
MEDICAL RECORD	MINOR PATIENT'S ASSENT TO PARTICIPATE IN A CLINICAL RESEARCH STUDY • Attach to NIH-2514-2, Consent to Participate in a Clinical Research Study
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INSTITUTE: National Institute of Environmental Health Sciences

STUDY NUMBER: 94-E-0165 PRINCIPAL INVESTIGATOR: Lisa G. Rider, M.D.

STUDY TITLE: Studies in the Natural History and Pathogenesis of Childhood-Onset and Adult-Onset Idiopathic Inflammatory Myopathies

Continuing Review Approved by the IRB on 09/28/10

Amendment Approved by the IRB on 09/28/10 (S)

Date Posted to Web: 11/03/10

Minor Patient

You are being asked to participate in a study to learn more about your muscle weakness and illness. We are trying to learn more about what may cause the weakness. This study may provide information to your doctor which may help him/her plan the best treatment for your disease. This study may not help you directly, but by participating in the study, others with similar problems may be helped.

Several doctors and other people who take care of sick people in the hospital will ask you questions and examine you. We would like you to give us a sample of your urine and also take a blood sample from your arm. It will hurt a little when we take the blood out and sometimes there will be a bruise afterwards.

Some blood samples will be used for isolating and studying DNA (genetic material), and some of your DNA, blood and urine will be stored for future study. Some blood samples will be used for isolating and studying DNA (your genes), and some of your DNA, blood and urine will be stored for future study. We will compare the spellings in your genes and from other people with myositis to the genes from people without myositis to find any differences. We plan to study many different parts of your DNA. Information about your genes and the genes of other myositis patients enrolled in this study will be placed together into an Internet database that does not identify you personally. But, your specific genetic information may be available to scientists who have approval to look at the DNA results and ask specific research questions. We will provide any clinically-relevant results of all studies to your referring physician. We will provide any clinically-relevant results of all studies to your referring physician.

Some of your blood and urine will be frozen with a code number but not your name on the label and may be sent to other researchers in the future for other studies.

Teenage girls may have a test to tell if you are pregnant. If this test shows that you are pregnant, we will tell you right away. We will also tell your parents of the results of this test after we tell you. If you do not want us to tell your parents the result of your pregnancy test, please tell the principal investigator of the study right now. If you are pregnant, you will not be able to be in this study.

For patients participating in this study through their local health care provider, if you agree to participate in this study, you will be enrolled during a visit to your local health care provider. This evaluation will involve obtaining your medical records for our review, answering questions about your medical history, completing written questionnaires, undergoing a physical examination and having your physician complete a questionnaire about your illness, and donating blood, urine and possibly other clinical specimens, including muscle biopsy, calcinosis samples and other tissues, for research purposes. All this information and your blood and urine samples, as well as tissue samples, will be returned to the NIH

PATIENT IDENTIFICATION

MINOR PATIENT'S ASSENT TO PARTICIPATE IN A CLINICAL RESEARCH STUDY

NIH-2514-2 (10-09)

P.A.: 09-25-0099

File in Section 4: Protocol Consent (3)

MEDICAL RECORD**CONTINUATION SHEET for either:**

NIH 2514-1, Consent to Participate in A Clinical Research Study

NIH 2514-2, Minor Patient's Assent to Participate In A Clinical Research Study

STUDY NUMBER: 94-E-0165

CONTINUATION: page 2 of 5 pages

or its designees for processing using a kit which we have sent to your local health care provider. When we receive this kit, you and your local health care provider will each receive \$100 to compensate you for your time and expenses. In order to process your samples at the NIH, you will first complete and sign a form, which gives us your contact information and permission to do these studies and assigns you an NIH number.

You may also be asked to undergo some or all of the following tests to see how other parts of your body function. These may be helpful to your doctors to see how sick you are.

