NATIONAL HAEMOGLOBINOPATHY PANEL

Annual Report 2022/2023

Chair: Professor Baba Inusa

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EXECUTIVE SUMMARY

The National Haemoglobinopathy Panel (NHP) will provide strategic direction and leadership of Haemoglobinopathy care and support the Haemoglobinopathy Co-ordinating centres (HCCs) from an operational and clinical perspective. 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, or will support their decisions. The year April 2022 to 31 March 2023 has witnessed a consolidation of the progress made in the framework, strategic and operational activities and increasing engagement with the MDT activities. The NHP provides the platform for a data collection/collation point between NHSE and the HCC/SHT/LHT network, particularly for patient and staff feedback that has fed into the national sickle cell service improvement endeavours.

Framework & Governance

The core NHP team (Chair, Deputy Chair, Operational Support) remains unchanged and all HCCs have been fully represented, with some changes in personnel due to retirements or new leadership roles. The NHP office requires substantial manpower/time to maintain databases, staff and contacts, and for meeting requests for data associated with the MDT and surveys.

The recent introduction of the Integrated Care Boards (ICBs), effective from 1st April 2023, was discussed at a number of NHP forums, providing better understanding for clinicians, patients and other stakeholders.

The biannual Business Operations/Governance meetings have held, as per requirements of the Commission and as set out in their Responsibilities and Governance document (*Attachment 1*), and have been an effective avenue for sharing strategic updates, concerns, Key Performance Indicators (KPIs) and milestone checks, and other governance issues. There also ensued other regular updates and meetings between the NHP subgroups, HCCs and working groups within and connected to the NHP, such has the Bone Marrow Transplant and Cellular Therapy subgroup, the National Sickle Pain Group, National Haemoglobinopathy Registry (NHR) and Transcranial Doppler QA programme, to name a few. All quarterly reports and the annual report for 2021/2022, were completed and submitted to the accountable CRG/NHSE personnel.

NHP MDT

The key NHP function - the national MDTs - has remained popular and with full participation of all HCCs, majority of SHTs, as well as multiple clinical observers referred by MDT core members. The monthly MDT covers cases in haemoglobinopathies and rare inherited anaemias, and has offered opportunity for learning and knowledge exchange, while ensuring the provision of equitable expert access for complex patient cases. A total of 62 cases were discussed in

2022/2023, 51.2% more than 2021/2022. 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

One of the key areas identified for education and training is the gap in addressing inequalities and the provision of quality service. Among the highlighted issues are access to care and patient experience, particularly for rare disorders, and the need to develop a better understanding and skill set in supporting patients. A rolling schedule of training has been developed by the HCCs/ SHTs but these need better uptake by the targeted staff. There is a need for Hospital Trust leadership to enforce the portfolio for training, including these to mandatory training, while engaging Academy of Medical Royal Colleges to improve teaching curriculum. The NHP endeavours to provide learning via the MDT as well as curating training events such as webinars and publicising events from HCCs and partner organisations to members.

Policy & Guidelines

The NHP published the Voxelotor SOP in May 2022 as well as Crizanlizumab and currently working on a number of treatment policies such as the standardisation for Liver Iron Concentration investigations (MRI T2*/FerriScan), paediatric bone marrow transplantation for sickle cell disease. NHP reports the uptake of immunomodulatory treatment- Eculizumab, Rituximab and increasing access to Tocilizumab use.

No One's Listening

The NHP/HCCs have coordinated the collation of responses from SHTs. LHTs and stakeholders in light of the recommendations made in the All-Party Parliamentary Group for Sickle Cell and Thalassaemia (SCTAPPG) report, *No One's Listening (Attachment 2)*. All HCCs, SHTs and LHTs have been engaged in meeting these recommendations and contacting Trust and affiliate leadership to do their part in meeting recommendations.

Our Network/Partnerships

Ongoing partnership with key organisations such as Sickle Cell Society (SCS), UK Thalassaemia Society (UKTS), UK Forum for Haematological Disorders (UKFHD), NHS Blood and Transplant (NHSBT) and NHR (National Haemoglobinopathy Registry) Steering Group/MDSAS, help us build a strong network with unique reach in our trend identification, information dissemination, and expert input.

1. NHP FRAMEWORK

The NHP is to carrying out its duties in line with the commission model of care as laid out by the Responsibilities and Governance 2021/2022 (*Attachment 1*) document from Commissioners. This is accomplished via the National MDT, HCC bilateral engagement, the designation of subgroups, and the strategic partnership with bodies such as the Sickle Cell & Thalassaemia Association of Nurses, Midwives and Allied Professionals (STANMAP), Sickle Cell Society (SCS), the UK Thalassaemia Society (UKTS), and the UK Forum for Haemoglobin Disorders (UKFHD). The NHP also acknowledges the impending Integrated Care Boards, and has sought further understanding of how to engage with this new entity, notably due to its different geographical structure from the NHP/HCC regional structure.

At the national MDT (both scheduled and emergency email cases), the NHP is able to provide expert input and advice on complex clinical cases for all HCC regions. These meetings also serve as a rich learning experience, which the NHP is regularly optimising. 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 maintains regular engagement at HCC meetings, while the biannual Business Operations/Governance meetings, and dissemination of information via emails 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 a few key initiatives that aim to further attain equity across the national landscape of haemoglobinopathy. 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 Anaemias, Newborn Screening, Transcranial Doppler Quality Assurance (TCD QA), Adult and Paediatric Sickle Cell Transplant. Various clinical leads have been appointed to coordinate this development with representatives across the regions.

The external network of partner organisations such as National Sickle Pain Group, the National Haemoglobinopathy Registry (NHR), Sickle Cell Society (SCS), UK Thalassaemia Network (UKTN), and UK Forum for Haemoglobin Disorders (UKFHD) continues to be an invaluable source of knowledge, reach and perspective that strengthens our ability to learn of, amplify empower patient voices and experiences, as well as clinical and service development.

Fig 1.1

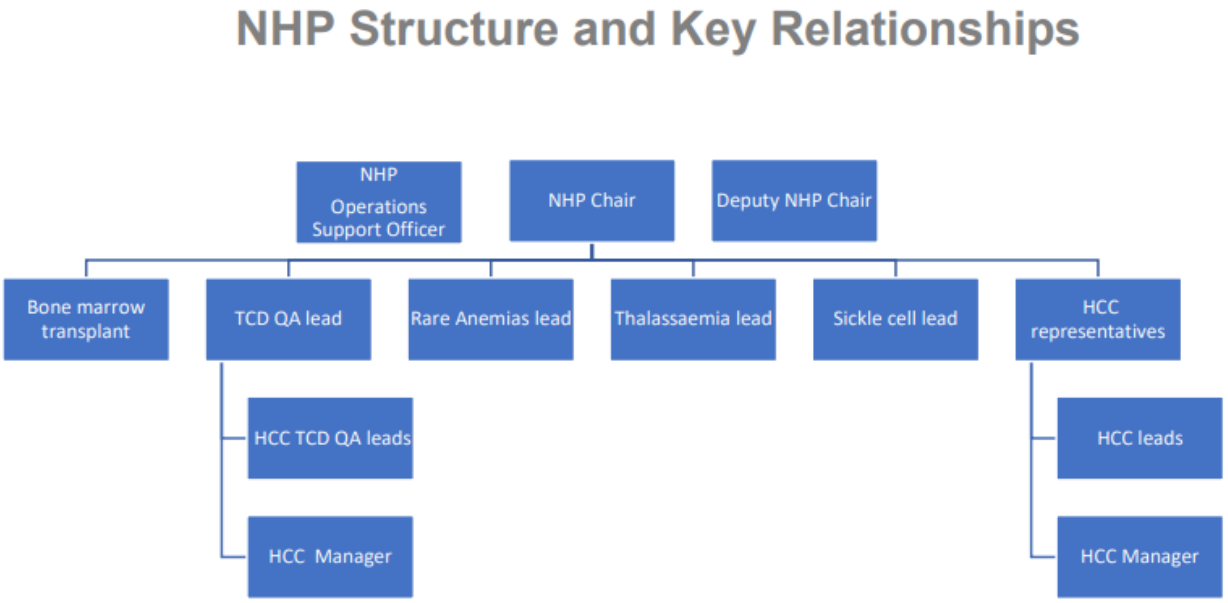

NHP Structure and Key Relationships
NHP Operations Support Officer
NHP Chair
Deputy NHP Chair
Bone marrow transplant
TCD QA lead
Rare Anemias lead
Thalassaemia lead
Sickle cell lead
HCC representatives
HCC TCD QA leads
HCC Manager
HCC leads
HCC Manager

