**NATIONAL HAEMOGLOBINOPATHY PANEL**

# Annual Report 2021/2022

Chair: Professor Baba Inusa

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## EXECUTIVE SUMMARY

The NHP has had a noteworthy 2021/2022 period. Despite staff shortage and many other limitations, including the Covid-19 pandemic, there have been worthwhile achievements on the back of dedicated and highly skilled panel members and other contributors.

### Framework & Governance

The biannual Business Operations/Governance meetings have held, as per requirements of the Commission and as set out in their Responsibilities and Governance document (*see A1*), and have been an effective avenue for sharing strategic updates, concerns, KPI 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 Transplant subgroup, New-born Outcomes screening programme, the National Sickle Pain Group, National Haemoglobinopathy Registry (NHR) and Transcranial Doppler programme, to name a few. All quarterly reports and the annual report for 2020/2021, were completed and submitted to the accountable CRG personnel.

### NHP MDT

The core aspect of the national MDTs continued with quorate monthly meetings, covering a wide range of issues, including rare anaemia cases, and continues to be a substantial forum for learning and knowledge exchange, while providing equitable expert access for complex patient cases. There were a total of 41 cases; 5 less than 2020/2021, which were dominated by transplant referrals. The SOP for this forum will also be reviewed to consider the increased input from observers.

### Education & Training

Education and training has continued to be an increasing need and demand, with a clear indication of the need for non-haemoglobinopathy practitioners and stakeholders to develop a better understanding and skill set in supporting and treating Haemoglobinopathy patients. Hence, the role of SHTs/LHTs/Trust leadership and Academy of Medical Royal Colleges is key to bringing about the needed changes. This is starkly evinced in light of implementing recommendations from the '*No One's Listening'* APPG report on failings in Sickle Cell and Thalassaemia care at secondary level. Communication and feedback is an ongoing challenge and focus, both within clinical and operational settings.

## Policy & Guidelines

There have also been a number of guidelines published by the NHP and network, including the Crizanlizumab and Voxelotor SOPs, and an acute pain action plan for services nationally. A number of policy and guideline documents are also in process- an iron chelation policy, which is currently under consultation; the adult sickle cell transplant protocol, an amendment to the paediatric Sickle Cell Transplant Protocol, and a protocol for the Sickle Adult Haploidentical trial due to commence by autumn 2022 in a number of centres nationally.

## No One's Listening

There have been significant and ongoing efforts by the NHP/HCCs, as to how to elicit practical responses, collaboration and action plans from SHTs/LHTs/Trusts in light of the recommendations made in the All-Party Parliamentary Group for Sickle Cell and Thalassaemia (SCTAPPG) report, *No One's Listening* *(See A2)*. 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, with NHSEI setting a deadline of June 2022 for Trust feedback on their progress. These are to be collated by HCCs and subsequently reviewed by the CRG.

## 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.

## Summary of Key Progress Highlights

-Holding 12 quorate and well-attended national MDTs, discussing 41 cases, including 4 emergency/email cases.

-Transfer of the Newborn Screening process to the new Newborn Outcome (NBO) system, for almost all centres nationally.

-Production and publication of the Crizanlizumab and Voxelotor guidelines.

-Successful roll-out of Novel Therapies, such as Crizanlizumab and Voxelotor, across the regions.

-National pain audit carried out with subsequent publication of an acute pain action plan via the pain subgroup.

-Successful completion of the Organisational mapping for the National haemoglobinopathy Registry (NHR).

-Publishing of three free educational sessions on New Therapies education, from the ASCAT2022 virtual congress.

-National Haemoglobinopathy Registry (NHR) being fully functional for significant data access, including tracking of new-born positive haemoglobinopathy diagnoses, TCDs performed, significant events and their comorbidities, Covid-19 vaccinations, patient care plans, and more.

-MDT referral form reconfiguration to facilitate a more accurate reviewing, reporting and analysis of cases pre and post MDT discussion.

## 1. NHP FRAMEWORK

The NHP continues to carry out its commission of a new model of care as laid out by the Responsibilities and Governance 2021/2022 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 STANMAP, Sickle Cell Society (SCS), the UK Thalassaemia Society (UKTS), and the UK Forum for Haemoglobin Disorders (UKFHD).

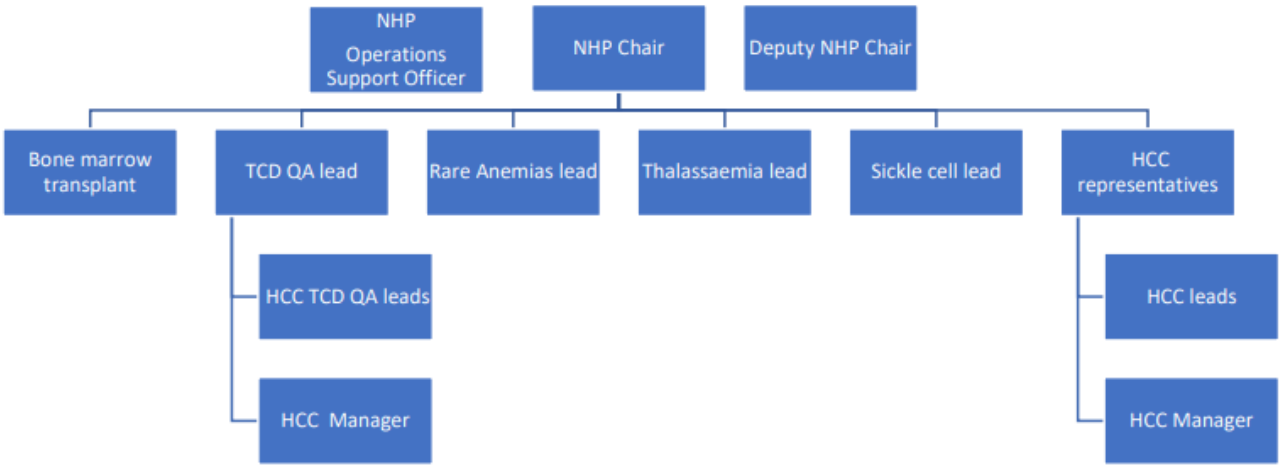
At the national MDT (both scheduled and emergency email cases), the NHP is able to provide expert input and advice on complex clinical cases for all HCC regions. These meetings also serve as a rich learning experience, which the NHP is in the process of optimising, and an avenue of highlighting challenges 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.

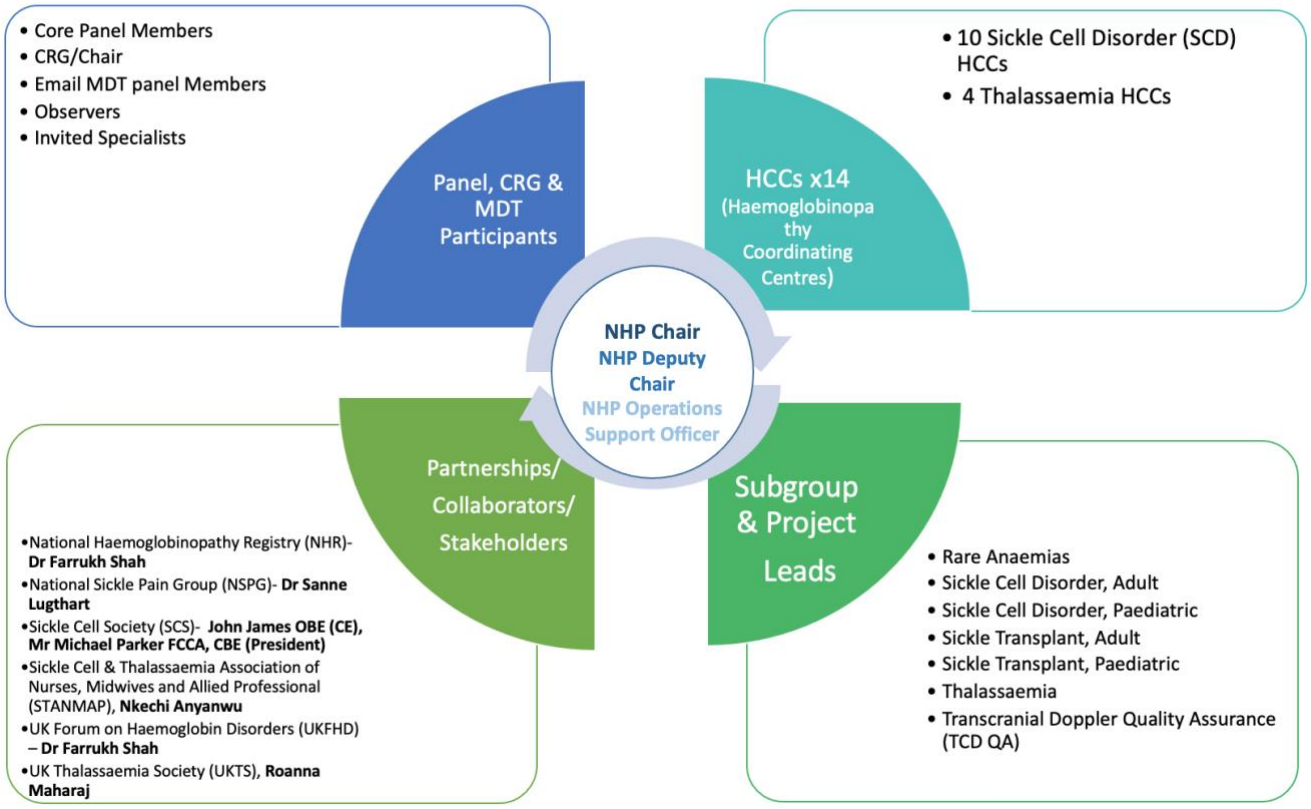
The NHP is also responsible for policy development, advice and input, as well as a few key initiatives that aim to further attain equity across the national landscape of haemoglobinopathy. The organisation's comprehensive framework continues to see that leadership is brought to the various focus areas 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 hear and empower patient voices and experiences, as well as clinical development.

