NATIONAL HAEMOGLOBINOPATHY PANEL

Annual Report 2024/2025

Chairs:

Professor John Porter (April-Dec 2024)

Dr Kate Gardner (Jan-Mar 2025)

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HAEMOGLOBINOPATHIES COORDINATING CENTRES (HCC) & THEIR ABBREVIATIONS

SICKLE CELL DISORDER HCCs

North West (NW) HCC
North East and Yorkshire (NE&Y) HCC
East Midlands (EM) HCC
West Midlands (WM) HCC
East London and Essex (EL&E) HCC
South East London and South East (SELSE) HCC
West London (WL) HCC
North Central London and East Anglia (NCLEA) HCC
Wessex and Thames Valley (WTV) HCC
South West (SW) HCC

THALASSAEMIA HCCs

North HCC (managed with North West SCD HCC)
Midlands HCC (managed with West Midlands SCD HCC)
London and South East HCC (managed with East London & Essex SCD HCC)
London, South Central and South West HCC (managed with and North Central London and East Anglia SCD HCC)

EXECUTIVE SUMMARY

The National Haemoglobinopathy Panel (NHP), established January 2020, is a means to actualise the aims of NHS England within the realm of red cell disorders, as part of a new model of care for haemoglobinopathies Sickle Cell Disorder (SCD) and Thalassaemia and Rare Inherited Anaemias (RIA). The main aim being to galvanise improvement and equity in services and patient experience in the haemoglobinopathy community. Further, to support access to specialist services and clinical expertise, aiming to provide equitable access across the country.

The NHP 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 (MDT) meeting, with membership drawn from all the HCCs, provides timely advice on complex cases that need access to a wider range of expertise or opinion that may not be available to the SHT/HCC in question. The MDT is also a place for assessment and approval of high-cost therapies, such as stem cell transplantation, and evidence building and remunerations for treatments, as with novel use of drugs such as Eculizumab for transfusion reactions.

The year 1 April 2024 to 31 March 2025 witnessed increased engagement with MDT activities - mainly due to the emergence of gene therapy – requiring sound management of demand for each session. The NHP provides a platform for data collection/collation between NHS England/the Haemoglobinopathies CRG, and HCCs/Specialist haemoglobinopathy Team (SHT)/Local Haemoglobinopathy (LHT) network, particularly for patient and staff feedback. Said feedback has fed into national and local service improvement endeavours.

The NHP also serves as a conduit for coordinating and disseminating communication and actions nationally, as well as experience sharing and consensus on best practice and addressing emerging issues.

All these and more, discussed further in this document, show how the organisation carries out its 4 key objectives summarised in Fig. 1. below, to improve the haemoglobinopathy landscape.

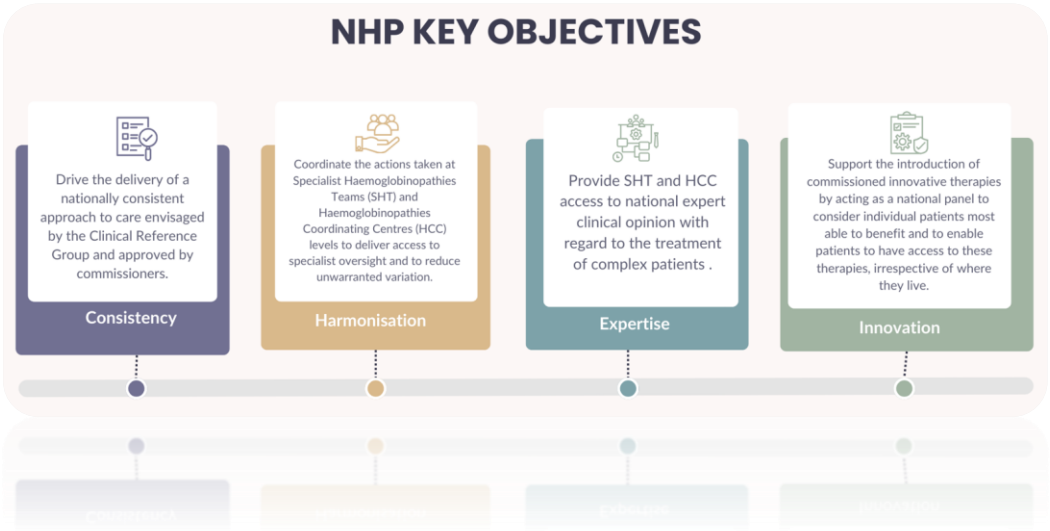


Fig. 1 NHP Key Objectives

Framework & Governance

The NHP reports to the Haemoglobinopathies Clinical Reference Group (CRG) and NHS England Specialised Commissioning (National Programme of Care for Blood and Infection).

Key changes in this group involve the Deputy Chair, Professor John Porter, taking on the additional role of Acting Chair, while recruitment of a new Chair was underway, following the departure of Professor Baba Inusa, founding NHP Chair. Dr Kate Gate Gardner took up the official post of NHP Chair in January 2025.

It was announced that NHS England would be abolished in March 2025. It is unclear where the NHP will sit in the future and in particular if it will be subsumed into the DHSC.
In the year 2024/2025, the NHP continued its role providing an expert input forum via the monthly MDT, and as an action and communication channel between the CRG, HCCs and various other stakeholders.

NHP MDT

The monthly national MDTs, which are a core function of the NHP, have continued with healthy engagement of all HCCs, and provides expert input across patient 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 and high-cost treatment evaluations/approvals. A total of 92 cases were discussed in 2024/2025, a 37.3% increase from the 67 total in 2023/2024. These referrals, as in the previous year, were dominated by stem cell transplant and the new feature of gene therapy cases. Other overarching clinical, professional and operational matters of national import were also discussed at these meetings, particularly with the new Chair including an operational slot for each MDT since January 2025.

Education & Training

The NHP recognises the key role that learning and education plays in addressing inequalities and improving the provision of a quality haemoglobinopathy service. The MDT is recognised to provide some of this. The NHP also serves as a conduit for sharing learning activities, both by email distribution and the options of HCCs listing events on the NHP website.

The globally renowned annual ASCAT (Academy of Sickle Cell and Thalassaemia) conference, which faculty features various members of the NHP, including ASCAT founder/former NHP Chair, Prof Inusa, was made available to many network staff and patients, at varying levels of discounted entry.

Policy & Guidelines

The NHP continues to work collaboratively with, or as a support to, its delegated clinicians in various policy and guideline developments and changes. In particular, (1) the standardisation of investigations for Liver Iron Concentration (LIC) project and (2) building of evidence towards editing guidelines for the use of immunomodulatory treatments including Eculizumab and Tocilizumab.

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. This financial year, the NHP welcomed the DBAS UK (UK's Diamond Blackfan Anaemia Syndrome charity).

Key Achievements 2024-2025

- Ongoing MDT enlargement
    - 37% increase in MDT case numbers in 2024-2025 compared to 2023-2024
    - 100% increase in case numbers in 2024-2025 compared to 2020-2021
- Build an active database of non-haematology clinical experts (hepatology, obstetrics, etc.)
- Introduction of gene therapy case review for thalassaemia and sickle cell.
- Development of gene therapy forms
- Cellular Therapies Group (CTG) - Terms of Reference agreed.
- Recruitment of a new Chair for NHP
- TCD – Power BI analytics
- National acute clinical management issues
    - Managing the withdrawal of voxelotor from the market
    - Managing the amber alert for red cell stocks
    - Platform for sharing med tech funding application advice
- Website overhaul – share learning opportunities and resources

1. NHP FRAMEWORK

The NHP carries out its duties in line with the commissioning precepts and aims outlined in the NHS England Responsibilities and Governance 2021/2022 by document, as well as its Terms of Reference which is currently being updated. These aims are 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 increasingly vital role played by the Integrated Care Boards in their role of driving the HCC/SHT initiatives forward, particularly within community services, and in future the likely commissioners of red cell services.

At the national MDT meetings (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. This forum is also an avenue to identify challenges and trends, as well as spotlighting, and/or agreeing consensus in approach and best practice. Panel members also work together outside of MDT meetings (as occurred in identifying and the 2024 wave of Parvovirus infections), creating a working group, which developed an exploratory survey to monitor incidences and severity nationally.

NHP continues efforts to engage at the national HCC Managers meetings for better understanding of issues at hand and potential support. 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 an equitable flow of information, contact and oversight with HCCs and organisations within the network.

Core Structure

The core operational group comprises the Chair, Deputy Chair and Operations Support Office. The core panel membership is made up of 41 haemoglobinopathy experts from all HCCs, including HCC Clinical leads and their deputies, as well as Clinical Nurse Specialists (CNSs), Transplant clinicians, Psychologists and Scientists. There are also 149 registered

Observers who join the monthly MDTs and a pool of listed non-haemoglobin experts (Hepatology, Neurology, Obstetrics) who can be called on for specific cases.

The organisation's comprehensive framework continues to see cohesion and 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 NHP member representatives across the regions (Fig 1.a).

