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Participant Information Sheet/Consent Form

Interventional Study - *Adult providing own consent*

Kingston Centre

Title	The efficacy of rehabilitation for hereditary cerebellar ataxia. A randomised controlled trial.
Short Title	Rehabilitation for ataxia trial
Protocol Number	Version 7. 13/11/2019
Coordinating Principal Investigator/ Principal Investigator	Dr Sarah Milne Ms Melissa Roberts
Associate Investigator(s)	Professor Martin Delatycki Ms Shannon Williams (Perth) Ms Jillian Chua (Sydney) Ms Alison Grootendorst (Darwin) Ms Aleka Freijah (Darwin) Dr Louise Corben Dr Phillipa Lamont Dr David Szmulewicz Prof Joshua Burns Prof Carolyn Sue Dr Christina Liang Ms Libby Massey Mr Paul Gerken
Location	Kingston Centre, Monash Health

Part 1 What does my participation involve?

1 Introduction

You are invited to take part in this research project. This is because you have been diagnosed with a genetic ataxia. The research project is testing the effects of physical rehabilitation for people with hereditary cerebellar ataxia.

This Participant Information Sheet/Consent Form tells you about the research project. It explains the tests and treatment involved. Knowing what is involved will help you decide if you want to take part in the research.

Please read this information carefully. Ask questions about anything that you don't understand or want to know more about. Before deciding whether or not to take part, you might want to talk about it with a relative, friend or your local doctor. If you require an interpreter, please ask the study doctor and you will be provided with an interpreter to explain this research study and the information on this form.

Participation in this research is voluntary. If you don't wish to take part, you don't have to. You will receive the best possible care whether or not you take part.

If you decide you want to take part in the research project, you will be asked to sign the consent section. By signing it you are telling us that you:
• Understand what you have read
• Consent to take part in the research project
• Consent to have the tests and treatments that are described
• Consent to the use of your personal and health information as described.

You will be given a copy of this Participant Information and Consent Form to keep.

2 What is the purpose of this research?

Aims and objectives of the project: This project aims to examine whether six weeks of outpatient rehabilitation followed by a 24-week home exercise program with fortnightly support from a physiotherapist will improve the ability to move and function in everyday activities. This project will compare the effectiveness of this program with the effectiveness of the therapy or exercise people are currently completing.

Background: A recent review of all available research found that rehabilitation for people with ataxia improves balance, the ability to function and walking speed. However, published studies are mainly based on very small numbers of people and have used methods that don't provide conclusive evidence. Studies have also shown that the benefits from attending outpatient rehabilitation are difficult to maintain.

Justification and significance of the project: This study is aims to determine if outpatient rehabilitation and home exercises can improve physical ability, balance and ataxia symptoms. It aims to recruit 80 people with ataxia across Australia. The findings from this study will guide services, clinicians and individuals with ataxia in deciding on the most appropriate therapy for people with a genetic ataxia.

This research has been initiated by the study doctors, Prof Martin Delatycki and Dr Sarah Milne.

This research has been funded by a National Health and Medical Research Council (NHMRC) Medical Research Future Fund Lifting Clinical Trials and Registries Capacity Grant.

This research is being conducted by researchers at the Murdoch Children's Research Institute, Monash Health, Alfred Health, Royal Perth Hospital, Sir Charles Gairdner Hospital, Royal North Shore Hospital, Ryde Hospital, Royal Darwin and Palmerston Regional Hospitals, and the MJD Foundation.

3 What does participation in this research involve?

Consent: If you are willing to participate, you will be asked to sign this consent form to indicate that you wish to participate in the study. No research will be conducted unless your consent is provided, and you are willing to participate.

