NIHR | Research Design Service East Midlands

News from the Research Design Service East Midlands
Spring 2019

# Minimum Clinically Important Difference (MCID), also known as the effect size – when working out how many patients are needed

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**Suppose that you want to evaluate a healthcare intervention, and are designing a quantitative research study – such as a population survey or a follow-up cohort study or a clinical trial. You want to be as sure as reasonably possible that you are recruiting a large enough number of patients (or healthy people, or community centres, or whatever 'unit' you are studying) to answer your question well. The funding panel and their reviewers will also want to be sure!**

There are only a few exceptions to this, such as some feasibility studies [1] and purely exploratory studies where little or no previous work has been done – and then you would justify the size of your study more informally.

## The Research Question

Start by writing down your Main Research Question, for example: 'Following an acute cardiac event, does cardiac rehabilitation lead to an increase in physical activity, compared with usual care?'

This question will then be focussed so that it applies to the specific clinical situation to be studied. The PICO framework is a good way of doing this [Fig 1].

**P** - Population
**I** - Intervention
**C** - Comparator
**O** - Outcome

Figure 1

Next you need to decide the 'primary outcome measure' to assess patient improvement. In this example it would be around the physical activity – for example, the number of minutes of physical activity that the patient carries out, on average, each week.

You will also need to determine what method of measurement will you use to collect this data: a patient diary, a pedometer or maybe an accelerometer?

How long will the patient be followed up before the principle assessment is made? In this example it might be 8 weeks after the end of a 6-week period of rehabilitation therapy.

## Sample Size Calculations

The RDS EM Newsletter - Spring 2017 [2] explains sample size calculations in greater detail and where you can go for further help and information.

Now you are nearly ready to carry out the calculation – and it is best to consult with RDS staff or your own team's statistician to help you with this process.

One of the key ingredients will be: The **Minimum Clinically Important Difference (MCID)** also known as the 'effect size'. In this example - How many extra minutes per week of physical activity, averaged over patients, in the treatment group compared with the control group, counts as a success for the treatment?

## The following are both crucial criteria -

### 1. 'Can I believe that?'

The MCID *must be a small enough average improvement to be plausible*.

In our example if patients without cardiac rehabilitation generally averaged around 90 minutes per week physical activity and you claimed that rehabilitation would bring this up to 3 hours, you would need to provide evidence to justify such a dramatic improvement.

Sources of evidence:

- Previous published studies, using similar treatments with similar patients (needn't be exactly the same) – even better are systematic reviews, such as those in the Cochrane Library.

- RDS Information Officers can offer advice on which databases to search to find the evidence.
- A feasibility or pilot study is unlikely to be large enough to give useful evidence by itself, although it can help make the argument.
- Data from well-maintained clinical or research databases.

**and also –**

### 2. 'So What?'

The *MCID must also be a large enough average improvement* to persuade clinicians and – even more important - the commissioners of healthcare that it is worth making the change in standard clinical practice.

If you were a commissioner of NHS services, would you be interested in an extra 10 minutes average physical activity per week by a patient, or 20 minutes, or 30 minutes?

This is a question of judgement which may take into account:

- What this specific average improvement is likely to represent, in broader terms, for the patients' benefit.
- How common and how severe adverse events are.
- The cost in money terms, and also logistic factors involved in introducing the new intervention - these might include the provision of staff training, medical equipment, floor space in wards or hospital outpatient areas, and so on.
- RDS staff won't usually have the medical and NHS management expertise to answer these questions, but we can
  - Help you find good clinical and NHS opinions.
  - Discuss with you and review how well we think the argument has been made.

The Minimum Clinically Important Difference is a key aspect of any study that evaluates a healthcare intervention. It is not only a number needed (too often, at the last minute) to 'plug into' a sample size calculation. It encourages you, as a researcher, to think about and try to cautiously estimate the impact that the intervention, and therefore your research, can make.

For more detail, when designing a clinical trial, see the new DELTA[2] guidance in the BMJ[3].