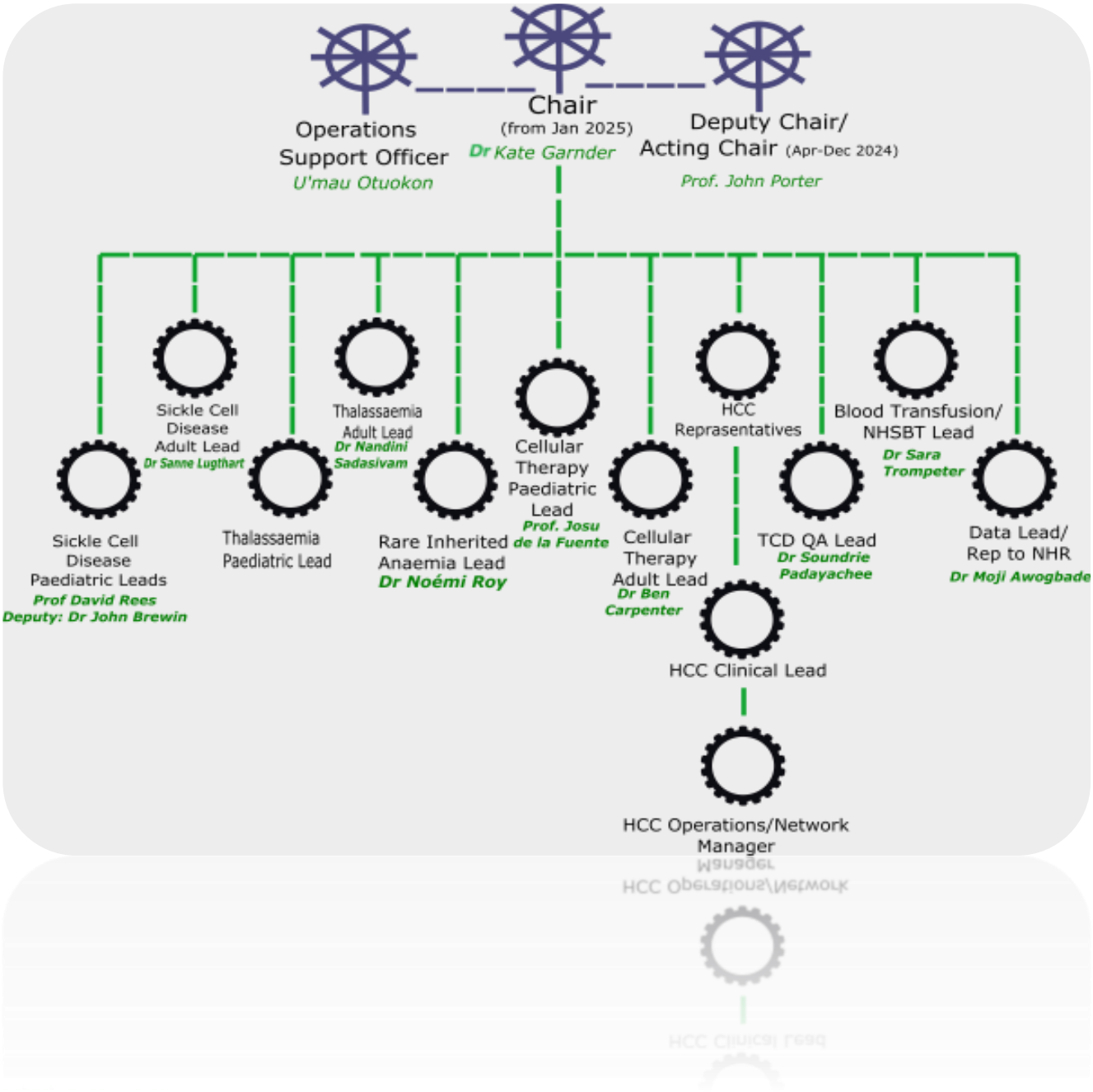


Fig. 1.A. Core NHP structure

Wider Network

The external network of 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 work with all healthcare professionals and empower the patient voice and experience. During this period, the NHP also welcomed DBAS UK, the UK's Diamond Blackfan Anaemia Syndrome charity to its wider network. The central positioning of the NHP with the HCCs, enables it to offer and relay key national 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.

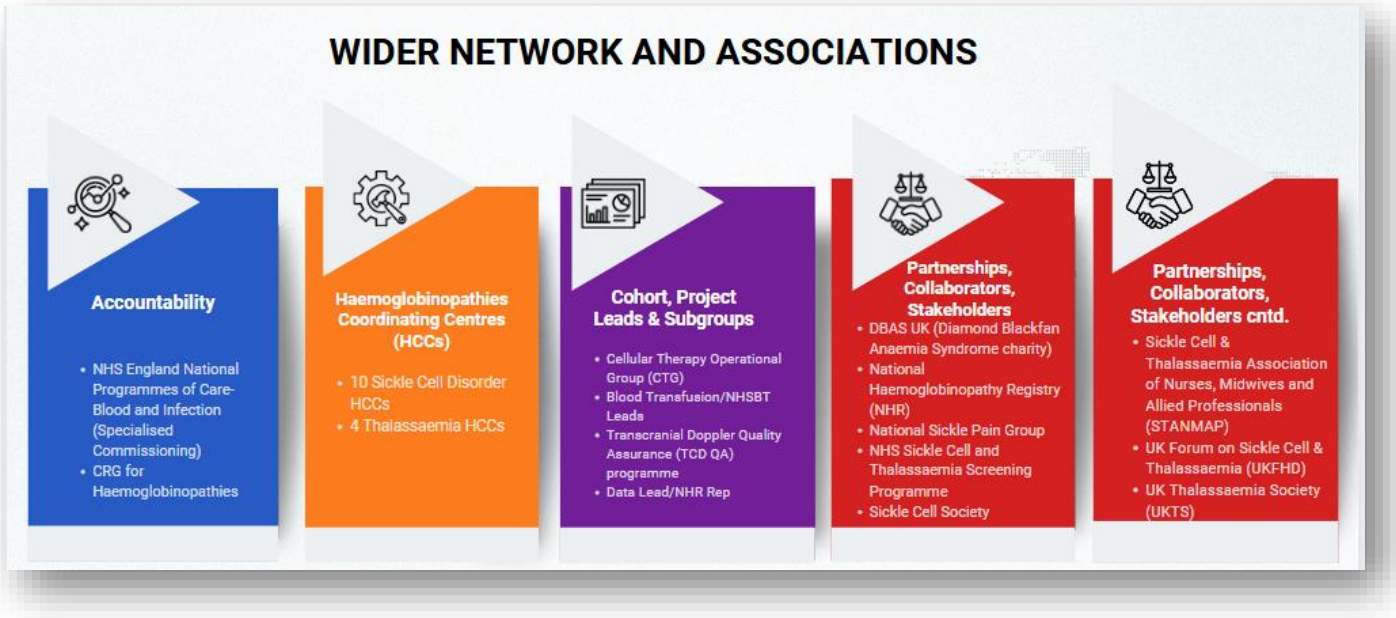


Fig 1.B. NHP Wider Network

2. THE NATIONAL MDT MEETING

Consistency | Expertise | Harmonisation | Innovation

2.1 OVERVIEW

Capacity

All 12 scheduled monthly MDT meetings took place as planned in 2024/2025, with 4 additional urgent email MDTs, resulting in 92 cases discussed for the year- a 37.3% increase on the 62 cases reported in 2023/2024. Each quarter reported increases in cases discussed. The issues of managing meeting demand capacity reported in 2023/2024 was thus compounded in 2024/2025, particularly with the authorisation of gene therapy, added to the historical trend of stem cell transplantation being a high demand factor. Of the various solutions considered, including longer meetings (>2hrs), a key approach initiated towards the end of this period was referral form revisions to create a more streamlined referral process and increased discussion focus. Inclusion of ringfenced slots and waiting lists also helped manage cases and allow dynamic case management. The impact on back-end processing that undergirds all this is pending amelioration.

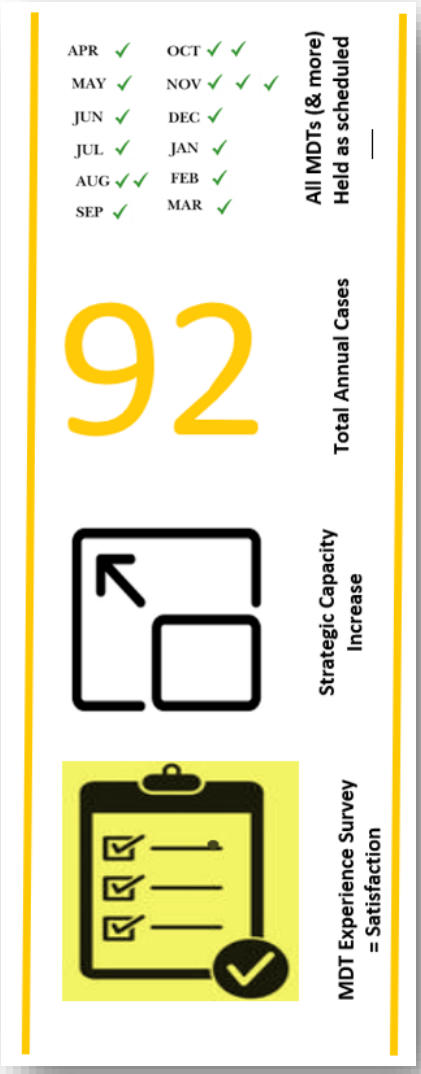


Fig. 2A Overview Highlights

Expert Input

The presence of varied clinical specialists such as psychologists, neurologists, nurses and clinicians from the paediatric and adult subspecialties makes for 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 the decision-making as evidenced by the large number of referrals for curative therapies; the NHP remains indebted for their expertise. Dr Kate Gardner began the process of updating details of invited specialists (e.g. neurologists, neuroradiologist & hepatologists).

The MDT Experience

A national MDT Survey was carried out in March 2025, regarding participants' experience of the MDT over the financial year.

- Amongst all responders, there was an affirmative expression as to the relevance and usefulness of the MDT to their clinical specialty, their patients' clinical care and access to specialist input.
- Majority of responders agreed that timelines for sharing of NHP updates and reports are good, with 1 responder in slight disagreement.
- While about 80% feel attendee engagement is appropriately open for their input, relaxed and respectful, there were some responders who disagreed or had no opinions either way.
- Also expressed was a desire for more variation in cases discussed, noting the dominance of cellular therapy cases each month.
- There was 100% overall satisfaction, to varying degrees, with responders' experience

Matters Arising and Learning

Over this period, the NHP MDT included various non-patient case discussions and consensus on trends, concerns, and other emerging topics. A helpful practice, which the new Chair, Dr Kate Gardner, established in January 2025, was including an operational element at the start of the meeting, allowing for matters of national import to be broached or fully discussed. The paragraphs below give examples of just some topics that occurred at the MDT meetings over the year in question.

Diversity of Topics

Clinicians were, on occasion, encouraged to submit a variety of cases for discussion, which featured a broad spectrum of patient cohorts, though, the majority of patients discussed had a diagnosis of homozygous sickle cell disorder.

