IUPUI and CLARIAN INFORMED CONSENT STATEMENT
INTERNATIONAL SPINAL MUSCULAR ATROPHY PATIENT REGISTRY

Study Number: 0203-03

PURPOSE OF REGISTRY

The Department of Medical and Molecular Genetics, Indiana University School of Medicine, is collecting and maintaining information on individuals affected by spinal muscular atrophy (SMA). The information that is being collected includes demographic information (e.g., name, date of birth, address) and medical history information.

PROCEDURE FOR PARTICIPATING IN THE REGISTRY

You are invited to participate in a research registry for individuals diagnosed with SMA. If you agree to participate, you will be one of approximately 3000 individuals who will be participating in this registry. Please read this informed consent statement carefully. Please make certain to have any questions or concerns answered. You need to sign this informed consent statement and then complete the patient questionnaire. The completed informed consent statement and patient questionnaire are to be returned to the Department of Medical and Molecular Genetics by mail (stamped business reply envelopes will be provided).

When a researcher is interested in contacting individuals affected by SMA, he/she will be required to submit a protocol, which is a detailed description of the research study, to the Medical Advisory Board and Scientific Advisory Board of Families of SMA. The Medical Advisory Board and Scientific Advisory Board of Families of SMA are responsible for reviewing the protocol to make sure that it is a well-planned study, will not harm participants and will provide new information relating to SMA.

The researcher is also required to submit the protocol to his/her institutional review board. The Federal Government requires every study involving people to be reviewed by a group of individuals, referred to as an institutional review board (IRB). Institutional review boards are responsible for making sure the study is well planned, that risks to participants are as small as possible, that the informed consent statement is accurate and that the participants' confidentiality is protected. Most universities and medical schools involved in research utilizing people are required to have an IRB. Research studies involving people need to be approved by the IRB before the study can begin and are reviewed by the IRB each year.

Once a protocol has obtained IRB approval and approval of the Medical Advisory Board and Scientific Advisory Board of Families of SMA, the Department of Medical and Molecular Genetics will send a letter summarizing this researcher's study to you and to other individuals participating in the International Spinal Muscular Atrophy Patient Registry. If you are interested in learning more about the study and/or in participating in the study, you will be asked to reply to the Department of Medical and Molecular Genetics with written permission allowing your name and contact information (phone number and address) to be released to the researcher. Your name and contact information will <u>never</u> be released to researchers or anyone without your written permission.

BENEFITS
Although you will not receive any direct benefit from participation in the International Spinal Muscular Atrophy Patient Registry, your participation may provide additional information regarding SMA. You will also learn of research studies in SMA, which may include research projects looking at possible treatments for SMA. By learning of these studies, you will have the option of participating in these research studies.

RISKS
The risks from participating in the International Spinal Muscular Atrophy Research Registry are minimal. Your demographic and medical history will be entered into a secure computer system in the Department of Medical and Molecular Genetics, Indiana University School of Medicine. There is a slight risk that there could be a breach of the security of this computer system resulting in the access of information. Safeguards are in place to minimize this risk.

ALTERNATIVES TO TAKING PART IN THE STUDY:
Instead of being in the study, you may choose not to participate. Declining to participate does not jeopardize your medical care.

CONFIDENTIALITY
If you choose to participate in the International Spinal Muscular Atrophy Patient Registry, all information that could identify you will be protected in accordance with very strict safeguards. It should be noted that the IUPUI and Clarian Institutional Review Board (IRB) or its designees may review research records.

By signing this document, you give permission to use other information, which will not identify you by name, for medical and scientific purposes that include research and/or publication. Your name will never be involved in any report or publication.

VOLUNTARY PARTICIPATION
Your participation in this project is totally voluntary. You can ask to discontinue your participation at any time without jeopardizing the quality of your medical care.

COMPENSATION
There is no monetary compensation for your participation in this project and no cost to you. The participation of individuals affected by SMA in research studies is invaluable in the quest for treatments and cures for this disease.

PEOPLE TO CONTACT
If you have any questions regarding this registry, you can call Dr. P. Michael Conneally at (317) 274-2218 between 8:00 AM and 5:00 PM, Monday through Friday. For questions about the rights of research subjects or any study-related injury, you may call a patient representative who is not associated with this research at (317) 274-8265.

SUBJECT'S CONSENT

By signing this statement I am only agreeing to receive letters from the Department of Medical and Molecular Genetics describing research studies for which I may be eligible. For each study I decide to participate in, I will need to read and sign a separate informed consent statement, which will be provided by the researcher.

I have read the above statements and am willing to participate in the International Spinal Muscular Atrophy Patient Registry.

SUBJECTS SIGNATURE: _____ Date: _____
(must be dated by the subject)

IF SUBJECT IS A MINOR:
SIGNATURE OF PARENT: _____ Date: _____

SIGNATURE OF PARENT: _____ Date: _____

SIGNATURE OF CHILD: _____ Date: _____
(AGE 7 AND ABOVE)

SIGNATURE OF WITNESS: _____ Date: _____
(May be investigator or person obtaining consent.)

IRB Approval Date: JAN 13, 2004

Continuing Review Due: JAN 13, 2005