Stratified Medicine Paediatrics (StratMedPaeds) - a study looking at genetic changes in children' s cancer

Submission date	Recruitment status	[X] Prospectively registered
09/01/2019	No longer recruiting	☐ Protocol
Registration date	Overall study status	Statistical analysis plan
28/01/2019	Ongoing	Results
Last Edited	Condition category	Individual participant data
07/02/2025	Cancer	[X] Record updated in last year

Plain English summary of protocol

https://www.cancerresearchuk.org/about-cancer/find-a-clinical-trial/a-study-looking-at-thegenetics-of-childrens-cancer-stratmedpaediatrics (added 07/12/2020)

Background and study aims

This is a UK research study testing tumour (somatic) and normal (germline) DNA and RNA for genetic and gene-expression changes in children, teenagers and young adults with relapsed /refractory cancer. The aim of this study is to identify patients who may be eligible for new targeted anti-cancer therapies. The study will aid research that will help us to more precisely diagnose cancer and understand why some patients do not respond to standard treatments.

Who can participate?

UK children and young adults whose cancer has either come back (relapsed) or not responded to treatment (refractory) and have undergone or will undergo a routine biopsy/surgery to obtain tumour tissue or bone marrow.

What does the study involve?

Participants with a solid tumour provide a blood sample and a piece (or pieces, if available) of tumour collected from their most recent biopsy or surgery, or some of the tumour sample from the original cancer diagnosis (if the hospital still has it). Participants with leukaemia provide a bone marrow sample. The results of the tests are relayed back to the patient's doctor via an expert group of doctors who make recommendations on any available treatments. Patients and /or their parents are asked in advance to consider what information they which to receive in relation to any abnormal genetic results either in the tumour or their normal (germline) genetic code. In addition, the data collected is used and shared for the purposes of clinical research.

What are the possible benefits and risks of participating?

There may not be any individual benefit for the patient and the greatest benefits of the study may not be expected for several years and therefore will predominantly help future patients. However, should something be found in the genetic information of the tumour sample which may help in the understanding or treatment of the patient's cancer (for example, it may provide

a treatment option i.e. clinical trial); then the patient's clinical team will be able to use this information. For solid tumour patients, as the tumour sample will already have been or is due to be taken as part of the care at the hospital, the patient will have only one blood test taken. The discomfort of this blood test is just like any other blood test. For leukaemia patients, the bone marrow sample will already have been or is due to be taken as part of the care at the hospital.

Where is the study run from?

- 1. Royal Aberdeen's Children Hospital
- 2. Royal Belfast Hospital for Sick Children
- 3. Birmingham Children's Hospital
- 4. Bristol Royal Hospital for Children
- 5. Addenbrooke's Hospital
- 6. Noah's Ark Children's Hospital for Wales
- 7. Royal Hospital for Sick Children Edinburgh
- 8. Royal Hospital for Children
- 9. Leeds General Infirmary
- 10. Leicester Royal Infirmary
- 11. Alder Hey Children's Hospital
- 12. Great Ormond Street Hospital for Children
- 13. Royal Manchester Children's Hospital
- 14. Royal Victoria Infirmary
- 15. Queen's Medical Centre, Nottingham
- 16. John Radcliffe Hospital
- 17. Sheffield Children's Hospital
- 18. Southampton General Hospital
- 19. University College London Hospital
- 20. Royal Marsden Hospital Sutton

When is the study starting and how long is it expected to run for? January 2018 to April 2027

Who is funding the study? Cancer Research UK

Who is the main contact? Amina Bukhari Smpaeds@trials.bham.ac.uk

Contact information

Type(s)

Scientific

Contact name

Ms Amina Bukhari

Contact details

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Birmingham
United Kingdom
B15 2TT
+44 (0)121 414 7851
Smpaeds@trials.bham.ac.uk

Additional identifiers

Clinical Trials Information System (CTIS)

Nil known

Integrated Research Application System (IRAS)

246557 (England, Wales, NI), 264925 (Scotland)

ClinicalTrials.gov (NCT)

