Parent Information Sheet

A Longitudinal Laboratory and Real-World Study of Gait and Balance in People with Friedreich's Ataxia

We would like to invite your child to take part in our research study.

In this study we want to investigate different ways of measuring ataxia symptoms, especially balance control and walking (gait), in people with Friedreich's Ataxia. We also want to look at how these symptoms change over one year.

- Before you decide whether or not you would like your child to take part to take part, it is important that you understand why we are doing this research and what it will involve for you and your child.
- Please take time to read this information carefully. Feel free to discuss it with your family, friends or your child's GP.
- You are free to decide whether or not you would like your child to take part in this study. If you decide you would not like them to take part, it will not affect the treatment your child receives from their doctors.
- Please ask if you have any questions or if anything is unclear.

This information sheet contains several sections. In the first few sections, we explain what the study is about, why we are doing it and why your child is invited to take part.

We will then describe what will happen if you decide you would like your child to participate.

In the final few sections of the information sheet, we explain what we will do with the data collected from this study and how the study is managed and overseen.

What is this study about?

Ataxia is a term used to describe several symptoms related to impairment of coordination, movement, walking, balance and speech.

Friedreich's ataxia is one of the most common recessively inherited disorders affecting movement and balance. It is thought to affect one in every 50,000 people.

People with Friedreich's ataxia experience balance and co-ordination difficulties. This causes clumsiness, unsteadiness and frequent falls. It can also cause speech and swallowing difficulties, weakness in the legs and sensory issues (e.g., numbness, tingling).

It is important to have effective ways of measuring symptoms of conditions like Friedreich's ataxia. The methods we currently use have limitations, for example they are not that good at detecting changes over a short period of time.

In this study we want to look at new ways that are sensitive enough to detect ataxia symptoms (especially balance and gait) and their changes over one year. Being able to measure disease progression over a short period may be useful for designing future clinical trials of new treatments and will potentially reduce the number of participants needed for these trials.

We are planning to recruit 16 individuals (aged between 14 and 65 years) with a confirmed genetic diagnosis of Friedreich's ataxia into this study.

The assessment of ataxia will be performed using three different approaches:

- Assessment of signs and symptoms including physical examination and questionnaires.
- Analysis of posture and walking (in a special room called a gait laboratory).
- Assessment of movement and everyday activities via wearable activity monitor.

We will assess each person who takes part at three study visits over one year. These visits will be at the start of the study (Baseline), Month Six and Month 12 (End of Study).

Why is my child being asked to take part?

Your child is being invited to take part in this study because they have a diagnosis of Friedreich's ataxia and are able to stand and walk without any aid for at least 20 metres.

Do we have to take part?

It is completely up to you to decide whether you want your child to join the study. If you agree for your child to take part, we will ask you to complete a consent form. We will also ask your child to complete an assent form to confirm their agreement to take part.

Throughout the study period, you are free to change your mind about your child's participation at any time.

What happens if I change my mind?

You can withdraw your child from this study at any time, without giving any reason and without their care being affected. If your child is withdrawn, we will keep all the information we have collected from you and them up until that point.

What happens if I agree for my child to take part?

If, after reading this information sheet, you are interested for your child to take part in the study, we will arrange to talk to you about the study and what it will involve for both of you.

You and your child will then be invited to the study site (the Clinical Ageing Research Unit, CARU) in Newcastle for three study visits. Each visit will last approx. four hours.

Before we conduct assessments or collect information from your child, we will ask you to sign a consent form and your child to sign an assent form. This confirms that you and they are happy for them to take part and fully understand what is involved. We will also double check that your child is suitable to take part.

To be suitable your child will need to be able to stand and walk unaided for 20 metres and be happy to take part in all the study assessments. If they have any conditions other than Friedreich's ataxia which affect their balance or movement it may not be possible for them to take part.

If your child is not suitable to take part, we will explain this to you and them and their involvement in the study will then end.

If your child is suitable to take part they will undergo the following assessments:

Collection of medical history, demographic and lifestyle information: We will ask you and your child questions about their medical history, any medications they are taking and about their lifestyle.

