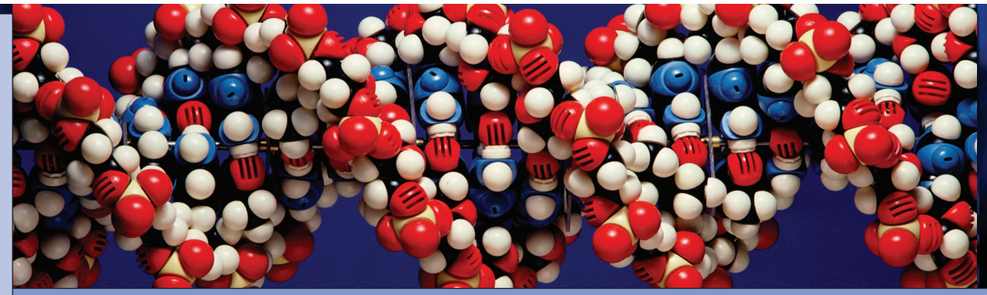

Taking Part in Alpha-1 Research

ALPHA-1 RESEARCH

BE PART OF FINDING A CURE

ALPHA-1 RESEARCH

What is done with my private information obtained during the study – who has access to it?

- The informed consent form that you signed to be part of the study should say what is done with your private information. The form should explain how it is stored (often in a password protected computer or locked file cabinet with limited access). It should state who has access to your private information (usually the study staff and study sponsors), how long it is kept and when it is destroyed, and whether it will be used in any papers or presentations.
- There is a Federal law in place, the Health Insurance Portability and Accountability Act of 1996 (HIPAA), which provides safeguards to keep your health information private.

Where can I find out about current and upcoming Alpha-1 Antitrypsin Deficiency-related studies?

Alpha-1 Foundation website:
www.alphaone.org/
Alpha-1 Association website:
www.alpha1.org/main.html

One way to be sure you are notified of and are given the chance to take part in clinical trials is to join the Alpha-1 Research Registry by calling 877-886-2383, sending an email to alphaone@musc.edu or visiting the Registry website at www.alphaoneregistry.org

What should I expect when I enroll in a research study?

- The first thing you will do when you decide to enroll in a study is provide your informed consent to be in the study. During this process, staff will explain the details of the study, answer your questions, address any of your concerns and then have you read and sign a consent form. The consent form should contain the following: reason for the study, study sponsor, rules that guide who can or cannot be part of the study, current knowledge about new drugs or treatments, risks and benefits that may be involved in taking part in the study, other options that you may choose instead of being in the study and expected study activities. The consent form should also state who is responsible for the costs related to your being in the study. It should also provide the following: a statement about keeping your identity private, a statement about taking part in the study being up to you and your right to drop out of the study at any time. The consent form should also list contact names and phone numbers that you may call to learn more about your rights in taking part in the study[6].
- Once you have read and signed the consent form, you are enrolled in the study and you may proceed with the outlined study activities. These may include taking part in: interviews or surveys, medical tests, educational sessions, or taking certain treatments, etc.
- Be aware that your taking part in the study may end for a number of reasons. Reasons may be that you no longer want to take part in the study, you have finished the treatment, or the treatment does not appear to work for you. The study may also be stopped because the treatment was shown to work well or to be harmful. It may also be stopped due to breaches of the study protocol.