# NHP Structure and Key Relationships




## NHP WIDER NETWORK AT A GLANCE



# 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 Noėmi 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, see the website.

# 2. THE NATIONAL MDT

## 2.1 OVERVIEW

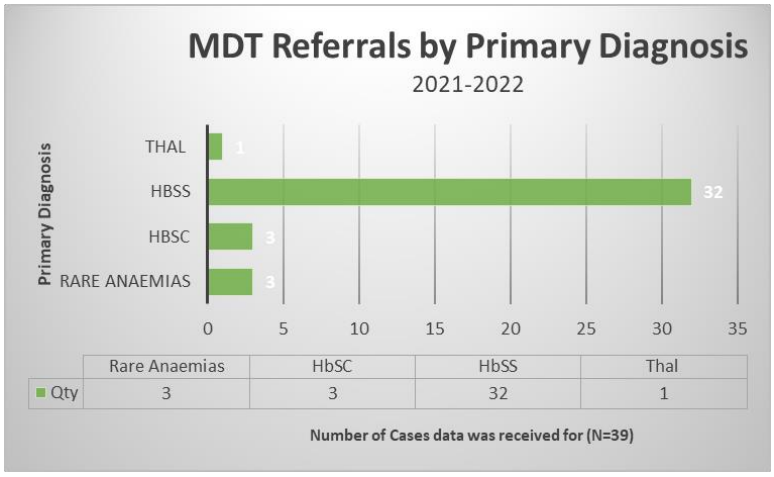
The NHP was able to hold all 12 national MDTs with quorate attendance. There was a slight fall in overall cases discussed, with 41 cases in 2021/2022, as opposed to 46 cases in 2020/2021. Within this, there were 4 urgent email MDTs, for which good feedback was received. Paediatric referrals made up 29% of referred patients. Of the 71% adults referred, the largest number referred (16) were from the age range of 17-30 years-old. As in the previous year, the dominant primary diagnosis was for the Sickle Cell Homozygous genotype (HbSS), mirroring the dominance of this genotype in the general population. Referrals were received from all but 1 HCC, with the highest number of referrals being from the North West HCC (12) followed by South East London South East HCC (11).

Haemopoietic Stem Cell Transplantation (HSCT) dominated the theme of referral requests. Of the 26 transplant requests for 2021/2022 (up from 23 requests in 2020/2021), 20 were approved, 3 were designated for possible future review, while 3 were deemed unsuitable for transplant. Approvals for sibling matched transplant were the highest recommendation at 15, while 8 haploidentical transplants and one matched unrelated were approved. It was observed and noted, during the March 2022 MDT, that there was indeed a slow increase in transplant requests for haemoglobinopathies, likely indicating that the funding for this treatment is creating access to satisfy an unmet need.

The main themes for patients presenting, in the discussed cases, were suitably varied, but an expected majority (18) were of frequent and/or severe VOC, with 9 presenting with Acute Chest Syndromes, most despite Hydroxycarbamide, but with 3 reporting intolerance or unresponsiveness to Hydroxycarbamide. There were 22 patients who were noted as receiving or had recently received transfusions, 10 of which presented with transfusion complications, mainly Delayed Haemolytic Transfusion Reaction or Hyper Haemolysis. There was 1 obstetrics management case and 1 mortality review. Ten discussed patients had current cerebrovascular issues with 2 others reporting this issue in their past medical history. Three cases comprised complex psychosocial matters. Other singular main presentations include Hypersplenism or splenic sequestration, fevers and widespread pain, frontal bossing, stuttering priapism, chronic pain and headaches of unknown cause, to name a few. Below are some highlights of the analysis of the MDT metrics (see Appx1 for metrix summary).

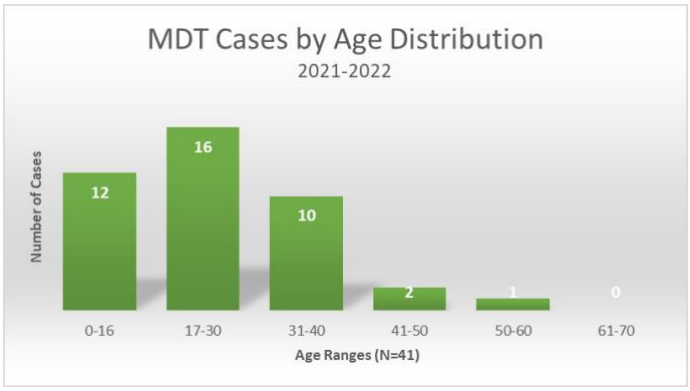
## 2.2 ANALYSIS OF REVIEWED CASES

### 2.2.a. [Fig 2.i] Referrals by Primary Diagnosis

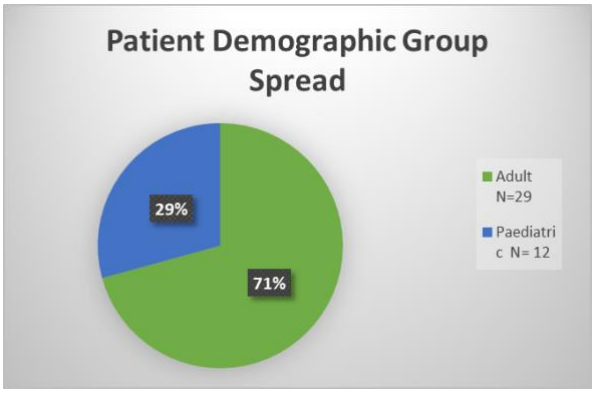



### 2.2.b. Referrals by Age demographic

[Fig 2.ii] By Age Distribution

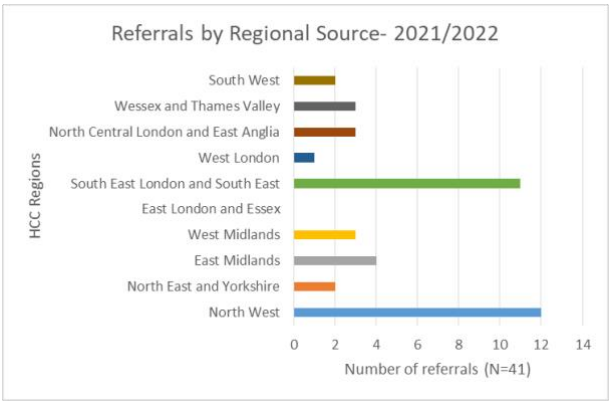



[Fig 2.iii] By Demographic Group Spread

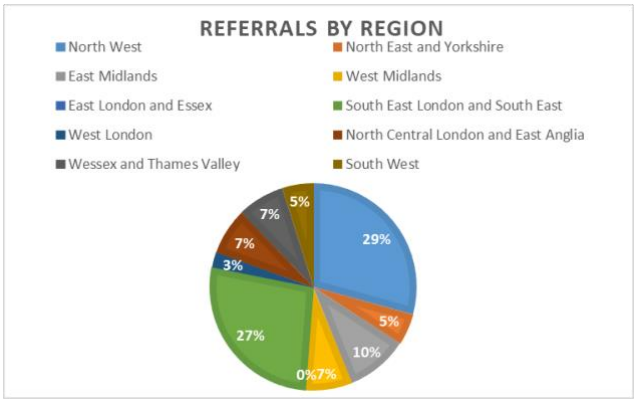



### 2.2.c. Referrals by Regional Source

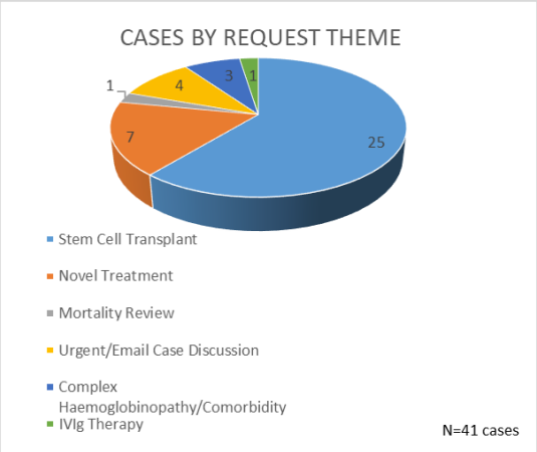
[Fig 2.iv]




