the trail configuration.

14. NO ONE'S LISTENING

During this period the NHP and HCCs continued work towards actualising the recommendations made in the *No One's Listening Report (2021)* and to hold Trusts accountable regarding the Trust-specific recommendations that are beyond the purview of Haematology teams.

The report has been a catalyst in creating new measures to address failings and deficits in the Haemoglobinopathy community, but also to infuse new impetus into already-existing improvement efforts. While there are still marked disparities in some areas, such as HCCs reporting difficulty in engaging with LHTs, there have been some positive outcomes, as listed below.

- ED priority signage and Sickle Cell representation
- Trust Sickle Boards
- The ACT NOW (**A**nalgesia, **C**ompassion, **T**est/Trigger, **N**otify, **O**xygen, **W**atch) acronym is aimed at supporting a rapid and effective response to a sickle cell crisis in patients attending any hospital. The NHP canvased the network and collated data during the process of selecting the most appropriate unit of elements, and hence, resulting acronym.
- On World Sickle Day, June 2023, the NHS England Health Inequalities Board announced the move to establish Sickle Hyper Acute units where Sickle cell patients can bypass Emergency Departments and attend a dedicated, fully-equipped and staffed clinic that can expertly handle their medical needs, particularly during acute episodes.
- A National Sickle Cell Sharing event took place on 3rd July 2024, organised by the Steering Committee of the London Sickle Cell Improvement Project – of which Prof Inusa was Chair – and supported by the Sickle Cell and Thalassaemia APPG. This was a great opportunity for regions, services and patients to celebrate the great work that has followed on since the publication of the report.

15. NETWORK PARTNERS/STAKEHOLDERS

The NHP continues to value and encourage rich engagement with its network partners, based on their varied perspectives and expertise which only enhances the work within haemoglobinopathies.

15.1 SCTAPPG (ALL-PARTY PARLIAMENTARY GROUP ON SICKLE CELL & THALASSAEMIA)

The NHP engage closely with the SCTAPPG in order to provide a joined-up and broad perspective of the national haemoglobinopathy landscape, to effectively bring attention and solutions to the areas the APPG is best placed to address and ameliorate in their Parliamentary position. The NHP presence at this meeting affords patients and their representatives opportunities to address relevant matters and raise any concerns with networked clinicians and policy-makers. The NHP participation in these forums allows other stakeholders to engage in service changes and provides a national platform for interaction.

15.2 SICKLE CELL SOCIETY (SCS)

The Sickle Cell Society continues to amplify the patient voice in society and halls of power, to educate the general populace on Sickle Cell matters, and to keep patients informed and empowered in their journey through managing their condition. The SCS also acts as the secretariat for the Sickle Cell and Thalassaemia AGGP (SCTAPPG). As such, their knowledge, perspective, reach and support has been a benefit to the NHP.

The Sickle Cell Society offers the NHP and wider network worthwhile insights on Sickle Cell Disorder patient views, concerns and needs, all of which affords better alignment of the network's activities and efforts in accountability and transparency. This is particularly so on the issue of reporting deaths, particularly those involving needful lessons to be learned, systemic errors, or significant events.

The Society notes a long history with working with UK Prison services and offered to collaborate with the Wessex & Thames Valley HCC who had begun work in addressing gaps and failures of patients within those services.

The NHP has supported their initiatives such as the Nursing Workforce Review in November 2023 and the earlier Appropriateness in Access to Automated Red Cell Blood Exchange survey, which took place in early 2023, the report for which was distributed by the NHP to panel members and HCCs in November 2023.

The Society has also been doing extensive work within London and the North of England HCCs with their peer-to-peer programmes and this incorporates ICB engagement, in light of their purview over community matters.

15.3 UK THALASSAEMIA SOCIETY (UKTS)

The UKTS is an organisation that provides support, awareness, engagement, advocacy and change instigation in the lives of patients with Thalassaemia. Their input during the NHP meetings is another valuable patient perspective and clinical overview that adds much value to the attendees.

