Health Technology Assessment

Alternate day caplacizumab for immune thrombotic thrombocytopenic purpura

Overview

Opportunity status:	Closing soon
Type:	Programme
Opening date:	5 August 2024
Closing date:	29 January 2025 at 1:00 pm
Reference ID:	2475

Ready to apply?

We recommend you get in touch as early as possible to benefit from our full range of support and expertise.

Apply now

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The Health Technology Assessment (HTA) Programme is accepting Stage 1 applications to their commissioned workstream for this primary research topic.

Timeline

29 January 2025, 1pm

Stage 1 deadline

March 2025

Applications considered by the HTA Funding Committee. Shortlisted Stage 1 applicants will be given 8 weeks to submit a Stage 2 application.

July 2025

Stage 2 applications considered by the HTA Funding Committee

How to apply

To apply for this funding opportunity you will need to log in through REALMS

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Commissioning brief

Introduction

The aim of the Health Technology Assessment (HTA) Programme is to ensure that high quality research information on the clinical effectiveness, cost-effectiveness and broader impact of healthcare treatments and tests are produced in the most efficient way for those who plan, provide or receive care from NHS and social care services. The commissioned workstream invites applications in response to calls for research on specific questions which have been identified and prioritised for their importance to the NHS, patients and social care.

Research question

What is the clinical and cost effectiveness of an alternate day caplacizumab treatment regimen in patients with immune thrombotic thrombocytopenic purpura (iTTP)?

1. Patient: Patients with new presentation of immune thrombotic thrombocytopenic purpura (iTTP) with confirmed normalisation of platelet counts (applicants to define and justify).
2. Intervention: Alternate day caplacizumab in addition to standard care.
3. Control: Continuation of daily caplacizumab in addition to standard care.
4. Important outcomes: TTP relapse and/or exacerbation; major and/or minor bleeding; maintenance of normal platelet count.

   Other outcomes: Adverse events; cost-effectiveness; quality of life.

   Existing Core Outcomes should be included amongst the list of outcomes unless a good rationale is provided to do otherwise. Applicants are encouraged to report recruitment and findings disaggregated by sex (and other demographic factors where relevant).
5. Setting: Secondary care.
6. Study design: A randomised controlled trial with an internal pilot phase to test key trial processes such as recruitment and adherence. Clear stop/go criteria should be provided to inform progression from pilot to full trial.
7. Minimum duration of follow-up: Applicants to define and justify.

Rationale

Thrombotic thrombocytopenic purpura (TTP) is a rare blood disorder that causes blood clots in small vessels around the body. Potentially leading to organ failure, it is a medical emergency with a mortality of 90% if left untreated.

More than 90% of cases are immune mediated, but usually with no identifiable cause. Patients are usually female (70%), and the median age of presentation is about 40 years old.

Plasma exchange and immunosuppression have been the mainstay of treatment for many years, and significantly reduce mortality. More recently, caplacizumab, an anti–von Willebrand factor humanized monoclonal antibody, has been added to treatment guidelines and further improved outcomes.

Following the acute treatment period, the current marketing authorisation for caplacizumab is for continued daily administration of ADAMTS13 (the metalloproteinase which is deficient in TTP) if levels remain below a recognised threshold.

Real world use of caplacizumab has confirmed its effectiveness as demonstrated in phase 2 and phase 3 trials, but there is now growing interest in whether the currently recommended treatment regimens could/should be revised for some or all patients.

Caplacizumab is an expensive drug and not without risk, as it can cause bleeding (although this is rarely severe). As such, there is a small but growing indication that alternate day (rather than daily ongoing) caplacizumab treatment might be sufficient to stop TTP relapse, while reducing the risk of bleeding and also saving the NHS money.

The NIHR HTA programme is therefore interested in applications for a randomised controlled trial which aims to investigate alternate vs standard everyday caplacizumab for TTP, following plasma exchange.

Additional background information

A background document is available that provides further information to support applicants for this call. It is intended to summarise what prompted the call and the existing evidence base, including relevant work from the HTA and wider NIHR research portfolio. It was researched and written on the basis of information from a search of relevant sources and databases, and in consultation with a number of experts in the field. If you would like a copy please email htaresearchers@nihr.ac.uk (mailto:htaresearchers@nihr.ac.uk).

Making an application

Your application must be submitted online no later than 1pm on 29 January 2025. Applications will be considered by the HTA Funding Committee at its meeting in March 2025.

Shortlisted Stage 1 applicants will be given 8 weeks to submit a Stage 2 application. The Stage 2 application will be considered at the Funding Committee in July 2025.

For commissioned topics, the Programme strongly discourages the practice of the same co-applicant joining more than one competing team, other than in unusual circumstances (for example, a lead from a named charity or a unique national expert in a condition).

For such exceptions, each application needs to state the case as to why the same person is included. The shared co-applicant should not divulge application details between teams, and both teams should acknowledge in their application that they are aware of the situation, and that study details have not been shared.

Should you have any queries please contact htagb@nihr.ac.uk (mailto:htagb@nihr.ac.uk)

Application support

In order to apply you will need to carefully review the:

- Stage 1 application supporting information (/documents/hta-programme-supporting-information-realms/27265)
- Stage 1 guidance notes (/documents/hta-programme-stage-1-guidance-notes-realms/27147)
- HTA tips for applicants (https://www.nihr.ac.uk/hta-tips-applicants)

Applications received by the advertised closing date will be considered at a first-stage funding committee meeting, and successful applicants will then be invited to submit a Stage 2 application. Applicants will have 8 weeks to complete and submit their Stage 2 application form, which will then be considered at the following HTA funding committee meeting. For more information, please read the commissioning brief.

All primary research projects are expected to establish a programme appointed Study Steering Committee and it is important that you read the Research Governance Guidance (/documents/research-governance-guidelines/12154) before completing your application. Costs incurred by this committee should be included in the budget as appropriate.

Word version of the Stage 1 application form

Download a template of the application form.

Please note that the Word version of the Stage 1 application form is to be used as a guide and to assist with completion of the online application form only, for example to see how many characters are accepted in each section and how the printed complete form is laid out. Please do not try to use this as an application form, you must apply using the online form available through the links available when calls are open. You should also refer to the application form guidance notes.

(/media/21716/download/)

Word-realms-version-stage-1-application-form.docx

DOCX

Last updated: 12 September 2024

Download document (124.92 KB)

Contact Details

- For help with your application contact htafunding@nihr.ac.uk (mailto:htafunding@nihr.ac.uk)
- For more information about the funding Programme, visit the HTA page (/node/62891)
- Got a research idea and not sure how to turn it into a funding application? The NIHR Research Support Service (RSS) supports researchers in England to apply for funding, and to develop and deliver clinical and applied health, social care and public health research post award. Find out how the RSS can help you (https://www.nihr.ac.uk/explore-nihr/support/research-support-service/) .