

## **Efficacy and Mechanism Evaluation Programme**

### **Commissioning Brief**

#### **Call for proposals for research into interventions in paediatrics**

Applications are sought for studies into interventions in paediatrics and neonatology<sup>1</sup>, which have the potential to contribute work of significant clinical advantage to the diagnosis, treatment, monitoring or clinical management of patients.

Applications may investigate novel or repurposed interventions and technologies. Studies of incremental or minor improvements to existing technologies or discovery of new biomarkers are not within the remit of the EME programme. Applications must concentrate on determining the efficacy of interventions, and may also include the evaluation of mechanisms.

In addition to applications within this broad area, the following areas are highlighted as being of particular interest:

- 1) interventions to improve neurodevelopmental outcomes in children (maternal interventions in the period immediately prior to birth are not within the scope of this call)
- 2) the emergency and critical care of babies and children

Applications to this call may also include initial stages such as:

- the limited steps needed to progress the development of an intervention to a stage suitable for use in an accredited clinical service
- prospective clinical work or retrospective research utilising existing clinical samples or data to inform the main study
- pilot or feasibility studies

As a rough guide it is expected that these early stages will be complete within the first 18 months of the project and contribute no more than 25% to the total cost of the project. The remaining costs should be for the main efficacy study which must be clearly specified within the application.

Applicants will need to make a strong case for the future importance of the intervention or technology through providing a measurable positive impact on health, innovation or future wealth creation and for the ultimate benefit of individual patients' or the wider NHS.

Projects must have a strong collaborative approach; the EME Programme is particularly keen to encourage collaboration with small and medium enterprises. Funding must be activated within 9 months of a decision being made.

#### **Programme remit**

Proposals must focus on a clinical study which is within the remit of the EME Programme ([www.eme.ac.uk/about/commissioned\\_remit.asp](http://www.eme.ac.uk/about/commissioned_remit.asp)). Applicants must make it explicit in their application how their proposed research meets the EME remit. The remit of the EME commissioned workstream includes clinical trials and evaluative studies in patients, which:

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<sup>1</sup> Call includes neonates, infants and children

- evaluate clinical efficacy of interventions;
- add significantly to our understanding of biological or behavioural mechanisms and processes;
- explore new scientific or clinical principles;
- include the development or testing of new methodologies.

The EME commissioned workstream supports:

- research which seeks to determine definitive proof of clinical efficacy and size of effect, safety and possibly effectiveness;
- studies that use validated surrogate markers as indicators of health outcome;
- laboratory based, or similar, studies that are embedded within the main study, if relevant to the remit of the EME programme.

## **Guidance on applications**

### **Proposed study design**

Applications to the EME commissioned work stream must be within the remit of the EME Programme and meet the specification detailed in the commissioning brief. In particular proposals must evaluate the clinical efficacy of an intervention and may also add significantly to our understanding of biological or behavioural mechanisms and processes. The EME Programme will not support confirmatory studies or trials of incremental modifications, 'confidence in effect' studies or refinements to existing medical interventions.

Applications are expected to set out programmes of work which contain distinct stages. It is expected that the early stages of the study will, if successful, lead onto a full evaluative clinical study or trial, which must also be included and clearly specified within the application. Any clinical trials embedded within a programme of work should be large enough to detect a meaningful effect.

### **Study start-up**

Applicants should ensure that they are in a position to start their proposed research rapidly and expenditure and funding must be activated within 9 months of a decision being made. Funding decisions will be communicated during November 2013.

### **Collaboration**

Applications to the EME commissioned work stream should be in the form of a significant collaboration. All applications should include significant contributions from at least two of the following partners; industry, academia, and the NHS. Evidence of this collaboration must be provided and a draft collaboration agreement will be required at the full proposal stage. The involvement of charities is also welcome.

### **Applicant eligibility**

The EME commissioned work stream welcomes applications from researchers in England, Scotland, Wales and Northern Ireland.

### **Team expertise**

Proposals should demonstrate a multi-disciplinary team with appropriate skills and experience, including an appropriately experienced statistician on the study team. The involvement of an accredited Clinical Trials Unit (CTU) is strongly encouraged in the design of clinical trials. Where appropriate, applicants are expected to work with suitably accredited clinical research facilities.

