Saving Faces - The Facial Surgery Research Foundation - funding

British Association of Oral and Maxillofacial Surgeons

The National Facial and Oral Research Centre NFORC

Saving Faces The Facial Surgery Research Foundation

NFORC – Step by Step Guide to Clinical Trial Proposal Development V5

NFORC can help you with all the listed activities. The suggested stages and the order of the activities in the flow chart are included as a guide and are in no way meant to be prescriptive.

Consider contacting your local Research Design Service at an early stage as they can help you to focus your research ideas and refine your research question as well as identifying suitable funders for your project

Stage 1

Define study Purpose

- Example 1 - To validate a new questionnaire for orthognathic surgery patients
- Example 2 - To identify biomarkers for radiotoxicity in mouth cancer patients

Undertake a literature review to determine:

- Is the research question original?
- What is already known?
- What do we need to know?

Think about PICOs

- Who are the relevant **P**atients?
- What is the **I**ntervention or **I**ndicator (e.g. diagnostic test) of interest?
- Is there an alternative management strategy (**C**omparator) or **C**ontrol?
- What is the expected patient-relevant **O**utcome of the intervention?

Stage 2

Patient and public involvement (PPI)

- Most funders require PPI
- PPI ensures that research is relevant to patient
- PPI ensures that outcomes are appropriate

Decide on a primary outcome measure (see notes below)

- Used to determine the overall result of the study
- Used to calculate the sample size

Stage 3

Survey your colleagues

- Are surgeons willing to participate?
- How many eligible patients are available?
- NFORC will help you to design and distribute your survey

Build an appropriate research team

- NFORC can provide statistical support
- You may need input from a health economist or other specialist

Undertake feasibility/pilot studies if needed to determine:

- Whether participants are willing to be randomised
- Whether centres will enter patients
- The characteristics of the proposed outcome measure
- Follow-up rates, response rates etc.

Notes on selecting a primary outcome measure

Although several outcomes may be measured it is usual practice to define one outcome as the "primary" outcome. A primary outcome is one which will be used to arrive at a decision on the overall result of the study. It is also the outcome used for the power calculation i.e. to work out how many patients should be studied in your trial. It should be the most important and relevant outcome from the patients' perspective. Primary outcome measures such as disease free survival in cancer or scores on a quality of life scale are usually measured at a specific time after the treatment or intervention. Avoid using any questionnaires or scales that have not been validated unless you can demonstrate that your measures are both accurate and consistent.

Primary outcome measures types

- A quantitative measurement representing a specific measure or count. They can be continuous (e.g. height or weight) or discrete (e.g. quality of life scale on a 10 point scale 1 to 10). These endpoints can be summarised as means or medians. When you have a normally distributed sample you can legitimately use both the mean or the median as your measure of central tendency. However, if the data are skewed the median may provide a better representation of your data.

- A binary clinical outcome indicating whether an event has occurred (e.g. death from any cause, the occurrence of disease signs or symptoms, the relief of symptoms). The proportions, odds ratios and risk ratios can be used to compare these endpoints.

- The time to occurrence of an event of interest or survival time (e.g. the time from randomisation of patient to death).

Data required for power calculations

A statistician will need relevant data from previous published research or from your own pilot studies in order to advise you on how many patients you need to study.

If your primary outcome measure is a normally distributed (think bell curve) **quantative variable** e.g. trismus in mm. or a patient reported outcome measure which produces a score of 0 – 100, you should provide the mean and standard deviation from previous research in similar populations. If you are unsure about the distribution of your variable, NFORC can advise you how to proceed.

If you are interested in the **relative frequency** you should provide examples from previous research that determine how often an event, for example PEG feeding, may occur in 100 patients following "Treatment 1" and how often following "Treatment 2."

If you have a categorical outcome measure e.g. the severity of xerostomia scored on a 4 point scale (none, mild, moderate and severe), you should give the expected proportions of the various categories for all arms of your study.

Size of effect

You will need to decide what size of change or difference in your primary outcome measure would be clinically important. For example, you might be looking for a reduction in the proportion of patients who suffer from severe dysphagia. If the literature suggests that 35% of patients typically suffer this complication, you might consider that reducing this proportion to 25% of patients would be clinically relevant. The statistician will need to know the expected proportions e.g. 35% and 25% not just the 10% difference.

Relationship between effect size and sample size

Detecting small differences requires more patients than detecting large differences. In the dysphagia example above the study would be powered to detect a 10% difference between treatment groups. If you wanted your study to detect smaller a difference of just 5%, you would need 4 times as many patients!

Ethical Approval

In general, it is not necessary or advisable to apply for ethical approval for your study until funding has been secured.

NFORC contact details

For further information or assistance from NFORC please contact: info@nforc.co.uk

How to apply for NFORC support for your proposed study

Submit a Summary Research Proposal for peer review

The summary (approximately 200 words) should highlight the nature of the problem, the need for the research, the hypothesis to be tested, the methods to be used, and the significance and unique features of the research.

You will receive feedback on your summary from NFORC and your Speciality Lead and may be invited to present your proposal at one of the twice-yearly NFORC Research Summits for peer review.
Summaries should be sent to the info@nforc.co.uk