Paediatric Special Interest Group: British Society of Haematology

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HYDROXYCARBAMIDE THERAPY AMONGST CHILDREN WITH HOMOZYGOUS SICKLE CELL DISEASE IN LARGE DISTRICT GENERAL HOSPITAL – A QUALITY IMPROVEMENT PROJECT

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Background Sickle cell Disease (SCD) is a chronic debilitating disease associated with recurrent vaso-occlusive crises, progressive organ damage, and other life-limiting complications. Hydroxycarbamide has been proven to be of clinical benefit in children with SCD and should be offered to all children with SCD who are aged 9 months and above irrespective of their disease severity. It reduces the incidence of vaso-occlusive crises, transfusion requirements, hospitalization and ultimately gives better quality and longer lives.

The clinical efficacy of hydroxycarbamide is achieved by dose escalation, compliance, and laboratory monitoring of toxic effects. Despite the proven benefits of hydroxycarbamide, it is usually underutilized by patients due to fear of side effects and lack of confidence by caregivers to comply with therapy.

Objectives Our study was set out to evaluate our practice of offering and monitoring hydroxycarbamide against the guideline produced by the British Society of Haematology in 2018 for the use of hydroxycarbamide in children with SCD. We also looked at the admission rate, quality of life and improvements in providing the service with the help of the specialist nurse.

This study highlights shortfalls of hydroxycarbamide dose escalation in clinical practice, logistic approach to overcoming barriers to therapy utilization and cost benefits of the appropriate use of hydroxycarbamide therapy amongst children with SCD.

Methods Retrospective single-centred study to evaluate how effectively children with SCD are offered hydroxycarbamide, treatment doses, and monitoring of the effects of the drug against current best practice guidelines. Data was collected by retrieving information from medical notes, electronic blood records, annual review documents, team hydroxycarbamide planner, and discharge summaries.

A qualitative arm of the study was also carried out by telephone interviews of parents by specialist nurse practitioners to understand barriers to the use of hydroxycarbamide and how obstacles were overcome.

Results All eligible children were offered treatment. 62% were started on the appropriate starting dose of 20 mg/kg. 76% had blood monitored within 2–4 weeks of commencement. 90% had regular blood monitoring every 3 months. Only 20% had appropriate dose escalation to the maximum tolerated dose. Only 25% of children had their quarterly review.

A significant reduction of 80% in hospitalisation with SCD related complications. An average increase in haemoglobin of 20–30 g/L was also noted.

An improvement in the quality of life was reported universally with increased parental confidence in medication.

Before the appointment of a specialist nurse into our service, 20% of eligible patients were treated with hydroxycarbamide. We overcame the barrier of poor uptake and communication gaps with the support of the specialist nurse.

Conclusions The guidance has helped us to offer hydroxycarbamide and help families to improve uptake. The role of a specialist nurse is invaluable in supporting the families and monitoring the toxic effect of medication. The quality of life of children has improved significantly with reduced hospitalisation and thus saving cost to NHS.

A nurse-led Hydroxycarbamide clinic is being introduced to further improve our service. We hope that this study will inform paediatricians on simple ways of improving therapy compliance and consequently provide better cost-effective care to children with SCD.

Quality Improvement and Patient Safety

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UNCOVERING THE ROLE OF A TELEHEALTH DEVICE IN PROVIDING QUALITY PAEDIATRIC CARE REMOTELY

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Background Innovation that improves the quality of care children and young people (CYP) receive is a major theme of the RCPCH 2040 vision. Paediatricians are being challenged to move beyond the virtual consultation adopted during the COVID-19 pandemic and utilise new technology to monitor, care for and treat CYP; empowering them to access services in a way that achieves this.

Bradford Teaching Hospitals NHS Foundation Trust (BTHFT) is the first NHS site in the UK to pilot the use of a handheld Telehealth device called TytoCare. The device enables healthcare professionals, CYP or their carers to link with clinicians remotely, in real time or offline, to enable examination of the heart, lungs, skin, ears and throat.

Objectives Our pilot aimed to assess the usability and potential benefit of TytoCare in CYP with chronic respiratory or life-limiting conditions to reduce face to face reviews by health care professionals, acute admissions and the burden of illness for these CYP and their families.

Methods 1. Stakeholder engagement with the BTHFT Executive, the Paediatric multidisciplinary team, Informatics, Information Governance Department, Clinical Engineering and the TytoCare Company.

2. Workflow design:

a. Professional workflow: a device was utilised by the specialist nurse in each team when a review from a senior doctor, other speciality or other allied health care professional was needed.

b. Patient workflow: families were chosen according to where the clinical team felt there would be greatest benefit

The device was used within an individualised care plan to assess acute or routine review with a member of the team from home.

3. Feedback on the usability, workflow and key outcomes was gathered at various stages of the project:

a. A feedback survey completed by the healthcare professional after each consult.

b. Data was collected via the TytoCare system for each consultation.

c. End of pilot surveys were completed by staff and families.