Nil known

Protocol serial number

CPMS 40156, IRAS 246557 (England, Wales, NI), 264925 (Scotland)

Study information

Scientific Title

Stratified medicine paediatrics: genomic characterisation of relapsed paediatric cancers for diagnostics and stratified therapy

Acronym

StratMedPaeds / SMPaeds

Study objectives

StratMedPaeds is a UK research study testing tumour (somatic) and normal (germline) DNA and RNA for genetic and gene-expression changes in children, teenagers and young adults with relapsed/refractory cancer. The results of the tests performed will identify patients who may be eligible for new targeted anti-cancer therapies and will aid research that will help us to more precisely diagnose cancer and understand why some patients do not respond to standard treatments.

Ethics approval required

Old ethics approval format

Ethics approval(s)

London - Camden & Kings Cross Research Ethics Committee (NHSBT Newcastle Blood Donor Centre, Holland Drive, Newcastle upon Tyne, NE2 4NQ, UK; +44 (0)207 104 8086; nrescommittee. london-camdenandkingscross@nhs.net), ref: not provided

Study design

Non-randomized; Interventional; Design type: Screening, Diagnosis, Other

Primary study design

Interventional

Study type(s)

Diagnostic

Health condition(s) or problem(s) studied

Solid tumours (including lymphomas) or leukaemia

Interventions

Current interventions as of 15/03/2023:

Patients with clinically relapsed/refractory progressive solid tumours (including brain and lymphomas) or leukaemia who undergo a biopsy as part of their standard of care are eligible for StratMedPaeds. Following receipt of valid informed consent, the patient can be registered in the central RDE database (MARVIN). In parallel, FFPE and FF tissue from the current relapse /refractory episode, along with blood and matching material from any previous diagnosis /tumour for solid tumour patients or Bone Marrow aspirate and Cerebrospinal fluid (CSF) if available, from the current relapse/refractory episode for leukaemia patients should be submitted to the central sample hub at Great Ormond Street Hospital for Children.

For each patient, a mixture of techniques will be used, depending on the availability and quality of the DNA, and primary cancer diagnosis and previous testing.

On tumour biopsy material - possible on Formalin fixed paraffin embedded (FFPE) and Bone Marrow/CSF

- 1. Customised next-generation sequencing panels (NGS panels)
- 2. Methylation sequencing

On tumour biopsy material – Fresh Frozen

- 1. Whole exome sequencing (WES)
- 2. RNA-sequencing (RNASeg)
- 3. Low coverage whole genome sequencing (lcWGS)

From blood: circulating tumour DNA (ctDNA)

- 1. NGS panel testing
- 2. Digital PCR

From blood collected for germline analysis

1. Exome or genome sequencing

Previous interventions:

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On tumour biopsy material - possible on Formalin fixed paraffin embedded (FFPE):

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1. Exome or genome sequencing

Intervention Type

Other

Primary outcome(s)

The proportion of cases referred that receive a Molecular Tumour Board Report within 28 days

Key secondary outcome(s))

Outcomes to be achieved within a timescale of up to 3 years:

- 1. The number and proportion of samples successfully analysed
- 2. The reasons for analysis failure
- 3. The proportion of patients with known or unknown somatic molecular alterations
- 4. The proportion of patients with actionable molecular alterations in tumours
- 5. The proportion of patients in which any treatment recommendation can be made
- 6. The proportion of patients in which a treatment recommendation with a molecularly targeted therapy can be made
- 7. The proportion of patients successfully registered on to clinical trials
- 8. The proportion of patients successfully registered on to clinical trials of molecularly targeted therapies
- 9. The proportion of patients who have their diagnosis changed following multi-omic analysis
- 10. Progression Free Survival (PFS)
- 11. Any complications of biopsy
- 12. The proportion of patients with germline events
- 13. The proportion of patients recommended for clinical genetics service referral
- 14. Collection of data on individual genomic, epigenomic and transcriptomic characteristics of refractory/relapsed paediatric malignancies

(added 15/03/2023)

- 15. The number and proportion of leukaemia samples successfully analysed on the DRP platform
- 16. The number and proportion of patients with leukaemia who have a potential therapy identified through the DRP platform

Numerical data will be taken from the case report forms from each participating hospital site. Case report forms will be completed using information taken from the patient's medical record.

