



BPSU Study guidance - Study follow-up assessment measures

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BPSU parent bodies:



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Introduction

Choosing the right outcome measures for a BPSU study, where follow-up is involved, is of critical importance to achieve maximum value from the research. Outcomes can be defined in many different ways, and the intention of this paper is to guide applicants through the semantics and practicalities of outcomes in order to improve their applications to the BPSU.

Why is this guidance needed?

1. Currently all BPSU studies use different outcomes frameworks simply because there is no standard agreed approach; yet many studies, especially newborn follow-up studies, are attempting to measure similar outcomes.
2. There is often confusion between the concepts and measurement of needs, outcomes, function and burden of disease.
3. There is value in being able to compare outcomes of different conditions between studies.
4. It is particularly important to be able to repeat studies of the same condition with the same outcomes over time.
5. On occasions it is valuable to be able to compare (similar) outcomes across different health systems/nations.
6. There are often confounding factors that influence impact outside the NHS that may interfere with outcomes, that should be collected when deciding on outcomes.

Common BPSU study requirements

1. disease/condition/intervention follow-up outcomes.
2. "burden of disease" for the individual, family, community, services.
3. sometimes costs of NHS support.

This paper primarily considers disease/condition/intervention outcomes simply because this data is relatively easily available from clinical notes. However, on occasions it is helpful to consider the burden of disease and sometimes costs, and while these often require a more in-depth study sometimes there are proxy measures available in medical records which provide some insight into these aspects. Examples might include access to specialist services or interventions, or support from other agencies.

BPSU study criteria

1. Data must be available within the health records.
2. Data must be consistently recorded
3. Data must be easily accessible

These are the practical issues that must be considered by applicants. If the data does not meet all three criteria, it is unlikely that the study would be approved by the BPSU Scientific Committee. Sometimes a pilot study is required to check whether the requested data does meet all three criteria.

Common outcome assessments required

1. One/two year neonatal follow-up
2. Any age follow-up
3. Burden on the NHS

Many BPSU studies are initiated in the newborn period or alternatively follow-up some event that occurs in the first year of life and therefore robust one/two year follow-up assessments are required. Events that can happen throughout childhood, require assessments that are equally appropriate, for example, both for follow-up of a three-year-old or a 13-year-old after a life-threatening episode. Only certain aspects of NHS burden are easily captured, often relating to days in hospital, interventions used or referral to specialist services.

Approaches to assessment

1. ***Physiological-measurement***, for example, lung function, renal function, hearing, vision etc.
2. ***Cognitive assessment***-what someone is able or not able to do using standardised tests. (Griffiths, Bailey)
3. ***Observational assessment***-usually based on observations in a clinic setting, usually structured but descriptive rather than quantitative.
4. **Functional assessment**. Based on observations of everyday living. Often based on additional support an individual requires to achieve maximum expected function for age. Sometimes descriptive but often using some form of graded proforma (Practical, good for service planning - see Appendix: the WeeFIM* approach)
5. ***Needs driven assessment***. Need is defined as the ability to benefit from an intervention. The intervention should be evidence-based and can be individual, family, community or service related. Often used by Commissioners for service

planning on a population basis, but can be used for individual care planning.

6. ***Burden of disease/condition.*** Can include the burden for families, communities, specific services or a combination. NHS burden is generally reduced to an estimation of the combination of ongoing services required at a certain point of that child's life.
7. ***Mixed methodologies*** tailored to particular conditions/circumstances.
8. ***Health economic outcomes.*** Usually not possible within BPSU studies, but may be possible to estimate some costs using proxy measures such as a well-defined intervention.
9. ***Quality of life.*** There are many questionnaires available that estimate quality of life both for individuals and families. However, few are in routine use within the NHS, but there may be occasions where they are available within certain subgroups, for example they are quite frequently used within mental health services.

Pros and cons

Physiological assessment

- usually quantitative.
- well defined normal range.
- abnormalities more difficult to categorise "mild, moderate, severe".
- physiological severity may not correlate to functional impairment.