Fig 1.2

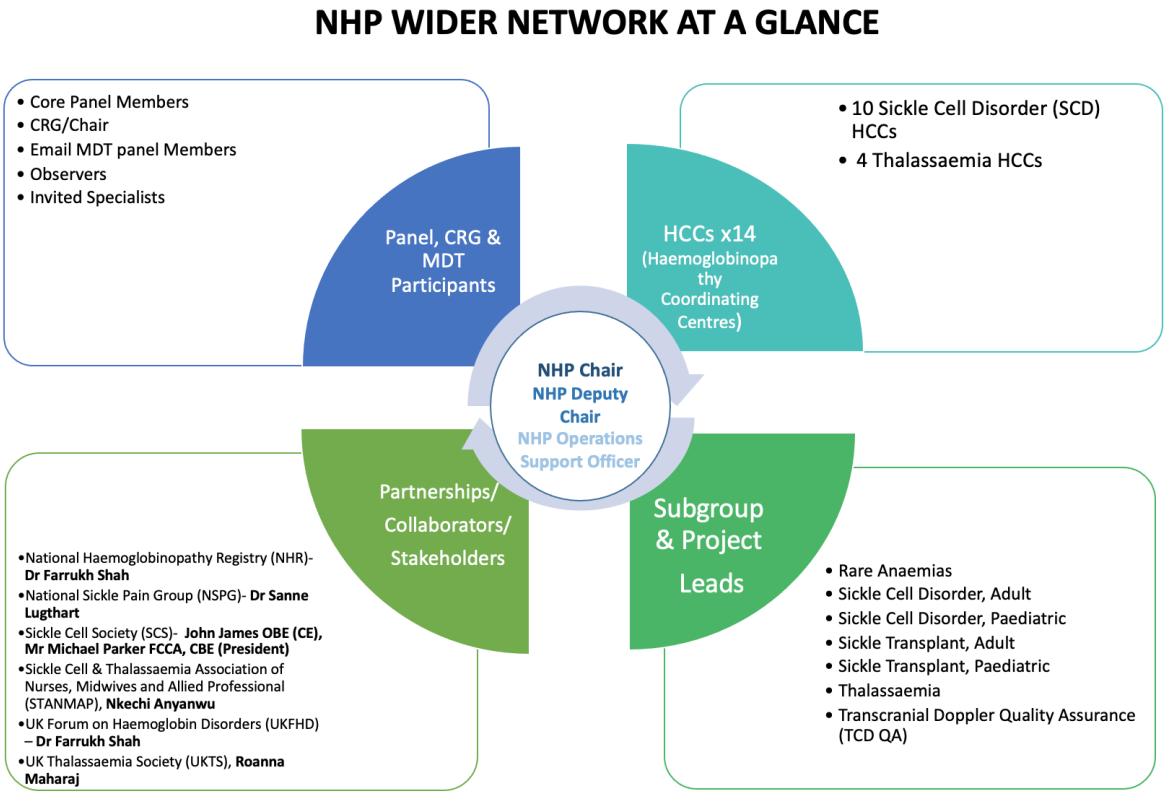

NHP WIDER NETWORK AT A GLANCE
• Core Panel Members
• CRG/Chair
• Email MDT panel Members
• Observers
• Invited Specialists
Panel, CRG & MDT Participants
• 10 Sickle Cell Disorder (SCD) HCCs
• 4 Thalassaemia HCCs
HCCs x14 (Haemoglobinopathy Coordinating Centres)
NHP Chair
NHP Deputy Chair
NHP Operations Support Officer
Partnerships/ Collaborators/ Stakeholders
•National Haemoglobinopathy Registry (NHR)- Dr Farrukh Shah
•National Sickle Pain Group (NSPG)- Dr Sanne Lugthart
•Sickle Cell Society (SCS)- John James OBE (CE), Mr Michael Parker FCCA, CBE (President)
•Sickle Cell & Thalassaemia Association of Nurses, Midwives and Allied Professional (STANMAP), Nkechi Anyanwu
•UK Forum on Haemoglobin Disorders (UKFHD) – Dr Farrukh Shah
•UK Thalassaemia Society (UKTS), Roanna Maharaj
Subgroup & Project Leads
• Rare Anaemias
• Sickle Cell Disorder, Adult
• Sickle Cell Disorder, Paediatric
• Sickle Transplant, Adult
• Sickle Transplant, Paediatric
• Thalassaemia
• Transcranial Doppler Quality Assurance (TCD QA)

Table 1.1

NHP Leads

NHP Chair: Professor Baba Inusa
NHP Deputy Chair: Professor John Porter

Domain	Lead
National Haemoglobinopathy Registry (NHR)	Dr Farrukh Shah
Rare Anaemias	Dr Noémi Roy
SCD, Adult	Tbc
SCD, Paediatric	Dr Sue Height
Stem Cell Transplant Subgroup, Adult	Dr Victoria Potter and Dr Ben Carpenter
Stem Cell Transplant Subgroup, Paediatric	Prof. Josu de la Fuente
Thalassaemia	Dr Nandini Sadasivam
Transcranial Doppler Quality Assurance (TCD QA)	Dr Soundrie Padayachee

For further information on the NHP terms of reference, framework and structure, please see the website.

2. THE NATIONAL MDT MEETING

2.1 OVERVIEW

All 12 scheduled MDT meetings held in 2022/2023 with an additional 5 email MDTs and 1 emergency video MDT. There were 62 cases discussed - a 51.2% increase on the 2021/2022 figure (41) - with the highest number of cases in Quarter 4 (N=20). The 5 urgent email MDTs were spread across each Quarter and all with good patient outcomes. There was also an emergency video MDT for a case from the North West HCC.

There was an increase in referrals for all primary referral themes compared to the previous year, but, Haemopoietic Stem Cell Transplantation (HSCT) referrals were the majority of cases discussed (32) in 2022/2023, followed by Complex Haemoglobinopathy/Comorbidity discussions (19). There were 4 discussions for novel use of drugs (Eculizumab and Rituximab)/new therapy(Voxelotor), with 2 being retrospective. All 4 were approved by the panel. There were 6 mortality reviews, reflecting a positive response to discussions encouraging these reviews. The majority of cases (50) were based on patients with a main diagnosis of homozygous sickle cell disease (HbSS) followed by Rare Inherited Anaemia cases at 6. Adults made up the majority (64.5%) of patients discussed. The highest number of referrals for the year were from South East London & South East (19), followed by North East and Yorkshire (11).

The main presenting issue of patients discussed were recurrent or severe Vaso-occlusive Crisis (VOC) despite treatment, including Hydroxycarbamide (HU) and transfusion. Complex disease management was the next most common patient presentation, which included the safety of administering pegylated interferon, or further management of a difficult Beta Thalassaemia Intermedia case, all in combination with other typical disease presentations. Cerebrovascular/neurovascular disease was the third most common patient presentation theme, followed by transfusion reactions (DHTR/HH) and severe phenotype. Others presentations include splenic sequestration, end organ damage, sickle retinopathy and transfusion difficulties such as venous access issues. Typically, most cases had a combination of the noted symptoms but were often distinguished with some prominent, while others presented to a lesser degree or were part of the patients' past history.