- **Physical examination:** A doctor will perform general and neurological examinations of your child. In addition, their height, weight, heart rate, respiration rate and blood pressure will be recorded.

- **Ataxia and disease severity assessment scales:** These are specific examination tools assessing the severity of ataxia and other Friedreich's ataxia-related symptoms.

- **Assessment of upper limb co-ordination:** We will ask your child to perform two tasks to measure their hand co-ordination. These involve using a keyboard and putting pegs in holes in a board whilst being timed.

- **Assessments of cognitive function:** We will ask your child to complete some tests of their memory and the way they understand things

- **Questionnaires:** Your child will complete several questionnaires related to balance, physical and mental well-being, and quality of life. These questionnaires can either be completed online (via computer or smartphone) from home or can be completed on paper.

- **Walking and balance assessments:** These assessments will take place in a special area called a gait laboratory. Wearable sensors will be placed on different parts of your child's body (e.g., lower back and/or feet) (see the below Figure for examples of the types of sensor we may use) and we will ask them to walk at different speeds. We will also assess their balance whilst standing in a variety of stances (different standing positions). We will record your child on video during the assessments in the gait lab so that we can check the data we collect. Your child will be recognisable in these videos however they will be stored securely by the gait lab team at Newcastle University. We may wish to use these videos in the future for teaching and training purposes however you can choose on the informed consent form whether you would like your child's videos to be used for these purposes.

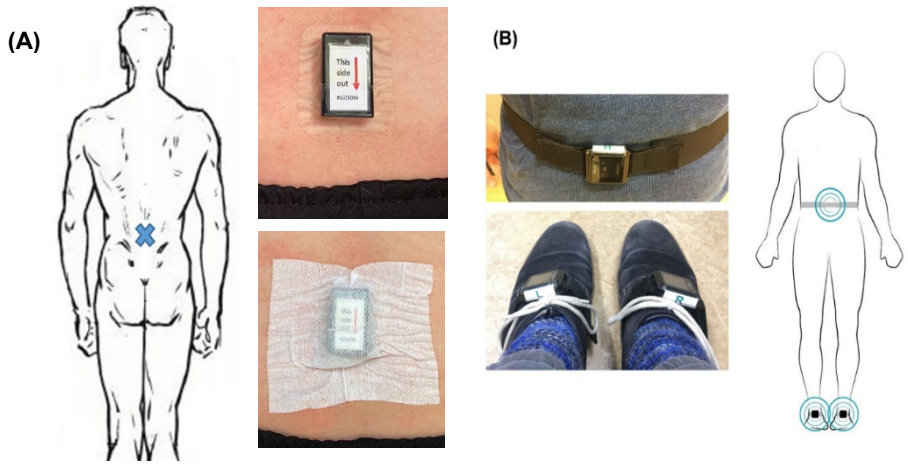

Figure. (A) Axivity, AX6 (single sensor). (B) Opal sensors, one placed in the lower back and two others on feet.

- **Collection of Information from Medical Records:** We will ask your permission to collect any relevant clinical information from your child's medical records from before and during the time they are in the study (this includes results from any clinical tests or investigations).

- **Wearable technology for seven-day monitoring:** During the visit, the Axivity AX6 wearable sensor will be placed on your child's body (as described above). We will ask them to keep this on for the next seven days to measure their physical activity in their usual environment. They will need to keep the sensor on at all times unless they are doing activities in water such as swimming At the end of this time, they will post the sensor back (we will provide a special pre-paid envelope to return the sensor). We will give you and your child full instructions on wearing and using the sensor and a separate instruction sheet will also be provided.

The above assessments will be repeated at each of the following study visits (Month Six and Month 12) at CARU.

Once your child has completed the Month 12 Visit (and returned their wearable sensor) their participation in the study will be complete. At the end of the study, we will ask you and your child for feedback on your experiences of taking part.

Completion of Questionnaires Online

As explained above, your child will have the option to complete the study questionnaires online (via computer or smartphone). We will ask them to provide an email address which will be used to send them a link to a webpage where they can complete the questionnaires.