[Fig 2.v]



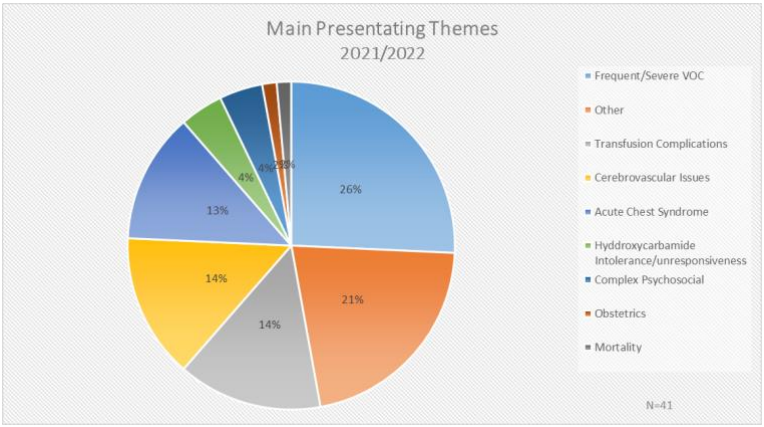
### 2.2.d. [Fig 2.vi] Cases by Request Theme




### 2.2.e. Cases by Theme

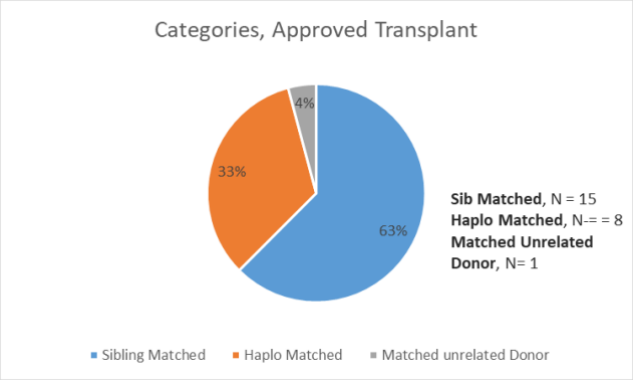
| Case Theme | Number of presentations |
|---|---|
| Frequent/Severe VOC | 18 |
| Other | 15 |
| Transfusion Complications | 10 |
| Cerebrovascular Issues | 10 |
| Acute Chest Syndrome | 9 |
| Hydroxycarbamide Intolerance/unresponsiveness | 3 |
| Complex Psychosocial | 3 |
| Obstetrics | 1 |
| Mortality | 1 |

[Fig 2.vii]

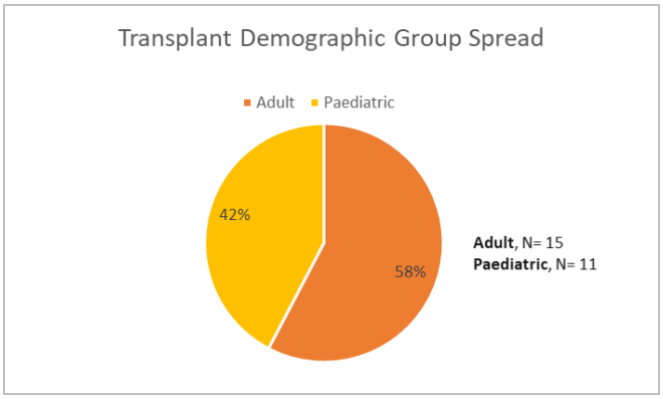


### 2.2.f. Transplant Cases Referred

[Fig 2.viii] Approved Transplant – Categories




[Fig 2. ix] Transplant referrals age demographic




## 3. MANPOWER AND STAFFING

2021/2022 was a difficult year for the already minimally-staffed NHP. The substantive Coordinator, Shane Nagle, who was key in the establishment and coordination of foundational processes and infrastructure - under the leadership of Professor Baba Inusa - was unfortunately, diagnosed with a brain tumour in April 2021. The demands of managing this huge health challenge meant that he was away often and then for an indeterminate stretch of time. Unfortunately Shane lost his health battle and passed away on 10th April 2022. We remain extremely grateful for all his incredible hard work for the NHP and to have known Shane as a person- he will be sorely missed. Our thoughts go out to his family and loved ones.

Sara Kemp, Operational Manager, for South East London and South East, was an incredible support for the NHP during Shane's absences, juggling her own very demanding role (HCC Operational Manager) while keeping the group NHP forward. However, in January 2022, Sara left the Trust and leaves us very grateful for her highly skilled assistance at such a difficult time. Since February 2022, U'mau Otuokon has supported the panel in an interim capacity.

The NHP wider network also saw staff departures from some key and senior figures due to retirement and resignation, leading to a great chasm in the knowledge and experience landscape. Lola Oni (Nurse Director of the Brent Sickle Cell and Thalassaemia Centre) and Jo Howard (HCC Lead and CRG Chair) are two examples.

This issue of inadequate staffing has been highlighted within a number of HCCs (more on this in *HCC Updates*), with some having one consultant supporting a whole Specialist Haemoglobinopathy Team (SHT) or Local Haematology team, often with a substantial haemoglobinopathy patient population. Causes stem mostly from funding deficits but low uptake of some roles was also noted.

## 4. EDUCATION AND TRAINING

As mentioned, education and training is greatly needed, more so for non-haemoglobinopathy services, Emergency Services being somewhere at the top of the list. The NHP and its partners continue to find ways to influence and encourage this training via the available networks. The MDT is a core avenue of clinical education and training of the NHP and various clinical staff within the network, and across the UK. The NHP also serves as a conduit through which the HCCs and partner establishments share training events with the wider network. Below are just a few noteworthy development initiatives.

### 4.1 ASCAT 2022

The Annual Scientific Conference on Sickle Cell and Thalassemia (ASCAT) 2022 conference, in collaboration with European Haematology Association (EHA), British Society for Haematology (BSH), took place virtually on 26th to 28th January 2022, spearheaded by the Chair of the NHP, Professor Baba Inusa. The theme for the year was 'Improving the lives of people living with Sickle Cell Disease and Thalassaemia: A focus on new therapies and person-cantered care.' This was hosted live online, due to covid-19, with over 300 attendees (399 registered). A roster of highly skilled and esteemed faculty lead 80+ sessions ranging from abstracts, live lectures and patient sessions. All registered attendees were given post-event access to all sessions. Attendee feedback showed a 94.2% satisfaction rate in the respondents, and of the balance, none were dissatisfied. There will be an in-person ASCAT conference in London, running 20th to 22nd October 2022, also in partnership with EHA and BSH.

### 4.2 STANMAP EDUCATION HALF-DAY

The Sickle Cell & Thalassaemia Association of Nurses, Midwives and Allied Professional session (STANMAP) held a half day educational webinar on 13th January 2022, which was chaired by Dame Professor Elizabeth Anionwu, with nearly 90 attendees. Presenters included Prof Baba Inusa, NHP Chair, regarding covid-19 research on Haemoglobinopathy patients and the Newborn Bloodspot Screening Outcome and the Alert card project; Roanna Maharaj from UKTS presenting on living with Thalassaemia; and the Sickle Cell Society Parliamentary and Policy Officer giving an overview of the '*No One's Listening'* Report.

### 4.3 NEW THERAPIES SESSION ONLINE

One of the sessions, from ASCAT2022 features 3 great presentations on new Therapies, given by Dr Julie Kanter MD (*Early Evaluation of the Use of Crizanlizumab in Sickle Cell Disease: A*

*National Alliance of Sickle Cell Centers Study*), Professor Biree Andermarian (*Real-Life Experience of Novel Therapies Use: Voxelotor RETRO/PROSPECT Studies*) and Dr Bart J Biemond (*Emerging Sickle Cell Disease*). This resource has been shared with the NHP/HCC network and made available on the new NHP YouTube page and the NHP website.

## 4.4 HCC NATIONAL ROTATIONAL TEACHING

The NHP is supporting a newly-developed national learning programme which runs monthly learning events curated by a different HCC over the course of the year. The Wessex and Thames Valley HCC are coordinating this year's programme (theme selection and allocation etc.). This will be handed over to another HCC to coordinate in the year 2022/2023. Information for that programme can be found on the NHP website.

## 4.5 MDT LEARNING OUTCOMES

An exercise is underway to analyse the MDT cases and draw out themes for specific learning and discussion. The final output of this will hopefully be available towards the end of the calendar year.

# 5. CLINICAL REFERENCE GROUP FOR HAEMOGLOBINOPATHIES

Professor Jo Howard, CRG Chair up to May 2022, shared a CRG progress update, starting with the impending new commissioning model which ushers in the Integrated Care System (ICS), a source of considerable uncertainty and concern for a number of service leads. The network was made to understand that there would not be any immediate changes for at least another year, giving time to absorb and adapt to the long-term service implications this would bring.