Screening for participation in the project: People who have a diagnosis of hereditary cerebellar ataxia and who attend the Neurogenetic or Ataxia Clinics at Monash Medical Centre, Caulfield Hospital, Royal Children's Hospital, Royal Melbourne Hospital, Royal Perth Hospital, Royal North Shore Hospital Royal Darwin and Palmerston Hospitals and the MJD Foundation will be screened by their physiotherapist or neurologist to determine if they may be eligible to participate. They will be asked to attend an initial appointment to discuss the study and sign this

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consent form. For some people, this may involve a consultation with a neurologist to confirm their diagnosis and establish a medical contact for the duration of the study. All people meeting the eligibility criteria will be given the opportunity to participate in the project.

Random allocation: If you are willing to participate, you will be randomly allocated to an intervention group or a control group. The intervention group will be provided with a six-week outpatient rehabilitation program followed by a 24-week home exercise program with alternating fortnightly home visits and a video or phone call from a physiotherapist to support you complete the program. The control group will be asked to maintain their current physiotherapy or exercise program for 30 weeks.

This is a randomised controlled research project. Sometimes we do not know which treatment is best for treating a condition. To find out we need to compare different treatments. We put people into groups and give each group a different treatment. The results are compared to see if one is better. To try to make sure the groups are the same, each participant is put into a group by chance (random). You have a one in two chance you will receive the rehabilitation intervention in this study, and a one in two chance you will receive no intervention.

Participation for the intervention group: If you are allocated to the intervention group, you will be asked to attend a structured rehabilitation program and four assessment sessions at the Kingston Centre, Cheltenham.

The rehabilitation will involve outpatient physiotherapy three times per week for six weeks. Each session of rehabilitation will consist of two hours of physiotherapy: one hour of physiotherapy on land and one hour of physiotherapy in a hydrotherapy pool. A physiotherapy assistant will support the physiotherapist. Your program will be focused on improving your physical function and you can set personal goals in conjunction with the physiotherapist. The program will target balance, co-ordination, core-stability, daily function and strength. After the six-week outpatient rehabilitation program, you will be provided with a home exercise program. You will be asked to complete the home program for one hour, five days per week, for 24-weeks. Your home exercise program will involve fortnightly physiotherapy support. This will be through alternating home visits and video calls. The physiotherapist will modify and progress your home exercise program and provide you with encouragement to continue the program.

If you are allocated to the intervention group, you will be asked to attend four, two-hour assessment sessions. At these sessions you will be required to undergo a physical exam of co-ordination, sensation, balance and walking or sitting. You will also be asked questions about your quality of life, your independence in completing daily activities and the amount of exercise you are completing. If you can walk, you will also be instructed to wear a Fitbit for seven consecutive days once you have returned back home. The Fitbit is worn on your wrist and contains sensors that will measure the number of steps taken. You will be required to complete this activity during your normal day to day routine. The four assessment sessions will occur: at the beginning of the study, after 6 weeks, 18 weeks and 30 weeks.

Participation for the control group: If you are allocated to the control group, you will be asked to continue the same amount of exercise and/or therapy you were doing when you commenced the study. You will be asked to continue this for 30-weeks. You will also be asked to attend four assessment sessions at the Kingston Centre, Cheltenham.

If you are allocated to the control group, you will be asked to attend four, two-hour assessment sessions. At these sessions you will be required to undergo a physical exam of co-ordination, sensation, balance and walking or sitting. You will also be asked questions about your quality of life, your independence in completing daily activities and the amount of exercise you are

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completing. If you can walk, you will also be instructed to wear a Fitbit for seven consecutive days once you have returned back home. The Fitbit is worn on your wrist and contains sensors that will measure the number of steps take. You will be required to complete this activity during your normal day to day routine. The four assessment sessions will occur: at the beginning of the study, after 6 weeks, 18 weeks and 30 weeks.

Video recording: We will ask up to 20 adult participants permission to record their first assessment with a video recorder. This will be used by the principal investigator to train all the physiotherapists who are completing the assessments. This is to ensure their assessments are accurate and consistent. We may also ask adult participants in the rehabilitation group permission to record one treatment session with a video recorder. This will be used by principle investigator to work with the other physiotherapists to ensure the treatments are consistent and best practice across the sites.

