Government of Western Australia
Department of Health

TELETHON KIDS INSTITUTE

Participant Information Sheet/Consent Form - Parent/Guardian, Participant (if able)

Perth Children's Hospital/Telethon Kids Institute

Title	**Developing Predictors of Disease Progression in Children with Neuromuscular Disorders to Prevent Future Respiratory Failure**
Short Title	**Clinical Predictors of Respiratory Failure in Paediatric Neuromuscular Disorders**
Protocol Number	**2015185**
Project Sponsor	**Telethon Kids Institute**
Coordinating Principal Investigator/ Principal Investigator	**Dr Andrew Wilson** **Dr Adelaide Withers, Prof Graham Hall, Dr Jenny Downs, Dr Peter Rowe**
Associate Investigator(s)	**Ms Hayley Lethlean, Muscular Dystrophy Association of WA**
Location	**Perth Children's Hospital**

Note: The term 'Your Child' refers to the child participating in the research. If you are the child participating in the research 'Your Child' refers to you.

1. Introduction

This is an invitation for the child in your care to take part in this research project. This research project is aiming to identify problems with breathing during sleep in children with neuromuscular disorders and to see if we can diagnose this problem earlier than if we rely on asking about symptoms alone. To do this, we also need information about the lung function of healthy children. We are looking to recruit healthy children who will provide a control group for the children with neuromuscular disorders.

This Participant Information Sheet/Consent Form tells you about the research project. It explains the tests and research involved, which will help you decide if you want your child to take part in this study.

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 the child can take part, you might want to talk about it with a relative, friend or local doctor.

Participation in this research is voluntary. If you do not wish for the child to take part, they do not have to. They will receive the best possible care whether or not they take part.

If you decide you want the child 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 the child taking part in the research project
- Consent to the child having the tests and research that are described
- Consent to the use of the child's personal and health information as described.

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

2. Why are we doing this study?

Children with neuromuscular disorders have weaker muscles, and this can affect their breathing. This is especially a problem while they are asleep as the muscles relax. Difficulty breathing during sleep can cause oxygen levels to drop and carbon dioxide levels to rise during sleep – this is called hypoventilation. Hypoventilation is often the first sign that lung function is worsening and can be a signal to doctors that more treatment is needed for the breathing muscles. If hypoventilation is not treated, muscle weakness can progress, eventually leading to lung failure.

Unfortunately, it is very difficult to identify when hypoventilation starts and when to begin treatment. The best test is with a sleep study – this requires an overnight stay in hospital, is an expensive test and not easily available at all places.

Currently we rely on asking about symptoms a person might have when they have hypoventilation to let us know when a sleep study is required. The problem is, the symptoms of hypoventilation are vague, may not be present or may be present because of another problem that is not hypoventilation. It is very difficult to detect when hypoventilation occurs just by asking about symptoms.

The main aim of this study is to use other tests (such as lung function testing) to see if we can detect hypoventilation earlier than just asking about symptoms. We are looking to recruit healthy children who will provide a control group for the children with neuromuscular disorders. We hope that by earlier diagnosis and treatment of muscle weakness during sleep, we can possibly prevent or slow the development of lung disease. This study will also allow us to collect information about lung function that will allow us to design clinical trials in the future, which may also help improve lung health in children with neuromuscular disorders.

This research is also being conducted by our collaborators at the Lucile Packard's Children Hospital at Stanford University, California USA, however the healthy control group is being recruited from Perth only.

3. Who is carrying out the study?

This research project is being conducted by Dr Andrew Wilson, Dr Adelaide Withers, Professor Graham Hall, Dr Jenny Downs and Dr Peter Rowe. Dr Andrew Wilson is the primary investigator/study doctor. This research project is being funded by the National Health and Medical Research Council (NHMRC), and partially funded by The Muscular Dystrophy Association of Western Australia (MDAWA) and the Duchenne Parent Project Netherlands (DPP NL). No member of the study team will receive a personal financial benefit from your child's involvement in this research project (other than their ordinary wages).

4. What is involved in this study?

If your child participates in this research project, we will ask you to sign the consent form before the study commences.

If you choose for your child to participate in this study, we will ask you and your child to visit Perth Children's Hospital (PCH) on six separate occasions over a two year follow up period. Each visit will take about 2 hours in total. At each visit, we will ask your child to perform lung function and motor function tests. Some extra information will be collected by questionnaire which can be completed at home. These tests are described in more detail below.

Lung function tests

Respiratory mechanics: This test will involve your child breathing normally into a machine through a mouthpiece, similar to a snorkel mouthpiece, while wearing a nose clip. The machine uses a sound wave to measure the stiffness or elasticity of your child's lungs. This works in a similar way to the way fishermen use sonar to find fish. We measure the sound waves that bounce back from your child's lungs. The way they are changed by the lungs gives us information about lung function. This test takes about 5 minutes.

Multiple breath washout test: This test measures the efficiency with which gases mix in the lungs and helps us to know if the different areas of your child's lungs are ventilating evenly. For this test, your child will be asked to breathe normally through the mouthpiece while wearing a nose clip. Then we will ask your child to breathe 100% oxygen until most of the nitrogen in their lungs is replaced by oxygen. The 100% oxygen your child breathes during this test does not taste or feel any different to normal air but may make the mouth a little dry. How long this test takes varies from person to person but usually doesn't exceed 5 minutes and will be repeated 3 times, with a rest period of up to 10 minutes in-between each test.

Spirometry: This test measures how much air your child breathes in and out. For this test, your child will be asked to wear a nose clip and to breathe normally through a mouthpiece. After a couple of normal breathing in and out, your child will be asked to breathe in as much air as they can at a moderate pace and then slowly exhale all of this air. The amount of air exhaled will give us a result called slow vital capacity (SVC). This process usually lasts for about 1- 2 minutes and will be repeated 3 times to get accurate measurements that match each other.