Completion date

30/04/2027

Eligibility

Key inclusion criteria

Current inclusion criteria as of 15/03/2023:

- 1. Patients with a relapsed or refractory paediatric tumour (all solid tumours, central nervous system (CNS) tumours and lymphoma) or leukaemia
- 2. For solid tumours: Formalin fixed paraffin embedded (FFPE) tumour available from a biopsy, resection or other surgical procedure that was taken within 8 weeks of trial entry*
- 3. For leukaemia: Viable fresh or frozen Bone Marrow aspirate sample taken at a prior assessment within 8 weeks prior to trial entry**
- 3. Written informed consent of patient/parent/guardian/legal guardian***
- * To allow full multi-omic analysis both fresh frozen and Formalin fixed paraffin embedded (FFPE) tumour plus a blood sample for constitutional (germline) and circulating tumour (ct) DNA will need to be available.

Original diagnostic slides should be submitted at the same time as block from current relapse /refractory episode either in same shipment.

- ** Where available, a cerebrospinal fluid (CSF) sample in the event of an isolated or combined CNS relapse should also be provided in addition to the bone marrow aspirate.
- *** Some adult patients with brain tumours or brain metastases may be incapable of providing their own consent due to the neurological effects of their disease. In such cases, these patients will be classed as an incapacitated adult and a consultee will be sought.

Previous inclusion criteria:

- 1. Patients with a relapsed or refractory paediatric tumour (all solid tumours, central nervous system (CNS) tumours and lymphoma)
- 2. Formalin fixed paraffin embedded (FFPE) tumour available from a biopsy, resection or other surgical procedure that was taken within 8 weeks of trial entry*
- 3. Written informed consent of patient/parent/guardian/legal guardian**
- * To allow full multi-omic analysis both fresh frozen and Formalin fixed paraffin embedded (FFPE) tumour plus a blood sample for constitutional (germline) and circulating tumour (ct) DNA will need to be available.

Original diagnostic slides should be submitted at the same time as block from current relapse /refractory episode either in same shipment or via PathXL (see laboratory manual for further details).

** Some adult patients with brain tumours or brain metastases may be incapable of providing their own consent due to the neurological effects of their disease. In such cases, these patients will be classed as an incapacitated adult and a consultee will be sought.

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Mixed

Sex

All

Total final enrolment

806

Key exclusion criteria

Does not meet inclusion criteria

Date of first enrolment

01/02/2019

Date of final enrolment

31/01/2024

Locations

Countries of recruitment

United Kingdom

England

Northern Ireland

Scotland

Wales

Study participating centre Royal Aberdeen's Children Hospital

Westburn Road Aberdeen United Kingdom AB25 2ZG

Study participating centre Royal Belfast Hospital for Sick Children

180 Falls Road Belfast United Kingdom BT12 6BE

Study participating centre Birmingham Children's Hospital

Steelhouse Lane Birmingham United Kingdom B4 6NH

Study participating centre Bristol Royal Hospital for Children

Uhbristol Education Centre Bristol United Kingdom BS2 8AE

Study participating centre Addenbrooke's Hospital

Hills Road Cambridge United Kingdom CB2 0QQ

Study participating centre Noah's Ark Children's Hospital for Wales

Heath Park Cardiff United Kingdom CF14 4XW

Study participating centre Royal Hospital for Sick Children Edinburgh 9 Sciennes Road Edinburgh United Kingdom EH9 1LF