Mortality Reporting

Mortality reporting has been a regular topic, in the MDTs and Business Operations & Governance meetings, with agreement that more cases eliciting learning could be brought to the NHP while clear parameters were pending clarification.

Reporting and Evidence building for Transfusion Reaction Treatments

A regular topic of discussion over the years, including this (2024/2025) period, has been the publication and reporting on SHOT, for cases where novel use of drugs such as Eculizumab, Rituximab and Tocilizumab, were implemented for treating transfusion reactions. These publications would also help towards the potential amendment of the commissioning policies which some clinicians have indicated are needed, particularly for the prophylactic use of Eculizumab.

Ophthalmology and Acetazolamide Use in Sickle Cell

In June 2024, a representative of Moorfields Eye Hospital Glaucoma service presented and initiated discussion on the uncertainties of impact and aftercare for Sickle Cell Disorder and Sickle Cell Trait patients treated with Acetazolamide. It was concluded that there is a small risk of triggering VOC (Vaso-occlusive Crises) with this drug, but the risk of losing sight must be weighed against the onset of a sickle crisis. The risk in sickle cell trait is very small. It was agreed that the Ophthalmology team would put together a draft patient information document confirming each patient's receipt of Acetazolamide treatment and include details of whom to contact (including haemoglobinopathy teams) if adverse symptoms occur. General outcomes from the meeting will inform SOP/policy for Moorfields and, possibly, a future national policy. NHP agreed to review the initial draft for further comment.

Cord Blood Collection

A Cord Blood Collection issue was brought to the Panel to have an open discussion for a consensus on the value of pursuing this policy. In summary, it was understood that there is generally little appetite among NHP members for this to be pursued as a policy or viable option for potential patients, and that funding and the effort required might be better routed to other areas of need.

Blood Stocks: Amber Alert July 2024

In July 2024, an extraordinary meeting was called and chaired by Dr S. Chakravorty, to gain a national understanding and concerted response regarding concerns about blood stocks, including shared experience from the King's College Hospital (KCH) and Guy's & St Thomas'

(GSTT) teams, who had been implementing alternative pathways for transfusions in the midst of a Synnovis cyber-attack and the subsequent Pre-Amber Alert for blood stocks. This meeting received input from the NHSBT Medical Director for Transfusion (Dr L. Estcourt), and some interventions included prioritizing on patient severity to decide delayed or reduced unit transfusion, depletions, moving some from exchanges to top-ups, and adjusting target HbS levels. This meeting was positively instructional.

Fig. 2.B. MDT Emerging Topics and Other Discussion Points

2.2 HIGHLIGHTS FROM MDT ACTIVITY DATA

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. The largest increments were between 2021/2022 to 2022/2023 (+51.21%) and 2023/2024 and 2024/2025 (37.2%)

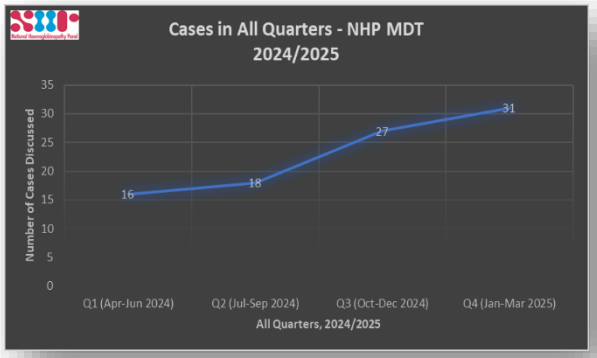


Fig. 2.C. Incremental Caseload Per Quarter

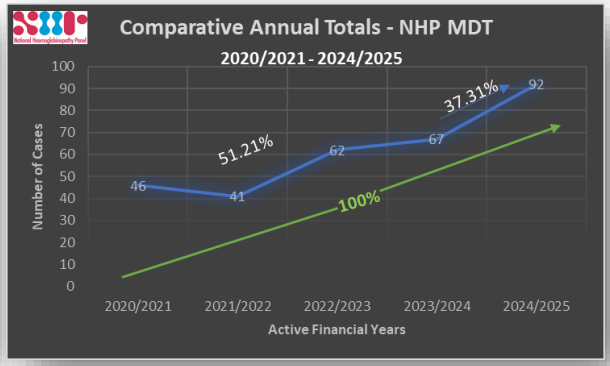


Fig. 2.D. Incremental Caseload Per Year

Referrals by region: The highest number of referrals came from North East & Yorkshire (NEY) HCC (17) and South East London & South East (SELSE) HCC (17). Noteworthy is that referrals which would normally be from North West HCC were registered under the Thalassaemia North HCC for Gene Therapy for Transfusion-dependent Thalassaemia (TDT) patients. Similarly, clinicians who normally referred from West London HCC now referred via the London South, South Central & South West Thalassaemia HCC.

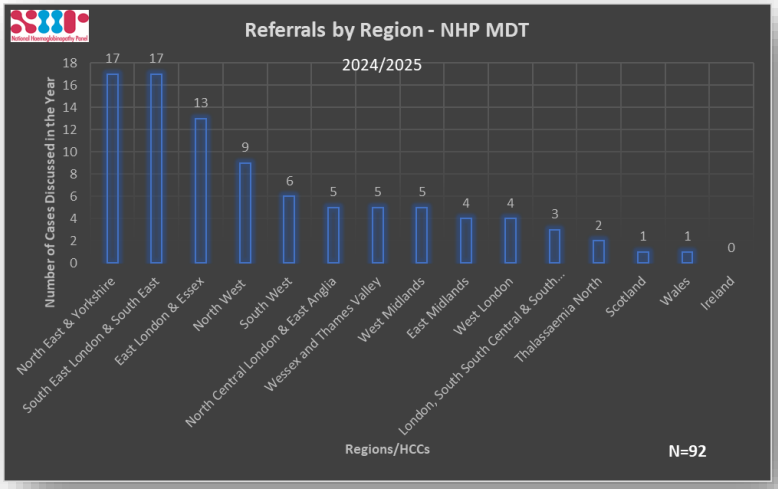
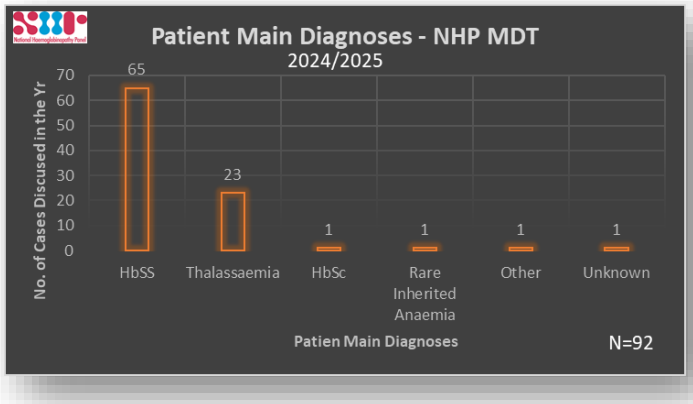


Fig. 2.E. Referrals by Region

Patient Main Diagnosis & Demographics: Patients with a homozygous sickle cell disorder (HbSS) were the majority of patients referred to the NHP MDT (65/92), in keeping with all previous years. There were 23 thalassaemia patients referred, which is a stark increase from 4 cases reported in 2023/2024. Nineteen (19) of



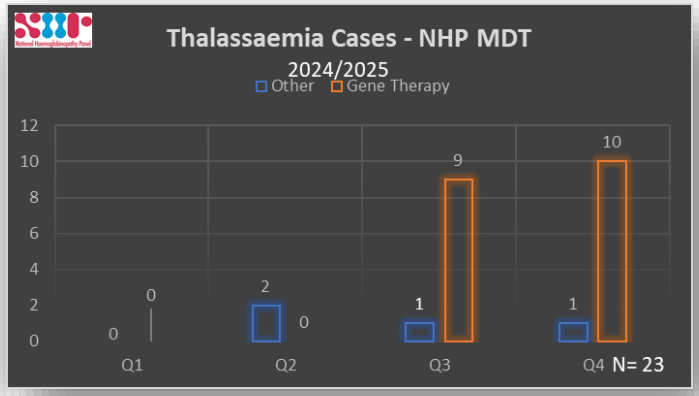


Fig. 2.G. Thalassaemia Incremental Trend per Quarter

the thalassaemia cases were for gene therapy approvals. In a change from 2023/2024, which had adult and paediatric referrals fairly on par, 2024/2025 received markedly more adult referrals than paediatric referrals (57:35). The age range most referred to the NHP MDT was 21-30 years of age, which had 27 referrals, followed by 11-16 with 20 referrals (Fig.2.I).

Referral Themes:

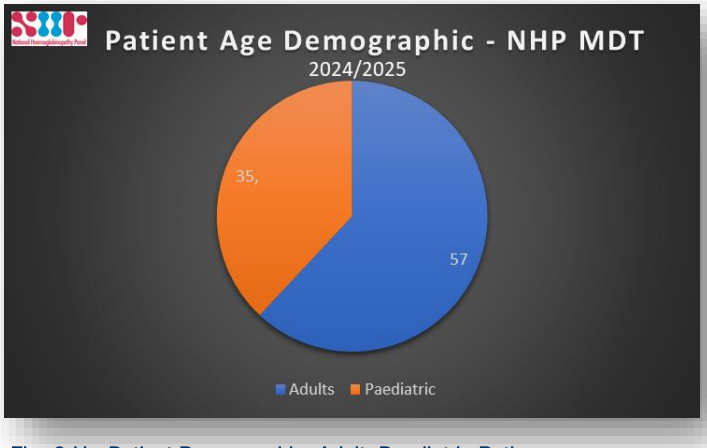


Fig. 2.H. Patient Demographic: Adult: Paediatric Ratio

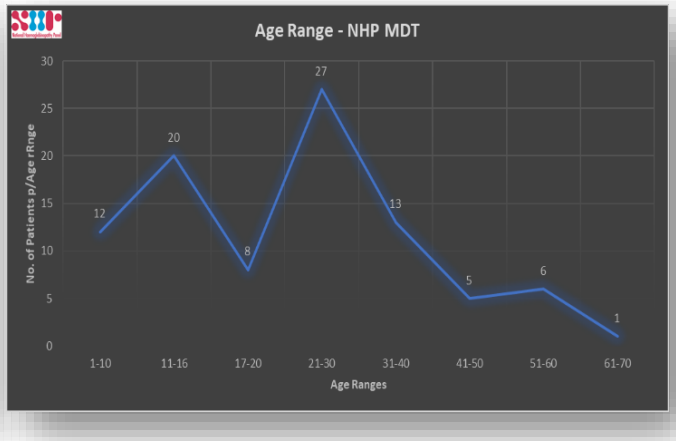


Fig. 2.I. Prevalence of Referrals by Age Range

Referrals for Haemopoietic Stem Cell Transplantation (HSCT) made up the highest category of referrals (40). However, despite gene therapy only being approved in August 2024 (with first referrals received in Sep 2024), gene therapy is the second highest in demand, at 25 cases.