Peak Cough Flow (PCF): This test assesses the strength of the muscles your child uses for breathing by measuring how much air they can push out when they cough. For this test, your child will breathe normally through a mouthpiece and then we will ask them to take a full breath in and then cough out as hard as possible. We may perform this test up to five times.

Sniff Nasal Inspiratory Pressure (SNIP): This also assesses the strength of the muscles used for breathing. We will insert a tube (nasal probe) into one of your child's nostrils and ask them to breathe normally for a while through their nose. With your child's mouth closed, we will then ask them to do a short and fast sniff at the end of a normal breathe out. This will be repeated up to 4 times on each nostril (8 times total).

6-Minute Walk Test: We will also ask your child to perform a 6-minute walk test. This will involve measuring the distance your child is capable of walking in 6 minutes as well as their blood pressure and heart rate before and after the test.

Questionnaire: We will also ask you and your child to complete a short questionnaire which asks for information regarding the mood of your child, and their general breathing and sleep

patterns. The online links to your parent/guardian questionnaire will be emailed to you prior to your scheduled visit and can be completed at home before your child's visit. This will take around 20 minutes to complete. A member of the research team will then verify your completed questionnaires with you during your visit. You may also choose to complete the questionnaires on paper which you will be able to do during your visit. A member of the research team will read and help your child answer their questionnaire during the visit.

There are no costs associated with participating in this research project, nor will you or the participant be paid. We will reimburse you for parking at the Perth Children's Hospital/Telethon Kids Institute at each visit.

This research has been approved by and will be monitored by the Child and Adolescent Health Service Ethics and Research Governance Committee.

5. What will my child have to do?

This study requires six visits to the Perth Children's Hospital over a two year period, as outlined below. Each visit will take about 2 hours in total. We will try to find appointment times that suit you and your child.

Visit 1: baseline review, time of enrolment
Visit 2: 1 month
Visit 3: 3 months
Visit 4: 12 months
Visit 5: 18 months
Visit 6: 24 months

There are no lifestyle, dietary or other restrictions for your child. Your child should continue to take their usual medications (if any) and there are no restrictions on using any medications.

There are no restrictions on participating in this study, however you and your child are free to withdraw from the study at any time for any reason.

6. Does your child have to take part in this research project?

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

If you do decide that your child can 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 your child can or cannot take part, or take part and then be withdrawn, will not affect their routine treatment, relationship with those treating them or relationship with Perth Children's Hospital or the Telethon Kids Institute. Your child does not have to take part in this research project to receive treatment at this hospital.

7. What are the possible benefits of taking part?

Although there will be no clear benefit to your child from their participation in this study, we hope the findings will benefit other children with neuromuscular disorders and improve their quality of life.

8. What are the possible risks and disadvantages of taking part?

This research does not involve any treatments, so it is very unlikely that there are any risks associated with being involved in this study. While some children may not wish to perform the lung function tests, there are no risks associated with these tests.

9. What if your child is withdrawn from this research project?

You and your child can withdraw from this study at any time. If you do decide to withdraw your child from this research project, please notify a member of the research team before withdrawal. There are no specific requirements linked to withdrawing.

If you do withdraw consent during the research project, the study doctor and relevant study staff will not collect additional personal information from your child, 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. If you do not want this information to be used in the study, please tell us,

10. What happens when the research project ends?

At the end of the research project, a report outlining the results and findings of the study will be provided to participants. We anticipate this would be in the form of an information evening/seminar and all participants and their families would be invited to attend. Because of the length of the study, it may be some time before we are able to do this. You and your family will also be invited to our regular community forums, which we hold in association with Muscular Dystrophy WA, to provide updates during the course of the research project.

It is likely that the results from this study will be published in medical journals in the future but group findings will be reported and you and your child will not be identifiable.

11. What about my child's privacy?

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

Data collected will be de-identified, meaning that it will be coded, and only the study staff will have access to the information which identifies your child. Data will be kept at the Telethon Kid's Institute on secure computers stored in an area with swipe access only. Your child's 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 your child may be obtained from their health records held at this and other health services, for the purpose of this research. By signing the consent form you agree to the research team accessing health records if they are relevant to participation in this research project.

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 the participant cannot be identified, except with your permission. Confidentiality will be maintained by removing all identifiers and using the participant's code number for data presentation.

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

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

12. Complaints and compensation

If your child suffers 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 your child is eligible for Medicare, they can receive any medical treatment required to treat the injury or complication, free of charge, as a public patient in any Australian public hospital.

13. Who has approved 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 Children and Adolescent Health Services.

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.

14. Who do I contact if I have any questions?

If you would like any further information about this study, please do not hesitate to contact one of the research team.

Name	**Contact Number**
Dr Andrew Wilson	08 6456 5424
Dr Adelaide Withers	08 6456 5414
Prof Graham Hall	08 6319 1594

If you have any concerns and/or complaints about the project, the way it is being conducted or your rights or child's rights as a research participant, and would like to speak to someone independent of the project, please contact Executive Director of Medical Services at CAHS on08 6456 2222. Your concerns will be drawn to the attention of the Ethics Committee who is monitoring the study.

Consent Form – Parent/Guardian, Participant, if able

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Location	**Perth Children's Hospital**

Declaration by Parent/Guardian

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

I freely agree to the child participating in this research project as described and understand that I am free to withdraw them at any time during the project without affecting their future health care.

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

Name of Child (please print) _____

Name of Parent/Guardian (please print) _____

Signature of Parent/Guardian _____ Date _____

Signature of Participant (if able) _____ Date _____

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 parent/guardian of 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