1 American Thoracic Society/European Respiratory Statement: Standards for the Diagnosis and Management of Individuals with Alpha-1 Antitrypsin Deficiency (2003). American Journal of Respiratory and Critical Care Medicine, 168, 818-900.
2 U.S. Department of Health and Human Services. HHS fact sheet, Protecting human research subjects. (June 6, 2000). Available at http://www.hhs.gov/news/press/2000pres/20000606a.html. Accessed July 2003.
3 National Institutes of Health, Office of Human Subjects Research. Guidelines for conduct of research involving human subjects at the National Institutes of Health. (1995). Available at http://www.nihtraining.com/ohsrsite/guidelines/graybook.html. Accessed July 2003.
4 U.S. Department of Health and Human Services Public Health Service. Grant Application PHS 398 forms (updated May 2001; revised September 2003). Available at http://grants1.nih.gov/grants/funding/phs398/phs398.html
5 National Institutes of Health: The Belmont Report – Ethical Principles and Guidelines for the Protection of Human Subjects. The National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research. (April, 1979). Available at http://ohsr.od.nih.gov/mpa/belmont.php3. Accessed July, 2003.
6 American Cancer Society: Clinical trials: What you need to know. Available at http://www.cancer.org/docroot/ETO/content/ETO_6_3_Clinical_Trials_-_Patient_Participation.asp. Accessed July, 2003.

About the Alpha-1 Foundation

The Alpha-1 Foundation is a not-for-profit organization dedicated to providing the leadership and resources that will result in increased research, improved health, worldwide detection, and a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1). The leading experts in the field of Alpha-1 research are working with the Foundation through their participation on the Board of Directors, as members of the Medical And Scientific Advisory Committee (MASAC), directing Clinical Resource Centers, as members of Working Groups, or as participants at Foundation-sponsored scientific conferences and workshops. These experts, together with respected members of the Alpha-1 medical, professional, scientific, and patient communities are teaming up with the Alpha-1 Foundation to identify the most critical areas of research and support the development of new therapies. Importantly, the Alpha-1 Foundation has also formed collaborative relationships with government and industry to promote needed research and create awareness of this genetic disorder.

Additional information on the Foundation and its research programs is available at www.alphaone.org.

Alpha-1 Foundation

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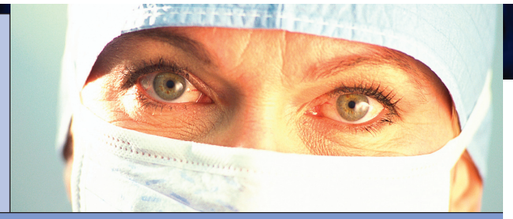
....ALPHA-1



TAKING PART IN ALPHA-1 RESEARCH

Introduction

- Intense research on Alpha1-Antitrypsin (AAT) deficiency over the past four decades has advanced our knowledge about the condition. Advances include understanding the genetics, the related emphysema and liver disease, and treatments[1].
- Increased research is necessary to find a cure for AAT deficiency and to improve current treatments. Research is also needed to find better means of detecting the condition, and to better understand what causes emphysema and/or liver disease in only some people with the condition. It will help to fully understand (and to improve) the social and legal implications of having the condition.

Overview of human research studies

- Great progress in disease prevention and treatment has been made through research. One of the most crucial parts of research is human subjects (people) taking part in it.[2]
- The National Institutes of Health (NIH) defines human subjects as those living people from whom a researcher collects data or private information[3].
- Research refers to a step-by-step approach (often called a "study") used to create or add to current knowledge. A clinical trial is a study of people that is designed to answer questions about what does and does not work in medicine and other fields of research. Trials may answer questions such as, does a new drug or treatment work? Does a new method of using known drugs or treatments work? Does a new method of changing people's health behavior work?
- The purpose of clinical trials is to find out whether or not new drugs or treatments, methods of taking drugs or treatments or methods for changing behavior really work and whether or not they are safe to use. Trials may go through four phases[4]:

  ✔ **Phase 1** trials are performed to find out the safety of a new drug or behavior treatment. They are helpful in finding out the safe dose and any side effects of a drug. They are performed in a small group (20 – 80) of people.

  ✔ **Phase 2** trials are performed in a larger group of people (hundreds). They find out whether or not a given treatment works. It's safety is studied further.

  ✔ **Phase 3** trials are performed in still larger groups of people (hundreds to thousands). The purpose of these trials is to study whether or not a given treatment should be used in the general population. In this phase, the treatment is compared to other standard or experimental treatments and is closely studied for any harmful effects.

