

## ECMC Paediatric Network - Highlights and Impact Summary - Summer 2025

### Scientific & Clinical Excellence

- **StratMedPaeds:** The paediatric ECMC network is the critical framework allowing successful conduct of our genomic medicine programmes StratMedPaeds (SMPaeds1) and SMPaeds2.

This [programme](#) is first of its kind and focused on using liquid biopsies to advance precision medicine for children and young people with cancer and aims to improve treatment outcomes by identifying actionable genetic changes in tumours through genetic testing.

SMPaeds1 has enrolled 850+ relapsed childhood cancer patients and enabled NHS adoption of circulating tumour DNA (ctDNA) and RNA fusion panels and Whole Genome Sequencing diagnostics. ECMC infrastructure was critical to this study with patient recruitment and sample collection and transport being dependent on the paediatric ECMC network infrastructure. The renewed CRUK-funded SMPaeds2 programme, which opened in 2025, will also utilise and benefit from the paediatric ECMC Network for the collection, processing and dispatch of patient samples, and for sharing of critical data.

- **BEACON-2 Trial:** A pan-European neuroblastoma trial building on UK-led BEACON, now open in 8 ECMC sites, will compare the adjuvant therapies in a phase 2 randomisation and will include small non-randomised signal-seeking phase I arms to test novel combinations.
- **MINivAN:** Recruitment to the final expansion cohort was extended because of promising response data, with recruitment of 43 patients (36 from UK) in total. Preliminary toxicity and response data was presented at the international Advances in Neuroblastoma Research congress in Washington in May 2025.
- **HEM-iSMART:** A precision medicine platform trial for relapsed haematological malignancies is due to open in the UK in Q2/3 2025. The ECMC Network's Trial Development theme has been instrumental in leadership and enabling trial setup and molecular profiling.
- **Therapeutic Drug Monitoring (TDM) Programme Expansion:** As part of the Network's ongoing focus on improving access to research for Teenagers and Young Adults, the Paediatric ECMC Network's Pharmacology theme has updated their approach so that the programme now includes TYA patients (<30 years).

Their work through this theme has demonstrated impact on developing dosing strategies for patients, especially in neonates and infants based on sound pharmacological rationale. A recent manuscript published in the British Journal of Cancer in 2024 highlights the benefits of utilising novel dosing approaches for carboplatin and the benefits of a TDM approach to treatment in neonates and infant retinoblastoma patients.

## Early Phase Trial Delivery & Operations

- **Industry Collaborations:**

- **FaR-RMS:** Bayer-funded trial in relapsed Rhabdomyosarcoma (RMS) led by Birmingham ECMC.
- **LIGHTBEAM:** Merck's platform trials in paediatric cancers. Currently open in Cardiff, Newcastle and London
- **DETERMINE:** National precision medicine trial with Roche and Novartis, integrating advanced molecular profiling and translational research. The Paediatric programme of DETERMINE is being led by Prof Lynley Marshall.

**Leveraging Funding and Activity:** SMPaeds2 has received add-on funding to support data integration aligning with development of the ECMC network data sharing tools. SMPaeds2 received £41k from as part of the funding grant to link genomic datasets to the NHSE Data Access Request Service (DARS) gateway and to Registry data in 2024. Additionally, SMPaeds2 is linking anonymised, clinically-annotated genomics data to the EU-based PedCanPortal through £500k received from the Data Hopp Foundation.

A follow-on sub-study of SMPaeds2 is currently under planning and has the support of the ECMC Translational Research Forum. This study will plan to enrol patients with high-risk malignancies at the time of diagnosis, with a high chance of relapse, with the aim of validating ctDNA as measurable residual disease (MRD) biomarkers of relapse in childhood cancer patients.

- **Trial Setup Success:** UK was first globally to open, and fast-tracked approvals enabled first global patient recruitment in April 2025 to **GLO-BNHL**, a rare B-cell non-Hodgkin lymphoma international trial.

## Equity of Access & Patient/Public Involvement (PPI)

- **Equity of Access:** The four regional panels (London & South-East, North & Scotland, Midlands & South West, and North-West & Yorkshire), at which children who might be eligible for trials are discussed, ensures equitable access to trials. 2000+ children have been discussed at relapse panels since their creation in 2018 (constituting the overwhelming majority of children relapsing in this time); with 13% enrolled in early phase trials (all others have standard treatment available or are not fit for further curative treatment).

As of March 2025, there were 18 early phase trials available for children with cancer in the UK. *[This is excluding ATIMP trials - Advanced Therapy (ATIMP) trials are not included in the trials above as they can only be conducted at a smaller number of ECMC sites with specific facilities and capabilities].* 15 of these 18 are open with sites in each regional network. The other 3 were open at a single site (RMH/ICR), which has been deemed appropriate for those trials as recruitment was likely to be very low across the UK.

- **PPI:**
  - **ECMC Pharmacology theme's Therapeutic Drug Monitoring (TDM) App:** Co-designed with The Young Person's Advisory Group North East (YPAGne) to improve patient understanding.
  - **Development of the Paediatric ECMC Data Integration Platform:** PPI reps critically embedded and involved in procurement and governance, to ensure a patient-centric approach to development of this national system that will bring together clinical and research data to support improved treatment and access to clinical trials for children and young people with cancer. The platform will support real-time data recording during relapse discussions and integrates diverse datasets, including imaging and molecular data from various sources, enabling new research opportunities.
  - **Clinical Trials:** PPI reps involved in strategic planning of the trial development theme. This happens both at a local level within each Centre, and also at a strategic level with PPI representation (Sara Wakeling) taking a leading role in the Trial Development theme, ensuring trials are mapped out with patient perspective front of mind.

## Collaborative Activities

**SACHA:** The **Secured Access** to innovative medicines for **CH**ildren, adolescents and young adults with **cA**ncer (**SACHA**) study is an international collaboration to collect real world data on compassionate/off-label use of anti-cancer medications in CYP. In the UK, it is strongly recommended that all potential patients are discussed in the ECMC regional relapse meetings to ensure that there are no suitable clinical trial alternatives before inclusion in the study.

- **IMPACCT:** The Paediatric ECMC is a key partner in IMPACCT, a National initiative to streamline trial setup and delivery, addressing pharmacy, R&D, and workforce challenges. Particular involvement includes input from Prof Darren Hargrave, Prof Amos Burke and members of the ECMC Programme Office.

## Workforce Development

- **Fellowships:** Two fellows have been appointed for 12 month paediatric-focused fellowships at Southampton and GOSH. They have gained hands-on experience in trial delivery, data analysis, and international collaboration. Both won awards at the ECMC Junior Investigator Network Group (JING) meeting in January 2025.