In light of Professor Howard demitting her CRG role in May 2022, Dr Subarna Chakravorty stands in while there is a reshuffle of CRG roles and a new 'National Specialty Advisor' (role replacing the CRG Chair) appointed.

In the overview, it was noted that the impact of covid-19 caused limitations, or the absence of some activities, such as review of SHT/HCC dashboards. This will be remedied going forward with dashboards to be reviewed in June/July 2022 against service specifications. The self-assesement, which went alongside the data collection for the dashboards, has unfortunately been withdrawn for this year, with no clinical input into this decision. The CRG has also been working on the NHP Governance Review. A compliance review of SHTs will follow, in the form of a self-assessment as well as a performance of HCCs against the procurement standards. This should feed into a review and update of service specificaitons. Due to block contracts during covid-19, discussions on financial matters were not fortchoming with NHSEI. However, following a NICE report, apheress has been designated as a mandatory technology and NHS funding may follow. The importance of HCCs knowing their funding sources, structure and flow was reiterated.

## 5.1 NHR

NHSEI continue to fund NHR but no longer the Clincial Lead post. Most of the dashboard data e.g. Hydroxyurea (HU), TCD reports etc. is now available on the system. The patient care plans on the NHR are positively anticipated. A discussion about the minimal data set is needed, as to whether it will be an active clinical or research tool etc. and how it will be funded, as NHSE will only fund aspects within its direct scope of interest.

## 5.2 POLICY DEVELOPMENT

Policy development has been good with the iron chelation policy out for consultation as of April 2022.

Rituximab and Eculizumab in DHTR/Hyper haemolysis policy and HSCT in adults with SCD policy have gone through.

Professor Josu de la Fuente and Dr Banu Kaya are designated as leading on the Paediatric HSCT policy. This follows on from a discussion last year about the NHP making the recommendation that all children aged 5 and under, and who have a matched sibling, should have a funded transplant. There is data to support this.

### 5.3 NEW TREATMENTS

The CRG has been working with Voxelotor coming on the market and with the Crizanlizumab Managed Access scheme and supporting curatvie therapies such as the funded haplo transplang in adults trial. Unfortunately, Gene Therapy was not approved by NICE.

### 5.4 ONGOING WORK

Professor Jo Howard and Sharon Hodgson (NHSEI) drafted a letter to all Trusts asking for responses to the APPG *No One's Listening* report. It is important that members engage Trust senior leadership in this and not allow it to be pushed to the haematologists within the Trust. The deadline from NHSE for responses is June 2022 and HCCs are responsible for collating these responses, which the CRG will review. Furthermore, HCCs are responsible for reporting any deaths that flag a concern.

The CRG are awaiting a summary document from Dr Elizabeth Rhodes/Armed Forces/Sickle Cell Society review of sickle trait.

The CRG has been supporting the Peer Review process.

There is a CRG-commissioned review of provision of fertility services, particularly sperm preservation. Drs Banu Kaya and Annette Wood will carry out an evidence review.

### 5.5 COVID WORKING GROUP

Professor Howard set up this goup which worked on guidelines, clinical support, patient information etc. related to covid-19, for haemoglobinopathy patients. However, due to the changes in the direction of the pandemic, the group, as an actively meeting and responding body, has been stood down, pending any further developments. There remained, however, a question on what to do regarding the data and research in this area.

Professor Mark Layton and Dr Paul Telfer were also involved in the research aspect of this group and shared that data collection became difficult as things became less organised. There is an awareness that the data would be less complete, as a high prevalence of patients are presenting locally and not in hospitals where the data is being collected. Further, there is a vast amount of covid data in the public domain that there may not be a unique perspective in what they might do with the data.

A consideration is that the data for the first 3 waves can be collected but there is no certainty that anyone has an apetite to curate and analyse this, though the option remains open.

The data for inpatients is fairly complete and would be good to continue collecting along with vaccination data. There was a protocol put together for the statistical analysis etc. which went through authorisation, but things have stalled as each HCC needs their respective R&D departments to authorise the study.

From a Sickle Cell Society perspective, the group (SCS) would like to see data completed and including updates reflecting hospitallised patients. It would be helpful to present this to the Sickle Cell community. This may address some views that may not be based on empirical data.

### 5.6 CHALLENGES

-HCCs have been set up and now need to be reviewed.

-How best to build services in context of NHS financial constraints.

-How will the new NHS structure - particularly Integrated Care Systems (ICS) – be managed?

-How do we meet recommendations of APPG report?

-How do we integrate reasearch into what we do?

-More effort is needed for NHP MDT attendees to read the papers in advance to make things run smoother.

-There is a need for a better understanding of the CRG and what it can and cannot achieve. CRG advises NHSEI but NHSEI may not take things on board. Some great work and listening has been done but often it is a challenge. Sharon Hodgson has been a real champion of the cuase but sadly will be moving to an upward role. Zoe Hamilton is the new National Programme of Care Manager – Blood and Infection.

# 6. 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 10 project regional leads continue to meet regularly and develop the project nationally.

## 6.1 QUALITY ASSURANCE

The TCD practitioner listing has had a few reviews and 50 practitioners have been identified, though only 26 were on the Register. Training continues locally, in-person for the early stages/clinical skills training, but there are also resources available online.

The project hopes to run a hybrid (in-person/online) structure for training. It is has also been noted that to keep practitioners' skills updated – aiming for approximately 40 scans every 6 months - practitioners with a small demographic, and hence less opportunities to practice, will go to larger local centres to perform scans. These rotations will be organised by TCD regional leads. The issue of gaining practice rights and access is being discussed as the honorary and observer contracts can take time. A 'letter of access', early application for honorary contracts, and possibly a 'research passport' are being considered to address this.

[Fig 6.i] **TCD Practitioners**

TCD PRACTITIONERS

| | SCD HCC | Practitioners | On Register |
|---|---|---|---|
| 1 | NORTH WEST | 3 | 1 |
| 2 | NE YORKSHIRE | 2 | 2 |
| 3 | E MIDLANDS | 4 | 1 |
| 4 | W MIDLANDS | 4 | 3 |
| 5 | E LONDON & ESSSEX | 6 | 4 |
| 6 | SE LONDON & SE | 8 | 5 |
| 7 | W LONDON | 11 | 5 |
| 8 | N CENTRAL LONDON & E ANGLIA | 5 | 1 |
| 9 | WESSEX & THAMES VALLEY | 4 | 3 |
| 10 | SOUTH WEST | 3 | 1 |
| | | 50 | 26 |

**Image credit; Dr S. Padayachee*

There are also site visits planned for observation of scans and collaboration on image QA. In a presentation given by East Midlands TCD lead, it was noted that NHR has been instrumental in attaining conformity of reporting across HCCs.

A few issues arose from more recent meetings, such as the impact of instrumentation and hence possibly introducing quality assurance on machines, which at times vary. The issue of data entry is also a consideration. While it is felt that all HCCs should have a data manager, it was also acknowledged that 1) Data managers will not only be dealing with TCD data and 2) many practitioners feel it is better and more conclusive to do it themselves.

Upcoming functionality on the NHR platform will include HCC TCD Output (now ensured with greater accuracy due to organisational/centre mapping exercise), Practitioner STOP/scan numbers, and Practitioner MCA velocity ranges. These reports will facilitate the QA process and can indicate anything from HCC or SHT engagement to likely practictioner needing scanning exercises for skills maintenance, or systemic shifts in data which could show poor protocol vs disease prgression. However, a trans-analysis may be required as some practitioners may operate in multiple centres.

### 6.2 NHR PLATFORM AND ENGAGEMENT

Currently, NHR generates and sends out, to subscribers, automated reports of scans per centre. More entry of scan data is encouraged as figures show some centres trailing below estimated real figures (see A3). Access problems have been resolved as have most NHR formatting issues. At 20th April 2022 there were still some location and other issues outstanding but being addressed.

Since the 2020/2021 annual report figure of 1500 TCD scans by November 2021, the number of reported TCD scans on 1 April 2022 stands at 2711 (see A3), with North Middlesex and Barts Health NHS Trust reporting 458 and 400 scan, respectively, while the range of numbers goes down to 8 and 1 reported in some Trusts.

Once centres and practitioners are putting in good, consistent data, it will enable a wealth of automated analysis that saves time and improves practice and services.

## 7. NATIONAL HAEMOGLOBINOPATHY REGISTER (NHR)

### 7.1 OVERVIEW

The NHR continues to be funded by NHS Engaland and NHS Improvement, though the funded Clinical Lead post, carried out so well by Dr Farrukh Shah, has been withdrawn. While she continues to support the role, this is, understandably, not sustainable in light of the demands of the role and her many other responsibilities. The NHR is also supported by a multidisciplinary steering group of experts and patient representatives from across the nation.