Limiting bias: This research project has been designed to make sure the researchers interpret the results in a fair and appropriate way and avoids chief investigators or participants jumping to conclusions.

If you participate in the study, you will be asked not to discuss the group you have been allocated to during your assessment with the physiotherapist. This ensures the assessing physiotherapist is not biased when he/she assesses you.

Costs and reimbursement: There are no additional costs associated with participating in this research project, nor will you be paid. All tests and physiotherapy care required as part of the research project will be provided to you free of charge. You will be reimbursed for any reasonable travel and parking associated with the research project visit.

GP information: It is desirable that your local doctor be advised of your decision to participate in this research project if you are allocated into the rehabilitation group. If you have a local doctor, we recommend that you inform them of your participation in this research project.

4 What do I have to do?

It is important to tell the research staff about any treatments or medications you may be taking, including over-the-counter medications, vitamins or herbal remedies, acupuncture or other alternative treatments.

You are unable to participate in this study if you are already participating or plan to participate in any drug trials during the 30-weeks you are participating in this study.

There are no other restrictions for participating in this study.

5 Other relevant information about the research project

We plan to include 80 people with hereditary cerebellar ataxia in this project, both adults and children over 15 years of age.

We plan to include 40 people at this site, Kingston Centre, Cheltenham.

This project involves researchers from the Monash Health, Alfred Health, Royal Perth Hospital, Sir Charles Gairdner Hospital, Royal North Shore Hospital, Ryde Hospital, Royal Darwin and Palmerston Regional Hospital and Machado-Joseph Disease Foundation Offices, Coconut

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Grove and Groote Eylandt working in collaboration with the researchers at the Murdoch Children's Research Institute.

6 Do I have to take part in this research project?

Participation in any research project is voluntary. If you do not wish to take part, you do not have to. If you decide to take part and later change your mind, you are free to withdraw from the project at any stage.

If you do decide to take part, you will be given this Participant Information and Consent Form to sign and you will be given a copy to keep.

Your decision whether to take part or not to take part, or to take part and then withdraw, will not affect your routine treatment, your relationship with those treating you or your relationship with Monash Health or Alfred Health.

7 What are the alternatives to participation?

You do not have to take part in this research project to receive treatment at this hospital. Other options are available; these include accessing physiotherapy or similar services such as exercise physiology in the community. Your study doctor will discuss these options with you before you decide whether or not to take part in this research project. You can also discuss the options with your local doctor.

8 What are the possible benefits of taking part?

We cannot guarantee or promise that you will receive any benefits from this research; however, if you are allocated to the intervention group possible benefits may include an improvement in strength and balance.

If you are allocated to the control group there will be no clear benefit to you from your participation in this research.

However, there are potential indirect benefits. This study will hopefully result in a better understanding of the effects of outpatient rehabilitation and supported home exercise in people for genetic ataxia. This will guide for clinicians who provide care to people with ataxia and will provide justification for rehabilitation care.

9 What are the possible risks and disadvantages of taking part?

While there are no obvious risks from participation in this study, possible risks could include fatigue from attending rehabilitation and an increased risk of falling due to fatigue and challenging balance.

This will be monitored by the researcher and the physiotherapist and rest breaks and the therapy provided will be modified accordingly. Please discuss any concerns with the researcher.

If you become upset or distressed as a result of your participation in the research, the study doctor will be able to arrange for counselling or other appropriate support.

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10 What if new information arises during this research project?

Sometimes during the course of a research project, new information becomes available about the treatment that is being studied. If this happens, your study doctor will tell you about it and discuss with you whether you want to continue in the research project. If you decide to withdraw, your study doctor will make arrangements for your regular health care to continue. If you decide to continue in the research project you will be asked to sign an updated consent form.

Also, on receiving new information, your study doctor might consider it to be in your best interests to withdraw you from the research project. If this happens, he/ she will explain the reasons and arrange for your regular health care to continue.

11 Can I have other treatments during this research project?

It is important to tell your study doctor and the study staff about any treatments or medications you may be taking, including over-the-counter medications, vitamins or herbal remedies, acupuncture or other alternative treatments. You should also tell your study doctor about any changes to these during your participation in the research project.