As highlighted during the February 2023 MDT, there is need for greater collaborative understandings between hepatology and haemoglobinopathy due to the prevalence of sickle hepatopathy and the bilateral implications and impact that liver and stem cell transplant bear on the patient. The clinicians at Kings College Hospital fortunately, have substantial experience in this area and are working with a hepatology clinician, Dr A. Suddle, with a keen interest in this setting and a will to find a mutual resolution.

The presence of varied clinical specialist such as psychologists, neurologists, nurses and clinicians from the paediatric adult subspecialties makes for a robust perspectives brought to all cases. The NHP is particularly grateful for the advance work, and input on the day, by the (NHS Blood and

Transplant) NHSBT and Stem Cell/Cellular Therapy teams, which are vital for decision-making as the prevalent themes require their input.

Haemopoietic Stem Cell Transplantation (HSCT) referrals for the year to March 2023 were up (32) from 26 recorded for 2021/2022. Data shows that transplant referrals and approval rates have increased year on year (see figs. 2.13, 2.14 and table 2.2). possibly indicating increased confidence and awareness in patients and clinicians to consider this treatment option, as well as increased confidence, conversance and consensus within the panel, to approve these therapies. Of the 32 transplant referrals made in 2022/2023, 26 were approved, 5 were recommended for further review and 1 was deemed unsuitable. Sibling matched HSCT was the highest requested transplant type, with 22 referrals, followed by haploidentical matched HSCT (8). The majority of referrals came from the South East London and South East HCC (17) followed by North East and Yorkshire HCC (5). There were no transplant referrals from East Midlands, South West, Wessex & Thames Valley and West Midlands HCCs.

The meetings continue to be a point of learning and later reference, echoed in feedback from the MDT attendees (*Attachment 3*

The forum is also used to highlight issues of national import, such as consensus on reconsidering the use of Oramorph, the benefits of the single prescriber practice for opiates, and the need for a single national transfusion record per patient, as per January 2023 MDT. When Voxelotor (Oxbryta®) gained market authorisation in July 2022, the NHP MDT initiated an update and discussion to address this and how it would affect patients who were accessing this via EAMS.

2.2 ANALYSIS OF REVEIWED CASES

Fig 2.1

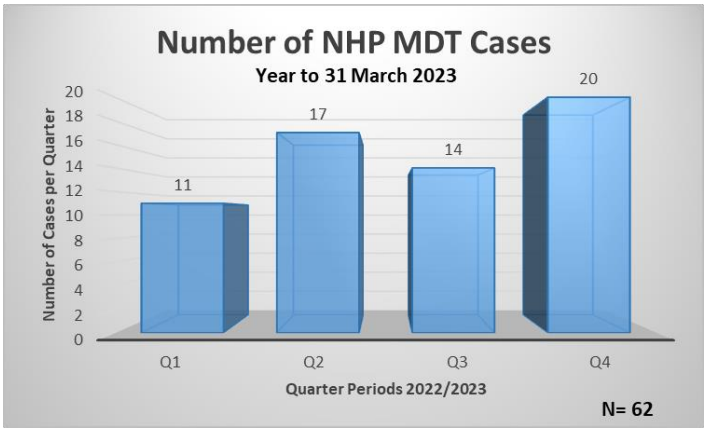


Fig 2.2

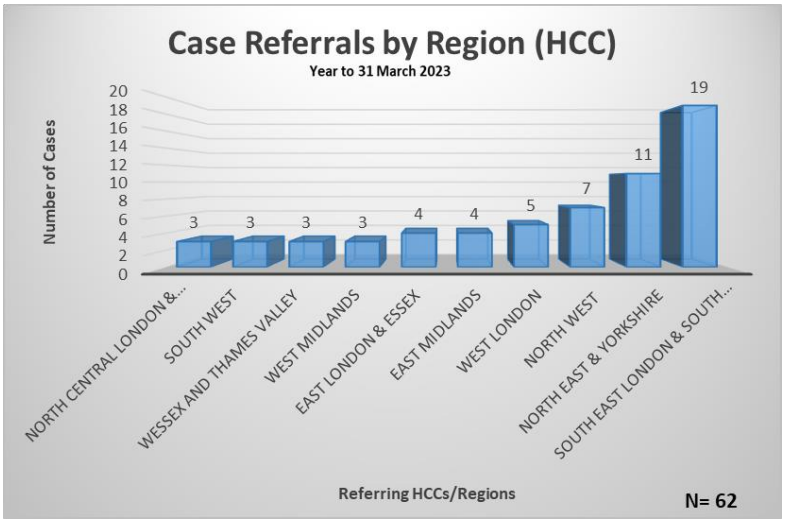

Case Referrals by Region (HCC)
Year to 31 March 2023
Number of Cases
20
18
16
14
12
10
8
6
4
2
0
3
3
3
3
4
4
5
7
11
19
NORTH CENTRAL LONDON &...
SOUTH WEST
WESSEX AND THAMES VALLEY
WEST MIDLANDS
EAST LONDON & ESSEX
EAST MIDLANDS
WEST LONDON
NORTH WEST
NORTH EAST & YORKSHIRE
SOUTH EAST LONDON & SOUTH...
Referring HCCs/Regions
N= 62

Fig 2.3

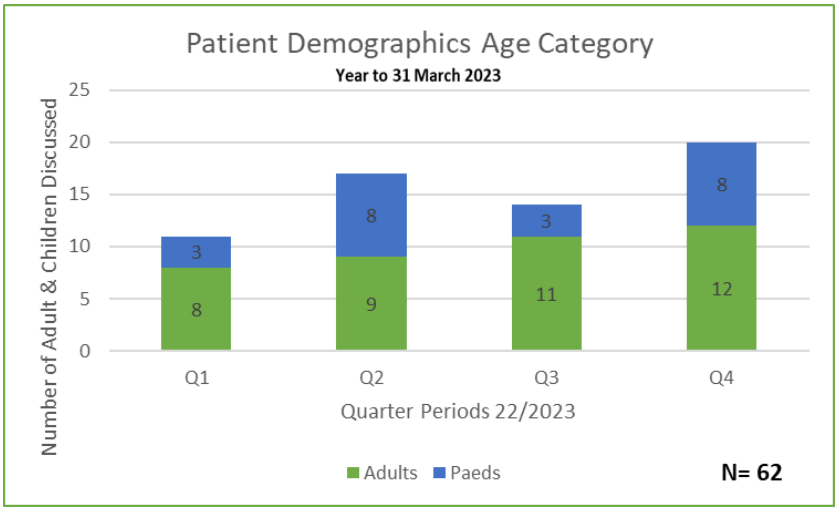

Patient Demographics Age Category
Year to 31 March 2023
Number of Adult & Children Discussed
25
20
15
10
5
0
3
8
8
9
3
11
8
12
Q1
Q2
Q3
Q4
Quarter Periods 22/2023
Adults
Paeds
N= 62