### **Monitoring of studies**

Projects funded through the EME commissioned work stream should be organised into distinct stages (usually up to three). At the end of each stage there should be clearly delineated go/no-go decision points with measurable criteria which will allow an assessment of whether the stage has completed successfully. The purpose of this delineation is to clearly identify critical points that determine whether the research should proceed to the next stage. It is anticipated that there will be a significant number of projects that will fail to meet criteria in the early stages. The EME Programme retains the right to reassess project progress in light of other new developments in the research area before subsequent stages of funding are released.

Within each stage it is expected that there will be a number of milestones which will allow the project team and EME Programme to track progress through routine project reporting.

### **Project management**

Applicants will need to demonstrate a clear management plan for all stages of the project as well as detailed plans for how they will actively manage individual stages.

### **Timescale**

There are no fixed limits on the duration of projects and proposals should be tailored to fully address the questions posed. However, it is anticipated that the early stages of a project will be completed within the first 18 months.

### **Funding**

Applicants should be aware that they are competing for limited funds and proposals should represent good value for money. All funding requested should be clearly justified. It is anticipated that the typical cost for an EME commissioned project will be in the range of £0.5-2million. However, there is no upper limit.

### **Public Involvement**

Patient and public involvement (PPI) in study design, implementation and dissemination of results is important to the EME Programme. Evidence of PPI will be sought within applications, and patient representation is expected on management and steering committees. Comments from public and patient reviewers will be obtained during peer review and at the EME Board. For further guidance please see the EME website ([www.eme.ac.uk/resources/involve.asp](http://www.eme.ac.uk/resources/involve.asp)).

### **Research Networks**

The EME Programme expects that applicants will work, where appropriate, with the relevant NIHR Clinical Research Network ([www.crncc.nihr.ac.uk/](http://www.crncc.nihr.ac.uk/)).

### **Governance and Regulation**

Applicants are asked to:

1. Follow the Medical Research Council's Good Clinical Practice guidelines ([www.mrc.ac.uk/Utilities/Documentrecord/index.htm?d=MRC002416](http://www.mrc.ac.uk/Utilities/Documentrecord/index.htm?d=MRC002416)) in planning how studies, particularly RCTs, will be supervised.
2. Note that trials involving medicinal products must comply with 'The Medicines for Human Use (Clinical Trials) Regulations 2004'. In the case of such trials, the NIHR expects the employing institution of the chief investigator to be nominated as the sponsor. Other institutions may wish to take on this responsibility or agree co-sponsorship with the employing institution. The NIHR is prepared to accept the nomination of multiple sponsors. Applicants who are asked to submit a full proposal will need to obtain confirmation of a sponsor(s) to complete their application. The NIHR reserves the right to withdraw from funding the project if they are not satisfied with the arrangements put in place to conduct the trial.

The MHRA ([info@mhra.gsi.gov.uk](mailto:info@mhra.gsi.gov.uk), [www.mhra.gov.uk](http://www.mhra.gov.uk)) can provide guidance as to whether your trial would be covered by the regulations. The Department of Health/MRC website ([www.ct-toolkit.ac.uk](http://www.ct-toolkit.ac.uk)) also contains the latest information about Clinical Trials regulations and a helpful FAQ page.

### **Making an application**

If you wish to submit an outline proposal in response to this commissioning brief please complete the web based application form ([www.eme.ac.uk/funding/Commissioning.asp](http://www.eme.ac.uk/funding/Commissioning.asp)) and submit it by 1pm on 18<sup>th</sup> February 2013 (further details in the guidance for applicants).

The EME Programme will undertake initial checks on all outline proposals submitted to ensure that they are within the Programme remit and meet the specification of the commissioning brief and are therefore eligible for consideration. Applications which do not fulfil this criterion will be rejected at this stage.

Applications which are within remit will be reviewed and prioritised by the EME Prioritisation Group prior to assessment by the Board.

Outline proposals will be assessed by the EME Board in June 2013. Shortlisted applicants will have approximately eight weeks to submit a full proposal.

*In line with the government's transparency agenda, any contract resulting from this tender may be published in its entirety to the general public. Further information on the transparency agenda is at: <http://transparency.number10.gov.uk/#>*