

Patient and Public Involvement and Engagement (PPIE) elements in the 18-months BTRU funding extension from October 2020 – March 2022 – Addendum to the PPIE Strategy 2018-2020

This document is an addendum to the Patient and Public Involvement and Engagement (PPIE) strategy 2018-2020. It sets out the continuing aims and additional elements for PPIE in ongoing research at the UCL BTRU, in the 18-months funding extension from 1 October 2020 until 31 March 2022.

We have excelled in progressing the embedding of PPIE into the UCL BTRU during the first 5 years of funding (1 October 2015 - 30 September 2020). Patient and Public Research Panel members (formerly Lay Panel) shaped this progress, gave input on various research projects and were key to identifying the following PPIE elements in ongoing research during the funding extension:

1. Patient and Public Involvement (PPI) to ensure relevant and appropriate research.

This will include the following elements in different research projects:

- a. Patient representation in [ToTem Trial Management Group](#).** This includes the patient perspective in managing the clinical trial and shaping communication plans for engagement with patients and their carers during and after the study (Theme 1).
- b. Dialogue about making data available as a public resource.** The usage of data for research is a topic of much interest and raises some public concerns. We will discuss how the use of anonymised stem cell donor data from one BTRU study can contribute to other areas of research with Patient and Public Research Panel members, to aid better understanding of the concerns and benefits of using data. We will share reflections of this dialogue in a blog post targeted at stem cell donors and recipients (Theme 1).
- c. Build connections to people living with Sickle Cell Disease (SCD).** This starts a dialogue about pre-clinical work for gene therapy research through hosting an online conversation about different research leading to potentially new and better treatments for SCD. This will include working with the BTRU in Red Blood Cell Products at University of Bristol. We will use this opportunity to explain our early work, learn about questions and concerns from people affected by SCD in the UK and we hope to build early relationships for possible involvement as the research progresses (Theme 2).
- d. Measure the impact of the documentary [‘War in the Blood’](#).** This involves Patient and Public Research Panel members in survey creation, outreach, analysis and reporting to inform how to present the outcomes of CAR T-cell early clinical trials (Theme 3).
- e. Discussion about current use of patient samples in CAR-T cell studies.** This conversation looks at the current use of samples in CAR-T therapy research studies and aims to gain the perspectives of Patient and Public Research Panel members (Theme 3).
- f. Encourage earlier involvement in lab-based and early clinical research.** This has a focus on our unit and beyond through a PPI workshop for UCL BTRU researchers and panel members, talks for a broader research audience, PPI advice for BTRU affiliated research and learning opportunities for panel members (all Themes).

g. Increase the diversity of people involved in research. This involves close collaboration with panel members, other BTRUs and NHS Blood and Transplant (NHSBT) to increase involvement of people with different ethnic backgrounds in BTRU research (all Themes).

2. Capture patient experience to inform future standards

a. Evaluation of clinical trial patient experience. This requires patient representatives and Patient and Public Research Panel members to design a questionnaire for trial participants and their carers, analyse the data and report results to learn about improvements for future trials (Theme 1).

3. Public engagement to increase transparency of research and public support

a. Video about gene therapy behind the scenes. This involves steering from the Young Person's Advisory Group (YPAG) at Great Ormond Street Hospital, to increase transparency and public support for this cutting-edge gene therapy research leading to new paediatric treatments for inherited blood disorders (Theme 4). It builds on recent work [explaining gene therapy](#) involving YPAG.

a. Lay summaries of selected research findings. These will be written with Patient and Public Research Panel members for the UCL BTRU website and other communication channels for patients and the public.

b. Twitter account to engage with patients and members of the public online. This will continue to highlight BTRU affiliated research and Patient and Public Involvement in research. A Patient and Public Research Panel member and twitter expert developed the strategy ahead of time and runs the account together with the PPIE Lead.

c. Public engagement activities at two or more public engagement events. This will aim to increase public support for UCL BTRU research, in collaboration with other BTRUs and NHSBT. In addition, we will undertake at least one educational school visit about new gene and cell therapies.

4. Collaboration with key stakeholders in health research to use synergies

We established strong working relationships with stakeholders across UCL, other BTRUs, NIHR funded institutions and NHSBT. We will maintain these relationships and further explore areas for collaboration in addition to building links to relevant charities.

5. Put standards into practice and assess impact of PPIE

We are capturing feedback and making time to reflect on the process of involvement in research to improve future practice and processes. We are communicating changes made to research following the input of patient and public members with everyone involved and externally. We are communicating the impact of PPIE and shared learnings in internal and external meetings, within annual reports, blog posts and through selected case studies on the BTRU website.

