**Case study**

**Background**

Asthma is a very common illness in children and young people. On average, it affects two children in every classroom in the UK.

Asthma is usually controlled by a ‘preventer’ inhaler, usually brown in colour. Children with asthma also have a ‘reliever’ inhaler, usually blue in colour. The blue inhaler is taken on demand to relieve symptoms of breathlessness, while the brown inhaler is taken regularly to prevent symptoms occurring, or reduce their intensity. Where a child’s asthma is inadequately controlled with these two forms of inhaler, a third ‘line of defence’ is needed. Thus the three ‘lines of defence’ are:

**Stage 1 Defence**: the use of a blue inhaler only, when necessary to relieve symptoms (e.g. for mild asthma)

**Stage 2 Defence**: the use of a brown inhaler on a regular basis to control asthma, plus a blue inhaler on demand in response to symptoms

**Stage 3 Defence**: an additional control (in conjunction with the use of the brown and blue inhalers), which includes two options:

* Drug A or
* Drug B

The protocol seeks to compare the efficacy of Drug A and Drug B, for a particular subgroup of children: children with asthma in a particular Genetic Group X

**Aim**

To test the efficacy of Drug A and Drug B in two groups of 30 children aged between 7 and 18 years of age.

**Hypothesis**

Overall, based on previous randomised controlled trials, children at Stage 3 Defence seem to do better with Drug A rather than Drug B. However, the evidence suggests that these results from randomised controlled trials are not reliably replicated in real-life situations.

We have also observed that children who are in a particular genetic group – Genetic Group X – appear to be at *increased* risk of asthma attacks if they take Drug A with their brown inhalers.

We therefore wish to test the theory that, for children in Genetic Group X, Drug B is more effective and safer than Drug A as a Stage 3 Defence. No other studies have yet been published that either compare Drug A or Drug B for children in Genetic Group X.

The main focus of the proposed research concerns which of these two ‘third line defence’ options work better. It also aims to collect data on both clinical measures and qualify of life measures (which can be easily reported online), in order to help improve the outcome measures used for children’s asthma research in the future.

**Method**

***Research subjects***

We would like to take 100 children with persistent asthma, who require Stage 3 Defence and randomly assign them to two groups. Group A would be tested to find out whether they are in Genetic Group X. If they are, they will receive Drug B, and if they are not they will receive Drug A. The children in Group B will not be tested to see if they are in Genetic Group X, and will receive Drug A, based on existing research.

The research subjects will be recruited from asthma clinics in hospitals in the Brighton area. Asthma doctors will be invited to identify children who meet the eligibility criteria (ie need a stage 3 line of defence as their asthma is not adequately controlled by blue & brown inhalers) and invite them to participate in the study.

***Additional requirements for participation***

Participants will need to discontinue their current Defence Stage 3 medication for a period of two weeks before the research begins. The purpose of this requirement is that the research subject must ‘wash out’ the effects of that medication so that an accurate test can be performed. For this period, participants must only take their brown or blue inhalers to alleviate their symptoms. Ideally, this wash out period would be four weeks, but this was felt to be likely to be unacceptable to the children. Given the length of the follow up (a full year), two weeks is proposed as an acceptable minimum wash out period.

***Outcome measures***

There are two outcome measures which we propose to use.

*Measure I*

The first outcome measure we propose to use in comparing Drug A with Drug B will focus on each child’s attendance at school.

Each participant will begin to take Drug A or Drug B at the start of the school year (September 2014). For a period of one school year (ending July 2015), we will record and compare the number of absence days recorded by each child’s school register. We will seek permission from the child’s parents and their schools to obtain these data.

*Measure II*

The second outcome measure will focus on whether each Group are able to use their blue inhaler less while taking Drug A or Drug B. To measure this, we propose to use an online questionnaire, which allows the data to be collected without the need for children to visit clinics, and potentially miss a day of school. These questionnaires will be completed at the start of the research (when the research subject stops taking their current Stage 3 Defence drug), two weeks later (i.e. at the point of randomisation), and then at three-monthly intervals for the remainder of the school year.

*Collecting additional data on effectiveness of outcome measures*

As part of this research project, we would also like to improve our understanding of the outcome measures used in children’s asthma research. Participants will therefore be invited to contribute additional information as part of the study, in order to improve the accuracy of outcome measures in the future. Thus, in addition to the online data collection described above, participants will also be asked to visit their hospital four times during the year’s research study, to undertake a number of tests (including lung function tests, and exercise tests). This additional data collection will not directly benefit the children participating in the research, but will contribute to a very valuable evidence base of the relationship between clinical data of this kind, and the quality of life data collected online, thus improving research methods in the longer term.

**Recognition**

We would like to give each child who takes part in the research a £20 gift voucher for Amazon. This will be a surprise for the children, as they will not be told about this until after the research is completed.