Fig 2.4

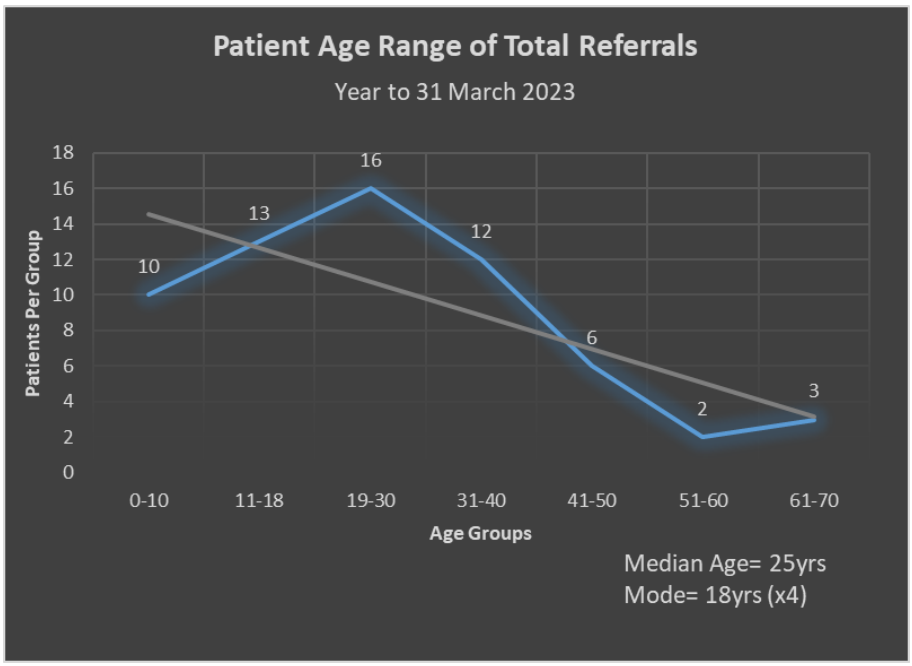

Patient Age Range of Total Referrals
Year to 31 March 2023
Patients Per Group
18
16
14
12
10
8
6
4
2
0
10
13
16
12
6
2
3
0-10
11-18
19-30
31-40
41-50
51-60
61-70
Age Groups
Median Age= 25yrs
Mode= 18yrs (x4)

Fig 2.5

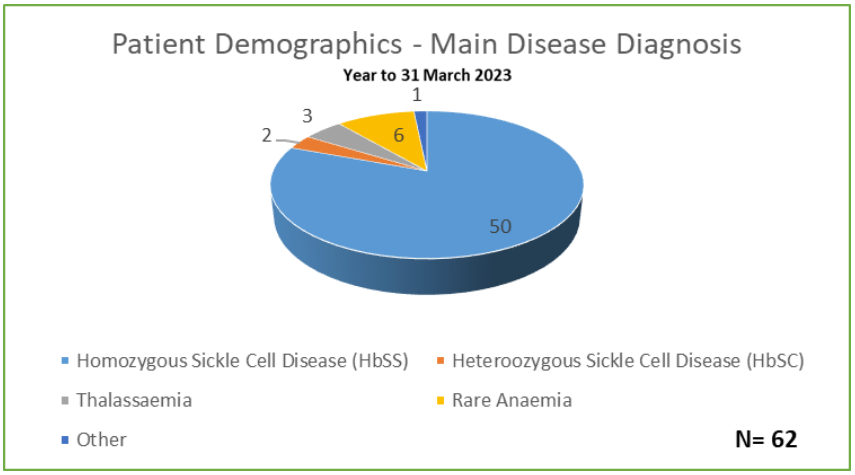

Patient Demographics - Main Disease Diagnosis
Year to 31 March 2023
1
3
2
6
50
Homozygous Sickle Cell Disease (HbSS)
Heteroozygous Sickle Cell Disease (HbSC)
Thalassaemia
Rare Anaemia
Other
N= 62

Fig 2.6

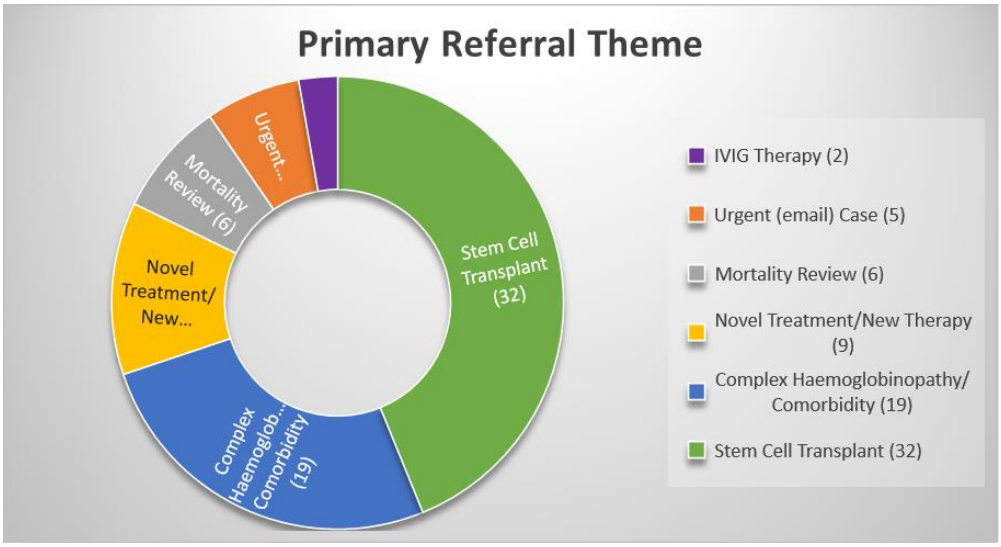


Fig 2.7

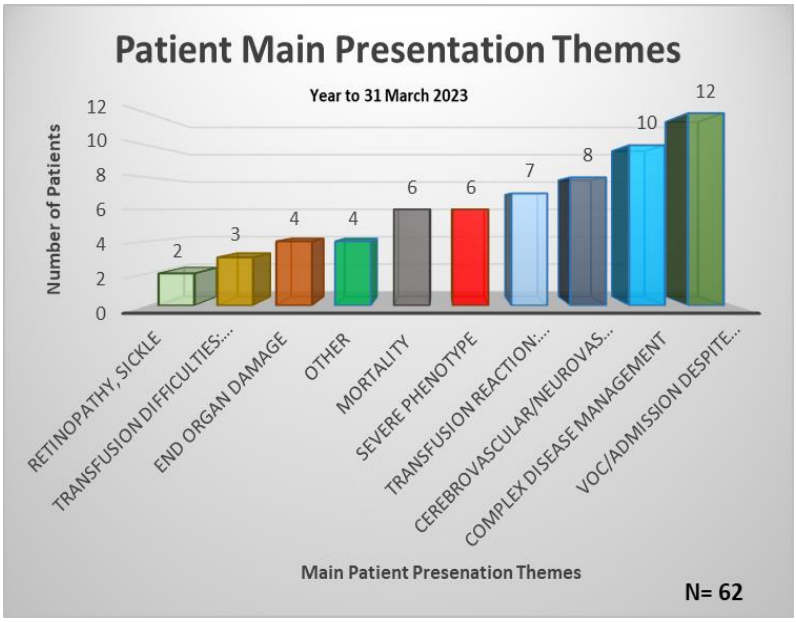


Table 2.1

Main Themes	Quantity
Retinopathy, Sickle	2
Transfusion difficulties: iron overload/chelation, vascular access	3
End Organ Damage	4
Other	4
Mortality	6
Severe Phenotype	6
Transfusion Reaction: DHTR/HH/Haemolysis	7
Cerebrovascular/Neurovascular Disease	8
Complex Disease Management	10
VOC/Admission despite treatment	12