During this period, the UKTS updated the NHP network on matters ongoing regarding some London and North England Trusts which have required escalation to Executive teams as well as possible safety concerns for patients.

There were updates on the ongoing supply problems regarding chelation agents and needles, as well as information on a possible alternative for the latter, which has been of great benefit to patients so far, and a likely improvement over what was previously available. This was very useful for the services who were also affected by the supply issues and in turn were able to signpost the UKTS representative to staff who could help review the product, reach more patients in need, as well as participate in a survey to gather patient experience to further assess product efficacy.

The UKTS representative highlighted issues that were being overlooked. For example, ageing patients did not have enough understanding and helpful expectations and thus, there needed to be more information on this phase of life for patients. Pancreatic insufficiency seemed to be an emerging issue that clinicians were asked to have on their radars.

The Society published 2 very significant pieces of work during this period, which hard work and broad range should improve the clinician education and harmonisation of the peer review processes amongst many things. These will also positively impact patient empowerment through knowledge, and shared patient experiences. The first was the highly useful and comprehensive handbook, *It's In Our Genes: Service User Experiences and Feedback on the Communication of Screening Results for Sickle Cell and Thalassaemia*. This was in collaboration with the NHS England Newborn Screening programme and Sickle Cell Society and published in April 2023. Following, in December 2023, was the publication of *Standards for the Clinical Care of Children and Adults Living with Thalassaemia in the UK* (4th Edition), with input and support by the UKFHD, British Society for Haematology (BSH) NHR, NHP and a number of other clinicians and groups.

The UKTS was also very engaged in the NICE process of assessing Exagamglogene Autotemcel for treatment of Transfusion-dependent Thalassaemia, from the consultation to committee meeting stages.

15.4 STANMAP (SICKLE CELL & THALASSAEMIA ASSOCIATION OF NURSES, MIDWIVES AND ALLIED PROFESSIONALS)

STANMAP is a national professional organisation dedicated to supporting nurse specialists, midwives, health and allied care professionals looking after clients and their families with, and at-risk of, haemoglobinopathies. Their work has a substancial impact on healthcare professionals working in the community.

The group held a virtual half-day educational day on 29th June 2023, which was attended by over 64 participants including Sickle Cell Society and UK Thalassaemia Society (UKTS) representatives who presented updates. There was an open forum discussion from members regarding developments and work-related issues in their local areas. This event included an update of the RCN competencies for haemoglobinopathy nurses from Sekayi Tangayi, Consultant Nurse North Middlesex University Hospital.

The Group participated in the Sickle Cell Nursing Workforce Project led by Aiden Rylatt – for the Sickle Cell Society.

STANMAP reports supporting and encouraging members to engage in a number of key clinical and professional training events such as the ASCAT annual conference, UKFHD Educational Day, and the regional HCC nurse forums that took place during the period.

15.5 UK FORUM FOR HAEMOGLOBIN DISORDERS (UKFHD)

The UKFHD is a multidisciplinary body of experts, previously led by Dr Farrukh Shah, dedicated to optimise care for all who live with inherited haemoglobin disorders, through advocacy and development of policy, best practice, research, patient and professional education, and preventative action. The Chair for this is now Dr Rachel Kesse-Adu. The goals and objectives of the UKFHD are highly complimentary to the NHP. The two organisations have key trustees and members in common, and collaborate on matters such as promoting and sharing updates on best practice, creating equity in the quality and access of good support for Haemoglobinopathy patients, and identifying gaps which necessitate increased learning and awareness for staff within and without the Haemoglobinopathies.

In addition to high level and inclusive educational events and clinical and professional enhancement, the UKFHD supported the UKTS in the process of publishing their 'Standards for the Clinical Care of Treatment of Children and Adult's living with Thalassaemia in the United Kingdom (4th Edition)', in December 2023.

16. HAEMOGLOBINOPATHY COORDINATING CENTRE (HCC) UPDATES

As the NHP is made up of representatives from the various HCCs, there is a continued effort to increase understanding and communication between the NHP and HCCs, so as to better champion and guide the process of harmonisation.

