

## What's it all about

### What does the clinical trial aim to do?

- To improve treatment for children and young people with severe asthma. These children need to take a 'Stage 3 Defence' drug to control their asthma, in addition to their regular inhalers.
- To find out if testing children and young people for a particular gene (the MAS gene) helps doctors give them the Stage 3 Defence medicine that will be best for them.

### How is this research different from what happens at the moment?

There are several Stage 3 Defence medicines. At the moment, a child or young person who has severe asthma will routinely be given a drug called Exhalin as their Stage 3 Defence. This is the medicine that seems to work best for most children.

However, doctors find that *some* children and young people don't do well when they take Exhalin. Instead, their asthma improves when they take a different drug called Verabreath. At the moment, though, there isn't a test to help doctors work out which children and young people will do better on Exhalin, and which will do better on Verabreath. This means that some people have to spend a long time trying different drugs before they find the one that is best for them; this is a 'trial and error' process.

One possibility is that children and young people who have a particular gene (the MAS gene) are the ones who do better on Verabreath. This research would like to see if testing research subjects for the MAS gene, and giving them Verabreath straightaway, means that their asthma improves.

### What will happen *before* the clinical trial starts?

- The research team will invite 200 children and young people with severe asthma to take part in the study. They can be aged between 7 and 18.
- Contact will be made through the child or young person's doctor.
- Children and young people will only be able to take part if their parent or guardian agrees.

## What will happen *during* the clinical trial?

### Preparations

- The researchers will ask the children and young people to stop taking their current Stage 3 Defence treatment (e.g. Exhalin or Verabreath) two weeks before the study starts. This is to make sure that it is ‘washed out’ of their bodies. They will still be able to use their inhalers (both blue and brown inhalers).

### Taking Exhalin or Verabreath

- After this ‘wash out’, the researchers will divide the children and young people into two groups of 100 (Group 1 and Group 2). The children and young people will not know what group they are in.
- **Those in Group 1** will be tested for the MAS gene using a saliva (spit) sample. (These samples will be kept and stored by the researchers to answer future research questions.)
  - If they have the gene = they take Verabreath
  - If they do not have the gene = they take Exhalin
- **Those in Group 2** will *not* be tested for the MAS gene, and they will all take Exhalin (= what usually happens when a child is first diagnosed with severe asthma).
- Each child and young person taking part in the research will be asked to take their treatment for one school year (from September 2015 – July 2016). However, if the drug they are taking does not seem to work for the individual child or young person, their doctor will be free to change it, just as they would do if the child or young person were not taking part in the trial. This applies to children/young people in both groups.

### Finding out the effects of the medicine on people’s asthma

The researchers will use a number of methods to measure how well the Stage 3 Defence drugs are working for all the children/young people in the study:

- **Effect on school life:** researchers will ask schools or colleges how many days of school have been missed because of asthma
- **Effect on ordinary life:** participants will be asked to fill in a confidential online questionnaire six times during the research, to say how their asthma is affecting their daily life. This information will be kept on a computer with a secure password.

- **Medical tests:** participants will also be asked to spend half a day in hospital four times during the research, to have medical tests of their asthma – for example measuring how deeply they can breathe, and doing exercise tests.

Participants can stop taking part in the research study at any time.

### What will happen *after* the clinical trial finishes?

#### Thank you gifts and travel costs

- The children and young people will all receive a £20 Amazon voucher for taking part.
- Any money spent travelling to the hospital for the study will be given back to the children and young people's parents.

#### How the results will be used

- The results from the clinical trial will be written into a report. The report may be published in an academic journal so that other asthma doctors know about the results.

### Who are the researchers?

The research team is made up of three people, who are all doctors. It is led by a professor who is also the doctor of some of the children and young people who will be invited to take part in the research study.