Transplant Referral Data

Fig 2.8

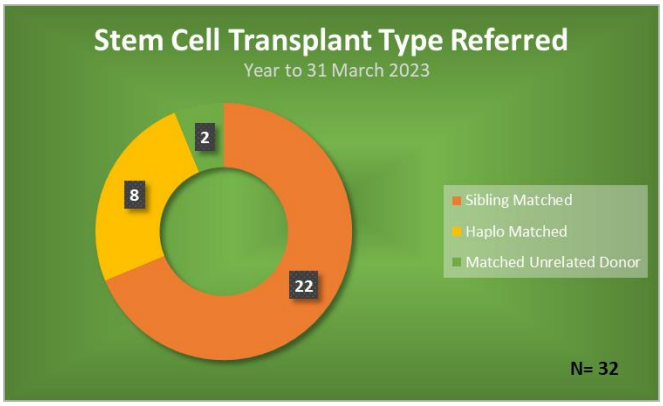


Fig 2.9

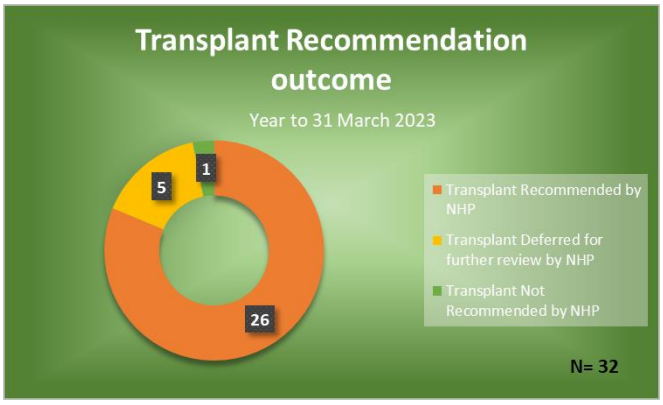


Fig 2.10

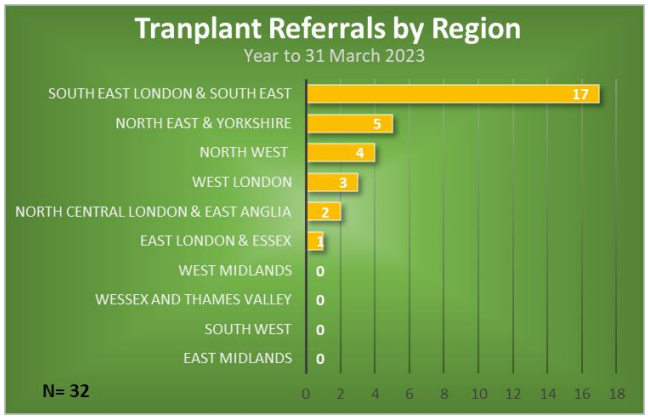


Fig 2.11

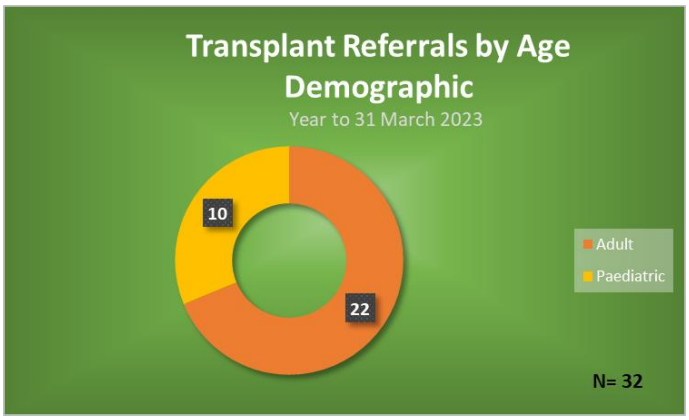
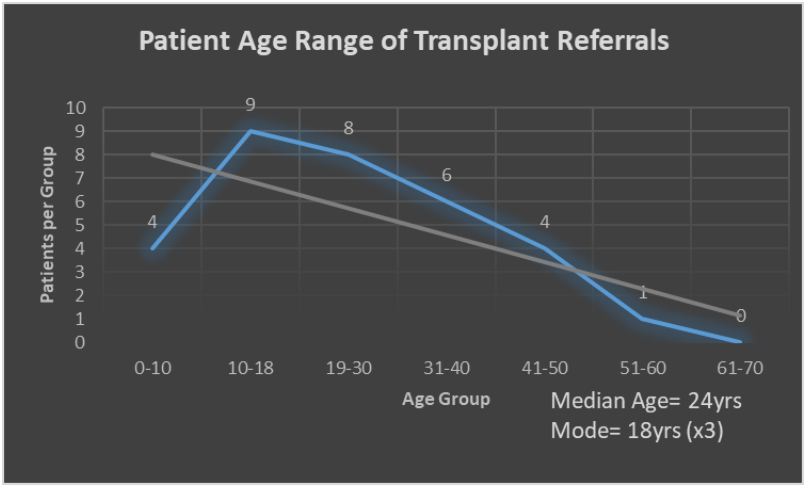


Fig. 2.12



Overview of Transplants Referred and Approved over the Years

Fig. 2.13

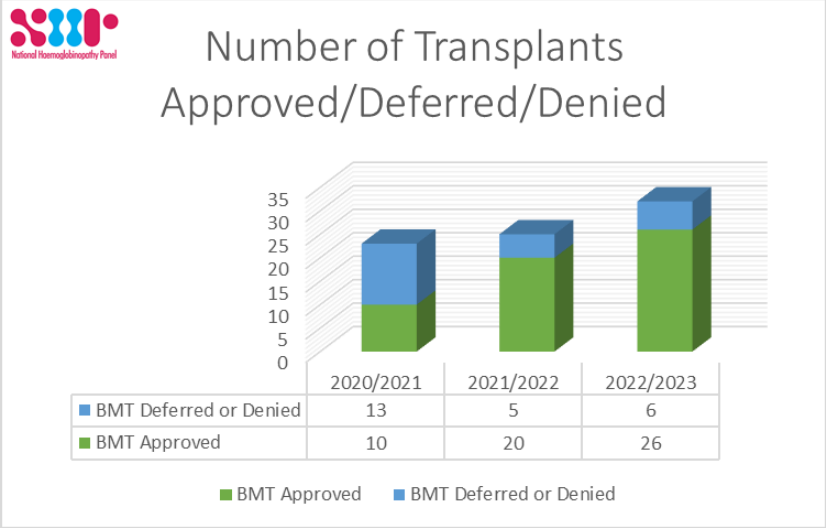


Figure 2.14

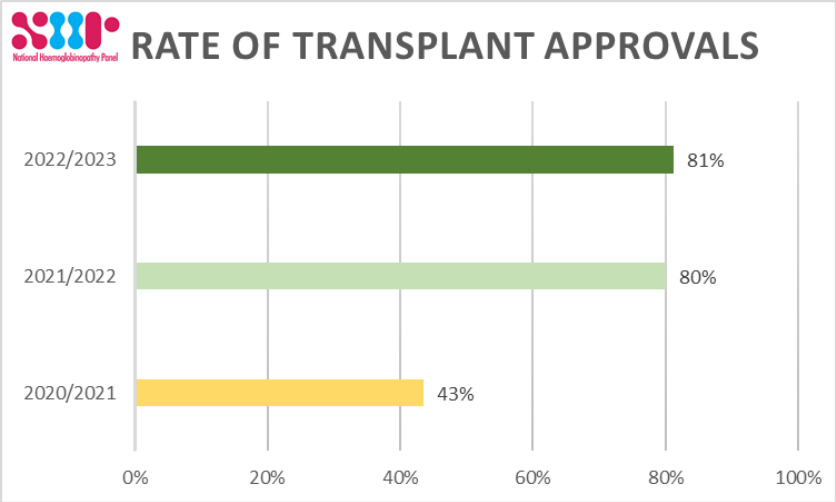


Table 2.2

	2020/2021	2021/2022	2022/2023
BMT Referred	23	25	32
BMT Approved	10	20	26

More data regarding the MDT can be found in Appendix 1.

3. MANPOWER AND STAFFING

Staffing stayed stable for the core/operational NHP team with the Chair, Deputy Chair and Operational Support officer remaining in place and re-established a steady structure and flow of processes and deliverables, as well including some developments with case summaries and indexing tor easy searching. However, as many network partners regularly report, the volume of work on small teams has not been without impact on members. NHP continue to provide data for a wealth of processes and stakeholders. This has, at times, not been without difficulty, particularly when data is not forthcoming from various other teams.

