

Applied Clinical Research on Very Rare Diseases

The National Institute for Health Research (NIHR) invites research proposals into interventions for Very Rare Diseases. Six of the NIHR research programmes are participating.

For the purpose of this call, a very rare disease is one that affects less than 1 in 100,000 of the general population, particularly those whose specialist care is provided through centres funded by the National Specialised Commissioning Group (NSCG) and equivalent arrangements from April 2013. Applications exploring the organisation of super specialised services or their commissioning will be considered where these fit under the remit of participating programmes.

Individuals who suffer from a very rare disease are often at a disadvantage due to the small number of people affected by the disease and the complexity of symptoms. In February 2012, the Department of Health launched its consultation of the UK's first plan of rare diseases (UK Health Departments, 2012). It identified that sufferers of Rare Diseases face a number of obstacles to receiving appropriate care. These can result from the limited scientific and clinical knowledge of the disease, leading to delays in diagnosis as well as the limited number of therapies of proven effectiveness. The availability of high quality evidence based information and support at the time of diagnosis is important.

Applications that are multidisciplinary in nature are encouraged. Due to the rarity of the conditions, innovative study designs or approaches to recruitment are also encouraged. Applications for the UK arm of multinational trials either led from the UK or as a participating country will be considered. The inclusion of patient views and experiences are considered important by each participating programme.

The participating programmes are:

- Health Technology Assessment (HTA) Programme – Opens Oct 12 – Closes on 7 Jan 2013
- Efficacy and Mechanism Evaluation (EME) Programme – Opens 30 Oct 2012 – Closes 18 Feb 2013
- Research for Patient Benefit (RfPB) Programme – Opens 1 Nov 2012 – Closes 23 Jan 2013
- Health Services and Delivery Research (HS&DR) Programme – Opens Nov 2012 (date tbc)
- Invention for Innovation (i4i) Programme – Opens 21 Nov 2012 – Closes 16 January 2013
- Programme Grants for Applied Research (PGfAR) Programme – Opens 4 Dec 2012 – Closes 19 Mar 2013

Please note that this call is specifically concerned with attracting applications for research into very rare diseases as defined above. Applications for research on rare diseases according to the EU definition of 5 in 10,000 of the general population are welcome as part of the normal business of participating NIHR programmes.

Funding

The funding arrangements for this call are specified by each of the six participating NIHR programmes and details are available on the individual programme websites.

Opening and closing dates

Opening and closing dates vary depending on the participating programme. Applications must be submitted via the relevant programme website by the specified date.