

NIAMS IRPartners

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A newsletter for patients of the Intramural Research Program (IRP), National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)

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All About Clinical Studies: An Interview

By Kelli Carrington

Have you ever wondered how pain relievers, X-ray devices and joint replacement procedures came to be? Clinical studies played a major role in their development. It can take many years to get from the concept stage to the testing and approval stages for these medical wonders, and clinical studies can be the most crucial part of development. Why? Because in these studies we find out if a new drug, screening device or surgical procedure actually performs as it was intended.

IRPartners recently interviewed Dorothy Cirelli (“DC” in the interview that follows), chief of the Patient Recruitment and Referral Center at the NIH’s Warren Grant Magnuson Clinical Center, to get her answers to some of the following questions NIH research participants frequently ask.

IRPartners: Why are clinical studies important?

DC: Clinical studies are a way for researchers to learn more about disease in people. These studies look at causes, who is at risk, and how best to prevent, diagnose and treat diseases. Many of today’s most effective standard treatments, like painkillers, are based on previous study results.



Dr. Raphaela Goldbach-Mansky (left) and nurse Mildred Wilson review X-rays of a patient in an NIAMS clinical trial.

In addition to helping researchers gain knowledge about disease, these studies may benefit the participant. For example, a test treatment or procedure may improve the participant’s health. If the research treatment or procedure is effective, the person can benefit long before the treatment is available from a doctor. Benefits are not guaranteed, however, so participants must understand that the clinical study may not improve their health.

From the Scientific Director . . .

We are pleased to welcome you to the winter issue of *IRPartners*. In our feature story, Ms. Dorothy Cirelli answers some of the questions we often get about clinical trials, such as: Why do we need them? What should patients consider before participating in one? How is patient safety assured?



We'll also meet the two NIAMS patient liaisons, helpful assistants who escort patients visiting the NIH from our Community Health Center. This issue also includes an article about overuse syndromes affecting performing artists.

In addition, we'll tell you about some muscle research being conducted at NIAMS that's important to our understanding of the human body and muscle diseases.

We hope you find this issue both informative and enjoyable. Please be sure to catch our spring issue, in which we'll cover the Children's Inn, interview an intramural scientist and update you on more NIAMS research.

*Peter E. Lipsky, M.D., Scientific Director
National Institute of Arthritis and Musculoskeletal
and Skin Diseases, National Institutes of Health*

CLINICAL STUDIES, *continued from page 1*

IRPartners: What are the different types of clinical studies?

DC: There are basically four different types of clinical studies: **prevention** studies look at lifestyle changes or drugs that may help prevent disease; **diagnostic** studies examine ways of detecting disease or finding out more about a disease; **natural history** studies observe the course of disease to better understand its development and impact; and **treatment** studies test new drugs, new combinations of existing drugs or other methods for treating disease.

IRPartners: Are there risks involved in participating in clinical studies?

DC: While researchers do their best to anticipate and prepare for any risks or side effects, clinical studies can carry unknown dangers as well as hoped-for benefits. Many medical treatments have known side effects, but if researchers are testing new treatments, they don't always know the risks ahead of time. It's extremely important for research participants to fully understand the known risks and benefits before they participate in clinical studies.

IRPartners: How is the safety of research participants at the NIH clinical center protected?

DC: There are several safeguards in place to protect research participants at the NIH. First, there's a protocol review, a process where an institutional review board (IRB)—consisting of doctors, nurses,

Questions To Consider Before Joining a Study

- What's the purpose of the study?
- What is required of me?
- Will the study benefit me or others?
- Are there risks? If so, what are they and what are the chances that they will occur?
- What discomforts are involved?
- How long will the study last?
- What will happen if I decide to leave the study?

scientists, medical ethicists, clergy and lay people—review a formal set of rules and procedures for a study to ensure its safety. To be approved by the IRB, the study must explore medically important questions in a scientific and responsible manner without posing undue risk to the participant.

Second, all research participants receive and sign a document called "Consent to Participate in a Clinical Research Study." This document, which is explained by a member of the research team, describes the study in detail. Research participants sign the consent document only after fully understanding the nature of the study and agreeing to the commitment.

Lillian Cosme, patient liaison

In October 2001, Lillian Cosme was a student working part time as an administrative assistant at the NIAMS when the NIAMS Health Partnership Program (HPP) team asked her if she'd like to join the program as a patient liaison. The job involved escorting patients referred from the HPP's Community Health Center (CHC) in Washington, D.C., to their appointments at the Warren G. Magnuson Clinical Center on the NIH campus in Bethesda, Md. Patients come to the clinical center to take advantage of the services and technology the CHC can't provide. For example, they come for X-rays, rehabilitation or to fill prescriptions at the pharmacy. Ms. Cosme says, "I was thrilled to become part of the HPP." She began assisting patients one month later.