Study participating centre Royal Hospital for Children

1345 Govan Road Glasgow United Kingdom G51 4TF

Study participating centre Leeds General Infirmary

Great George Street Leeds United Kingdom LS1 3EX

Study participating centre Leicester Royal Infirmary

Infirmary Square Leicester United Kingdom LE1 5WW

Study participating centre Alder Hey Children's Hospital

Eaton Road Liverpool United Kingdom L12 2AP

Study participating centre Great Ormond Street Hospital for Children

Great Ormond Street London United Kingdom WC1N 3JH

Study participating centre Royal Manchester Children's Hospital

Oxford Road Manchester United Kingdom M13 9WL

Study participating centre Royal Victoria Infirmary

Queen Victoria Road Newcastle upon Tyne United Kingdom NE1 4LP

Study participating centre Queen's Medical Centre, Nottingham

University Hospital NHS Trust Nottingham United Kingdom NG7 2UH

Study participating centre John Radcliffe Hospital

Headley Way Oxford United Kingdom OX3 9DU

Study participating centre Sheffield Children's Hospital

Western Bank Sheffield United Kingdom S10 2TH

Study participating centre Southampton General Hospital

Tremona Road Southampton United Kingdom SO16 6YD

Study participating centre University College London Hospital

235 Euston Road London United Kingdom NW1 2BU

Study participating centre Royal Marsden Hospital Sutton

Downs Road Sutton United Kingdom SM2 5PT

Sponsor information

Organisation

Institute of Cancer Research, London

ROR

https://ror.org/043jzw605

Funder(s)

Funder type

Charity

Funder Name

Cancer Research UK; Grant Codes: C34648/A24566

Alternative Name(s)

CR UK, Cancer Research UK - London, Cancer Research UK (CRUK), CRUK

Funding Body Type

Private sector organisation

Funding Body Subtype

Other non-profit organizations

Location

United Kingdom

Funder Name

CHILDREN with CANCER UK (Children with Leukaemia); Grant Codes: 17-325

Alternative Name(s)

Children with Leukaemia, CwC_UK

Funding Body Type

Private sector organisation

Funding Body Subtype

Other non-profit organizations

Location

United Kingdom

Results and Publications

Individual participant data (IPD) sharing plan

The datasets generated during or analysed during the current study will be available upon request from the StratMedPaeds Trial office (smpaeds@trials.bham.ac.uk).

Type of data: Data on the distribution and frequency (incidence/prevalence) of molecular alterations at time of diagnosis and relapse, clinical diagnosis, treatment and clinical outcomes data.

When the data will become available and for how long: : after publication, Indefinitely By what access criteria data will be shared including with whom: data may be shared with external investigators who wish to share clinical trial data.

All requests for data sharing are dealt with on a case by case basis

On receipt of a valid request to share data the trialists will ensure that:

- 1. External parties and trial oversight committees (e.g. Trial Steering Committee) are supportive of the request
- 2. The necessary legal, ethical and regulatory permissions to allow data sharing are in place
- 3. Anonymised data can be supplied
- 4. There is sufficient resource with to deal with the request

For what types of analyses: all requests for data analyses will be reviewed.

By what mechanism: Any request to share data should be submitted in writing to the Trial Management Group. The request should clearly document:

- 1. The scientific rationale of the proposal
- 2. Aims and objectives
- 3. Outcome measures
- 4. Data variables required
- 5. How the data will be analysed
- 6. Indicate what acknowledgements will be made on any publications resulting from the work. Whether consent from participants was obtained: either the patient, parent, guardian or consultee will have read and filled out a form giving written consent to take part in the SMPaeds

study, this includes consent for the use of, and sharing of data, for research, teaching, commercial and scientific publications.

Comments on data anonymisation: all data is subject to the General Data Protection Regulation and Data Protection Act 2018 for health and care research and will be kept strictly confidential. Identifiable information will be removed from the data and replaced by unique codes. The only exception to this is the signed informed consent form which is not anonymised in order to perform monitoring of the consent process.

Any ethical or legal restrictions: if the above conditions are met we will provide the requested data. In some circumstances this may be subject to a Data Sharing Agreement being put in place. Please note: data sharing will usually only be considered once the primary endpoint data has been published in a peer reviewed journal.

IPD sharing plan summary

Available on request

Study outputs

Output type Details Date created Date added Peer reviewed? Patient-facing?

Participant information sheet Participant information sheet 11/11/2025 11/11/2025 No Yes