1. Physical and occupational therapists may check how strong your muscles are and ask you questions about your ability to perform certain tasks.
2. You may be asked to swallow liquid (barium) or to have studies to check your speaking and swallowing.
3. You may be asked to blow into a machine to check your breathing and an x-ray picture of your chest may be made to check your lungs.
4. Stickers may be placed on your chest to measure the electrical activity in your heart and if necessary, other tests to measure your heart function may be done.
5. You may be asked to lie still in a large machine so that inflammation in the muscle can be measured by your doctors. This test is called a magnetic resonance imaging scan (MRI). This will not hurt, but you will have to lie very still in a machine with a small space. If you feel anxious about this, let us know and we will not do this research test. We may also do another MRI to examine the inflammation in all of the muscles throughout your body (called whole-body MRI), and if indicated, do another MRI test to examine the inflammation in the fat tissue under your skin. Each of these would be separate tests of about 30- 60 minutes each.
6. As part of your magnetic resonance imaging scan, you may be asked to perform some exercise. This may consist of you climbing up and down one stair repetitively OR taking a walk down the hall. It is important while doing this exercise that you tell the doctors when you feel tired and unable to do any more exercise. At the end of the exercise, you can choose if you want to have a blood test, which would be helpful to your doctors. You will be asked to rest after the exercise and then undergo some additional MRI scans. The entire test may take up to 2 hours. You will also be asked to tell us what types of activities and sports you did in the week before you came to NIH.
7. If you are older than 8 years of age and are able to ride an exercise bicycle, and if your doctors think that it is OK for you to undergo the exercise testing, you have the option to participate in a bicycle aerobic exercise test. In this test, the amount of effort on the bicycle will be gradually increased until you become tired, short of breath, or develop chest discomfort. You may ask to stop the testing at any time. During the test, your blood pressure, heart rate, heart waves (electrocardiogram) and oxygen uptake will be monitored. During the test, there is a possibility that certain changes such as muscle strain injuries, abnormal blood pressure, or fainting disorders of heart beat (too rapid, too slow or ineffective) may occur. You will also be asked to tell us what types of activities and sports you did in the week before you came to NIH. This exercise study may be useful to your doctors to advise you about how much exercise you can tolerate. On your return to NIH, you may be asked to repeat this test.
8. If your doctors determine your illness is affecting the way you speak (your voice quality), you may be asked to undergo some additional tests. This will include a voice analysis (having a computer analyze a tape recording of your voice); an ultrasound study of your voice box (vocal cords) to look for the disease (inflammation); and an indirect

PATIENT IDENTIFICATION**CONTINUATION SHEET for either:**

NIH-2514-1 (10-84)

NIH-2514-2 (10-84)

P.A.: 09-25-0099

STUDY NUMBER: 94-E-0165

CONTINUATION: page 3 of 5 pages

laryngoscopy. In this last test, a doctor looks at your voice box using a small, rigid scope (similar to a strong tongue depressor) and video camera. You may feel some discomfort or itchiness in your throat when the scope goes through your mouth, but this is often helped by spraying a numbing medicine into the back of your throat.

9. You will have some blood testing to check on your sugar and fat metabolism, and if you are entering puberty, to check on the hormone levels in your blood; for this testing you would have nothing to eat after a bedtime snack until after the blood tests are obtained the next morning. An endocrinologist will see you during your visit. If they think it is appropriate, a x-ray of your hand will be taken to see how your bones are growing (bone age). If they feel you have lost body fat or that your sugar metabolism seems abnormal, additional 2 hour tests of your blood sugar metabolism would be obtained (oral glucose tolerance test). For this special sugar metabolism testing, an IV catheter would be placed using a topical numbing medicine, so that you will not have to have repeated needle sticks. They also may recommend you have an ultrasound of your liver or ovaries (pictures obtained by sound waves) to check for abnormalities in those organs. If you take prednisone or a similar medicine, a blood level of prednisone may be tested one hour after the morning dose, along with blood tests related to its breakdown in the body. Some of these tests, not all, will be repeated at follow-up evaluations.

10. Standardized Muscle Strength Testing: If you are at least 5 years of age, a physical therapist would like to see how strong your muscles are using a machine to accurately check your strength. Here, you will be asked to move your arm or leg as strong as you can against a machine with weights. If you are at least 7 years of age, a muscle power test will also be obtained to see how much work your muscles can perform while you move a weight against different amounts of tension as rapidly as possible. The physiotherapist or physiatrist will also check the amount of distance you can walk in a 6 minute period of time, As part of this testing, you and your parents will be asked to complete a questionnaire regarding your activities during the week prior to your visit to NIH, and you will be asked general questions about your overall energy level and well being. With the muscle power test, there is a small risk of experiencing muscle soreness within 48 hours of the test. If you have problems with your heart or high blood pressure, you would be excluded from the muscle power test.