12 What if I withdraw from this research project?

If you decide to withdraw from the project, please notify a member of the research team before you withdraw. This notice will allow that person or the research supervisor to discuss any concerns you may have, or special requirements related to withdrawing.

If you do withdraw your consent during the research project, the study doctor and relevant study staff will not collect additional personal information from you, although personal information already collected will be retained to ensure that the results of the research project can be measured properly and to comply with law. You should be aware that data collected by the sponsor up to the time you withdraw will form part of the research project results. If you do not want them to do this, you must tell them before you join the research project.

13 Could this research project be stopped unexpectedly?

This research project will not be stopped unexpectedly.

14 What happens when the research project ends?

If you wish to know the results of this research project once it has been completed, we would be happy to send you a letter explaining our overall findings.

Part 2 How is the research project being conducted?

15 What will happen to information about me?

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By signing the consent form, you consent to the study doctor and relevant research staff collecting and using personal information about you for the research project. Any information obtained in connection with this research project that can identify you will remain confidential.

Hard copy data will be stored in a locked cabinet and securely stored at the Kingston Centre. You will be allocated a non-identifiable code for the purpose of analysing the results. Your data, including your name and date of birth, will be entered into a password protected database at the Murdoch Children's Research Institute, Melbourne. Only the research team listed in this form will have access to this information. The database will be set up so that your personal details cannot be exported.

If you agree to a recording of your assessment, the video of your assessment will only be shared with other researchers listed on this form, for the purpose of this research. The video will be stored for a minimum seven years on a password protected computer and will be permanently destroyed at this time.

Your information will only be used for the purpose of this research project and it will only be disclosed with your permission, except as required by law.

Information about you may be obtained from your health records held at this and other health services for the purpose of this research. By signing the consent form, you agree to the study team accessing health records if they are relevant to your participation in this research project. This will include obtaining your genetic test results for the purpose of confirming your diagnosis and the genetic variation that resulted in your diagnosis.

Your health records and any information obtained during the research project are subject to inspection for the purpose of verifying the procedures and the data. This review may be done by the study sponsor, Murdoch Children's Research Institute or the institution relevant to this Participant Information Sheet, Monash Health, or as required by law. By signing the Consent Form, you authorise release of, or access to, this confidential information to the relevant research personnel and regulatory authorities as noted above.

It is anticipated that the results of this research project will be published and/or presented in a variety of forums. In any publication and/or presentation, information will be provided in such a way that you cannot be identified, except with your permission. Confidentiality will be maintained as data will presented as a group in any publication/presentation arising from this project. Individual data will not be identifiable.

In accordance with relevant Australian and Victorian privacy and other relevant laws, you have the right to request access to your information collected and stored by the research team. You also have the right to request that any information with which you disagree be corrected. Please contact the study team member named at the end of this document if you would like to access your information.

Future research: The Murdoch Children's Research Institute are the custodians of the database and may share this data with other researchers after completion of the study. This data will be non-identifiable and will not be able to identify you. Your information will be stored on the database for use only in future research studies that are related to the original research project.

Any information obtained for the purpose of this research project *and for the future research described in Section 16* that can identify you will be treated as confidential and securely stored. It will be disclosed only with your permission, or as required by law.

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16 Injury

If you suffer any injuries or complications as a result of this research project, you should contact the study team as soon as possible and you will be assisted with arranging appropriate medical treatment. If you are eligible for Medicare, you can receive any medical treatment required to treat the injury or complication, free of charge, as a public patient in any Australian public hospital.

17 Who is organising and funding the research?

This research project is being conducted by Professor Martin Delatycki and Dr Sarah Milne.

Funding is provided by a National Health and Medical Research Council, Medical Research Future Fund grant. The Murdoch Children's Research Institute is administering the funds for this research project.

Monash Health will receive a payment from Murdoch Children's Research Institute for undertaking this research project.