Results 48 consultations were undertaken using TytoCare during the pilot. We had healthcare professional feedback for 46 of them reporting the following impact: 100% of consultations felt to provide reassurance to families, 98% had a positive impact on the CYP. Two hospital assessments, 3 inpatient admissions, 13 face to face clinic appointments, 4 home visits, 23 face to face physiotherapy reviews and approximately 329 miles travelled were saved.

Conclusions In this pilot the TytoCare device was found to be easy to use by professionals and carers and to be reliable and effective in providing safe and quality care for a select group of CYP at home. The pilot highlighted the impact technology can have in reducing the burden of chronic illness for families. It also demonstrated that technology could be used successfully to improve access to care for some of our most vulnerable families.

British Association for Paediatric Nephrology

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ALPORT SYNDROME IN CHILDREN: A 15-YEAR SINGLE CENTRE EXPERIENCE

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Background Alport syndrome (AS) accounts for 0.2%-0.6% adult and 2-3% paediatric patients with end stage renal disease (ESRD), with increased risk of chronic kidney disease in those with heterozygous mutations. Angiotensin-converting enzyme inhibition (ACEi), the current first line therapy, can delay the onset of ESRD. Clinical trials of newer therapies eg, bardoxolone methyl and anti-microRNA-21 are underway. Recent classifications adopt an inclusive genetic-based approach to help early diagnosis, monitoring and treatment.

Objectives The objectives of this study are to review the characteristics in our cohort of patients with AS, and to compare patient characteristics between the different genetic-based classifications.

Methods A retrospective review of all patients with pathogenic COL4A3, COL4A4 and COL4A5 variants followed up at Great Ormond Street Hospital between 2005 and 2020 was undertaken. Patients were classified as: (i) males with X-linked AS (XLAS); (ii) autosomal recessive AS (ARAS); (iii) autosomal dominant AS (ADAS); and (iv) females with XLAS.

Demographics, genetics, presenting symptoms, length of follow up, clinical parameters at the last follow up were collected.

Results Twenty-eight children with AS were included. The median age of presentation was 5.4 years (IQR 4.5-11.1) with a median follow up of 3.0 years (IQR 1.3-7.3). Median age at latest follow-up was 11.8 years (IQR 8.1-14.6). The median urine albumin-creatinine ratio at latest follow-up was 38.6mg/mmol (IQR 3.3-127.4), with median serum albumin of 41.5g/L (IQR 38.0-44.0) and median eGFR of 115.4ml/min/1.73m² (IQR 99.2-138.3). The most common presenting symptom was isolated microscopic haematuria (39%).

Eight patients transitioned to adults: 3 had male XLAS, 4 had female XLAS and 1 had ADAS. The median age of these patients at last follow-up was 15.9 years (IQR 14.6-16.4), with a median follow up time of 4.8 years (IQR 3.0-9.1). The median eGFR at last follow-up was 100.8 ml/min/1.73 m (IQR 95.6-107.5), median serum albumin was 46.0g/L (IQR 39.0-42.0), median urine albumin creatinine ratio was 45 mg/mmol (IQR 5.3-201.0). One patient with male XLAS progressed to ESRD at the age of 14.

Conclusions Microalbuminuria was the earliest event observed in the progression of AS in this cohort of patients, most significant in male X-linked and autosomal-recessive AS patients. Majority of children had normal serum albumin and eGFR during the study period but we have identified 2 patients for consideration of enrolment in clinical trials.

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Abstract 1397 Table 1 Comparison between the different groups of patients. Data presented as median (interquartile)

Males with XLAS	n=8 ARAS	n=7 ADAS	n=5 Females with XLAS	n=8	p-value
Age of presentation (years)	5.8 (4.6-10.5)	4.7 (3.4-9.2)	7.5 (4.9-12.8)	5.4 (4.1-11.8)	0.69
Age of genetic confirmation (years)	12.7 (6.2-13.6)	5.1 (3.9-8.8)	8.5 (5.8-13.2)	11.8 (5.0-13.8)	0.22
Length of follow up (years)	5.4 (2.0-9.1)	1.4 (0.5-4.9)	2.6 (1.4-3.0)	4.8 (1.6-8.5)	0.23
Age at latest follow up (years)	14.1 (9.3-15.0)	10.2 (5.3-11.9)	10.5 (6.7-15.1)	14.2 (7.7-16.1)	0.33
Urine albumin-creatinine ratio at latest follow up (mg/mmol)	40.0 (1.6-172.5)	135.8 (65.5-421.0)	1.2 (0.8-21.7)	9.5 (6.0-301.0)	0.03
Serum albumin at last follow up	39.0 (38.0-44.0)	38.0 (31.0-43.0)	45.0 (43.3-46.8)	41.5 (37.8-46.0)	0.07
eGFR at last follow up (ml/min/1.73 m ²)	131.6 (98.8-152.5)	104.8 (97.4-135.1)	142.6 (97.6-168.4)	106.0 (100.5-132.7)	0.58