Cognitive assessment

- usually quantitative.
- well developed widely used test with good psychometric properties.
- time consuming therefore only used for specific groups where there are concerns.
- dependent on cooperation of the child more accurate with older age groups.

Observational assessment

- very dependent on cooperation with the child/young person.
- often dependent on competence of observer/assessor.
- often time-consuming/expensive.
- often subjective.
- this type of data may well become more readily available in the future with the advent of patient completed electronic records.

Functional assessment

- generally based on questions of parents/carers.

- very practical life observations.
- subject to parental expectations.
- can be independently verified by other observers.
- relatively rapid
- rarely applied in a standardised way to all patients unless using an established format e.g. WeeFIM.

Needs assessment

- generally a multiagency assessment used for service planning.
- comprehensive, but time-consuming.
- uses mixed methodologies.
- requires an understanding of evidence-based interventions.

Burden of disease/condition

- other services involved generally available in health records.
- details/frequency of interventions often not included
- often difficult to cost.

Mixed methodologies

- focused on specific outcomes relevant to disease/condition
- not easily comparable between different studies

Health economic outcomes.

- Costs are generally not included within medical records.
- Access to specific interventions are often included and where these are standardised, for example the cost of a surgical procedure or admission to intensive care, this may be used as a proxy.

Quality of life

- Many quality of life assessment questionnaires are available, well researched with good psychometric properties.
- Rarely easily available within clinical records but being introduced e.g. PedsQL.

Guidance for BPSU applicants

1. There is no single "off-the-shelf" set of outcome measures that can be recommended for all BPSU studies. However attention to detail when considering outcomes is an important attribute of a good BPSU study proposal.
2. Review BPSU studies similar to your proposal that have been successful in the past, this will give you some ideas about outcomes that are practical.
3. In most BPSU studies there are a number of outcomes usually focusing on growth, physiological or functional assessment, as this data is generally recorded well in medical records.

4. Make use of standardised assessments where these are consistently available, for example neonatal follow-up.
5. With the increasing availability of linked electronic systems consider linking with Healthy Child Programme assessments (but note they may vary slightly from nation to nation).
6. Generally, functional assessments have been underused within BPSU studies, and careful thought has to be given as to what data is available and how consistently this is recorded to be retrieved retrospectively. However, this information may be available from other professional groups for example speech-language therapists, occupational therapists and physiotherapists.
7. It is essential to check that the information you are requesting is relevant and routinely available and easily accessible within medical records to reduce the burden of questionnaire completion by paediatricians.
8. Consider other services involved, contact times with services, particularly length of stay may provide some estimate of burden of disease on the health service.
9. Neonatal follow-up protocols based on standardised assessment can provide a wealth of information often not available for other conditions at other times of life.
10. When submitting a BPSU proposal it is essential to clearly define the proposed outcome measures.
11. It is important to consider confounding factors that may influence the outcome particularly when estimating the value of a health service-based intervention.
12. It is vital to complete the data analysis section of the BPSU application ensuring that all the data requested is actually used in the analysis.
13. Ensure there are sufficient numbers, particularly in subgroups, to ensure robust statistical analysis, where this is an integral part of the proposal.

Appendix: The WeeFIM approach

WeeFIM assesses individuals on a seven-point scale ranging from complete independence (for age) through to complete dependency. Not all levels are required for every function.

1- Totally normal (as expected for age)

2- Environmental change or technology only required to achieve normal function

3- Some adult supervision required to achieve normal function

4- Minimal help required (<25% of the time)

5- Moderate help required (<50% of the time)

6- Continuous observations/intermittent interventions required (<75% of the time)

7- Continuous interventions required

The areas of function are chosen depending on the underlying condition and could include:

Physiological/body functions

Breathing

Sleeping

Cardiovascular

Maintaining body temperature

Level of consciousness

Special senses

Vision

Hearing

Functions

Mobility

Feeding

Dressing

Language

Social interaction

Problem solving

Personal care

Toileting

Bladder management

Bowel management

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