In the period of 2023/2024, all HCCs continued to work incredibly hard to support and develop their STHs and LHTs in line with Service Specifications.

- Some Key Challenges Across Most HCCs:
    - Staff shortages; from Consultants, nurses and psychologists, to general admin, project management and data managers.
    - Difficulties with LHT engagement.
    - All HCCs are navigating the new ICB relationships and their impact on HCCs' ability to develop key projects and relationships, particularly in the community.
    - All HCCs have a website but most find it increasingly difficult to maintain due to reduced manpower and vastness of data due for upload and editing.
    - The impact of the Crizanlizumab negative decision by NICE, and other possible factors, has resulted in a marked drop-off of patients on the drug.
- Some Key Achievements Across Most HCCs:
    - Strong learning and education programmes being run by all HCCs
    - Continued efforts to engage with ICBs, particularly in light of developing community projects, though this has been a challenge for some HCCs.
- Other Noteworthy News from a few/individual HCCs:
    - Regional collaboration such as East Midlands HCC with West Midlands HCC, and North East & Yorkshire HCC with North West HCC
    - Innovative steps are being applied to overcome challenges such as:
        - Built-in learning time at the start of regional MDTs
        - Haematology staff cross-covering roles in ED (Emergency Department)
        - Alternative ED bypass measures such as 'unwells' programme (West Midlands HCC)
    - Emerging topics for ongoing addressing:
        - Prison health policy/mechanisms for Haemoglobinopathy patients

- Refugee/Asylum seekers relocation/housing impact on health planning (consistency of and accessibility to appropriate, continued healthcare) and hence quality of life
- Time to analgesia clarification of definitions and uniformity of audits
- The need for a uniformed approach to Annual Reviews

Further details of each HCC update can be found in the NHP Business Operations and Governance meeting minutes for May 2023 and November 2023, which has been distributed to authorised personnel.

17. NHP LOOKING FORWARD

-The instatement of a new Chair to set the vision for outworking the core objectives, responsibilities and governance.

-Commence update/streamlining of MDT referral process and meeting procedure

-Complete Website overhaul and platform transition process

-Greater engagement and collaboration with HCCs

-Greater clarity, access and monitoring of NHP funds in order to better respond to development needs and transformation projects.

18. FINAL WORD

The NHP are very grateful for the many staff (panel members, cohort and project leads, CRG and commissioning staff, HCCs, observes/trainees, invited experts and affiliates) who invest so much of their valuable time and expertise for the greater good that is our collective mandate. To do so with great grace and warmth, despite disparities in original approach, experience, schedules, responsibilities, expression and organisational models, truly drives the work, lifts the weight and is worthy of commendation.

Thank You.

MDT METRICS 2023/2024

2023/2024 - Year to Date					
	Q1	**Q2**	**Q3**	**Q4**	**Total**
MDT Cases	13	14	19	21	**67**
HCC Region	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
East London & Essex/Thal London & SE	**1**	**0**	**2**	**1**	**4**
East Midlands	**1**	**0**	**0**	**2**	**3**
North Central London & East Anglia/ Thal London South Central & South West	**2**	**0**	**0**	**3**	**5**
North East & Yorkshire	**3**	**4**	**7**	**1**	**15**
North West /Tha North	**2**	**4**	**4**	**1**	**11**
South East London & South East	**2**	**2**	**2**	**0**	**6**
South West	**0**	**0**	**0**	**1**	**1**
Wessex and Thames Valley	**0**	**0**	**1**	**2**	**3**
West London	**1**	**3**	**2**	**7**	**13**
West Midlands/thal Midlands	**1**	**1**	**1**	**0**	**3**
Scotland	**0**	**0**	**0**	**3**	**3**
NHP Total	**13**	**14**	**19**	**21**	**67**
Adult/Paediatric Split	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
Adults	**10**	**7**	**5**	**13**	**35**
Paediatric	**3**	**7**	**14**	**8**	**32**
Case Diagnosis	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**