### Further reading:


[1] 'Justifying sample size for a feasibility study' by Richard Hooper, RDS London and Queen Mary University of London https://www.rds-london.nihr.ac.uk/resources/#statistics

[2] Sample Size Calculation. News from the Research Design Service East Midlands, Spring 2017. https://www.rds-eastmidlands.nihr.ac.uk/news?tag=RDSEMNewsletter

[3] Cook JA, Julious SA, Sones W et al. DELTA[2] guidance on choosing the target difference and undertaking and reporting the sample size calculation for a randomised controlled trial. BMJ 2018; 363:k3750. doi: 101136.bmj.k3750


# "Make sure your research question makes sense to people who aren't interested in your area!"

***Dr Damian Roland** is a Consultant and Honorary Associate Professor in paediatric emergency medicine. He was awarded an NIHR fellowship in 2010 and successfully applied for RfPB programme funding in 2015. He has shared his advice on how an RfPB award as an early career researcher can be a stepping stone to a research career.*

### Why did you decide to apply for research funding?

My colleagues and I wanted to test the impact of a children's emergency department scoring tool I had created, in a different setting to the one in which we'd initially developed it, to find out whether it could be rolled out nationwide.

### Where did this research idea come from?

The tool is called the Paediatric Observation Priority Score (POPS), and I'd developed it with a colleague, Dr Ffion Davies, from the Leicester Royal Infirmary in 2010. It's a checklist that assigns a score to acutely ill children, based on components such as heart rate, breathing rate, temperature, and the clinical staff's gut feeling of how ill they are. This makes it easier for staff to assess, prioritise and treat children, helping spot the sickest children quickly and also decide which children can be safely sent home.

We had a few small publications that provided some preliminary evidence to support the tool's use, which we used to create an app http://bit.ly/2Kl4npR . The Pennine Acute Trust had also looked at our tool and developed a modified version (PAT-POPS), and they'd shown this had improved performance compared to their current in-house scoring system. It was clear that the next step should be a collaborative venture to continue to develop the tool.

### What made you apply to the RfPB programme?

It was a perfect fit for what we needed to do - take a developed idea and confirm it was fit for purpose in improving patient care.

### How did your NIHR fellowship help you to reach the position where you were applying for an RfPB grant?

The NIHR fellowship provided me with the core criteria to be able to apply for a grant, including some publications related to my thesis, credibility as a researcher given the competitive

nature of the NIHR Doctoral Research Fellowship and evidence I am able to complete complex tasks. Perhaps more importantly though, it developed my research leadership and organisational skills and the experience enabled me to develop a proposal that was both relevant and achievable.

## How did you develop your research idea into a full proposal?

The idea came initially from discussions between me and Prof Andrew Rowland from the Pennine Acute Trust, statisticians from Manchester University and academic nursing staff from Salford University - together we developed the proposal over a six month period.

We had all been working together on early PAT-POPS work, and I was the second most experienced researcher on the team. The most experienced person was nearing retirement and didn't want to lead, so I thought this was a good opportunity to develop chief investigator skills and build my research portfolio.

## What sort of support was available to you?

The most useful support came from PERUKI, the Paediatric Emergency Research Network for the UK and Ireland (which I now chair). Their members helped review the proposal and feedback comments which were very helpful to the success of the application.

We discussed the proposal with the Research Design Service (RDS) linked to the Pennine Acute Trust. They were very helpful in regard to patient and public involvement (PPI).

## How did patient and public involvement help shape your research?

The PPI group were extremely helpful. For example, they were clear that consent wouldn't be required in written format when children were being assessed – this enabled patient recruitment to happen at scale; hugely improving the capacity to reach the necessary sample size. The group were also clear and coherent in feedback about poster design and patient leaflets.

## How did you go about writing the application itself?

It was very iterative - we drafted, edited, drafted, edited. The key point was nailing down a core primary outcome measure. Once we had decided this, a lot of things fell into place. Peer review was also very useful and certainly helped improve the application.

## What was it like leading your first research project?

I was very excited but actually things were a little slow to being with. The research governance process can take a lot longer than you think - being able to employ a research manager for our project was especially helpful.

Having a team who are all willing to help with different parts of the project has been vital. Once the main study got up and running, having a clear agenda for our team meetings has been very useful to keep us on track.

## What are the main lessons you learned?

Prior planning is key. It is likely things will go wrong and you won't be able to collect information in the way you thought. Make sure you've thought about all eventualities, especially when they involve IT.

## What benefits do you hope this project will bring to patients and the NHS?

We don't know what the outputs are yet, but the study is gathering unique information on the case mix of children and young people presenting to small Emergency Departments and Urgent Care Centres. This type of work has never been undertaken before, so it will provide valuable information for other researchers.