4. EDUCATION AND TRAINING

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

4.1 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.

4.2 ASCAT 2022 – IN PERSON

Following the virtual ASCAT (Academy for Sickle Cell and Thalassaemia) conference in January 2022, there followed an in-person conference, the first for a long while due to the Covid-19 outbreak. Lead by Prof B. Inusa and his ASCAT team, and in collaboration with European Haematology Association (EHA) & British Society for Haematology (BSH), the conference took place at the Park Plaza Westminster Hotel, London, on 20th – 22nd October 2022. There were almost 400 registrants participating in lectures hosted by physicians, nurses, psychiatrists, and scientists, as well as abstract and patient sessions. Topics ranged from Transplantation, Novel Therapies and Epidemiological Studies to Comprehensive Sickle Cell Care, Preventing and Managing Priapism, Breaking Bad News, Patient Experience, and many more. Speakers included a diverse range of specialists including NHP and network members with a good level of salient data to share, owing to network activities and shared agreements. There were also great insights, challenges and advances shared by international contributors.

4.3 HCC NATIONAL ROTATIONAL TEACHING

The NHP continued to support the dissemination of information regarding regional teaching as well as other learning and development opportunities.

4.4 4.4 OTHER LEARNING AVENUES

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

4.5 OWN TRAINING EVENTS

The NHP has begun the process of preparing for a curated TCD training event in June 2023. There are a number of emerging topics from MDT meetings, not on featured in the HCC rotational education roster, that have been identified for future discussion and training.

4.6 NHP EDUCATIONAL FELLOW

The NHP is working on obtaining a clinical fellow, which should augment the literature review and research/evidence base aspect of the group learning, as well as proving clinical and unique managerial training for the chose young doctor. This should bolster the overall awareness of haemoglobinopathy practice at that level and possibly direct future consultants to this field.

4.7 WORLD SICKLE CELL & THALASSAEMIA DAY 2022

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. This includes NHP-curated fact-sheets with signposting for further learning. (*Attachment 4*)

5. CLINICAL REFERENCE GROUP (CRG) FOR HAEMOGLOBINOPATHIES/NHS ENGLAND

The CRG for Haemoglobinopathies continue to engage its affiliate body, the NHP, via guidance, governance and support. The NHP reports, as well as members who are officers of the CRG, provide accounts of the progress and issues within the Haemoglobinopathy community.

During this period, Dr Subarna Chakravorty was appointed to the National Specialty Advisor role (formerly known as the Chair) for the CRG as well as other clinical CRG roles. Zoe Hamilton remains the NHS England National Programme of Care Manager – Blood and Infection, Specialised Commissioning.

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 community. For this period, this included matters such as the progress of policy

development in Cord Blood collection, Sickle Cell Year of Care Tariff, the Universal Care Plan Programme, and the Emergency Services (ED) Bypass pilot, the MedTech Funding Mandate (MFM) to expand the use of automated red cell exchange, and other matters.

The NHP, along with HCCs, underwent a compliance exercise in December 2022, carried out by NHSE, which was submitted on time.

The CRG/NHSE Commissioning are also in the process of reviewing and updating the Service Specifications for the NHP as well as HCCs and these will be impacted by the aforementioned.

Various NHSE leadership also gave updates at the NHP Business Operations/Governance meetings, regarding regional and national initiatives and workstreams for improvements in haemoglobinopathy improvement.

6. GOVERNANCE AND OTHER RESPONSIBILITIES

As noted above, the NHP sends quarterly reports as well as an annual report to the CRG and NHSE Commissioner, as well as its members.

The Biannual Business Operations and Governance meetings are attended by a specific group comprising HCC leads, subgroup and project leads, health care practitioners, such as psychologists and neurologist, and network partners. More recently, HCC operational managers have been invited as observers and input according to intra-HCC agreements and requirements. This forum remains a vital opportunity for attendees to learn from each other and identify common challenges, as well as to celebrate innovative solutions and progress. All action points are recorded in the minutes and outcomes are reviewed intermittently and in subsequent meetings, to ensure matters are not lost to follow-up. With the presence of the CRG and NHSE Commissioners, this is an opportunity to maintain accountability to, and gaining guidance from, these organisations on issues raised.

The NHP continues to support policy and evidence base building for future policies such as its dissemination and encouragement of participation in the MRI/FerriScan survey linked to standardisation of liver iron concentration assessment, which emerged from a governance meeting as raised by Dr Nandini Sadasivam. Support for other national projects such as nursing manpower review by Sickle Cell Society and NHSE has also been supported by the NHP. NHP also follows up progress for other policy work such as the paediatric sickle transplant policy adjustment lead by Professor Josu de la Fuente and Dr Banu Kaya.

This year also saw the market authorisation of Voxelotor as well as a less-than-favourable decision from NICE regarding the drug. Considering the impact on patients receiving this via Early Access to Medicines Scheme (EAMS), the NHP made concerted efforts to get external advice and to address this with clinicians and NICE.

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

The TCD QA programme, led by Dr Soundrie Padayachee, continues to progress as she and her team of regional leads continue to meet regularly and develop the project nationally, working closely with MDSAS and the NHR team. The NHP also attend and input into the periodic TCD QA team meetings and continue other interactions with this project, including, as noted previously, work on an upcoming national education event.

The project reports increasingly good data entry volumes and accuracy. MDSAS has highly developed the reporting feature and HCC activity data has been circulated to the TCD clinical leads. The 6-month report is due for circulation. Establishing sound Key Performance Indicators to ensure sound quality assurance measures has been discussed in depth. The knowledge of eligible patient numbers was a key factor, in order to identify if regions were reaching sufficient patients, hence, optimal scanning levels. This data, which would need to be provided by HCCs/SHTs, was also identified at the NHP Business Operations/Governance meeting as helpful to understand the demographics of regions and in relation to their MDT engagement.

There is an ongoing training programme for new and existing TCD practitioners both online and in-person.

8. NATIONAL HAEMOGLOBINOPATHY REGISTRY (NHR)

The NHR, led by Dr Farrah Shah, continues to oversee the development of the Registry data integrity and platform, working closely with MDSAS.

There are concerted efforts to firm up links of allocation of patients to the correct SHT and LHT, and HCCs/SHTs/LHTs are encouraged to check this section whenever patient records are accessed, to ensure they are assigned to their proper regions and categories.

There has been ongoing work with linking the NHR with NHSBT data sources, which should greatly increase the accuracy, robustness and relevance of the data and service provided by both organisations. This is particularly helpful with registering antibodies, considering Trusts which do not send this antibody data to Hematos.

The patient care plan can now be accessed nationally and the NHP receives and reviews monthly data sent from the Registry regarding Significant Complications (*Attachment 5*) and TCD scans (*Attachment 6*).

The NHR are considering the request from NHP to deposit their anonymised MDT reports on the NHR platform, but acknowledge that it would require a lot of work and need to be part of a password-protected section of the site.

9. NATIONAL SICKLE PAIN GROUP (NSPG)

The NSPG, led by Dr Sanne Lugthart, aims is 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. Telfer, 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 NSPG have one of the lead clinicians from the British Pain Society on their board, and hope to get consensus about services specifically dedicated to chronic pain in Sickle Cell Disease and perhaps Thalassaemia as well.

The NHP continues to liaise with the NSPG to support and collaborate on this pervasive issue of pain within the Sickle Cell patient cohort and how it is managed by various services. The NHP and NSPG will also work towards incorporating the benefits of the forthcoming NHP Educational Fellow in the NSPG future research endeavours.