In the past year, the NHR functionality, which technical software is supported by MDSAS, has continued to evolve with increased capacity, allowing stakeholders, and soon; patients, have ready data on various key patient care features and activity such as regular reporting on the number of TCD scans, Covid-19 vaccinations, significant complications and patient care plans. The Covid-19 immunisation data reflects recent immunisation criteria for children and shows different age cohorts and level of immunisation (1st, 2nd vaccine etc.)

The system is gaining capacity for interrogations for TCD centre and practitioner data that will assist in the Quality Assurance mandate.

Patient Care Plans for individual patients can now be uploaded or a generic care plan for a service can be uploaded into the patient's records. When the Patient Portal goes live, the patients will be able to share their care plans with other health care providers such as an emergeny department in another region.

The New-born Outcomes system also now links into the NHR, and NHSBT teams will soon be able to upload red cell antibody data onto patient NHR records.

Organisational mapping has been a key feature of the progress of this increased functionality and accuracy of the database, which Tim Smith (Data Manager, Haematology, Kings College Hospital) and colleagues have been key in completing. This will be vital in linking up HCC and SHT dashboards and providing flexibility in interrogating data.

Overall, there is increased practitioner participation and good monthly report output though there are still some very minor discrepancies being worked out. The possibilities for how this system can pivot patient care, are exciting, particularly regarding the patient care plans, which has a direct impact on how patients can be treated in unknown settings.

### 7.2 NEXT STEPS

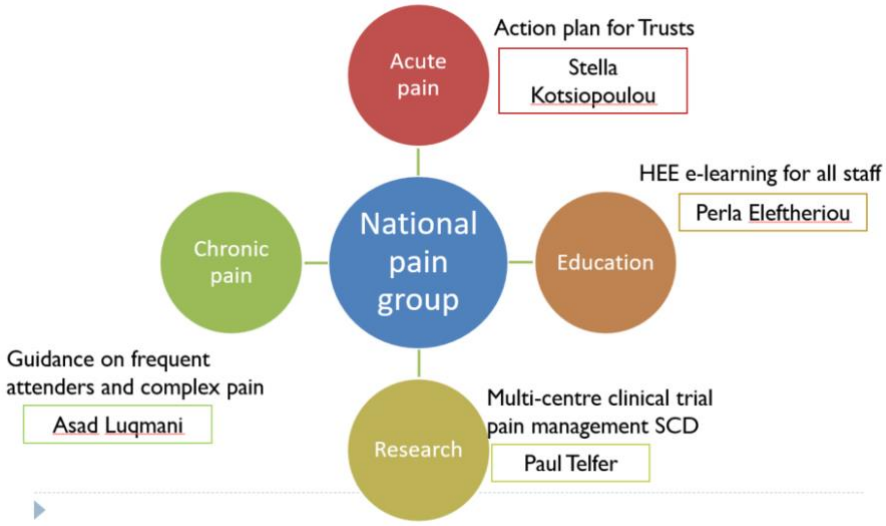
The below, reported in April 2022, are some key next steps for the NHR development.
-Upload of patient care plan
-Patient web app scoping
-Implementation of patient app
-Tim Smith and colleagues are working on how to download data automatically from electronic patient records, to better facilitate data entry.
-IRAS application for generic research is being led by Drs Noémi Roy and Dr Kate Gardner.
-Connection to the NHSE database of deaths.

## 8. NATIONAL SICKLE PAIN GROUP (NSPG)

The NSPG 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.

Since being set up in April 2021, the group, led by Dr Sanne Lugthart, have had 5 well-attended meetings so far and is currently comprised of 35 experts including adult and paediatric haematologists and CNSes, pharmacists, chronic and acute pain specialist, ED contulstants, and palliative care/pain specialists, to name a few. There are 4 subgroups within the group; Acute Pain, Chronic Pain, Education and Research. Details of the subgroup leads and some projects overseen are depicted below.

[Fig. 8.i] **Pain Subgroup Structure**




** Image credit; Dr S. Lugthart*

Amongst a number of significant outcomes of the NSPG so far, is the Acute Pain Management Action Plan which was preceeded by an Acute and Chronic Pain management national audit carried out in July 2021. The action plan has since been distributed to all HCCs for further disemination and is now published on the NHP Website. This action plan is a welcome resources in line with one of the recommendations in the *No One's Listening* report which states that '*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*'.

The response to the national pain audit consisted of 39 centres (56 responders) which completed the audit, with 54% of those being paediatric services and the rest adult. 51% of responders were LHTs, 33% were HCCs (all centres) and 15% were SHTs.

The conclusion of the study is summarised below.
-The majority of acute sickle cell pain presentations are managed via the emergency department and time to analgesia is often delayed (>30 minutes).
-Protocols of pain management in sickle cell disease show a large variation across centres.
-The most common analgesia used were Morphine/Oxycodone for adults and Morphone/Diamorphine for paediatrics.
-Centres with an ambulatory care service show a trend of reduced 'time to analgesia'.
-Education and training for different specialities (ED, acute med/paediatric, pharmacy) is lacking or given infrequently.

-Few centres (n=14) have a patient satisfaction questionnaire.

Below is a summary of key NSPG ouctomes in the year.

**[Fig 8.ii] Pain Subgroup outcomes**

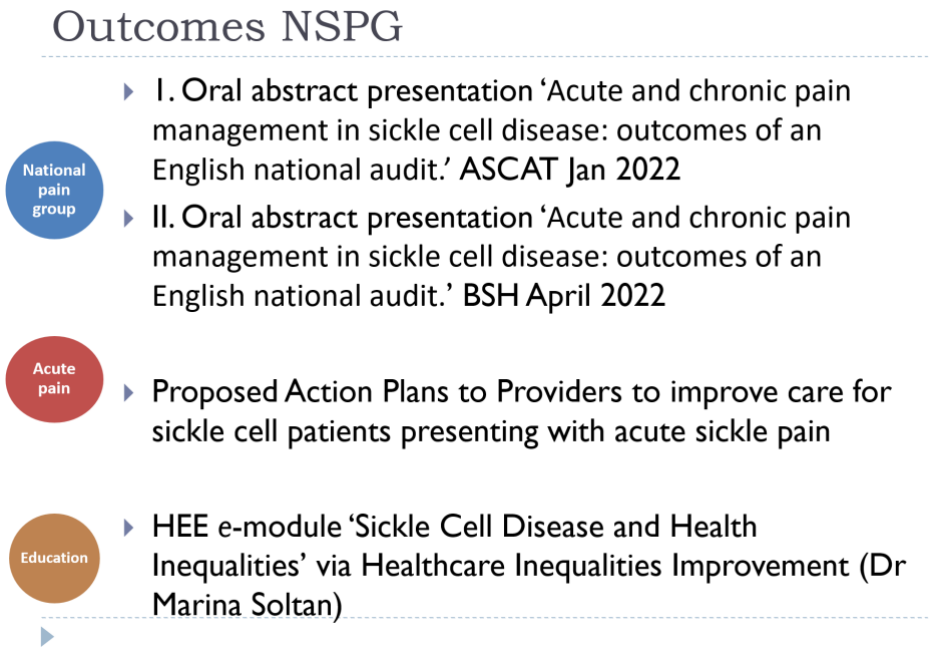



**Image Credit; Dr S. Lugthart*

## 9. NEWBORN OUTCOME SCREENING

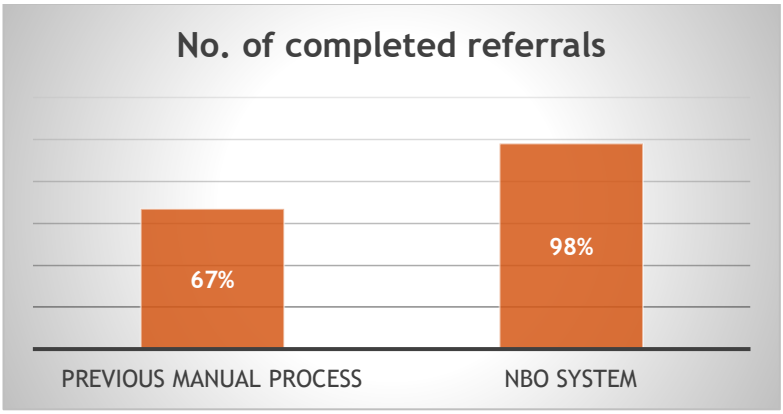
This project is led Amanda Hogan (PHE) and also facilitated by Emma Proctor (PHE). The New-born Outcomes (NBO) system is set up to refer babies, following a screen positive result (from the new-born bloodspot programme) for SCD and Thalassaemia, to clinical services.

All labs and associated clinical networks are now live on the system. This means all SCD and Thalassaemia positive babies born since March 2021 will be on the system. The first six months had the manual pathway and NBO systems running in tandem, to ensure the system worked well. Of the 13 labs, 10 have fully transitioned to NBO solution as a sole method of referral, while transition of the remaining 3 labs is posing a bit of a challenge, particularly in South West region, which has a small number of cases and testing is difficult. Because the NBO links into the NHR, it has been advised to transfer the baby records from NBO to NHR instead of creating a new NHR record.