- Complex haemoglobinopathy/comorbidity cases are the third highest theme with 12 cases.
- Reporting of novel use of drugs (Eculizumab etc. for transfusion reactions) is down at 9 from 12 instances in 2023/2024. Two cases reported in 2024/2025 were the bases of the urgent email MDTs, which patients were stabilised at the time of discussion.
- Mortality increased to 8 cases from just 3 in 2023/2024.

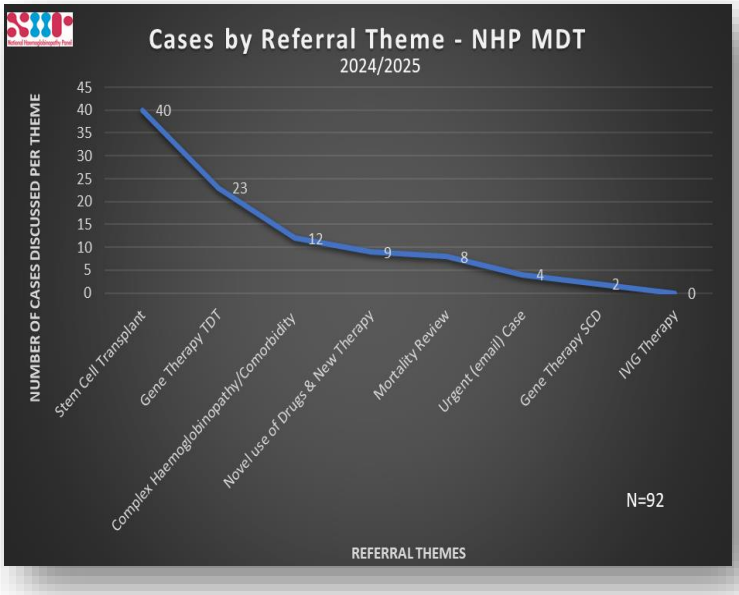


Fig. 2.J. Cases by Referral Theme

Mortalities:

Following many discussions and encouragements over the years, to encourage mortality discussions, there has been more engagement in this area with 8 cases submitted. The difficulties for the patient, family and involved staff in each sad and unfortunate case was a key factor taken on board.

- North West HCC presented a case in April 2024 which had underlying cerebrovascular involvement in a patient with sickle cell. This was a complex paediatric case where the parents did not disclose full medical history. The panel discussed the importance of good transfer of care documentation when patients move red cell centres. The panel also discussed the importance of communicating risk to patients/families when they decline certain treatments. It was also noted that escalation of pain relief, such as to ketamine, should have triggered senior clinician evaluation.

- South East London & South East HCC presented a mortality case in June 2024 in the context of severe DHTR/hyperhaemolysis in a patient with sickle cell. The panel sympathised with the patient's family and clinical team but acknowledged no omissions in care were identified.
- In August 2024 East London & Essex HCC presented two distressing mortality cases related to severe transfusion reactions in young adults with sickle cell. In both cases, there was a very rapid clinical deterioration which was distressing for the families – as well as the clinical team. The Panel noted there is no evidence around when to transfuse again in these severe cases. The Panel noted that the clinical team did everything possible with no identified omissions in care in either case. The team has used these cases as learning exercises locally. The Panel also acknowledged the need for more black blood donors to help with complex sickle transfusion cases.
- December 2024 South West HCC shared a mortality case in the context of recent second trimester pregnancy loss. The patient (female) had progressive sickle liver disease, and her case was reviewed by regional and national liver teams. It was acknowledged that patients with sickle liver disease tend to have poor outcomes and it was felt earlier referral to a liver team would not have changed her outcome. It was noted that King's College Hospital is progressing with a National Sickle Liver Centre and are drafting referral pathways for an MDT and specialist clinic. The Panel also acknowledged the multiple aspects to her case (pregnancy loss, progressive liver disease) that is not uncommon in sickle cell, unfortunately, but that can make clinical communication harder.
- Wessex and Thames Valley (WTV) reported an expected death of a patient with sickle cell in January 2025. It was a reminder of the positive role of palliative care in sickle cell.
- Wessex and Thames Valley (WTV) presented two mortality cases in February 2025.
    - One case of a patient with sickle cell who had an expected death. There were different speciality teams involved in multidisciplinary discussions, including palliative care and the benefits of this was recognised.
    - The second case was an unexpected death in a patient with transfusion-dependent β-thalassaemia. The death was unrelated to thalassaemia. However, the Panel noted the importance of good clinical communication and transfer of care when a patient moves between areas, and between private and NHS practice.

Main Presenting Themes: Patients presented with a wide range of conditions, but transfusion-depended thalassaemia accounted for the majority of patient main presentations (23) which correlates with the gene therapy demand beginning in Q2, whereas in 2023/2024, cerebrovascular disease was the highest presenting theme.

Patients with severe/frequent VOC/ACS despite disease modifying treatment were the second largest presenting cohort with 21 patients.

Cerebrovascular/Neurovascular disease was the third highest presentation at 12, followed by Complex haemoglobinopathy presentation in 9 patients.

Presenting Themes	No. of patients per theme
Transfusion-dependent Thalassaemia	23
Severe/frequent VOC/ACS despite DMT	21
Cerebrovascular/Neurovascular Issues/Disease	12
Complex Haemoglobinopathy presentation	9
Mortality	8
Transfusion reaction (DHTR/HH etc.)/Ecu/Ritux/Toclzmb use	8
End Organ Damage	5
Other	4
Transfusion difficulty (iron overload/chelation, venous access)	2
TOTAL	**92**

Table 2.1. Patient Presenting Themes at NHP MDT – 2024/2025

The 'other' category comprises splenomegaly/splenic sequestration cases and obstetrics.

CELLULAR THERAPY REFERRALS

As previously noted, though stem cell transplantation (HSCT) referrals were the highest referral category for the year, gene therapy (GT) referrals outstripped HSCT referrals in quarter 4. This trend will be watched over the coming months.

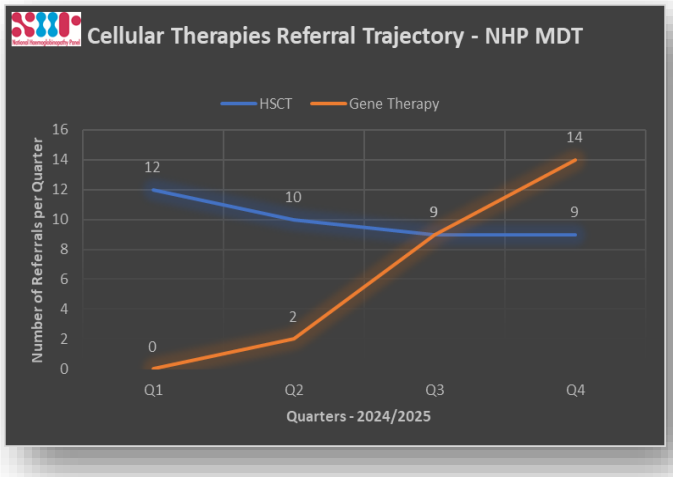


Fig. 2.K. Trajectory of Cellular Therapy Referrals

Adult and Paediatric HSCT & GT referrals

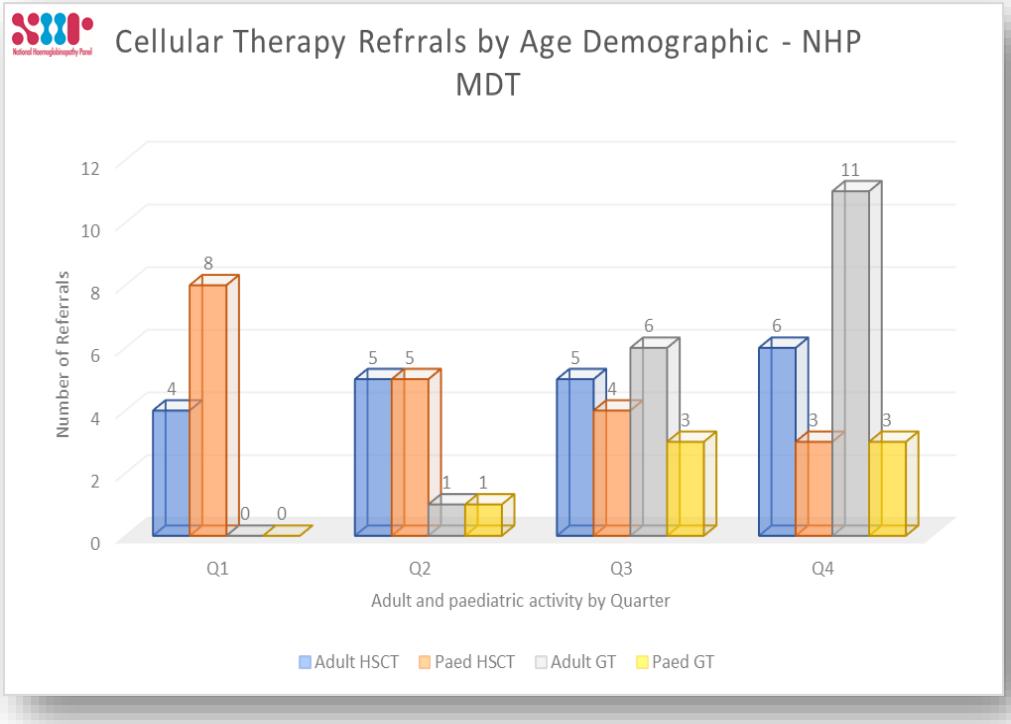


Fig. 2.L. Cellular Therapy Referrals by Age Demographic

CT Type	Referrals Received
Adult HSCT	20
Paed HSCT	20
Adult GT	18
Paed GT	7

Table. 2.2. Cellular Therapy Referrals by Age Demographic

- Paediatric HSCT referrals were highest of the cellular therapy referrals in Q1.
- Adult and paediatric HSCT referrals were on par in Q2.
- Adult GT referrals outstrip other categories in Q3 and markedly in Q4.