Action Plan

PPIE element	Project Leads	Groups involved	Timeframe	Status
1. Patient and Public Involvement (PPI) to ensure relevant and appropriate research				
a. Patient representation in ToTem Trial Management Group	Principal investigator	ToTem study Patient Representative	Entire study duration (beyond BTRU funding)	Ongoing
b. Dialogue about making data available as a public resource	Theme Lead 1/ PPIE Lead	BTRU Patient and Public Research Panel (BTRU Panel)	December 2021 – March 2022	In preparation
c. Build connections to people living with Sickle Cell Disease	Theme 2 Lead/ BTRU Panel member and PPIE Lead	BTRU Panel	Oct 2020 – March 2022	In preparation
d. Measure the impact of the documentary 'War in the Blood'	PPIE Lead	BTRU Panel	Aug 2021 – March 2022	In preparation
e. Discussion about current use of patient samples in CAR-T cell studies	Theme 1 Lead/ PPIE Lead	BTRU Panel	Nov – 2021 – March 2022	In preparation
f. Encourage earlier involvement in lab-based and early clinical research ➤ PPI workshop in lab based and early clinical research	Theme Leads/ PPIE Lead Panel members/ PPIE Lead	BTRU Panel BTRU researchers/ BTRU Panel	Oct 2020 – March 2022 Feb/March 2021	Ongoing Delivered
g. Increase the diversity of people involved in research	Theme Leads/ PPIE Lead/ BTRU Panel	BTRU Panel	Oct 2020 – March 2022	In preparation
2. Capture patient experience to inform future standards				
a. Evaluation of clinical trial patient experience	Principal Investigator/ Patient Representative/ PPIE Lead	BTRU Panel	Oct 2020 – March 2022	On hold: delayed recruitment
3. Public engagement to increase transparency of research and public support				
a. Video about gene therapy behind the scenes	Theme 4 Lead/ PPIE Lead	YPAG	March 2021-2022	In preparation
b. Lay summaries of selected research findings	Theme Leads/ BTRU researcher/ PPIE Lead	BTRU Panel	Oct 2020 – March 2022	Ongoing
c. Twitter account	BTRU panel member/ PPIE Lead	Patient and public members	Oct 2020 – March 2022	Ongoing
d. Activities at two or more public engagement events	BTRU researcher/ PPIE Lead	Patient and public members	Oct 2020 – March 2022	In review due to pandemic

Acronyms and explanations

BTRU	Blood and Transplant Research Unit (BTRU), a partnership between NHSBT and universities funded by NIHR, supporting the needs of NHSBT for research to improve the supply of blood, blood products, stem cells and tissues and organs for transplantation, https://www.nhsbt.nhs.uk/research-and-development/current-research/btrus/
BTRU in Stem Cells and Immunotherapies at UCL	The BTRU in Stem Cells and Immunotherapies focuses on improving stem cell transplants (transfer of stem cells, which lead to new blood cells in the recipient) and the clinical use of novel therapies both to treat inherited genetic disorders and to repair or strengthen the immune system's ability to combat infection or disease. <ul style="list-style-type: none"> • Website: https://www.ucl.ac.uk/cancer/research/centres-and-networks/nihr-blood-and-transplant-research-unit-stem-cells-and-immunotherapies • Twitter: https://twitter.com/btruinstemcells?lang=en-gb
CAR-T cell therapy	Chimeric Antigen Receptor (CAR) T cells are a type of white blood cell that have been modified to find and kill specific types of cancers cells. Watch the TED-Ed animation 'How to biohack your cells to fight cancer', https://www.youtube.com/watch?v=Mt5C5fhuU_0
Gene Therapy	A potentially curative treatment adding or replacing a faulty gene with a functioning one in the DNA (our body's instruction manual) for severe inherited disorders. Watch our animation 'Gene Therapy explained': https://www.youtube.com/watch?v=i9sng5l-IVI
Immunotherapies	Immunotherapies are treatments that use part of the body's immune system to fight diseases such as cancer. This can be stimulating the body's natural defence system or modifying cells of the immune system to find and attack cancer cells (CAR-T cell therapy).
NHSBT	NHS Blood and Transplant (NHSBT) manages NHS blood donation services in England and transplantation services across the UK, https://www.nhsbt.nhs.uk/
NIHR	National Institute for Health Research (NIHR) funds, enables and delivers world-leading health and social care research, https://www.nihr.ac.uk/
PPIE	Patient and Public Involvement and Engagement (PPIE) in research <ul style="list-style-type: none"> • Involvement: Working with patient and public partners to ensure research is relevant, acceptable and meets the needs of those it is designed for. • Engagement: Two-way dialogue about research.
SCD	Sickle Cell Disease (SCD) is a group of inherited disorders that cause red blood cells to form a sickle cell shape leading to severe complications including increased risk of serious infection, painful episodes (sickle cell crisis), stroke and organ failure.
Stem Cells	Young cells that can develop into many different cells in the body. Blood stem cells are produced by bone marrow (spongy tissue in the centre of some bones), which turn into different types of blood cells.
ToTem trial	A first in human phase I clinical study transferring memory T cells (a type of white blood cells) after a donor stem cell transplantation to help with the recovery of the patient's immune system, https://gtr.ukri.org/projects?ref=MR%2FR025436%2F1
'War in the Blood' documentary	BBC Two documentary about CAR-T therapy that follows medical researchers, two patients and their families taking part in a first in-human CAR-T study. Watch on vimeo: https://vimeo.com/347541240

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