11. Muscle Ultrasound: This study produces a picture of your thigh muscles using ultrasound or sound waves. You will be asked to lie very still for the test, which may take less than 60 minutes, and if you are at least 5 years of age, you will be asked to also tighten or contract your thigh muscles. The thigh will be measured while lying down, and a pen mark will be drawn on the leg muscle to mark the landmarks of the muscle. A small ultrasound transducer (a wand like tube) and a water gel, which may feel cold or wet, will be placed on the skin of the thigh and moved slowly along the marked line. You may be asked to repeatedly climb a small step until you feel tired or for no more than 10 minutes. Sound wave pictures will be taken while you are lying still, when you tighten your thigh muscles, and after the stair stepping exercise. There are no known risks to this procedure.

12. Blood flow test: If you are at least 7 years of age and have undergone certain blood tests to evaluate the blood vessels, then a test of the blood vessel function may also be performed. While lying down, an ultrasound machine will take a picture of your large blood vessel in the arm. A blood pressure cuff will then be tightened on your arm to try to make the blood vessel shrink in size. The blood pressure cuff will then be let loose, and then the rush of blood will be examined by the ultrasound machine. During this test, you may experience some tingling or discomfort in the arm or leg, which will stop quickly after the blood pressure cuff is loosened. You will be watched carefully during and after this test.

13. Skin Evaluation: An evaluation of how the myositis is affecting your skin will be made by the doctors that see you. If the skin doctor finds that you have certain rashes that may require more medicine, pictures may be taken of the rashes and a skin biopsy may be advised. Photographs of skin rashes, nail fold capillaries and other findings are taken

STUDY NUMBER: 94-E-0165

CONTINUATION: page 4 of 5 pages

for a number of reasons, including for research, to teach other doctors about myositis, and for research publications, including in medical journals and on the internet. The possibility exists that you may be identifiable in some of the photographs. For the skin biopsy, the skin is numbed with a numbing cream or by injecting numbing medicine under the skin.

Following this, a clean, tiny blade is used to cut a very small hole in the skin. It is very uncommon, but you can develop an allergic reaction to the numbing medicine. With the skin biopsy, there is a slight risk of infection or bleeding at the site, pain when the needle is placed into the skin, and the possibility of a very small scar at the site of biopsy and itching during healing.

Other tests not listed may be recommended to help us plan the best treatment for you. For all of the tests, you and your parent or guardian will be consulted in advance, given full information, and asked to approve them. If you or your parent or guardian does not approve of any of these tests, they will not be done. You may benefit directly from participating in this study: we will share the results of this very thorough assessment of your disease and how it affects all aspects of your body with the doctor who takes care of you. This information may help them plan the most appropriate treatments for you. You will **not** receive treatment for your myositis at NIH at this time or in the future. The doctors you meet at NIH will **not** become your regular doctors. We will, however, work with your myositis doctor and continue to make treatment recommendations to that doctor.

We plan to re-contact your parents or guardian and your regular doctor at one time in the future to obtain follow-up information about what additional medicines you received for your myositis and how you responded to them, whether you developed any new problems related to your myositis, and how you are doing with your myositis since the last time we saw you at NIH.

When you become 18 years of age, you may re-contact us to sign an adult consent form or even choose instead to withdraw your samples and data from the study and stop doing the study. You are not required to sign an adult consent form when you become 18 years old to remain enrolled in the study.

COMPENSATION

You will receive \$100 for the time and inconvenience involved in participation, upon your initial enrollment into the study and each time you return to the NIH to repeat some of the research tests. If you enroll through your local health care provider, you and your local health care provider would each receive \$100 for the time and inconvenience involved in participation. Compensation would not be made if you are enrolling primarily in another NIH research study.

CONFLICT OF INTEREST STATEMENT

The National Institutes of Health reviews NIH employees at least yearly for conflicts of interest. The following link contains details on this process <http://ethics.od.nih.gov/forms/Protocol-Review-Guide.pdf>. You may ask your research team for additional information or a copy of the Protocol Review Guide.

This protocol has investigator(s) who are not NIH employees. They are expected to comply with their Institution's conflict of interest policies.

STUDY NUMBER: 94-E-0165

CONTINUATION: page 5 of 5 pages

I have had this study explained to me in a way that I understand, and I have had the chance to ask questions.
I agree to take part in this study.

Signature of Minor Patient: _____ Date: _____

Print Name: _____

Signature of Investigator: _____ Date: _____

Print Name: _____