- Overall, transplant referrals for adult and paediatric patients for the year to 31 March 2025.

Cellular Therapies by Region

- The Highest HSCT referrals are from London and the North East

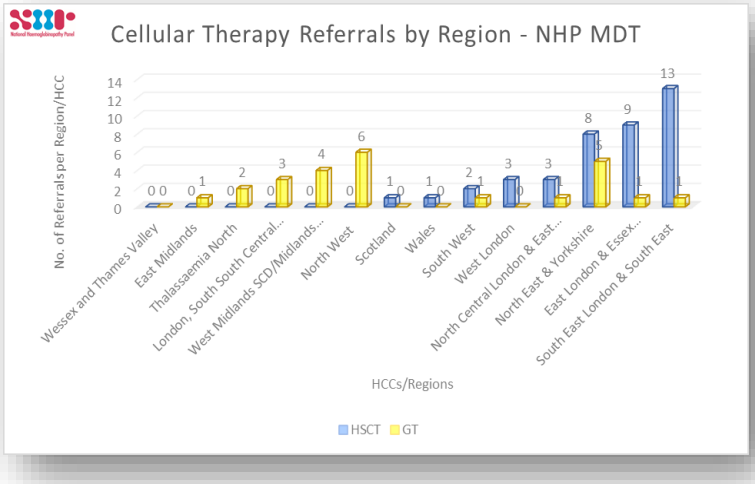


Fig. 2.M. Cellular Therapy Referrals Region

Transplant by Donor Type

- Sibling donor HSCT is the highest requested (15). This is for adult recipients only (child recipients of sibling HSCT do not need to be presented at the NHP).
- Haploidentical donor and other alternative donor requests are nearly on par at 12 and 11 respectively)

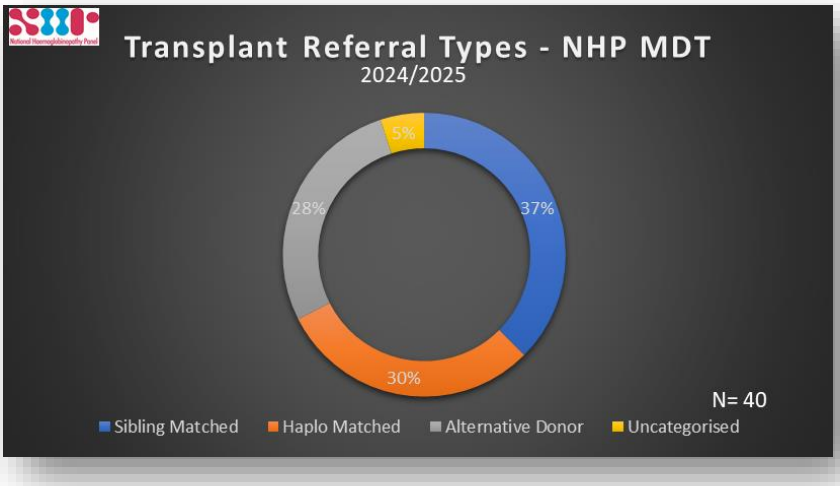


Fig. 2.N. Referrals by Transplant Donor Type

Transplant Type	No. of Refer
Sibling Matched	15
Haplo Matched	12
Alternative Donor	11
Uncategorised	2
Total Referrals-->	40

Table. 2.3. Referrals by Transplant Donor Type

HSCT Approval Rates

Adult HSCT referrals received a slightly higher rate of approvals than the paediatric cases. However, direct comparison is not meaningful as in the adult setting, only matched sibling donor cases are discussed in the NHP (alternative donors are only available via REDRESS haploidentical donor trial), and in the paediatric setting, only alternative donor cases are discussed (as paediatric matched sibling transplants are not discussed at NHP).

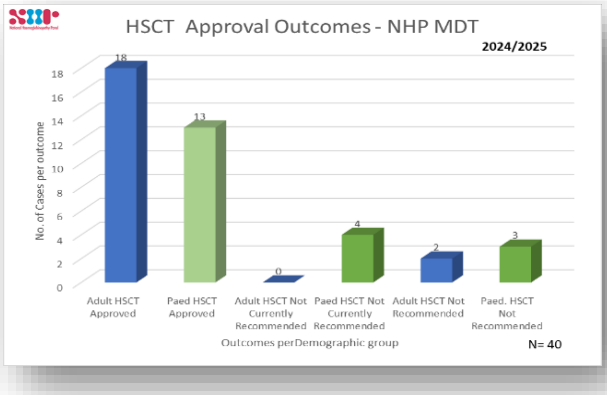


Fig. 2.O. Stem Cell Transplant Approval Outcomes (N)

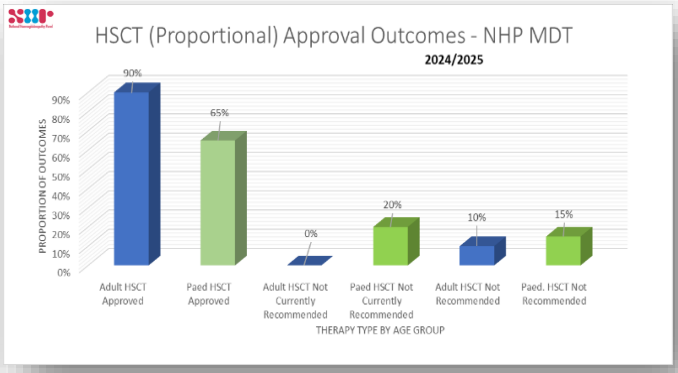


Fig. 2.P. Proportional Stem Cell Transplant Approval Outcomes (%)

Gene Therapy Approval Rates

All paediatric GT referrals for the period were approved while one of the adult referrals was not recommended at the time, but open to resubmission pending further action.

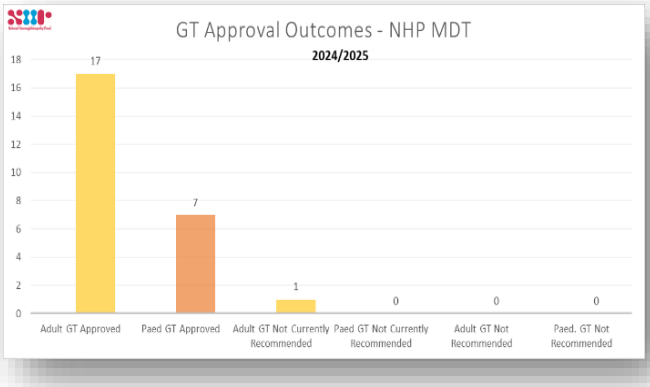


Fig. 2.Q. Gene Therapy Approval Outcomes (N)

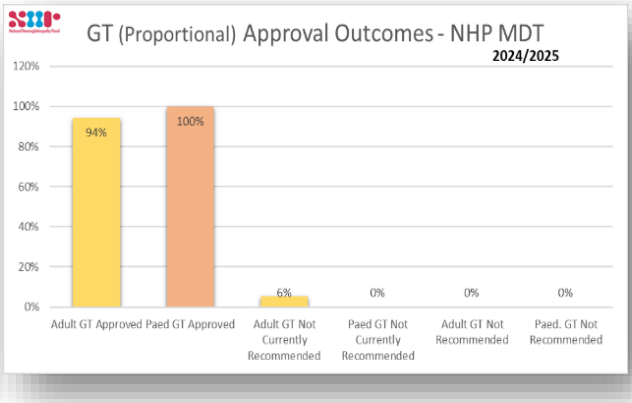


Fig. 2.R. Proportional Gene Therapy Approval Outcomes (%)

Overview of Transplants Referral Outcomes over the Years

Stem Cell Transplants (HSCT) approvals for 2024/2025 were slightly lower than the previous 2 financial years. It is unclear why this is the case.