The system used for these questionnaires is secure and only authorised members of the research team will be able to see their responses.
The questionnaires your child completes will not identify them by name. They will be sent a new link before each of the study visits which will take them to the relevant questionnaires.

We can send you and your child a text message alert as a reminder to fill in any incomplete questionnaires if you and they opt in to provide a mobile phone number. This phone number will be stored on the same secure system as the email address and will not be shared with anyone else.

If your child doesn't have access to the internet, or if they would prefer to complete the questionnaires on paper, we can arrange this for them.

Who is the sponsor and data controller for this research study?

Newcastle University is the Sponsor for this study.

Newcastle University and The Newcastle upon Tyne Hospitals NHS Foundation Trust (Newcastle Hospitals) will use information from you and your child to undertake this study and will act as the data controllers. This means that Newcastle University and Newcastle Hospitals are responsible for looking after your child's and your information and using it properly.

The lawful basis for carrying out this study under the General Data Protection Regulation (GDPR) is Task in the Public Interest, (Article 6,1e). This is because research is seen as part of the University's and Trust's duties.

This study is part of a larger project involving researchers from the Hertie Institute for Clinical Brain Research and Centre for Integrative Neuroscience in Tubingen (Germany). Data collected as part of this study will be shared with the research team in Tubingen however this data will be de-identified.

This means that the researchers in Tubingen will not know who your child is (instead of their name they will be identified by a study-specific code only).

Representatives from the Sponsor, Newcastle Hospitals, or the regulatory authorities may request access to the study records to check that the study is being conducted correctly. This may include accessing identifiable information about you and your child. Any staff accessing this information will work to strict codes of confidentiality.

Identifiable data from this study will be kept by Newcastle Hospitals for at least five years after the study has ended.

De-identified data (e.g., where your child is not directly identifiable) will be held by Newcastle University and the project partners (Germany) for longer (20 years or more).

How will you use information about me and my child?

We will need to use information from you and your child for this study.
This information will include:

- Your name and your child's full name
- Your child's date of birth
- Your child's NHS number
- Your and your child's postal address
- Your or your child's email address (optional)
- Your and your child's telephone number (including mobile phone number for text message alerts; optional)
- Details of your child's medical history, genetic diagnosis and demographic information (e.g., sex, ethnicity and educational level)
- Videos of your child's assessments in the gait laboratory

We will use this information to do the research or to check your child's records to make sure that the research is being done properly.

As described above, people who do not need to know who your child is will not be able to see your or your child's name or contact details. The data will have a code number instead. We will keep all information about you and your child safe and secure.

Once we have finished the research, we will keep some of the data so we can check the results. We will write our reports in a way that no-one can work out that your child took part in the study.

The study data will be de-identified and may in future be shared with other researchers in the UK and overseas.

It may also be made available as "open data" through a research data repository. This means the de-identified study data will be publicly available and may be used for purposes not related to this study.

We will make every attempt to ensure that it is not possible to identify your child from this open data.

With your permission, we will notify your child's general practitioner (GP) so they are aware they are taking part in this study.

What are my choices about how my and my child's information is used?

You can stop being part of the study at any time, without giving a reason, but we will keep information about you and your child that we already have.

We need to manage your child's records in specific ways for the research to be reliable. This means that we won't be able to let you or your child see or change the data we hold about you.

What can I find out more about how my and my child's information is used?

You can find out more about how we use your information

- at https://www.ncl.ac.uk/data.protection/ and/or by contacting the Newcastle University Data Protection Officer rec-man@ncl.ac.uk or the Newcastle Hospitals Data Protection Officer nuth.dpo@nhs.net.
- at www.hra.nhs.uk/information-about-patients/
- via a leaflet available from www.hra.nhs.uk/patientdataandresearch
- by asking one of the research team
- by sending an email to **nuth.mitoresearch@nhs.net** or
- by ringing us on **0191 2083105**

If you have any concerns about the way your data, or your child's is handled as part of this study, you have the right to lodge a complaint with the Information Commissioners Office (ICO), https://ico.org.uk/.

Are there any benefits to participating in this study?

