ISRCTN11966493 https://doi.org/10.1186/ISRCTN11966493

Post haematopoietic stem cell transplant follow-up

Submission date
30/09/2016

Recruitment status
No longer recruiting

Registration date
10/10/2016

Overall study status
Completed

Last Edited
18/11/2022

Condition category
Haematological Disorders

[X] Prospectively registered
[X] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English summary of protocol

Background and study aims
Primary immunodeficiency (PID) is a group of rare conditions (affecting 1 in 15-20,000 people) that reduce the function of the immune system and result in severe infections. Many PIDs start in childhood and are life-threatening. Treatment includes haematopoietic stem cell transplant (HSCT), which involves replacing the patient's own immune cells with donated healthy stem cells taken from the bone marrow or blood. Due to improved techniques, more transplants are undertaken and patients are living longer. However long-term complications can impact significantly affect future health and quality of life. Previous research has focused on short-term medical outcomes. Little is known about health outcomes in adulthood or the social and psychological circumstances of this population. Therefore, the aim of this study is to find out about the long-term physical, social and psychological outcomes for a growing population of adults who underwent HSCT for PID during childhood.

Who can participate?
Patients aged over 16 years who underwent HSCT for PID at Great Ormond Street Hospital 5 or more years previously

What does the study involve?
Participants are asked to complete questionnaires and practical tasks to assess their current health and circumstances. Physical health, psychological health, anxiety, depression, physical health, health service usage, NHS costs, and costs borne by patients and families are assessed. Information is also gathered from medical notes. The data is compared with population norms and a control group of siblings or nominated friends.

What are the possible benefits and risks of participating?
There are no immediate benefits to individual participants. However the information gathered will inform how patients are approached, treated and counselled before and after transplant. Little is known about the longer-term outcome of patients after HSCT. Greater understanding of the outcome of adult survivors of HSCT, and identifying areas of potential strength and difficulties within this population, would allow us to provide more information for the young people and their families to better guide their treatment decisions, particularly when planning for the future. It is also anticipated that the information gathered will inform clinical practice,

policy-making and service delivery. There is no potential for any additional adverse effects, risks, or hazards, pain, discomfort, distress, or inconvenience to the patients as a result of participating in this study.

Where is the study run from?
1. Royal Free London NHS Foundation Trust (UK)
2. Great Ormond Street Hospital for Children NHS Foundation Trust (UK)

When is the study starting and how long is it expected to run for?
May 2016 to April 2019

Who is funding the study?
National Institute for Health Research (UK)

Who is the main contact?
Dr Mari Campbell

Contact information

Type(s)
Scientific

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Additional identifiers

Protocol serial number
1

Study information

Scientific Title
Investigating the long-term outcomes of adult patients who underwent haematopoietic stem cell transplant for primary immunodeficiency during childhood

Study objectives

The overall aim of this project is to evaluate the long-term health outcomes of adult patients who underwent haematopoietic stem cell transplant (HSCT) for primary immunodeficiency during childhood, and to identify factors associated with long-term health outcome. In line with the World Health Organisation (1948) definition of health the study has been designed to look at outcome in a holistic way. Within this aim there are five main objectives:
1. To examine the mental health outcomes of patients following HSCT. This will document the psychological symptoms and cognitive functioning of this population, compared to a control group and general population norms. It is hypothesised that there will be high variability in psychological and cognitive functioning in patients who underwent transplant, and that there will be higher rates of psychological distress and lower cognitive ability compared to population norms
2. To examine the physical outcome of patients following HSCT. We will determine the full range of physical health needs of adults following HSCT by examining immunological outcome and late effects of transplant. It is hypothesised that physical outcome contributes to psychological functioning and social outcomes in patients following HSCT
3. To examine the social outcomes of patients following HSCT, including employment, relationships and socioeconomic status. It is hypothesised that a significant proportion of patients will have altered social outcomes compared with population norms and the control group
4. To examine the mediating and moderating factors of health outcomes (e.g., physical, mental and social) in patients with primary immunodeficiency following HSCT. This will aim to establish the factors that impact on poor and good outcomes in this population. It is hypothesised that:
4.1. There will be an association between cognitive function and social outcomes
4.2. There will be an association between psychological distress and social outcomes
4.3. That patients experiencing greater physical difficulties will report higher levels of psychological difficulties
4.4. That patients reporting physical and psychological complications following transplant will experience more limitations in their social circumstances
5. To measure health service utilisation and costs of adult survivors of HSCT compared with the control group, and factors associated with such usage. Costs borne by patients and families will also be measured

Ethics approval required
Old ethics approval format

Ethics approval(s)
Wales REC 6, 02/06/2016, ref: 16/WA/0147

Study design
Cross-sectional and cohort study

Primary study design
Observational

Study type(s)
Quality of life

Health condition(s) or problem(s) studied
Primary immunodeficiency

Interventions

The study will employ a cross-sectional and cohort design, examining the current functioning of adult patients who underwent HSCT during childhood and comparing it with healthy controls and population norms. Patients, aged over 16, who underwent HSCT for PID at Great Ormond Street Hospital, five or more years previously, will be invited to participate. Each patient will be asked to nominate a sibling or close friend who will also be invited to take part. Participants will be asked to complete questionnaires and practical tasks to assess their current functioning and circumstances. Information will also be gathered from medical notes.