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

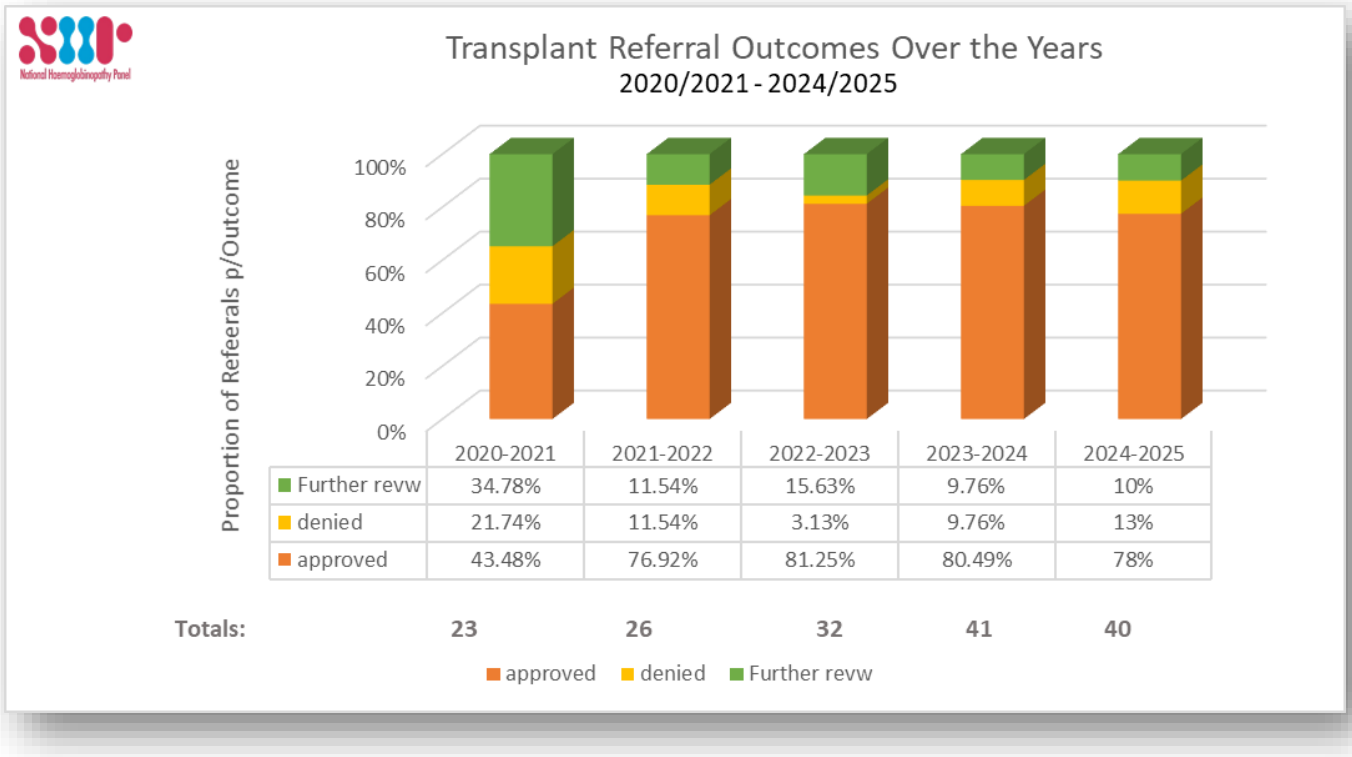


Fig. 2.S. Transplant Referral Outcomes over the Years (2020/2021 – 2024/2025)

3. STAFFING

The NHP operational team consists of the Chair, Deputy Chair and Operational Support officer. From April 2024, Professor John Porter, NHP Deputy Chair, assumed the additional duties of Acting Chair from April 2024 to December 2024, following the departure of Professor Baba Inusa, founding NHP Chair. The NHP and wider network are extremely grateful for the efforts of Professor Porter, during this interim period. January 2025 welcomed the new NHP Chair, Dr Kate Gardner, who is an adult haematology consultant based at Guy's & St Thomas' Hospital NHS FT.

The operational team have continued to maintain service and key deliverables despite the strain of substantial manpower and time to maintain the work demands, including managing patient and activity data collection, analysis and requests; website updates; member and contacts changes; and meetings, with their concomitant documentation and actions. This workload continues to increase as engagement has increased.

With the increasing demand for cellular therapies, the work of the NHP Cellular Therapy Operational Group (CTG) has also increased as the group redefined its position and remit during this period, as well as curate the clinical pathways and guide documentation to support the new therapies. The NHP Operational office provides administrative support for this subgroup of the NHP.

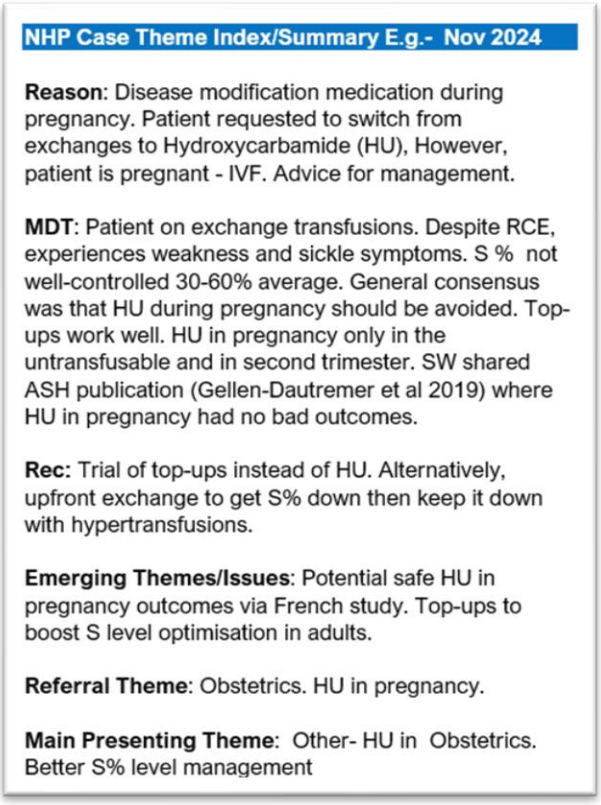
4. EDUCATION AND TRAINING

The 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. Case notes are sent in advance for preparation by all, external experts are invited to join for specific cases (e.g. hepatology opinion), then expert opinion is shared on the day, exploratory prompts and guidance by the Chair allow for deeper assessment of 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 learning topics. There is an aim for these anonymised sessions to be published on the NHP website once updated. Since 2022 NHP has made great efforts to create indexes for each case, with a brief summary and key words, in addition to the detailed minutes, to aid better identification, searchability and data extraction. It is hoped that the anticipated NHP Fellow post will be able to take on and expand on this as well as take over proposed publishing of MDT and other learning on the RCPath learning hub.



NHP Case Theme Index/Summary E.g.- Nov 2024

Reason: Disease modification medication during pregnancy. Patient requested to switch from exchanges to Hydroxycarbamide (HU), However, patient is pregnant - IVF. Advice for management.

MDT: Patient on exchange transfusions. Despite RCE, experiences weakness and sickle symptoms. S % not well-controlled 30-60% average. General consensus was that HU during pregnancy should be avoided. Top-ups work well. HU in pregnancy only in the untransfusable and in second trimester. SW shared ASH publication (Gellen-Dautremer et al 2019) where HU in pregnancy had no bad outcomes.

Rec: Trial of top-ups instead of HU. Alternatively, upfront exchange to get S% down then keep it down with hypertransfusions.

Emerging Themes/Issues: Potential safe HU in pregnancy outcomes via French study. Top-ups to boost S level optimisation in adults.

Referral Theme: Obstetrics. HU in pregnancy.

Main Presenting Theme: Other- HU in Obstetrics. Better S% level management

Figure 4.A: Current state of NHP MDT Case Theme Summary/Indexing

4.2 ASCAT 2024

The renowned, international, annual conference of the Academy for Sickle Cell and Thalassaemia (ASCAT) – founded by the founding NHP Chair, Professor B. Inusa and curated in collaboration with European Haematology Association (EHA) and the British Society of Haematology (BSH) - took place in London, on 2nd – 5th October 2024.

As well as contributing to the ASCAT committee, the NHP facilitated discounts to some network members and free entry to patients. The NHP exhibition stand saw high engagement with delegates which included substantial international interest to join the forum or have possible future collaboration.

4.3 HCC NATIONAL TEACHING

The NHP continues to support the dissemination of information on HCC/regional learning events for national audiences, as well as other learning and development opportunities. HCC pages are collated here: https://www.nationalhaempanel-nhs.net/science-and-innovation/learning/hcc-learning

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 SICKLE CELL & THALASSAEMIA AWARENESS DAYS 2024

In 2024, the NHP participated in curating awareness materials and dissemination, as well as sharing other network partner activities and resources for the Global and National awareness days for Sickle Cell Disorder and Thalassaemia. Both campaigns were geared towards galvanising people to take positive and progressive action, such as donate, give blood, fundraise, volunteer, with extra provided resources of groups or organisations contacts that were already established in various recommended actions.

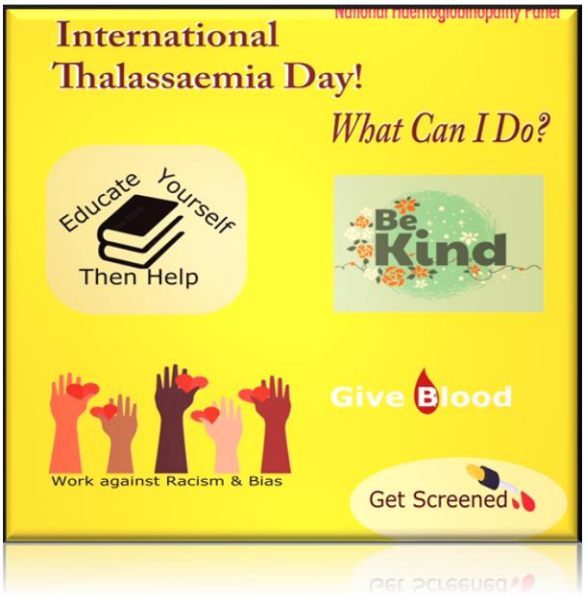


Fig. 4.B: 8 May 2024– International Thalassaemia Day Campaign Poster

Fig. 4,19 June 2024 World Sickle Cell Day Campaign Poster: 8 May 2024– International Thalassaemia Day Campaign Poster

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

Consistency Harmonisation

The CRG for Haemoglobinopathies and NHS England Specialised Commissioning continue to oversee the NHP via guidance, governance and support, particularly in the persons of Dr Subarna Chakravorty (CRG National Specialty Advisor) and Zoe Hamilton (National Programme Manager, Blood and Infection, Specialised Commissioning) respectively. The NHP Biannual Business Operations and Governance meeting, quarterly and annual reports, as well as panel members communications, provide the CRG and Commissioners with accounts of the progress and issues within the organisation and the Haemoglobinopathy community from various

perspectives. The London NHS England Commissioner, Kathy Brennan has also been very instrumental in providing insightful updates, experience/learning and helpful guidance.

The CRG and NHS England 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 by HCCs and patient groups during the meetings. Their efforts are gratefully recognised in advocating for NHR Chair role remuneration, updating HCC and SHT service specifications, clarifying contractual parameters for NHP recruitment, chasing up on delays with MedTech Funding Mandate for Spectre Optia red cell exchange funding, working towards the gene therapy roll-out, and many more.

6. GOVERNANCE AND OTHER RESPONSIBILITIES

As noted above, the NHP reports to the Haemoglobinopathies Clinical Reference Group (CRG). All quarterly and annual reports for this financial year, were completed and submitted to the accountable CRG/NHSE Commissioning personnel.