There will be no direct benefits to you or your child for taking part in this study. However, we hope that the information we collect will help us to identify tools that can robustly measure the severity of ataxia in people with Friedreich's ataxia. The data collected from this study will inform the design of future studies looking at treatments for Friedreich's ataxia.

Are there any disadvantages to taking part?

The research study visits will last much longer than your child's usual clinic appointments. We will provide assistance with travel and parking costs for the research visits. We will also provide you and your child with refreshments/reimbursement for the cost of light refreshments during the visits.

The questionnaires we ask your child to complete at home will take approximately one hour and, if you choose to complete them online, will require the use of a smartphone or computer and internet access.

The study assessments that will be performed are safe and well-tolerated by most people and these will be performed by researchers who are experienced in delivering them. We will ensure before your child enters the study, and again before each assessment, that they are suitable to undergo the assessment.

Your child may also feel unsteady when they do some of the balance assessments, or when they are asked to walk at a faster pace in the gait laboratory, however trained staff will be with them at all times during these assessments to ensure that they are safe and comfortable.

If your child finds any of the study assessments physically or emotionally challenging, a member of the research team will be on hand to provide support. If you or your child would prefer to discuss any difficulties with a member of their usual clinical care team, we will arrange this for you.

For the wearable sensors, your child may experience slight irritation from the adhesive (glue) used to attach the sensors to their body. Any irritation should not last long and you and they will be provided contact details for the research team so that you can seek advice if you have any concerns.

We do not expect to find any area of clinical concern (i.e., indicating a potential problem) or any health issues that you and your child were not previously aware of, during the gait and balance assessments. If anything is found which may need further investigation or treatment, the study doctors and Principal Investigator will discuss this with you and your child and inform their GP or refer your child to the appropriate specialists for further follow-up.

What happens once the study has finished?

Once your child has attended the three study visits they will have completed the study. We think that it will take us about 18 months to complete the study for all participants.

Once the study has been completed and the results analysed, we hope to publish the findings in medical journals and present them at conferences. We will also publicise the findings through patient groups and charities.

It will not be possible to provide you with your child's individual results from the study assessments. However, once all the study data have been analysed, a summary of the overall study findings will be available. We will send a copy of this summary to you and your child along with a thank you letter to them for taking part.

Who has reviewed this study?

All research in the NHS is looked at by an independent group of people, called a Research Ethics Committee, to protect your interests. This study has been reviewed and granted a favourable opinion by East of England -Cambridge South Research Ethics Committee.

Who will be managing this study?

Newcastle University are sponsoring this study which means they are responsible for its conduct and management.

The Chief Investigator for this study is Dr Yi Shiau Ng. Dr Ng has responsibility for the overall conduct of the study.

Newcastle University work closely with Newcastle Hospitals, where the study visits will be taking place.

How is this study funded?

The French Friedreich's Ataxia Association (AFAF) have provided funding for this study.

Who should I contact if I have any concerns?

Chief/Principal Investigator: Dr Yi Shiau Ng
Email: **nuth.mitoresearch@nhs.net**
Study Contact Name: Isabel Barrow
Tel: 0191 2083105
Email: **nuth.mitoresearch@nhs.net**

If you wish to discuss the study or have any concerns, you can contact a member of the study team on the details above.

If you wish to discuss the study or raise a complaint with someone independent of the study team, please contact Newcastle University at:
Email: sponsorship@newcastle.ac.uk

If you wish to raise a complaint on how your personal data is handled, you can contact the Newcastle University Data Protection Officer who will investigate the matter by emailing rec-man@ncl.ac.uk

If you are not satisfied with their response you can complain to the Information Commissioner's Office (ICO): https://ico.org.uk/

If you have received this information sheet via your child's clinical team and would prefer to raise your concerns with someone not involved in their care, you can contact the local Patient Advice and Liaison Service (PALS).

This service is confidential and can be contacted on:
Freephone: **0800 032 0202**
Email: NorthofTynePALS@northumbria-healthcare.nhs.uk
Address: Freepost PALS

Thank you for reading this information sheet