

Information and Consent Form

TITLE: CANADIAN REGISTRY OF PATIENTS WITH ALPHA-1 ANTITRYPSIN DEFICIENCY

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You are being asked to participate in the Canadian and International Registry for Alpha-1 Antitrypsin Deficiency. Before agreeing to participate, it is important that you read and understand the following explanation of the proposed procedures. The following information describes the purpose, procedures, benefits, discomforts, risks and precautions associated with this study. It also describes your right to refuse to participate or withdraw from the registry at any time. In order to decide whether you wish to participate in the registry, you should understand enough about its risks and benefits to be able to make an informed decision. This is known as the informed consent process. Please ask the study doctor or study staff to explain any words you don't understand before signing this consent form. Make sure all your questions have been answered to your satisfaction before signing this document.

Background

What is Alpha-1 antitrypsin deficiency?

Alpha-1 antitrypsin deficiency is an inherited disorder that may cause liver and/or lung disease. Not everyone with an alpha-1 antitrypsin deficiency will develop lung disease or emphysema. In most cases the amount of alpha-1 antitrypsin present will be enough to protect the lung from severe destruction.

Alpha-1 antitrypsin is an important protein produced by the liver. Once the protein is released into the bloodstream and travels to the lung it protects the lungs from the destructive actions of common illnesses and exposures, particularly tobacco smoke. People with a deficiency of this protective protein often suffer from progressive lung damage known as emphysema. Unlike the common form of emphysema seen in otherwise healthy individuals who have smoked for many years, this alpha-1 antitrypsin deficiency form of emphysema may occur at unusually young age and after minimal exposure to tobacco smoke.

Emphysema is an irreversible condition that results from destruction of air sacs in the lungs. The air sacs (or alveoli) normally allow for absorption of oxygen into the blood and for the elimination of waste carbon dioxide. If they are destroyed, shortness of breath and chronic cough may result.

Present treatment is to help relieve symptoms, especially the breathlessness. The use of inhaled bronchodilators (e.g. Ventolin, Atrovent, Bricanyl) often help. Intravenous (injection) replacement therapy with a purified blood product (Prolastin®) has been approved for prescription use and is targeted at deficient subjects who have evidence of significant pulmonary

diseases. However, the role of this replacement therapy remains controversial. Further studies need to be undertaken.

Purpose of Registry

You have been asked to participate in the registry because you have been diagnosed with Alpha-1 Antitrypsin Deficiency. The Canadian Alpha-1 antitrypsin Deficiency Registry's main objective is to identify and gather information about Canadians with alpha-1 antitrypsin deficiency so that they and Canadian scientists can participate in an international effort.

At present time an International Registry has been set up with approximately 4,000 participants. There are several goals of this registry. Initially, the registry will gather existing medical information about people who are deficient in the protein to understand better why some people remain healthy and free of lung disease and why some people develop emphysema. In the longer term, people who are participants in the registry may be asked if they are willing to participate in research trials that might improve our diagnosis, assessment or management of the disease. By coordinating research efforts at this international level, the registry scientists hope to make more rapid progress than individual scientists would achieve working in isolation.

Procedures

What is expected of you?

You will be asked to sign a consent form and medical release of information form. We ask that either you or your doctor send these forms back to the Data management Centre.

You will be sent a yearly two page questionnaire that may be filled out by you with the assistance of your doctor, if required. This will keep us informed of your present condition. We will be looking at medical history, breathing test and other routine laboratory results.

Your Doctor

A four-page questionnaire will be sent to the doctor or doctors familiar with your deficiency. We are looking only for a simple medical history and breathing test and other routine laboratory results.

Risks and Benefits

There are no known risks or benefits to this registry. Information learned from this registry may benefit other patients in the future with Alpha-1 Deficiency.

Confidentiality

All information obtained for the registry will be held in strict confidence. You will be identified with a study number only. No names or identifying information will be used in any publication or presentations. No information identifying you will be transferred outside the investigator in this study or this hospital. In the event of data sharing with the International Registry for the analysis of large amounts of shared data, code numbers will identify all patients.

Participation

Your participation in the registry is voluntary. You can choose not to participate or you may withdraw at any time without affecting your medical care. Your permission to gather health information will not be considered permission to involve you unknowingly in trial research. You will be contacted to seek permission for clinical trial participation in the event of intervention trials.

Compensation

As this is a data based study, no compensation is available.

Questions

If you have any general questions about the registry, please call the doctor in charge of the registry, Dr. Kenneth Chapman at 416-603-5499 or you may also call the Alpha 1 Canadian Registry coordinator at 416-603-5020 or toll free number 1-800-352-8186. Email address: info@alpha1canadianregistry.com. Please note that this email is not secure and should be used for general communication purposes only. Personal health information should not be transmitted via email.

If you have any questions about your rights as a participant, please call the Chair of the University Health Network Research Ethics Board or the Research Ethics office at 416-581-7849. These people are not involved with the research registry in any way and calling him will not affect your participation in the registry.

Consent

I have had the opportunity to discuss this registry and my questions have been answered to my satisfaction. I have been informed about both the Canadian and the International Registry for Alpha-1 Antitrypsin Deficiency. I have read and have understood the Information written above for this registry. I, hereby, give my consent to have my name entered in the Canadian Registry and the International Registry. I may withdraw at any time without affecting my medical care. At no time will my name be used or given out for future studies without first being contacted. I agree that authorized persons may have access to my personal data, provided that confidentiality on the information obtained is maintained.

On this understanding, I agree to have my name and relevant health information entered into the registry. I have received a signed copy of this consent form. I voluntarily consent to participate in the Alpha 1 Canadian Registry.

The registry has my permission to inform my regular doctor and/or respirologist about my being in the study (please initial on line) YES _____ NO _____

Participant's name _____

Participant's signature _____ Date _____

Parent or Legal Guardian's name (for minors) _____

Parent or Legal Guardian's signature _____

Witness's name _____

Witness's signature _____

Principal Investigator: Dr. Kenneth Chapman _____ Date _____