Business Operations & Governance meetings:

The biannual Business Operations/Governance meetings have held, as per requirements of the Commission, and as set out in their Responsibilities and Governance document. This meeting is attended by HCC representatives that form the actual Panel, various NHS England and CRG representatives, including the National Programme of Care Manager for Blood and Infection, Specialised Commissioning (Zoe Hamilton) and CRG National Specialty Advisor (Dr Subarna Chakravorty). Also participatory are project leads from the TCD Quality Assurance, Newborn screening programmes, and representatives from stakeholder organisations such as the National Haemoglobinopathy Registry (NHR), Rare Inherited Anaemia lead, and UK Forum on Haemoglobinopathy Disorders (UKFHD), to name a few. Patient representative groups such as Sickle Cell Society, UK Thalassaemia Society (UKTS) also attend, bringing updates, challenges and emerging issues for discussion. As usual, this has been an effective avenue for the NHP, HCCs and other invited stakeholders to share strategic updates, national concerns, Key Performance Indicators (KPIs)/milestone checks, and other governance issues.

Some key topics and actions that have taken place during this meeting include the above-mentioned mortality reporting issue, consensus for new national haemoglobinopathy educational material for the Learning Hub, discussion on the impact of Age of Blood restriction removals, means of escalating clinical practice issues, particularly with LHT non-engagement when a mortality is concerned, in light of the Patient Safety Incident Response Framework (PSIRF).

While acknowledging the benefits of this meeting for the community, there is a recognised need to find a balance between making the meetings more robust to fully address weightier issues,

yet simplified and a shorter strain on time. These biannual meetings run alongside regular updates and meetings between the NHP and subgroups, HCCs and working groups within and connected to the NHP.

Policy Development: 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. There is continued work towards standardisation of investigations for Liver Iron Concentration (LIC), with a survey of centres highlighting much variation in methods and interpretations of results in this arena. While the initial policy proposal was declined, the NHP believes that this is a worthwhile endeavour and continues to seek avenues to strengthen future attempts to re-apply for this policy to be approved.

NHP records and reports the uptake of immunomodulatory treatment such as Eculizumab, Rituximab and, increasingly, Tocilizumab. The panel particularly encourages clinicians to present their cases to the panel as well as fill out SHOT (Serious Hazards of Transfusion) reports and publish cases of these drugs used, in order to increase evidence base for potential future policies on treatments for transfusion reactions, as well as for facilitating departmental reimbursement of the drug costs. The question of amending the policy to allow use of Eculizumab to be used prophylactically would also benefit from this data and experience collection.

There have been suggestions that the VOC requirement outlined in the gene therapy commissioning document needs modifying, particularly to take into account severity of disease as a whole and not just reported hospital visits but this will be under the auspices of the CRG in liaison with NHSE commissioners.

Learning from Mortalities: The NHP continues to use the MDT platform for learning, including regular encouragement to clinicians/HCCs to report and discuss incidences of patient deaths at the NHP MDTs for learning, as well as ensure timely and accurate reporting on NHR.

Evidence Building 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. Many of the potential policy amendments and applications noted in the Policy Development section will owe their justification from the exercise of evidence-building.

Good Financial Stewardship: The nature of the commissioning of the NHP requires that it is hosted by an existing entity by whose infrastructure the NHP is supported for auxiliary functions (e.g. HR and Finance.). The complexities of the hosting Trust's departmental and financial structure have, for a number of years, posed challenging to ascertain clarity of NHP funding access and monitoring. At a time, this resulted in the NHP website being terminated for approximately 4+ months. 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. One potential solution initiated by the new Chair, Dr Kate Gardner, is in requesting a transfer of management, including funding, to their Trust (Guy's & St Thomas' Hospital) which is a co-host of the NHP and where it can be more easily monitored an accessed by the organisational lead.

7. NHP CELLULAR THERAPY OPERATIONAL GROUP (CTG)

The NHP Cellular Therapy Operational Group (CTG), is Chaired by the NHP Chair and CRG National Specialty Advisor, in an executive and administrative capacity, while Dr Ben Carpenter, Dr Victoria Potter, and Professor Josu de la Fuente, are the cohort leads for adult Thalassaemia, adult Sickle Cell Disorder (SCD), and Paediatric care, respectively. This leadership group continues to support the direction, approval, implementation and audit processes surrounding NHP Haematopoietic Stem Cell Transplantation (HSCT) and all other Cellular Therapies. The CTG Group meets monthly and comprises other non-transplant clinicians, nurses, psychologists etc.in the discussion of transplant matters. During the period, the CTG completed a review of their Terms of Reference (ToR) in light of the group's reviewed structure and mandate.

With the aforementioned issues with MDT demand pressures, due to an increase in cellular therapy requests at the NHP MDT meetings, this group has been instrumental in working with the NHP operational team to improve communication and support for the NHP MDT, which requires expert cellular therapy input during the meetings for high cost therapy approvals and cellular therapy complications within the haemoglobinopathy context. Other efforts towards NHP MDT capacity management include updated referral forms with targeted fields to elicit specific enquiry and investigations. This also includes secondary discussions at a clinical segment of the CTG meetings, for MDT cases requiring deeper cellular therapy discussion and assessment.

In December 2024, the group hosted a review meeting for the adult sibling transplant programme looking at, and learning from, all the transplants centres' experience to date. There was particular focus on the pattern and possible implications of mixed chimerism and graft failure in haemoglobinopathies.

With the anticipation gene therapy [Casgevy™/ Exagamglogene Autotemcel ('Exa-Cel')] approval for Transfusion-dependent Thalassaemia (TDT) and Sickle Cell Disorder, this group has been instrumental in working on various protocols and pathway coordination to ensure a

smooth practical rollout of the treatments and encourage sound national data collection and analysis as this develops. A particular aspect of the roll-out which this group has guided, is the thresholds and manner of assessments of liver health in order to ensure safe and efficient therapy for patients. The first of such large meetings, which included national hepatology experts, took place in February 2025.

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 (produced and manage NHR platform) 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, there had been a focus on updating TCD Standards and how the QA data can be accessible and shareable across networks (e.g. for CQC visits and peer reviews), around which MDSAS has been key in facilitating. A new focus was on the benefits of interactive Power BI Analytics for visual data oversight, providing, and not exhaustive to, dashboards, showing TCD scan status, operator data, and stop categories, all within customizable time parameters. While the tool was still in its early stages, it received positive feedback. Challenges included a need for individual licenses (£10) for the Power BI software per key HCC TCD staff. Despite which, this was seen as a useful and worthwhile tool for quality assurance processes.

9. NATIONAL HAEMOGLOBINOPATHY REGISTRY (NHR)

Dr Noémi Roy, formerly Deputy Chair of the NHR, took up the role of Acting Chair following the departure of former Chair, Dr Farah Shah, but shared with the Business Operations group the impending call for expressions of interest for the role.

Achievements during the period included better NHSBT data linkage aiding automatic uploads of red cell antibodies, enhancing data quality and accessibility. Another key achievement was in additional fields for annual reviews in thalassaemia and rare inherited anaemia patients.
Challenges for the period reported included data entry (timeliness, accuracy and completeness) in part attributed to HCC staff shortages, particularly for mortality reporting. NHP Business Operations & Governance meetings, in addition to email correspondence, have been forums for sharing directives and collaborated troubleshooting with HCCs regarding NHR data issues.

Case studies shared on the experience of rare inherited anaemia patients and their clear under-representation on NHR highlighted the importance of their identification on the system and the role it plays in their access to life-changing expert input.

10. NATIONAL SICKLE PAIN GROUP (NSPG)

The National Sickle Pain Group, led by Dr Sanne Lugthart and team, 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 pain 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 group reported NIHR clinical trial submissions, collaborations with a US group for SCD pain management improvements, and abstract submissions in various conferences.

11. NEWBORN OUTCOME SCREENING

The National Screening Programme for Sickle Cell Disorder (SCD) and Thalassaemia, led by Amanda Hogan, of NHS England, comprises antenatal and new-born screening facets. This programme encapsulates addressing inequalities, implementing prenatal (including fathers) and newborn testing, educational activities and managing the Newborn Outcome (NBO) system implemented in recent years. The NBO system is a web-based solution used by laboratories, nursing centres and treatment centres to make referrals for babies screened positive for SCT and links into the NHR, ensuring infants with confirmed haemoglobinopathy diagnosis are connected to expert teams.

Reporting shared is that this programme as greatly expanded over the year and that the numbers of carriers had been reducing annually in the London region but increasing for the rest of

England, with confirmed cases likely to follow a similar trend. This correlates with general decline in birth rates.

Challenges affecting this programme included lack of follow-up, postal strikes, language barriers and lab mislabelling. More recently, web-based training for Healthcare Professionals (HCPs) has been made available, which includes a genetic counselling course.

The interconnectivity at the NHP Business meetings allowed for the STANMAP Chair to negotiate access to genetic counselling training from the national screening programme, for community nurses who would not have such access otherwise. Data sharing on pneumococcal infections in children under 5 years of age was agreed to be shared with the HCCs for service planning. Further collaborative work being considered and put to the NHP paediatric Sickle Cell Disorder leads (Prof. D. Rees and, Deputy, Dr J. Brewin), is a potential review/input of the public resource '*A parent's guide to managing Sickle Cell Disease'* for possible update.

12. NOVEL AND NEW THERAPIES

Withdrawal of Voxelotor for treating haemolytic anaemia caused by sickle cell disease

On 25th September 2024, Pfizer issued an announcement of its 'voluntarily withdrawing all lots of OXBRYTA ® (Voxelotor) for the treatment of sickle cell disease (SCD) at this time, in all markets where it is approved'. Pfizer announced 'also discontinuing all active Voxelotor clinical trials and expanded access programs worldwide', noting that the 'decision is based on the totality of clinical data that now indicates the overall benefit of OXBRYTA no longer outweighs the risk in the approved sickle cell patient population.' (https://bit.ly/4jmprr8).

Due to the lack of further information from Pfizer, and the proliferation of varied information and guidance, The NHP members, with GSTT clinicians providing an initial draft, put out an initial letter as an option for clinicians to share with patients during the time of uncertainty until further advice was available from Pfizer. An updated letter from Pfizer was subsequently shared with the network.