Of the 579 referrals received in the last 3 years, only 12 were incomplete- i.e. 98% completion. In the previous manual process there was a 60/70% completion rate re outcome data. 83% of records are now closed when the new-born pathway is complete with a majority transferred to

NHR. Centres also receive automated emails when standards are breached or at 6-motnhs of age, when records need to be closed.

**[Fig. 9.i] Number of completed referrals- Old v New NBO system**




There were discrepancies regarding matching parental screening results and pre-natal diagnostic (PND) testing results due to the myriad manual processes. However, an electronic alert card has now been created and is currently at user acceptability testing stage. This will automatically link parental screening, PND reports to new-born positive screen records. This will be available to the labs interpreting the data and for clinicians seeing the families.

## 10. NEW THERAPIES

A number of centres have reported the roll-out of Crizanlizumab and Voxelotor treatment with their patients, as captured in the HCC updates further below. Crizanlizumab is currently available under a Managed Access Agreement (MAA) while Voxelotor is available via an Early Access to Medicines Scheme (EAMS) with a market license due to be published June 2022 with a committee meeting due at the end of 2022.

Both drugs have had national guidelines produced by NHP Working Groups, and been disseminated and published on the NHP Website. There is a patient leaflet also in progress and currently in patient consultation stage.

Use of other novel drugs haven been reported via the NHP MDT as retrospective cases, noted below. The NHP will continue to encourage more of these cases to be shared with the panel in order to monitor the use and trends, as there is awareness that cases presented are not representative of use within the services.

| Novel Drug | Reported Instances of Use |
|---|---|
| Eculizumab | 5 |
| Rituximab | 2 |

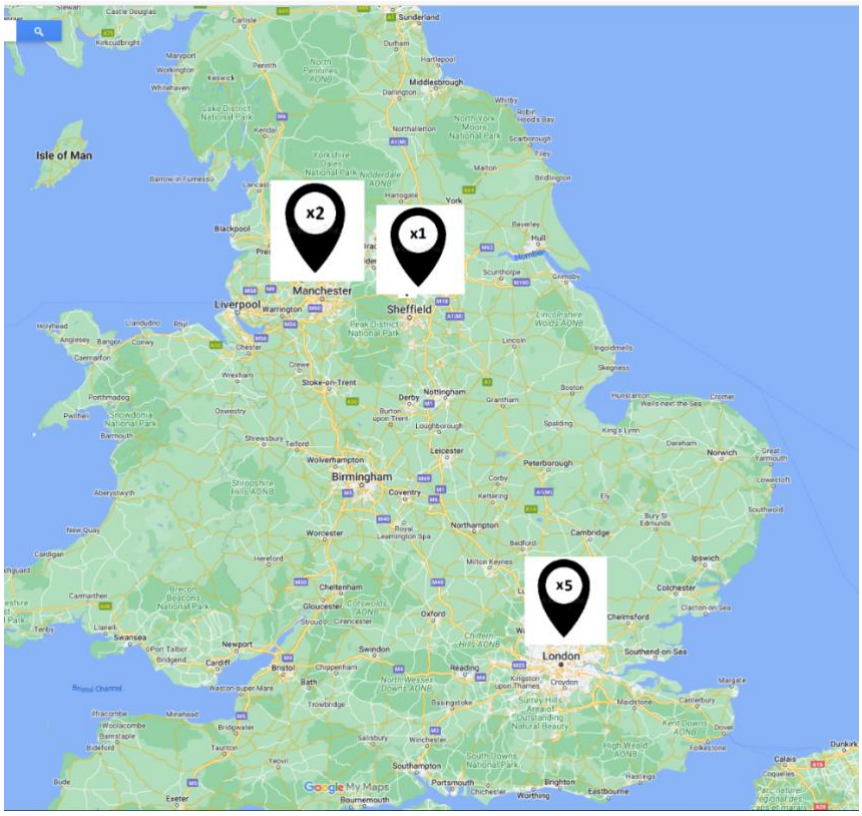
## 11. STEM CELL TRANSPLANTATION FOR SICKLE CELL

The Sickle Stem Cell Transplantation subgroup, led by Drs Ben Carpenter and Victoria Potter, continue to meet regularly. Recent focus has been on designing the Adult Sickle Cell Sibling Allogeneic Transplant protocol, the Adult Sickle Cell Haploidentical Hematopoietic Stem Cell Transplant protocol, and the monitoring of the current, and growing, sickle transplant cohort from group members' centres.

The Group have recently submitted their final draft of the REDRESS protocol -haploidentical HSCT study for SCD- for Ethics Committee (REC) review, with a view to hopefully roll out the study in Autumn 2022. The primary end point being Sickle-free survival, based on occurrence of VOC and transfusion requirement. A national protocol for the Haploidentical HSCT has been ratified by the group- based on the Vanderbilt/St Marys experience- this protocol will be used within the REDRESS study, and for patients receiving haploidentical HSCT outside of this study.

The group's regular meeting also serves as a sharing and learning platform for discussing patients undergoing transplant. Of the represented centres, there are approximately 8 patients having undergone transplant from sibling donors; Kings 4, UCLH 1, Sheffield 1, Manchester 2. Each patient case progress is reviewed as well as general joint learning and consensus on matters including management of sirolimus levels; rightly interpreting the meaning and composition of HbS levels, particularly actions that should be taken in the case of mixed chimerism.

**[Fig 11.i] NHP Transplant Group – Current patient spread**




A formal analysis of the outcomes of the first 10 patients will be taken when this milestone is reached. The group have also hosted a guest colleague from the Netherlands (Dr Erfan Nor), to share on their advanced experience of the alemtuzumab TBI protocol for siblings and handling mixed chimerism. There is a potential for further collaboration.

## 12. 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. 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 implementation of the recommendations made by the SCTAPPG. Haemoglobinopathy services have been working hard, before and after the report to improve the care and wellbeing of their patients, however, this report brings to the fore the key issue that most of the changes that need to be made are in

areas outside of and beyond the realms of Haemoglobinopathy teams. Hence a lot of communication and collaboration has been undertaken by HCCs. Particularly with Emergency Department protocols and training for non-Haemoglobinopathy clinical staff who come in contact with SCD patients. This includes letters, training modules and other direct communication to SHT/LHT leaders, Royal Colleges, Deans, and Heads of nursing. Social media and presentations at Trust Risk and Governance meetings have also been a means by which awareness and dialogue towards change are being encouraged. The network was also encouraged to reach out to NHS Confederation and NHS providers.

### 12.1 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.

# 13.NETWORK PARTNERS

## 13.1 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 conditions. 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.

Recently, Chief Executive, John James MBE, shared the Society's stance on the importance of reporting to the NHP all deaths in order to allow proper oversight the circumstances surrounding the deaths but also to ensure these deaths are processed appropriately on an LHT, SHT and HCC level. They are aware of deaths in the community that indicate possible systemic failures but which have not been reported to, and hence reviewed by, the NHP.

There is a question on how effectively SHTs and HCCs are communicating regarding Voxelotor, as some patients were not aware of this drug. SCS believes this falls to the SHTs and HCCs as other bodies like SCS and GBT are not ethically allowed to promote these drugs.

Most pressing to note was the status of a greater London SHT, in light of their damning CQC report published in February 2022. It is felt that there should be a clear, fair and consistent process of assessing whether a Trust can keep their SHT status, in light of such indicting reports, while taking into account any fragility in the matter and positive efforts that surround the service. It was noted that there was another Trust in a similar situation a few years ago, and their SHT status was in question the, but that does not seem to be the case at all for NMH. This is being taken under advisement by the NHP to consider what action to be taken though, on the face of it, the HCC would be responsible for handling this.

## 13.2 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.

In recent reporting, Roanna Maharaj shared that the North of England are facing a lot of issues-racism being a huge factor. Patients fear for their lives following transfusions because of the low level of care received, and in listening meetings they shared about being told to stop being ungrateful because the care here (UK) is better than the countries they are coming from and that if in their home country, they would be dead. This had been shared with Sajid Javid, who was sympathetic and expressed commitment to the cause.

There were problems noted with initiation of transfusion, particularly with patients transitioning to new practices/centres, say for university.

Emergency and inpatient education and training still needs a lot of work with a lot more Thalassaemia patients presenting than anticipated. There needs to be more done for Thalassaemia in these areas, just as there is for Sickle Cell Disease.

Other prominent issues for patients in the North were CVAD (Central venous access devices) maintenance, Exocrine pancreatic insufficiency (EPI) being wrongly diagnosed even as presentations increase, Liver fibrosis, bone pain, and nutrition (there needs to be a formalised guidance and specialised Thalassaemia nutrition). Dr Nandini Sadasivam has been instrumental in this latter area.

Following the UKTS Survey, one of the outcomes was that ethnicity is an issue that affects life expectancy in the Thalassaemia community. E.g. life expectancy is a lot lower in the Asian population than in the Mediterranean population. The Asian population have more secondary conditions that other Thalassaemia populations.