No member of the research team will receive a personal financial benefit from your involvement in this research project (other than their ordinary wages).

18 Who has reviewed the research project?

All research in Australia involving humans is reviewed by an independent group of people called a Human Research Ethics Committee (HREC). The ethical aspects of this research project have been approved by the HREC of Monash Health.

This project will be carried out according to the *National Statement on Ethical Conduct in Human Research (2007)*. This statement has been developed to protect the interests of people who agree to participate in human research studies.

19 Further information and who to contact

The person you may need to contact will depend on the nature of your query.

If you want any further information concerning this project or if you have any medical problems which may be related to your involvement in the project, you can contact the principal study doctor, Dr Sarah Milne on (03) 8341 6442, or any of the following people:

Clinical contact people

Name	*Professor Martin Delatycki*
Position	*Friedreich Ataxia Clinic Director*
Telephone	(03) 8341 6290

For matters relating to research at the site at which the participant is taking part, the details of the local site complaints person are:

Complaints contact person

Name	*Deborah Dell*
Position	*Manager, Human Research Ethics Committees*
Telephone	(03) 9594 4611
Email	*Deborah.dell@monashhealth.org*

If you have any complaints about any aspect of the project, the way it is being conducted or any questions about being a research participant in general, then you may contact:

Reviewing HREC approving this research and HREC Executive Officer details

Reviewing HREC name	Monash Health
HREC Executive Officer	*Deborah Dell*
Telephone	(03) 9594 4611
Email	*Deborah.dell@monashhealth.org*

Local HREC Office contact (Research Governance Officer)

Name	Michael Kios
Position	Research Governance Officer
Telephone	(03) 9594 4606
Email	michael.kios@monashhealth.org

Consent Form - *Adult providing own consent*

Title	The efficacy of rehabilitation for hereditary cerebellar ataxia. A randomised controlled trial.
Short Title	Rehabilitation for ataxia trial.
Protocol Number	Version 7. 13/11/2019
Coordinating Principal Investigator/ Principal Investigator	Dr Sarah Milne Ms Melissa Roberts
Associate Investigator(s)	Professor Martin Delatycki Ms Shannon Williams Ms Jillian Chua Ms Alison Grootendorst Ms Aleka Freijah Dr Louise Corben Dr Phillipa Lamont Dr David Szmulewicz Prof Joshua Burns Prof Carolyn Sue Dr Christina Liang Ms Libby Massey Mr Paul Gerken
Location	Kingston Centre, Monash Health.

Consent Agreement

I have read the Participant Information Sheet or someone has read it to me in a language that I understand.

I understand the purposes, procedures and risks of the research described in the project.

I give permission for my doctors, other health professionals, hospitals or laboratories outside this hospital to release information to Murdoch Children's Research Institute concerning my disease and treatment for the purposes of this project. I understand that such information will remain confidential.

I have had an opportunity to ask questions and I am satisfied with the answers I have received.

I freely agree to participate in this research project as described and understand that I am free to withdraw at any time during the study without affecting my future health care.

I understand that I will be given a signed copy of this document to keep.

Declaration by Participant – for participants who have read the information

Name of Participant (please print) _____

Signature _____ Date _____

Declaration – for participants unable to read the information and consent form

Witness to the informed consent process

Name (please print) _____

Signature _____ Date _____

* Witness is not to be the investigator, a member of the study team or their delegate. In the event that an interpreter is used, the interpreter may not act as a witness to the consent process. Witness must be 18 years or older.

Declaration by Study Doctor/Senior Researcher†

I have given a verbal explanation of the research project; its procedures and risks and I believe that the participant has understood that explanation.

Name of Study Doctor/
Senior Researcher† (please print) _____

Signature _____ Date _____

† A senior member of the research team must provide the explanation of, and information concerning, the research project.

Note: All parties signing the consent section must date their own signature.

By signing this consent section, I consent to the storage of my de-identified information on the database managed by the Murdoch Children's Research Institute, as described in *section 15*, for any related future research. By signing this consent form, I agree to the use of my information for the purposes of any further research related to this project.

