



## Screening Information Sheet

## **SANTA Study**

### **(Simvastatin in Neurofibromatosis Type 1-Autism Study)**

We would like to invite you to take part in our research study. Before you decide we would like you to understand why the research is being done and what it would involve for you. You can ring us if there is anything that is not clear or if you would like more information.

#### ***What is the purpose of the study?***

Our recent studies suggest that 25% of children with NF1 have Autism Spectrum Disorder (ASD). ASD is a condition which affects 1 in 100 people. It affects communication and how someone relates to other people. It also affects how people make sense of the world around them. It is a 'spectrum' condition which means that difficulties vary from child to child from mild to severe.

This discovery suggests new ways of understanding families' problems and approaching treatment. In other research, a commonly used group of medicines called statins (such as Simvastatin — which are used safely in other childhood conditions) have been shown to improve learning problems in mice and potentially improve brain functioning in older children and adolescents.



### **Contact details**

**Ruth Ellicott**

**Senior Paediatric Clinical Research Nurse**

**The National Institute for Health Research/Wellcome Trust  
Clinical Research Facility**

**At Central Manchester University Hospitals NHS**

**Royal Manchester Children's Hospital**

**Hathersage Road**

**Manchester**

**M13 9WL**

**Phone:** 0800 634 0102 (answerphone – please leave a message)

**Email:** [hannah.tobin@manchester.ac.uk](mailto:hannah.tobin@manchester.ac.uk)

**Thank you for reading this and best wishes to you and your family!**

### ***What happens to the result of the screening questionnaire?***

We will only contact you if your child meets the screening questionnaire criteria for the study.

### ***What if there is a problem?***

If you are worried or unhappy about something in the study, you can contact Ruth Ellicott on 0800 634 0102 and we will do our best to answer any questions or concerns. If we are unable to resolve your concern or you wish to make a complaint regarding the study, please contact Patient Advice and Liaison Service (PALS) on 0161 276 8686 or by email to [pals@cmft.nhs.uk](mailto:pals@cmft.nhs.uk).

### ***Who is organising and funding the research?***

The research is organised through the department of Genetic Medicine at the Central Manchester University Hospitals NHS Foundation Trust. The funding comes from the Central Manchester Foundation Trust.

### ***Has anyone checked the study?***

An independent group of people, called a Research Ethics Committee, looks at all research in the NHS to protect your safety, rights, wellbeing and dignity. This study has been checked and approved by them.

Simvastatin is approved in Europe for treating children over the age of 10 years who have high cholesterol but it is not yet approved for use in younger children and in children with NF1 and autism.

We want to know if Simvastatin is well tolerated by children aged 5-8 years with NF1 and autism and if Simvastatin improves the communication and social problems in these children. In order to test this, we will compare Simvastatin with a dummy medication (placebo). We will divide the children — one group receiving Simvastatin and the other receiving the dummy medication. The results will be compared to see if one is better.

### ***Why are you asking us to take part?***

You have been invited to take part in this study because your child has NF1 and is aged between 5 and 8 years.



### ***Do we have to take part?***

No, it is up to you to decide to join the study. Please ring us if you have any questions. You are free to withdraw from the study at any time, without giving a reason. This would not affect the standard of care you receive.

### ***What will we have to do?***

Before you and your child are enrolled in this study we will screen your child for autism. If you are interested in taking part, please fill in the screening questionnaire and indication of interest form and send it back to us in the pre-paid envelope provided. We will contact you if your child meets the screening criteria for participating in this study.



### ***What happens if my child meets the screening criteria?***

If your child meets the screening criteria we will invite you and your child to Manchester Children's Hospital for a detailed assessment of Autism Spectrum Disorder. If this detailed assessment suggests your child has Autism Spectrum Disorder, we will ask whether you would wish to participate in the study.

Participating in the study would involve:

- Three visits to Manchester children's hospital (your travel expenses will be covered).
- We would give your child Simvastatin medication or dummy medication to take for 12 weeks.
- Your child would have some blood tests and a brain scan to monitor the effects of the medication.

### ***What are the possible risks or discomfort?***

There are no risks associated with filling in the screening questionnaire. The risks associated with participating in the trial may include the burden of travelling to Manchester for the study visits; your child may experience some distress during the blood tests or the brain scan. Also the medication Simvastatin used in the study may have some side-effects.



### ***Are there any benefits?***

A study like this tells us more about children with NF1 and their difficulties in communication and making friendships. The study may help to indicate whether your child may have an underlying Autistic Spectrum Disorder. Understanding these difficulties better may allow you to access educational and other services suitable to meet your child's needs. We will also do some genetic testing as part of this study and share the result with you if you wish.



### ***Will others know we are taking part?***

No. Everything that you and your child will tell us will be kept confidential, which means that only the people in our research team will know.