HbSS	**12**	**10**	**17**	**19**	**58**
HbSc	**0**	**1**	**0**	**1**	**2**
Thalassaemia	**0**	**3**	**0**	**1**	**4**
Rare Inherited Anaemia	**1**	**0**	**2**	**0**	**3**
Primary Theme	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
Stem Cell Transplant	**4**	**10**	**14**	**13**	**41**
Gene/Cellular Therapy	**0**	**0**	**0**	**0**	**0**
Novel Treatment	**3**	**2**	**2.5**	**4**	**11.5**
Mortality Review	**0**	**1**	**1**	**1**	**3**
Urgent (email) Case	**0**	**0**	**0**	**1**	**1**
Complex Haemoglobinopathy/Comorbidity	**6**	**2**	**1.5**	**6**	**15.5**
IVIG Therapy	**0**	**0**	**0**	**1**	**1**
Transplant Cases	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
Sibling Matched	**3**	**3**	**3**	**3**	**12**
Haplo Matched	**1**	**6**	**7**	**3**	**17**
Matched Unrelated Donor	**0**	**1**	**4**	**7**	**12**
Transplant NHP Outcome	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
NHP recommended to refer	**3**	**9**	**11**	**10**	**33**
NHP - **not currently** recommended **** n1***	**0**	**1**	**1**	**2**	**4**
NHP - **not** recommended	**1**	**0**	**2**	**1**	**4**
Successful Referral Rate	**75%**	**90%**	**79%**	**77%**	
Transplant Referrals by Region	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**

East London & Essex	**1**	**0**	**1**	**1**	**3**
East Midlands	**1**	**0**	**0**	**0**	**1**
North Central London & East Anglia	**0**	**0**	**0**	**2**	**2**
North East & Yorkshire	**1**	**1**	**6**	**1**	**9**
North West /Thalassaemia North	**0**	**4**	**2**	**0**	**6**
South East London & South East	**1**	**1**	**1**	**0**	**3**
South West	**0**	**0**	**0**	**0**	**0**
Wessex and Thames Valley	**0**	**0**	**1**	**1**	**2**
West London	**0**	**3**	**2**	**6**	**11**
West Midlands	**0**	**1**	**1**	**0**	**2**
Scotland	**0**	**0**	**0**	**2**	**2**
NHP Total	**4**	**10**	**14**	**13**	**41**

Novel Treatment Confirmed Instances of Use reported to NHP	**Q1**	**Q2**	**Q3**	**Q4**	**2023/2024**
Eculizumab	**3**	**1.5**	**1.5**	**3**	**9**
Rituximab	**1**	**0.5**	**0.5**	**0**	**2**
New Therapy	0	0	1	1	**2**
Total Instances of Use * *n2*	**4**	**1.5**	**2.5**	**4**	**12**
Retrospectively Referred **** n3***	4	1.5	**1.5**	3	**10**
Retrospectively Referred %	100%	100%	60%	75%	83%

*** Note** - n1 - Outcome is not currently recommend, but can be reviewed pending future action/changes

*** Note** - n2 - The number of instances might exceed NHP MDT case numbers since drug consideration might be additional to the primary theme and or multiple drugs might be considered.

*** Note** - n3 - NHP is aware instances of use might not yet have been presented to the NHP MDT. The NHP will seek to retrospectively review any such instances.

REFERENCE NHP DOCUMENTS

**An nhs.net email address is required to access links*

Item log	Name	Links
RefD 1	NHP Governance and Responsibility 21-22	*Return to reference paragraph*
RefD 2	MDT Experience Survey 2023/2024	Return to reference paragraph
RefD 3	MDT Case Indexing & Theme Evolution	Return to reference paragraph
RefD 4a&b	International Thalassaemia Day 2023 1) Image 1 2) Image 2	Return to reference paragraph
RefD 5a&b	World Sickle Cell Day 2023 1) Report 2) Shared experience sheet	Return to reference paragraph
RefD 6	NHP LIC Survey Results (Dr N. Sadasivam)	Return to reference paragraph
RefD 7	NHP TCD in SCD Webinar	Return to reference paragraph
RefD 8	NHP Post transplant approvals and indications	Return to reference paragraph