Yes ☐ No ☐

Declaration by Participant – for participants who have read the information

Name of Participant (please print) _____

Signature _____ Date _____

Declaration – for participants unable to read the information and consent form

Witness to the informed consent process

Name (please print) _____

Signature _____ Date _____

* Witness is not to be the investigator, a member of the study team or their delegate. In the event that an interpreter is used, the interpreter may not act as a witness to the consent process. Witness must be 18 years or older.

Declaration by Study Doctor/Senior Researcher†

Name of Study Doctor/
Senior Researcher† (please print) _____

Signature _____ Date _____

† A senior member of the research team must provide the explanation of, and information concerning, the research project.

Note: All parties signing the consent section must date their own signature.

By signing this consent section, I consent to my first assessment session to be recorded, for the purposes of this research project only. By signing this consent form, I agree this video to be shared with other researchers listed on this protocol.

Yes ☐ No ☐

Declaration by Participant – for participants who have read the information

Name of Participant (please print) _____

Signature _____ Date _____

Declaration – for participants unable to read the information and consent form

Witness to the informed consent process

Name (please print) _____

Signature _____ Date _____

* Witness is not to be the investigator, a member of the study team or their delegate. In the event that an interpreter is used, the interpreter may not act as a witness to the consent process. Witness must be 18 years or older.

Declaration by Study Doctor/Senior Researcher†

Name of Study Doctor/
Senior Researcher† (please print) _____

Signature _____ Date _____

† A senior member of the research team must provide the explanation of, and information concerning, the research project.

Note: All parties signing the consent section must date their own signature.

By signing this consent section, I am willing to be contacted by the researchers named in this study about future research for which I may be eligible. I understand I am under no obligation to participate if contacted.

Yes ☐ No ☐

Declaration by Participant – for participants who have read the information

Name of Participant (please print) _____

Signature _____ Date _____

Declaration – for participants unable to read the information and consent form

Witness to the informed consent process

Name (please print) _____

Signature _____ Date _____

* Witness is not to be the investigator, a member of the study team or their delegate. In the event that an interpreter is used, the interpreter may not act as a witness to the consent process. Witness must be 18 years or older.

Declaration by Study Doctor/Senior Researcher†

Name of Study Doctor/
Senior Researcher† (please print) _____

Signature _____ Date _____

† A senior member of the research team must provide the explanation of, and information concerning, the research project.

Note: All parties signing the consent section must date their own signature.

Form for Withdrawal of Participation - *Adult providing own consent*

Title	The efficacy of rehabilitation for hereditary cerebellar ataxia. A randomised controlled trial.
Short Title	Rehabilitation for ataxia trial.
Protocol Number	Version 7. 13/11/2019
Coordinating Principal Investigator/ Principal Investigator	Dr Sarah Milne Ms Melissa Roberts
Associate Investigator(s)	Professor Martin Delatycki Ms Shannon Williams (Perth) Ms Jillian Chua (Sydney) Ms Alison Grootendorst (Darwin) Ms Aleka Freijah (Darwin) Dr Louise Corben Dr Phillipa Lamont Dr David Szmulewicz Prof Joshua Burns Prof Carolyn Sue Dr Christina Liang Ms Libby Massey Mr Paul Gerken
Location	Kingston Centre, Monash Health

Declaration by Participant

I wish to withdraw from participation in the above research project and understand that such withdrawal will not affect my routine treatment, my relationship with those treating me or my relationship with Monash Health or Alfred Health.

Name of Participant (please print) _____

Signature _____ Date _____

Declaration by Study Doctor/Senior Researcher†

I have given a verbal explanation of the implications of withdrawal from the research project and I believe that the participant has understood that explanation.

Name of Study Doctor/
Senior Researcher† (please print) _____

Signature _____ Date _____

† A senior member of the research team must provide the explanation of and information concerning withdrawal from the research project.

Note: All parties signing the consent section must date their own signature.