Participants will complete a questionnaire book which includes the SF-36, GAD-7, Patient Health Questionnaire, Inventory of Health Status, FACIT-Fatigue, Insomnia Severity Index, Coping with Health Injury and Problems, Intolerance of Uncertainty Scale, Primary Antibody Deficiency Acceptance & Control Scale, Perceived Stress Scale, the Work and Social Adjustment Scale and the Social Network Index. A general self-developed questionnaire will further detail educational attainment, receipt of benefits, and further aspects of general health including smoking status, current medications, recreational drug use and alcohol intake (incorporating the AUDIT-C).

The practical tasks are the information, digit span, letter number sequencing, symbol search, coding and matrix reasoning subtests of the Weschler Adult Intelligence Scale (WAIS-IV), which measure cognitive functioning. These tasks are administered as a face-to-face assessment by the assistant psychologist.

Historical data will be collected from medical notes at Great Ormond Street Hospital. This will include information regarding pre-, peri- and post- transplant, and early social and psychological functioning. Data taken from medical notes at the Royal Free Hospital will include demographic data, and current physical health including laboratory data and clinical information regarding aspects such as the immunological outcome of HSCT, and long-term complications of the HSCT procedure.

As this is a cross-sectional design, data is collected using these methods just once (at baseline) for each patient.

Patients will also undertake the Client Service Receipt Inventory, a telephone survey developed specifically for this study, measuring health service use and costs borne to patients and their families. This data is collected over a year, via five telephone assessments at baseline and then three monthly intervals.

Intervention Type
Other

Primary outcome(s)
1. Physical functioning, measured using the SF-36 PCS at baseline
2. Psychological functioning, measured using the SF-36 MCS at baseline

Key secondary outcome(s))
1. Anxiety, measured using the GAD-7 at baseline
2. Depression, measured using the PHQ-9 at baseline
3. Physical health, measured using the Inventory of Health Status at baseline
4. Health service usage, NHS costs, and costs borne by patients and families, measured using a self-developed questionnaire at baseline

Completion date

04/09/2019

Eligibility

Key inclusion criteria
1. All patients aged 16 and over who have had an HSCT at Great Ormond Street Hospital five years or more previously
2. Patients with learning difficulties, who have low levels of literacy or limited verbal communications skills will be supported by family members or carers to complete as much of the assessment pack as possible. All potential participants are known to the clinical team and, if able to communicate verbally, speak good English.

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
16 years

Sex
All

Total final enrolment
135

Key exclusion criteria
Participants not fulfilling the inclusion criteria

Date of first enrolment
11/01/2017

Date of final enrolment
30/09/2018

Locations

Countries of recruitment
United Kingdom

England

Study participating centre

Royal Free London NHS Foundation Trust
Pond Street
London
United Kingdom
NW3 2QG

Study participating centre
Great Ormond Street Hospital for Children NHS Foundation Trust
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Sponsor information

Organisation
Royal Free Hospital

ROR
https://ror.org/01ge67z96

Funder(s)

Funder type
Government

Funder Name
National Institute for Health Research

Alternative Name(s)
National Institute for Health Research, NIHR Research, NIHRresearch, NIHR - National Institute for Health Research, NIHR (The National Institute for Health and Care Research), NIHR

Funding Body Type
Government organisation

Funding Body Subtype
National government

Location
United Kingdom

Results and Publications

Individual participant data (IPD) sharing plan

Participant level data is not being made available in compliance with ethical and data confidentiality guidelines. All data will be stored at the Royal Free Hospital, London in password-protected documents on secure computer systems, or in a locked filing cabinet within a locked office, as appropriate.

IPD sharing plan summary

Not expected to be made available

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		20/06/2022	18/11/2022	Yes	No
Results article		17/05/2022	18/11/2022	Yes	No
HRA research summary			28/06/2023	No	No
Participant information sheet	Participant information sheet version 2.0	11/11/2025	11/11/2025	No	Yes
Protocol file		23/04/2017	18/11/2022	No	No