10. NEWBORN OUTCOME SCREENING

This project is led Amanda Hogan (PHE) and also facilitated by Emma Proctor (PHE). The New-born Outcomes (NBO) system is set up to refer babies, following a screen positive result (from the newborn bloodspot programme) for SCD and Thalassaemia, to clinical services. This system has been created by MDSAS, who built the NHR platform and this system also links in with the NHR. Almost all national systems have now transitioned to this new platform.

The newborn outcome programme is set to launch an e-learning programme in April 2023 aimed at HCPs working in antenatal and new-born screening programmes.

The team has also been working with the UK Thalassaemia Society (UKTS) and the Sickle Cell Society (SCS) on a publication due out in the following financial year.

11. NOVEL AND NEW THERAPIES

Crizanlizumab
In January 2023, an update of the STAND study indicated that there was no significant difference found between the patients administered the drug at various doses and the placebo. Noteworthy is that there were also not safety events or toxicity risks evinced. The potential impact of this being of great to concern considering the patients whom did derive some benefit.

Voxelotor
Following appointing of a multidisciplinary working group from the NHP, a guideline for the use of Voxelotor was finalised and published in May 2022, which therapy had been via an Early Access to Medicines Scheme. However, the subsequent commercial licence and interim negative appraisal by NICE posed challenges to patient access. The NHP collaborated on a feedback document to NICE following a conflict of interest scoping. Concerns were raised as to the impact on patients and investment of drug research if the new Haemoglobinopathy drugs were being rejected.

Gene Editing
The Gene editing 151 study (BCL11A erythroid study, Vertex product) was opened for children of 5 – 11 years. Professor J. de la Fuente shared details and basic patient criteria, inviting the NHP MDT network to consider patients for enrolment. In light of recent ASH published data on this study in other nations, the therapy holds promise.

Tocilizumab
There are a growing number of cases citing the use of Tocilizumab in the transfusion reaction setting where there was suboptimal or no resolution with the use of Eculizumab. There is general agreement that there needs to be greater evidence base and perhaps investigations into this, with perhaps a guidance document to follow.

12. STEM CELL TRANSPLANTATION/CELLULAR THERAPY SUBGROUP

The NHP Stem Cell Transplantation/Cellular Therapy subgroup, led by Drs Ben Carpenter and Victoria Potter, continues to meet regularly.

The presence of the various members at the monthly NHP MDT has been invaluable and the NHP operational staff provide some administrative support for this group.

In addition to discussing the adults currently undergoing sibling allogeneic stem cell transplantation, there has also been collection and collation of patient process and outcome data for analysis, as the 10-patient threshold has been reached. Other clinical matters such as chimerism management, trial recruitment and immunosuppression management and weaning have featured regularly in these meetings.

Further configuration and planning for the REDRESS haploidentical trail, due to open in the early 2023/2024 period, have been taking place during this period.

Infrastructural considerations for the clinical and operational management of gene therapy approvals and protocols have also begun, particularly with regards to what parts of the pathway would be managed by the NHP and what part by this sub-group.

13. NO ONE'S LISTENING

NHP members, as well as many other clinicians, service providers and patients, were involved in the enquiry led by the SCTAPPG (Sickle Cell and Thalassaemia All-Party Parliamentary Group) that was triggered by the death of Evan Nathan Smith in North Middlesex Hospital, resulting in the *No One's Listening* report published in 2021 (*Attachment 2*). This report confirmed the failures in care for sickle cell patients in secondary care. Following the publishing of the report in 2021, the NHP and HCCs have been working towards creating awareness and fuelling implementation of the recommendations made by the SCTAPPG. Haemoglobinopathy services have been working hard, prior to and following the report, to improve the care and wellbeing of their patients. However, this report brings to the fore the key perspective that most of the changes that need to be made are outside of, and beyond, the realms of Haemoglobinopathy teams, particularly with Emergency Department protocols and training for non-Haemoglobinopathy clinical staff and wards which have substantial contact with SCD patients.

This year the NHP, continued to follow up and collate data from HCCs on the progress of Trust feedback to the recommendations from the *No One's Listening Report (2021)*. As with other areas of interaction, HCCs reported most difficulty in engaging with LHTs on this. Some positive outcomes include Trusts setting up Sickle boards, an increase in ED reform, such as sickle cell priority signage, specialist team alerts when sickle patients attend other departments, and sickle champions and liaison nurses in emergency departments.

13.1 TRUST RECOMMENDATIONS

All HCCs have brought to the attention of their respective Trusts the 7 key APPG recommendations noted below.

1. NHS Trusts to share findings of all internal reviews into incidents involving serious sickle cell care failings with the National Haemoglobinopathy Panel so that learnings can be communicated to haemoglobinopathy teams across the country.

2. All NHS Trusts to develop an action plan setting out how they will ensure compliance with the NICE clinical guideline around the delivery of pain relief within 30 minutes for sickle cell patients, with appropriate advice from the NHS England Clinical Reference Group for Haemoglobinopathies pain sub-group.

3. Royal College of Emergency Medicine and Royal College of Physicians to develop guidance for staff working in A&E and on general wards making clear that sickle cell patients should be prioritised for treatment as a medical emergency due to the high risk of fast medical deterioration, to be distributed by NHS Trusts.

4. All NHS Trusts to require that haematology teams are informed whenever a sickle cell patient accesses or is admitted to the hospital to ensure the patients clinical history is known and advice can be passed on regarding their care, with compliance reported via the NHS England and NHS Improvement specialised services quality dashboards.

5. NHS Trusts to develop individualised care plans for, and in partnership with, each sickle cell patient, with the patient and any relevant carers provided with a copy of the plan.

6. NHSEI to require NHS Trusts to conduct and report regular audits of patient involvement in decisions about their care, utilising patient feedback in line with NICE clinical guideline stating that sickle cell patients (and their carer) should be regarded as experts in their condition.

7. All NHS trusts to ensure that specialised service funding is invested in meeting recommended sickle cell service staffing numbers.

14. NETWORK PARTNERS

The NHP continues to value and encourage optimal engagement with its network partners with their varied perspectives and expertise which only enrich the work within haemoglobinopathies.

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

The NHP engage closely with the SCTAPPG in order to provide a joined-up and robust perspective of the national haemoglobinopathy landscape to effectively bring attention and solution to the areas the APPG is best placed to address and ameliorate. A working example being the 5 Priority points exercise that the NHP carried out in January 2023, to ascertain the 5 most crucial points that the network felt were most vital for the APPG to get behind, amongst various Recommendations set out in the *No One's Listening* Report (2021) (*Attachment 7*). The NHP presence at this meeting is also a good opportunity for patient representatives and groups to address relevant matters and raise any concerns with policy-makers.. The NHP participation in these forums allows other stakeholders to engage in service changes and provides a national platforms for interaction .

14.2 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. As such, their knowledge, perspective, reach and support has been a benefit to the NHP.

Their input on the management on reporting deaths, reviewing the eligibility and parameters that guide SHT status, and potential collaboration on a prison policy, have been vital considerations, which they have contributed to the NHP forums.

The NHP has supported their initiatives of national benefit, such as their Automated Red Cell patient enquiry produced in collaboration with Patient and Public Involvment Team, Innovative Research and Life Sciences Group and NHS England, which underscored the MedTech Funding Mandate.

14.3 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.

The UKTS input during the NHP meetings is another valuable patient perspective and clinical overview that adds much value to the attendees. Their work with patients in the North of England has been particularly insightful as the long-standing patient challenges in that area would not have been otherwise known due to patient distrust of the clinical and operational staff in Trusts and hence abstinence to speak freely at listening events by those teams. The UKTS also works with clinical teams to get to the source of the issues and help build understanding between the patient and NHS groups.

In this period, amongst the many outreaches, training and awareness events and advocacy actions, the UKTS also published a standards document and patient/parent handbook. A lot of EDI (Equality, Diversity and inclusion) material has been developed and is available on their robust website.