The new clinical standards will have updates on previously-listed topics as well as a few new ones (e.g. qualitative and end-of-life care. It is hoped to be published 19th October 2022 (National Thalassaemia Day) or at the UK forum in November 2022.

UKTS have submitted their comments on the Lucpatercept (BMS) for Non Transfusion Dependent Thalassaemia (NTDT) as part of NICE HTA. Feedback is awaited. The Society are trying to update the website with news of new Clincal trials with the help of a new employing who has a PhD in genetics and microbiology.

Some clinical trials to note;

Gene Editing: Vertext Pharmaeuticals,

SLN-124: Silence Therapeutics,

iMR-697: Imara

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

STANMAP is a national professional organisation, formally established in 2016, dedicated to supporting nurse specialists, midwives, health and allied care professionals looking after clients and their families with, and at-risk of haemoglobinopathies in the UK and internationally. It is chaired by Nkechi Anyanwu.

A key update for this body is in the educational event which had been delayed by covid-19 but eventually took place as a webinar on 13th January 2022. It was well-attended by nurses, midwives and allied professionals, as well as UKTS and SCS representatives. This was Chaired

by Dame Prof Elizabeth Anionwu. Presentations from Prof Inusa (NHP), Emma Proctor (PHE) and Lola Oni (CNS, Brent).

Topics included updates on covid-19 implications and reasearch on haemoglobinopathy patients, as well as newborn blodspot screening. The Nursing Competencies review by the Royal College of Nurses was of particular note, in light of the *No One's Listenging* report which highlighted the reduced or absence of adequate nursing eduation in the area of haemoglobinopathies. Following this, Deans of universities and Heads of Nursing have been approached consider putting SCD and Thalassaemia care in the nursing syllabus. The competencies review has lead to agreement on the competencies and there is an approval awaited, which may take a while due to their hefty waiting list.

The *No One's Listening* recommendations are still being reviewed, with the SCS Parliamentary & Policy Officer giving and overivew on the education day. Retired members and the patron of STANMAP were acknowledged for their contribution to STANMAP and service delivery locally and nationally.

It is hoped that the next educational event planned for summer 2022 will be in-person, which should encourage more networking and collaboration.

There are a lot of workforce issues, including a number of upcoming retirements, presenting the issue of a loss of a wealth of experience. Retention of nurses is also a difficulty.

### 13.4 UK FORUM FOR HAEMOGLOBIN DISORDERS (UKFHD)

The UKFHD is a multidisciplinary body of experts who are 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. Dr Farrukh Shah, UKFHD Chair, shared UKFHD updates including several departures and retirements such as Dr Paul Telfer standing down as Vice Chair, and Lola Oni retiring. Expressions of interest were to be sent to members to join the committee as formal members – Scotland representatives included.

Peer review standards are updated with plans for the next cycle of peer reviews underway. Research meetings led by Dr Telfer have been going well. Roanna Maharaj has catalysed a revision of UKTS standards for the care of Thalassaemia patients. Chapters have been sent out for contribution.  There is also a new website due for launch in the new quarter, featuring the new logo.

## 14. 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. One such recent effort has been regular attendance of an NHP representative at HCC meetings and events, which elicits very insightful problem-solving beneficial to both parties. The recent agreement to host and promote the HCC curated learning on the NHP website has been one such other endeavour, as has liaising with the CRG on HCC queries.

HCCs have also been encouraged to share annual reports with NHP so as to reduce duplication of HCC reporting by excess audits and other reports, a sentiment that has been intimated by some HCC operational leads. It has also been suggested that perhaps it would be helpful for NHSEI to share with NHP, SSQD submitted by HCCs to their commissioners.

At the last governance meeting, it was agreed that HCCs should share mortality data with the NHP, so that appropriate cases, particularly where systemic failure is indicate, could be discussed at the national MDT.

The Business Operations/Governance meeting also serves as forum for HCC leads to share updates, challenges and achievements. Below is a summary of these, shared at the April 2022 Business Operations/Governance meeting (noted in alphabetical order).

### EAST LONDON & ESSEX

The HCC is currently in the process of preparing the annual report. They have worked hard to ensure mapping across the network actually works for them as well as NHR purposes. This has been good for the pan-London paediatric escalation pathway, of which development they are part. This is complete and with Commissioners now for approval. The next step is to see how/if it is adaptable for adults needing escalation in this specialist area.

MDT engagement is good, including approval for new therapies for local hospital cases. TCD QA engagement is good but there is a need to ensure training to maintain skill sets.

There are 2 main areas of challenge; 1) Block contracts – not meeting needs of SHTs to deliver on requirements, particularly affecting psychology support and staffing. Adoption of mandated technologies may help with delivering new therapies. 2) There is a problem with commissioning and designating of one of the SHTs (Queens Hospital) due to infrastructure of delivery of specialist care. This impacts other SHTs. This has been ongoing or a number of years. There are potentially some changes in working models in East London that may change this.

## EAST MIDLANDS

The region is still struggling with finances. Not because of unknown factors but due to the previous network in place and now it is not clear which is new or old money. Block contracts have further increased the confusion. Getting clarity on this has been challenging.

There has been patient reluctance re Crizanlizumab but Nottingham were due to start their first patient in the first quarter of 2022/2023. The HCC had been working with Citizens Advice on a welfare role and have appointed to that.

NHR mapping has brought clearer perspective on some centres, particularly east of the region e.g. Peterborough Hospital. Commissioners have been contacted and will be meeting to take things further.

## NORTH CENTRAL LONDON AND EAST ANGLIA

There was successful roll-out of Crizanlizumab at UCLH and across the network, including LHTs, though there is an issue of lack of capacity at the larger centres, due to no concurrent funding for staffing to deliver this intervention.

Cambridge has successfully become an SHT, however it has not been allocated any funds to carry out functions. Major challenges around staffing at the North Middlesex for which there are attempts to address. MDTs for sickle cell and thalassaemia/rare anaemia very successful and well attended across multiple regions. Educational programme ongoing and popular across the network.

## NORTH EAST & YORKSHIRE

The regional lead concurs with the summary of challenges shared by North West lead. North East & Yorkshire is up and running with Crizanlizumab and adult transplants are going through.

There have been good results aligning Bradford, which is done via outreach from Sheffield. While this is good for Bradford, it has raised issues regarding lines of responsibility and funding. There has been discussion about 24-hour cover rota for north of England, which is an important requirement that an SHT have 24-hour outreach advice available from specialists. There needs to be different rotas for paediatrics and adult haematologists but clinician numbers are not sufficient.

Difficulties engaging with SHTs is not always due to reluctance. As an example, two SHTs are run by one part-time haematologist who does a little in both centres, nurses have limited capacity, and all have multiple commitments. With increasing referrals and admissions, there is a worsening strain on skeleton staff available. While increased referrals are a sign of success of the Centres, it could backfire with inadequate infrastructure.

## NORTH WEST

Following the recent APPG on Thalassaemia report, there is clearly a need for more development in Thalassaemia and Rare Anaemias in the North. There has been one death, reported to the HCC, in March 2022. The HCC clinical lead is awaiting a report but aware it was a heavily iron-loaded patient found dead at home. The GP would not sign the death certificate and this is now a coroner's case. Once discussed at HCC it will be brought to the NHP.

It has been acknowledged that there are discrepancies in MRI iron assessment tools within the region. Two-monthly, hourly teaching sessions on Thalassaemia and rare anaemias have been running for 6 months and proving very effective, with good feedback. A significant number of iron overloaded patients have gone down to good levels once on the right dosage of treatment. Barriers to this balance is not knowledge, but time and manpower for the continuous monitoring etc., which are a real struggle, especially as a single haematologist with other haematology demands and clinics. Thalassaemia is an orphan service in the North and has many challenges and needs.

Training in the North/outside London is limited-to-absent for Thalassaemia, with variation in practice across the region. There needs to be more than HCC MDTs which are also very helpful.

Poor patient attendance is also due to patient/patient guardians' life demands (work/school etc.), multiple attendance and inconvenient Clinic/Day Unit times. Hence, not all non-compliance. These patients then slip through the net and develop morbidity, often not attending until very sick, which is often late.

A lot more attention and discussion is needed. HCC lead has proposed to do an audit to see if this is only an issue in the North, and to address issues such as: the general iron loading levels across the nation, whether there is variation in iron loading across ethnicities, whether there is adequate iron chelation support systems for patients struggling with this issue.

## SOUTH EAST LONDON AND SOUTH EAST

The HCC has been giving patients Crizanlizumab for about 6 weeks to April 2022, though currently only at King's College Hospital and Guy's & St Thomas' Hospital. The other SHTs in the region have not yet started and this need for progression is recognised. Tim Smith, Haematology Data Manager was kindly covering the HCC manager until Liliana Duarte commences the role on the role on 11th July 2022. There has always been a decent programme for regional education, led by Dr Subarna Chakravorty, but the HCC will also feed into the proposed national, HCC curated education scheme. There has also been noted a problem at Guy's & St Thomas' regarding iron assessments, though mainly for Sickle and not Thalassaemia patients. One workaround was booking FerriScans on transfusion days, which can reduce the substantial diary burden.