## What are the next steps for your research?

This particular piece of work will lead on to two or three different further pieces of investigation. Importantly, we want to find out if there are significant differences in patients' acuity (their level of illness) and disposition (whether are they admitted or discharged) between urgent care centres, district general and tertiary emergency departments. This is something that has not been studied objectively before in children.

We will also be able to use this work to determine whether change in score is a more valuable aid than a single score itself.

Finally this work also links to another NIHR study I'm involved with, looking at the implementation of scoring systems.

## How do you think NIHR RfPB funding has helped your research career?

This RfPB study has been a clear stepping point in my transition from a post-doctoral fellow to an independent researcher. It has been helpful in giving me research skills, as well as the kudos of a competitive grant, and I hope this is the springboard towards ongoing research output.

## Any advice for other early career researchers who are considering applying for funding?

The research question you're asking needs to be clear. It's easy to get tied up in the details when you're thinking about the feedback you might get, but what's important is to have a research question that makes sense to those not interested in your research area, and one that is deliverable. Your core team are vital and you need to know you can work well with them as you will be communicating very frequently!

Damian's article was originally published as part of the NIHR blog series
https://www.nihr.ac.uk/news-and-events/features/nihr-blog/

### Damian will be presenting locally at the RfPB seminar on 30 May 2019, Derby

https://www.rds-eastmidlands.nihr.ac.uk/rdsem-rfpb-seminar

# Noticeboard

## BAME toolkit to increase participation in health and social care research

This toolkit aims to capture such best practice and provide researchers with a framework on how to improve the participation of BAME groups in research.

Use the toolkit to develop more relevant research questions, consider engagement of BAME groups in a more structured way, and provide tips on better participation and dissemination of research findings.

The toolkit covers:

- Section 1: Consideration of the communities which your research needs to involve.
- Section 2: Undertaking effective patient and public involvement (PPI) in research
- Section 3: Conducting effective recruitment in BAME communities
- Section 4: Ensuring cultural competency in the conduct of your research
- Section 5: Providing effective feedback to research participants
- Section 6: Recognising the importance of recruiting BAME communities in research: preparing a grant application
- Top Tips

http://bit.ly/2GbpHKs

## Podcast about social care research

Dr Mike Clark, Director of School for Social Care Research talks about social care research and what NIHR is doing to support and improve the evidence base in this crucial area of research.
https://soundcloud.com/nihr/dr-mike-clark-and-social-care-research

## New NIHR funding and awards database

The NIHR have launched a new funding and awards database covering all of their funding streams, listing current and past projects.

Information on each award covers plain English summary and abstract, as well as the funding amount, investigators, start and end date, funding stream, research type and contracting organisation.

https://fundingawards.nihr.ac.uk/search

## The Interactive Costing Template

With improved usability, functionality, reliability and compatibility - the new CPMS-based system will ultimately make it quicker and easier to set up commercial studies in the NHS through increased accuracy and visibility of commercial costing.

The new iCT will be a fundamental tool underpinning the transition to a new single review process for all commercial studies - minimising the time required to negotiate the cost for delivering research at an individual site.

iCT functionality improvements at a glance:

- **Compatibility**: The new web-based iCT will work on any computer - overcoming software compatibility issues with the existing Excel-based version of the tool
- **Usability**: With a simple layout, the new iCT will be a fully automated costing solution - accessible from the NIHR Central Portfolio Management System (CPMS) and no longer involving any manual steps from users
- **Reliability**: Updates or changes to the new system will be a seamless process, improving system reliability
- **Improved audit functionality**: The new app will contain a central log of all adjustments made as part of the contract negotiation process - enabling NIHR to further streamline processes by identifying any issues or delays
- **Visibility**: The new iCT will enable resource requirements determined at a site level will to be shared with others involved in the study within the system, avoiding duplication of effort

http://bit.ly/2GeMjK9

## Public Co-Applicants in Research – guidance on roles and responsibilities

developed jointly by the NHS R&D Forum, the Health Research Authority and INVOLVE.

**This guidance is intended to help:**

- Researchers wanting to include public co-applicants in a study
- Public contributors wanting to become a co-applicant
- Research staff who coordinate public involvement activities or advise on funding applications
- Those working in or with research organisations to review or process research applications

http://bit.ly/2EGUtJ9

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