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Patient/ Carer information sheet

Introduction

Orphan drugs is the term applied to drugs that treat rare conditions. Rare diseases effect approximately 1 in 2000 people. These drugs may be expensive to develop compared to the small numbers of patients who receive them. As such, these therapies tend to be high-cost and rarely meet existing standards for showing that they are good value for money. Unless we consider orphan drugs as being special in some way, the NHS might find it difficult to make funding available for their use. In the UK, there are a number of policies in place to do just that. These are in addition to policies to help drug companies to produce orphan drugs.

However, it doesn't follow that all orphan drugs are funded by the NHS and there remains a debate on whether or not orphan drugs be assessed according to conventional standards for showing value for money, or whether alternative ways are more appropriate. To inform this debate, we are aiming to understand the preferences of the UK population for funding orphan drugs.

What is the aim of the study?

The aim of this study is to determine the most important factors that relate to funding drug treatments for rare diseases. The study includes 15 Factors that have been identified as important for orphan drug funding policy. This study aims to narrow this list down to 5.

Another aim of the study is to assess attitudes towards funding options for high cost gene therapy treatments. Here you will be given a scenario and asked whether or not the NHS should fund the treatment.

This study will also contribute the completion of a PhD qualification for Ms. Siobhan Bourke.

Why have I been chosen?

You are a patient, or the carer of a patient who has been diagnosed with a rare disease.

Do I have to take part?

No. It is up to you to decide whether or not to take part. If you do, you will be asked to agree to a consent form. You are free to withdraw from the study at any time by clicking the (x) in the top right hand corner and without giving a reason.

What will the study involve?

Participation in this study will involve you being asked to complete a questionnaire on the factors that have been identified as important in the debate for funding orphan drugs. Details on your background and your experience relating to orphan drugs will be asked.

What do I have to do?

The questionnaire should only take approximately 20 minutes to complete, however there is no time limit to complete the survey. The survey will be available for 3 months or until the quota of responses has been filled then it will be taken down.

What will be the benefits of taking part? Are there any disadvantages?

Whilst there are no direct benefits of you taking part in this research. It is important to understand the views of patients and their carers on how this funding should be prioritised amongst rare diseases we believe participants will see the value of assisting with progression in this field. By taking part in the study will improve our understanding of funding for these rare conditions which will help policymakers to deal with the issues surrounding funding priorities.

Contact Details:

For further information speak to: Siobhan Bourke (Bangor University) 01248 388894 or email: mhp401@bangor.ac.uk

Confidentiality

Measures to ensure confidentiality of personal data include:

- Reporting of data will be only on a group level
- Any direct quotes will be attributed to the group and not the individual patient
- Storage of personal data will be kept in a secured I.T file storage in Bangor University.
- Persons only directly involved with the study will have access to this data.
- All documents will be password protected.
- Responses to the questionnaire will be permanently deleted in accordance with the latest version of Bangor University's policy on data destruction and code of conduct for academic integrity and quality.

What will happen to the research results?

The issues raised in the survey will be published as part of a wider academic report, although nothing will be published which can be used to identify individual participants.

Complaints

If you have a concern or complaints about any aspect of this study, then you can contact Prof. Dyfrig Hughes in the Centre of Health Economics and Medicines Research (CHEME).

Who is organising and funding the research? The Centre for Health Economics and Medicines Evaluations based in Bangor University are funding, organising and conducting the study.