Stem Cell Transplantation

Stem cell transplantation continues to be in substantial demand though the advent of gene therapy may change numbers.

Gene Therapy

The commissioning and onset of gene therapy provision for Thalassaemia (August 2024) and Sickle Cell Disorder (January 2025) have shown keen uptake amongst patients, with MDT cases requests for this therapy exceeding all other MDT requests in the last quarter.

Eculizumab and Rituximab

The NHP records reported cases of Eculizumab and Rituximab use in the transfusion reaction setting. There were 8 instances of Eculizumab and Rituximab use presented at the NHP MDT (some instances of both within the same case), two of which were the bases of the urgent email MDT. There was also a resurgence of the debate on policy amendment to allow prophylactic use of Eculizumab in mitigating circumstances.

Tocilizumab

As noted previously, the NHP encourages clinicians to publish, report via SHOT, and present to the Panel, all cases of Tocilizumab use in the acute setting of transfusion reactions, and has suggested a review of the East London & Essex policy of using this as first line of treatment for delayed haemolytic transfusion reactions.

13. 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.

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

The NHP historically 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 policymakers. The NHP participation in these forums allows other stakeholders to engage in service changes and provides a national platform for interaction. During this period, this APPG were not in session, due to a hold ahead of a reconstituted group, following elections.

13.2 SICKLE CELL SOCIETY

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 Sickle Cell Society 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.

They have been a strong advocate for better reporting, learning and transparency in cases of patient deaths, as well as many insights on the patient community and working with local communities, Integrated Care Boards (ICBs) and HCCs on impactful national programmes such as their peer mentoring scheme.

13.3 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 time, the UTKS underwent changes, which meant that Roanna Maharaj, who has been a courageous, candid and knowledgeable patient representative and strategic advocate, would no longer be representing the group.

The NHP received updates and shared in discussions with the UKTS regarding the need for stronger and clearer information on the impending cellular therapy, as patients reported receiving conflicting and unclear information, which indicated a possible need for further nurse training and signposting for patients, to an established information resource, which was being organised.

The UKTS discussed with the NHP network issues regarding the infected blood inquiry and the NHP network committed to ensure that more patients who may be eligible but unaware, would know about the support and compensation available to them.

It was also pointed out that much more work needed to be done to ensure equity in the efforts and developments for thalassaemia were on par with that of Sickle Cell Disorder, as the latter seemed to garner more focus by clinicians/Trusts, HCCs/SHTs and policy-makers.

13.4 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 reported difficulties in coordinating members and organising the annual educational workshop due to the multitude of national and HCC haemoglobinopathy training forums. However, the group contined to carry out its main functions with a future learning event in the works.

13.5 UK FORUM FOR HAEMOGLOBIN DISORDERS (UKFHD)

The UKFHD is a multidisciplinary body of experts, led by Dr Rachel Kesse-Adu, dedicated to optimising 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 goals and objectives of the UKFHD are highly complementary 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.

14. 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 2024/2025, all HCCs continued to work incredibly hard to support and develop their STHs and LHTs in line with Service Specifications.

- **Summary of HCC common themes:**
    - All have robust learning events
    - A lot of the additional HCC funding went to community and most recruitment (HCC/Community) included psychology, social work support, nursing and data management
    - Most recently had peer reviews and aligning future actions and planning to address noted issues
    - Some still struggling with Local Haemoglobinopathy Team (LHT) engagement and also working on (Service-Level Agreements (SLAs) between (Specialist Haemoglobinopathy Teams) SHTs and LHTs
    - Trust limitations on access to funds and internal employment freezes were affecting recruitment
    - MedTech funding delays were affecting service provision and budgeting + extra time for revising business cases
    - Website and social media management a challenge, with some considering an extra full-time role to manage while others divested management to main Trust
    - Patients from all regions have used patient organisation and clinical teams to express their concerns on adverse effects of old blood in transfusions
    - A good number of HCCs report increase in patient numbers with no corresponding staff and resource increase.
    - Data management issues – affecting NHR entries
    - Low uptake on learning opportunities provided

Some unique Highlights & Challenges

- Sickle awareness film screened in several commercial cinemas (NCLEA)
- Launch of regional pain management MDT and regional psychology network (West Midlands/Midlands)

- A hospital at risk of losing its SHT status, but another hospital in view to gain a new SHT status (ELE)
- Network meetings optimisation (NCLEA)
- Key HCC substantive roles filled (NEY)
- Continued pan-midland collaboration
- Expanded patient management tools (North West HCC/North Thal HCC)
- National SpR Red Cell Training (SELSE)
- Management of cyber-attack which supported low blood stocks management (SELSE)
- Formalized and remunerated patient and public voice group (PPVG) (WL)
- Promoting LHT engagement by supporting staff to ASCAT2024 Conference (WL)
- Strong links with LHTs reported by West Midlands SCD/Midlands Thal HCC, which were established before the HCC structure but continued. The positive impact of the nurse network in that region was also good news.
- Shared learning highlights were noted regarding the PREMS exercise carried out by SELSE, which other HCCs found useful.
- EL&E's highlighting of the Patient Safety Incident Respond Framework (PSIRF), on the back of The Sickle Cell Society's raised concerns on managing and reporting deaths, was an opportunity for all to learn of this vital framework, as further emphasised by KB/NHSE.

15. NHP LOOKING FORWARD

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

-Develop a mortality reporting pathway, clarifying parameters for cases due for NHP discussion and encouraging clinicians to engage more in this practice

-Complete website overhaul and platform transition process

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

16. 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. Particularly during this time of great change and increased demands on everyone's time. 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 2024/2025

2024/2025 - Year to Date					
	Q1	Q2	Q3	Q4	Total
MDT Cases	16	**18**	**27**	31	**92**

HCC Region	Q1	Q2	Q3	Q4	2024/2025 Year to Date
East London & Essex	**3**	**5**	**1**	**4**	**13**
East Midlands	**0**	**0**	**2**	**2**	**4**
Ireland	**0**	**0**	**0**	**0**	**0**
North Central London & East Anglia	**1**	**2**	**1**	**1**	**5**
London South, South Central & South West Thal HCC	**0**	**0**	**0**	**3**	**3**
North East & Yorkshire	**5**	**3**	**5**	**4**	**17**
North West	**1**	**2**	**3**	**3**	**9**
Thalassaemia North	**0**	**0**	**0**	**2**	**2**
Scotland	**0**	**1**	**0**	**0**	**1**
South East London & South East	**6**	**2**	**6**	**3**	**17**
South West	**0**	**0**	**4**	**2**	**6**
Wales	**0**	**0**	**0**	**1**	**1**
Wessex and Thames Valley	**0**	**0**	**2**	**3**	**5**
West London	**0**	**2**	**1**	**1**	**4**
West Midlands	**0**	**1**	**2**	**2**	**5**
NHP Total	**16**	**18**	**27**	**31**	**92**

Adult/Paediatric Split	Q1	Q2	Q3	Q4	2024/2025 Year to Date
Adults	**5**	**10**	**18**	**24**	**57**
Paediatric	**11**	**8**	**9**	**7**	**35**

Case Diagnosis	Q1	Q2	Q3	Q4	2024/2025 Year to Date
HbSS	**16**	**15**	**16**	**18**	**65**
HbSC	**0**	**0**	**0**	**1**	**1**

Thalassaemia	0	2	10	11	23
Rare Inherited Anaemia	0	1	0	0	1
HbS/O Arab	0	0	1	0	1
Unknown (diagnostic dilemma)	0	0	0	1	1

Primary Theme (*n1)	**Q1**	**Q2**	**Q3**	**Q4**	**2024/2025 Year to Date**
Stem Cell Transplant	12	10	9	9	40
Gene Therapy TDT	0	2	9	10	21
Gene Therapy SCD	0	0	0	4	4
Novel use of Drugs & New Therapy	0	3	2	4	9
Mortality Review	2	2	1	3	8
Urgent (email) Case	0	1	3	0	4
Complex Haemoglobinopathy/Comorbidity	3	2	6	1	12
IVIG Therapy	0	0	0	0	0
	17	19	27	31	

Transplant Cases	**Q1**	**Q2**	**Q3**	**Q4**	**2024/2025 Year to Date**
Sibling Donor	2	5	4	4	15
Haploidentical Donor	5	2	2	3	12
Alternative Donor	5	2	2	2	11
Uncategorised	0	1	1	0	2
Total	12	10	9	9	40

Transplant NHP Outcome	**Q1**	**Q2**	**Q3**	**Q4**	**2024/2025 Year to Date**
NHP recommended to refer	9	7	9	6	31
NHP - **not currently** recommended ***n2***	2	2	0	0	4
NHP - **not** recommended	1	1	0	3	5
Successful Referral Rate	75%	70%	100%	67%	77.50%

Gene Therapy NHP Outcome	**Q1**	**Q2**	**Q3**	**Q4**	**2024/2025 Year to Date**

NHP recommended to refer	**N/A**	**2**	**9**	**13**	**24**
NHP - **not currently** recommended **** n2***	**N/A**	**0**	**0**	**1**	**1**
NHP - **not** recommended	**N/A**	**0**	**0**	**0**	**0**
Successful Referral Rate		**100%**	**100%**	**93%**	
Novel Use of Drugs & New Therapy	**Q1**	**Q2**	**Q3**	**Q4**	**2024/2025 Year to Date**
Eculizumab	**0**	**3**	**1.5**	**1**	**5.5**
Rituximab	**0**	**0**	**0.5**	**2**	**2.5**
Tocilizumab	**0**	**0**	**0**	**1**	**1**
Other New Therapy	0	0	0		**0**
Total Instances of Use * *n1*	**0**	**3**	**2**	**4**	**9**
Retrospectively Referred **** n3***	0	2	2	2	**6**
Retrospectively Referred %		67%	100%	50%	72.3%

*** Note** - n1 -Theme total may exceed total number of cases due to overlap from cases having multiple core themes and therapy applications.
*** Note** - n2 - Outcome is not currently recommend, but can be reviewed pending future investigations/action/review
*** 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.