  ✔ **Phase 4** trials take place after the treatment has been approved. Their purpose is to study how well the approved treatment works in the general population. Any harmful effects that may be due to widespread use of the treatment are closely studied.

- It is important to note that taking part in a research study is your choice. It is only up to you.

Who sponsors research studies?

- The U.S. Department of Health and Human Services, which includes the National Institutes of Health
- Drug companies
- Biotechnology companies
- Not-for-profit organizations

Who can take part in research studies?

- Any person who is eligible (meets the rules for study entry) and who is able to provide informed consent may take part in a study. Being able to provide informed consent means that you understand the research study and what taking part in it would involve. You also understand what risks may be involved in the study and what benefits you may receive from taking part in it. You can weigh the risks and benefits against each other. Also, it is your choice to be in the study.
- Each study has certain rules that guide who can and cannot be enrolled. The rules are used to ensure that the research questions that the study was designed to answer can, in fact, be answered. Some of the rules that may guide study entry concern:

  ✔ Age and gender

  ✔ Family history of a given disease or condition

  ✔ Stage of a given disease or condition

  ✔ Treatments that a person must have had before…or must not have had

  ✔ The length of time since the last treatment

  ✔ Results of certain lab tests

  ✔ Taking part in certain health behaviors like smoking 1 pack of cigarettes per day or exercising 3-5 times per week

  ✔ Using certain drugs or treatments

Is it safe to take part in research studies?

- There are many safeguards in place to look out for the welfare of people taking part in research.
- *The Belmont Report – Ethical Principles and Guidelines for the Protection of Human Subjects* provides the basis for the current laws guiding human subject research. This report formed and outlined 3 ethical principles (rules) that apply to all research with people[5]:

  1. **Respect:** this rule acknowledges dignity and independence. It calls for special safeguards for people who may be vulnerable or who have little independence. It also demands informed consent from all people before they take part in any research study.
  2. **Beneficence (quality of being kind):** this rule demands that expected benefits of a study outweigh the harms that may occur. It also demands that those who conduct research assure that any risks are minimized. This rule also demands that researchers disclose if there are ways to get the benefits other than being in the study.
  3. **Justice:** this rule demands that people taking part in research studies be treated fairly. This means that they should be chosen with care to ensure that certain people such as prisoners, the elderly, or people with financial problems are not consistently chosen or denied the right to be part of the study unless there are valid reasons for doing so.

- Institutional Review Boards (IRB's) are required to review all research studies that involve people. An IRB is a group of people in charge of guarding the welfare of people taking part in studies and for making sure that studies follow federal rules. The groups are often made up of doctors, researchers and lay people. Any researcher who wants to conduct a study must submit the study protocol (description and plan of action) for IRB review. The IRB then decides whether or not the benefits that you may receive from taking part in the study outweigh the risks that may be involved and that the risks have been minimized. The IRB also reviews the study's informed consent form to make sure that it is correct, easy to understand, and explains the risks and benefits as well as options other than taking part in the study[2].

Why should I think about taking part in a research study?

- Taking part in Alpha-1 research studies helps to advance science and knowledge about the condition. Progress made can lead to better treatments and/or even a cure.
- Being in a study may give you access to new treatments that are not otherwise able to be used by people with Alpha-1.
- Taking part in Alpha-1 research studies may help you and others who have the same condition.

How should I decide whether or not to take part in a research study?

- Speak to the study Principal Investigator (person in charge of the study) or other research staff to learn about the study. You should find out what the study needs from you in terms of time, effort, activities, medical tests, etc. Also, find out the risks that may be involved and the benefits that you may receive, treatments other than those proposed in the study and possible compensation for being in the study.
- Weigh the risks and benefits of being in the study.
- Talk with your doctor, family and friends to find out what they think about you taking part in a study. Their points of view may be helpful to you when you decide whether or not to participate.
- Again, only you can decide whether or not you will take part in a study.