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

STANMAP held a successful half-day educational day on Thursday 29th June 2023, which was attended by over fifty participants. There were updates from the Sickle Cell and the UK Thalassaemia societies as well as an update on the RCN Competencies for Haemoglobinopathy nurses by Sekayi Tangayi - Consultant Nurse North Middlesex University Hospital. There followed open forum discussions from members regarding developments and work-related issues in their local areas.

STANMAP members are participating in the Sickle Cell Nursing Workforce Project led by Aiden Rylatt – for the Sickle Cell Society.

There is currently in development a Sickle Cell & Thalassaemia Witness Seminar which is connected to a Wellcome-funded project (121648/Z/18/Z) run by Jenny Bangham, a historian based at Queen Mary University of London. J. Bangham is looking broadly at the history of Genetic Counselling. Following an interview with Dr Lola Oni, J. Banham agreed to embark on tracing the history of sickle cell and thalassaemia counselling, as part of the broader project on the history of genetic counselling in the UK. This is a RCN/STANMAP joint venture.

14.5 UK FORUM FOR HAEMOGLOBIN DISORDERS (UKFHD)

The UKFHD is a multidisciplinary body of experts, led by Dr Farrah 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.

In addition to educational events and clinical and professional enhancement, the UKFHD have been supporting the UKTS to complete chapters of the revised peer review standards, which

are now live. During this period there were invitations for new committee members including the role of Vice Chair demitted by Professor P. Telfer.

15. HAEMOGLOBINOPATHY COORDINATING CENTRE (HCC) UPDATES

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

The Business Operations/Governance meeting serves as forum for HCC leads to share updates, learning, challenges and achievements.

HCCs have shared great initiatives to improve services and overcome challenges. As such, the MDT and Business Operations/Governance meetings are a great resource.

Some common threads running through the HCC reporting, in terms of challenges, has been suboptimal experiences for patients presenting at Emergency Services, insufficient staffing and funding in clinical and operational red cell teams, the need for increased Thalassaemia awareness and service improvement, racism, and LHT engagement. There was also a weariness towards data requests expressed by HCCs.

Important outcomes and achievements included increased feedback from Trusts regarding the *No One's Listening* report (2021), very relevant and high-quality learning events and materials produced by HCCs/SHTs, incorporation of training sessions into network meetings to optimise attendees' presence. ED champions and regional education events have also featured positively in the regional reports.

Further details of each HCC update can be found in the Business Operations and Governance meeting minutes for May 2022 and November 2022. (*Attachment 8*, *Attachment 9*).

16.NHP LOOKING FORWARD

-The NHP has begun discussions towards a obtaining a Clinical Fellow in the coming year. It is hoped that this will create greater awareness and succession potential within the red cell clinical cohort as well as help with the data management, research and operational processes.

-Planning has begun for a TCD training event to take place in the upcoming quarter

-Greater engagement and collaboration with HCCs will be worked towards, especially for greater understanding and support for the ultimately shared roles and benefits.

-Greater clarity, access and monitoring of NHP funds in order to better respond to development needs and transformation projects such as the website overhaul and to end the ongoing personal funding of costs relating to its running.

APPENDIX

APPX1. MDT METRICS 2022/2023

2022/2023 - Year to Date					
	Q1	Q2	Q3	Q4	Total
MDT Cases	11	**17**	**14**	20	**62**

Quarter 4 - 2022/2023				
Months	Jan	Feb	Mar	Total
MDT Cases	**4**	**7**	**9**	**20**

HCC Region	Q1	Q2	Q3	Q4	2022/2023 Year to Date
East London & Essex	**0**	**0**	**3**	**1**	**4**
East Midlands	**2**	**0**	**1**	**1**	**4**
North Central London & East Anglia	**0**	**1**	**0**	**2**	**3**
North East & Yorkshire	**1**	**4**	**2**	**4**	**11**
North West	**2**	**2**	**1**	**2**	**7**
South East London & South East	**4**	**7**	**3**	**5**	**19**
South West	**0**	**1**	**1**	**1**	**3**
Wessex and Thames Valley	**0**	**1**	**1**	**1**	**3**
West London	**1**	**0**	**2**	**2**	**5**
West Midlands	**1**	**1**		**1**	**3**
NHP Total	**11**	**17**	**14**	**20**	**62**

Adult/Paediatric Split	Q1	Q2	Q3	Q4	2022/2023 Year to Date
Adults	**8**	**9**	**11**	**12**	**40**
Paediatric	**3**	**8**	**3**	**8**	**22**

Case Diagnosis	Q1	Q2	Q3	Q4	2022/2023 Year to Date
HbSS	**10**	**13**	**10**	**17**	**50**
HbSC	**0**	**0**	**1**	**1**	**2**
Thalassaemia	**0**	**2**	**0**	**1**	**3**

Rare Anaemia	1	2	3	0	6
Other	0	0	0	1	1

Primary Theme	Q1	Q2	Q3	Q4	2022/2023 Year to Date
Stem Cell Transplant	6	11	4	11	32
Novel Treatment/New Therapy	3	4	1	1	9
Mortality Review	1	1	2	2	6
Urgent (email) Case	2	1	1	1	5
Complex Haemoglobinopathy/Comorbidity	2	3	7	7	19
IVIG Therapy	2	0	0	0	2

Transplant Cases	Q1	Q2	Q3	Q4	2022/2023 Year to Date
Sibling Matched	4	6	3	9	22
Haplo Matched	2	4	1	1	8
Matched Unrelated Donor	0	1	0	1	2

Transplant NHP Outcome	Q1	Q2	Q3	Q4	2022/2023 Year to Date
NHP recommended to refer	6	9	3	8	26
NHP - **not** recommended, repeat review **** n1***	0	2	1	2	5
NHP - **not** recommended	0	0	0	1	1
Successful Referral Rate	100.0 %	81.8 %	75.0 %	72.7 %	81.25%

Novel Use/New Therapy Confirmed Instances of Use reported to NHP	Q1	Q2	Q3	Q4	2022/2023 Year to Date
Eculizumab	1	1	1	0	3
Rituximab	0	0	0	0	0
New Therapy	0	0	0	1	1
Total Instances of Use * *n2*	1	1	1	1	4
Retrospectively Referred **** n3***	1	0	1	0	2
Retrospectively Referred %	100%	0%	100%	0%	50%

*** 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.

ATTACHMENTS

Attachment 1	NHP Governance and Responsibility 21-22	 A1 - NHP Gov & Resp 21-22.pdf	*Return to reference paragraph*
Attachment 2	No One's Listening	 A2. No-Ones-Listening-Fi	*Return to reference paragraph 2a* *Return to reference paragraph 2b*
Attachment 3	NHP MDT Survey 2022/2023	 A3. Survey2022 NHP MDT2 - Summary.pdf	*Return to reference paragraph*
Attachment 4	World Sickle Day 2022	 A4. World Sickle Day-Did You Know2022_C	*Return to reference paragraph*
Attachment 5	NHR Significant Complications Report to 1 April 2023	 A5. NHR_Significant_Com	*Return to reference paragraph*
Attachment 6	NHR Report on TCD Scans, Annual to 1 April 2023	 A6. NHR_TCD_Scans_Rep	*Return to reference paragraph*
Attachment 7	NHP 5 Priority Points Analysis	 A7. NHP APPG 5 Priority Points Feedba	*Return to reference paragraph*
Attachment 8	NHP Business Operations/Governance Meeting minutes – April 2022	 A8. Business Operations Minutes 2	*Return to reference paragraph*
Attachment 9	NHP Business Operations/Governance Meeting minutes – Nov 2022	 A9. Minutes - NHP BusOp_Gov 2022111(	*Return to reference paragraph*