## SOUTH WEST

A Crizanlizumab pathway has been set up in the Bristol Trust and the first patient due to be infused in May 2022. The HCC have however found it difficult to engage other centres in the region in getting this up and running. There has been heavy involvement with the sickle pain CRG subgroup which is a fully staffed team with Pharmacist, Psychologist, MDT Coordinator and patient support worker.

## WESSEX AND THAMES VALLEY

The HCC has had an unexpected and unexplainable doubling in the amount of admissions for a wide range of issues including sickle crises. The cause is uncertain. They are working on the Crizanlizumab pathway with some patients on Voxelotor. Lesley McCarthy, education lead, is collaborating with other HCC education leads to share ideas and not duplicate. Proposal sent to Professor Baba Inusa. The service Managers group is still ongoing, coordinated by WTV HCC Manager, Manuela Sultanova, with variable attendance, but proving a good platform for support, and information sharing.

## WEST LONDON

Referrals for Crizanlizumab have been received from all SHTs and the first patient has been treated recently with no barriers to delivery of care. HCC MDT is going well with good quantity of referrals across North West and South West. Some challenges engaging LHTs with the MDT which may be due to the fact that consultants in LHTs are not Haemoglobinopathy specialists and cannot attend all MDTs for the broad areas they cover.

Medical Workforce challenges are present in North and South West London with vacant consultant posts. The recent departure of Lola Oni has left a gap in substantial specialist nursing expertise across the region. A new HCC education lead has now been appointed and the education programme is going well, with sessions available via the HCC's website and YouTube channel.

An adult sickle cell disease guideline for the region is close to completion, with the paediatric guideline in the works. Dr Kofi Anie MBE shared this with the PPV group which he facilitates. Of their various work streams, a key area is involvement in audits. They have also been involved in educational sessions; notably one on pain management and one regarding A&E education. The PPV are keen that the HCCs feedback on SUIs, particularly if involving system-wide issues. Professor Mark Layton and Dr Kofi Anie MBE wrote to all the Trusts regarding the *No One's Listening* report and Trusts have responded with promises of what they will do. PPV group are keen to know more about the outcomes of this.

## WEST MIDLANDS

The year in question started slow due to delays in recruiting a network manager, who has now been in post since circa November 2021. Things are now moving at a reasonable pace. The region's website has a due launch date of May 2022. There have been various non-haematology

targeted training/education events, arranged by Dr Shivan Pancham. Attendance to these events were the challenge as it was not people's core interest/need. There is collaboration with East Midlands to host an annual Midlands Education Day in June 2022, addressing adult and paediatric haematology. The HCC had their first Crizanlizumab patient early in February 2022, within the adult team. There have since been about 8 patients. Education is still generally a challenge. The national coordinated effort is good. Further challenge in recent months (to April 2022) has been trying to influence and improve the community teams, as HCC does not have direct influence on their staffing or finance. There are efforts to liaise with Commissioning teams on this but it has been hard. There are a significant numbers of parents with children under 5 awaiting new commissioning for SCT and concerned that once they pass that age, it will be more difficult as they will not automatically qualify.

## 15. NHP LOOKING FORWARD

Areas the NHP will be looking to strengthen in the coming year, will be the training and education roll-out, particularly in optimising the learning outcomes and content from the National MDTs, an exercise which is already underway.

The NHP are currently in talks with an organisation who are keen to support a training event for new therapies. It is hoped that this may come to fruition by the end of the calendar year.

The NHP intend to continue developing the website to be a resource for up-to-date policy, clinical and patient information.

A series of short profiles featuring significant personalities within the history and development of the haemoglobinopathy sector is also due to be developed in the latter part of the year.

There will be a focus on updating and monitoring clinical trials taking place nationally within our network.

The NHP plan to carry out an audit for HCC activities covering education, training and clinical trials, amongst others.

Following highlighted discrepancies observed in iron overload assessments, flagged by Dr Nandini Sadasivam in her update on the North West HCC, and the role that variations in FerriScans vs MRI T2 scans played in these, there will be an evidence gathering exercise and possible policy application regarding standardisation of this process. This issue has notable impact on patients' health and safety.

We are expecting, in place of the current governance and responsibility document, a service specification outlining key expectations/deliverables for the panel, which we welcome to position ourselves for 2023/2024.

Drive for more equity and range in regional representation at the monthly MDT meetings, as well as the variety of clinical scenarios etc.

Obtaining robust access to NHR data and reporting, to enhance activity oversight and avoid delayed action and duplication of data requests to HCCs.

Continued engaging and supporting of Trusts to achieve the 7 key recommendations for Trusts from the *No One's Listening* report around the following issues, and which effects are echoed in updates from HCCs:

1) Sharing internal reviews with NHP
2) Trusts developing pain action plans in line with National Sickle Pain Group
3) Royal College of Emergency Medicine and Royal College of Physicians developing guidance for A&E staff

4) Trusts informing Haematology teams on SCD patient admissions
5) Trusts developing individualised care plans in partnership with/accessible to patients/carers
6) Trust conducting regular patient audits and reporting to NHSEI
7) Trusts ensuring specialised service funding meets SCD staffing needs

APPENDIX

## APPX1. MDT METRICS 2021/2022

| 2021-22 - Year to Date | | | | | |
|---|---|---|---|---|---|
| | Q1 | Q2 | Q3 | Q4 | Total |
| MDT Cases | 8 | 13 | 7 | 13 | 41 |

| Quarter 4 - 2021/2022 | | | | | |
|---|---|---|---|---|---|
| | Jan | Feb | Mar | | Total |
| MDT Cases | 5 | 3 | 5 | | 13 |

| HCC Region | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| East London & Essex | - | - | - | - | 0 |
| East Midlands | 1 | 1 | - | 2 | 4 |
| North Central London & East Anglia | - | 3 | - | - | 3 |
| North East & Yorkshire | - | 1 | - | 1 | 2 |
| North West | 2 | 6 | 1 | 3 | 12 |
| South East London & South East | 2 | 2 | 4 | 3 | 11 |
| South West | 1 | - | - | 1 | 2 |
| Wessex and Thames Valley | 1 | - | 1 | 1 | 3 |
| West London | - | - | - | 1 | 1 |
| West Midlands | 1 | - | 1 | 1 | 3 |
| NHP Total | 8 | 13 | 7 | 13 | 41 |

| Adult/Paediatric Split | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| Adults | 6 | 9 | 6 | 8 | 29 |
| Paediatric | 2 | 4 | 1 | 5 | 12 |

| Case Diagnosis | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| HbSS | 7 | 12 | 5 | 8 | 32 |
| HbSc | 1 | - | 1 | 1 | 3 |
| Thalassaemia | - | - | 1 | 0 | 1 |
| Rare Anaemia | - | 1 | - | 2 | 3 |

| Primary Theme | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| Stem Cell Transplant | 5 | 10 | 3 | 7 | 25 |
| Novel Treatment | - | 1 | 4 | 2 | 7 |
| Mortality Review | - | 1 | - | - | 1 |
| Urgent (email) Case | 1 | - | - | 3 | 4 |
| Complex Haemoglobinopathy/Comorbidity | 2 | - | - | 1 | 3 |
| IVIG Therapy | - | 1 | - | - | 1 |

| Transplant Cases | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| Sibling Matched | 5 | 5 | 2 | 3 | 15 |
| Haplo Matched | - | 5 | 1 | 2 | 8 |
| Matched Unrelated Donor | - | - | - | 1 | 1 |

| Transplant NHP Outcome | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| NHP recommended to refer | 5 | 6 | 3 | 6 | 20 |
| NHP - **not** recommended, repeat review ***** ***n1*** | - | 2 | - | 1 | 3 |
| NHP - **not** recommended | - | 2 | - | 1 | 3 |
| Successful Referral Rate | 100% | 60% | 100% | 75% | 76.9% |

| Novel Treatment Confirmed Instances of Use reported to NHP | Q1 | Q2 | Q3 | Q4 | 2021-22 - Year to Date |
|---|---|---|---|---|---|
| Eculizumab | - | 1 | 3 | 1 | 5 |
| Rituximab | - | - | 2 | - | 2 |
| **Total Instances of Use * *n2*** | 0 | 1 | 5 | 1 | 7 |
| Retrospectively Referred ***** ***n3*** | 0 | 1 | 3 | 1 | 5 |
| Retrospectively Referred % | 0% | 100% | 100% | 100% | 100% |

*Return to reference Paragraph*

*** Note** - n1 - Outcome is recorded as not to currently recommend, but that a case might be brought back for repeat review at a later point once appropriate actions taken / criteria met.

*** 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* |
| Attachment 3 | NHR Report on TCD Scans, Annual to 1 April 2022 | A3 - NHR_TCD_Scans_Rep | *Return to reference Paragraph* |