A 1999 graduate of Wheaton High School in Wheaton, Md., Ms. Cosme began college as a business major at Barry University in Miami. In the spring of 2001, she returned home to study criminal justice at the University of Maryland in College Park. Unsure about where that degree may lead, Ms. Cosme says, "I want a career where I can meet people, maybe in social work, but I'm not sure yet. I know I want to help people." She also knows she wants a career where she can leave the office behind and go out into the field.

Ms. Cosme can do just that as a NIAMS patient liaison. A typical visit begins when she greets a patient in the clinical center south lobby. The majority of patients she assists speak Spanish, so she puts her fluent Spanish to good use. She escorts the patients to their appointments in the clinical center. She also arranges for interpreters to assist patients during their consultations or treatments so she can spend her time on more practical details, like getting meal vouchers, snacks or wheelchairs, if necessary. "The clinical center can be an overwhelming place. Patients are more comfortable knowing we know the staff and where they're going," Ms. Cosme says. "Just a smile at the end of their visit means it went well. That makes me feel good about what I do."

Many patients are curious about the clinical center and what goes on there. Ms. Cosme explains it is different from other hospitals because of its research focus. She also explains that the NIAMS is only one of the many institutes of the NIH doing research there.

Ms. Cosme believes the patient liaison program will likely expand to accommodate more patients from



Lillian Cosme (left) and Nicole Schuett greet Pete Patterson at the entrance to the NIH Clinical Center.

the CHC, which just celebrated its first anniversary. She says, "It's nice to be part of a work in progress like the CHC, which is helping so many people. The first anniversary was great, but I'd like to see where the CHC is in 10 years."

Nicole Schuett, patient liaison

When Nicole Schuett visited the NIH for a seminar as a junior in high school, she was not predicting a career here. In fact, she says, "I decided I would never work here. My first impression of NIH was that all of the research was conducted in a lab setting with no personal contact with the community."

Today she's using her bilingual skills and her degree in community health education from the University of Maryland as a patient liaison in the NIAMS Health Partnership Program. So much for never working for the NIH!

Ironically, Ms. Schuett's interest in community health stemmed from the very same seminar, a National Hispanic Youth Initiative for students interested in biomedical research and health policy, that turned her off to the NIH. During the program, Jose Carneiro, Ed.D., the Director of the U.S. Department of Health and Human Services' Office of Minority Health Resource Center, spoke to the group, and Ms. Schuett was so impressed she thought maybe she would like a job like his. "He had been to 60 countries overseeing and implementing community health programs. Everyone else was focusing on premed and science, but I really liked the community aspect of his work. It was the first time I had heard about the public health arena," Ms. Schuett says.

NICOLE SCHUETT, *continued on page 4*

Ms. Schuett is working on her own master's degree in public health at George Washington University in Washington, D.C. In the future, she would like to work on international health programs, either by traveling or by working on the U.S.-based operations of international programs.

About being a patient liaison, Ms. Schuett says, "I love it. It's rewarding to be there for patients. I try to lift their spirits so they are not so stressed when they come here. They already have diseases that add so much stress to their lives, or they're worried about finding out they might have one."

Did You Know?...

Performing artists such as dancers and musicians are vulnerable to certain injuries known broadly as overuse syndromes. Dancers often develop problems in their backs and lower limbs, such as sprains, aches, fractures and tendinitis. Ballet dancers especially may stress the ankles when they go *en pointe*; that is, dance on the very tips of their toes with special shoes. Musicians playing a piece with difficult fingering may overstress the joints and ligaments of the arms and hands. In particular, overuse of the wrist may cause swelling of the ligament in the wrist, which then irritates the nerve beneath. This particular problem is known as carpal tunnel syndrome.

When muscles contract to hold certain positions for a long time, they need a chance to relax. Joints and ligaments also have to rest. When muscles, tendons, ligaments and joints are stressed, they respond with inflammation and pain. Poor posture or technique may cause or contribute to these problems.

Many performers don't go to see a physician until the pain interferes with their performance. They may try to keep practicing and performing, working through the pain or discomfort. Health professionals agree that's a bad idea. When hurt, even if little or no pain exists, the body needs time to heal. Continuing to use injured muscles, ligaments and joints only makes the problem worse. Sometimes rest is all that's necessary. Other treatments may include compression using bandages or splints to prevent further swelling, applications of heat or cold to the affected area, physical or occupational therapy and medications. In extreme cases and depending on the injury, surgery

Ms. Schuett's parents grew up in Bolivia, and around her house, both Spanish and English are spoken. This bilingual background coupled with her interest in health education have been essential to Ms. Schuett's work as a patient liaison. "It can be difficult for anyone to find their way around the clinical center. Patients who don't speak English as a first language, or who do speak English but aren't accustomed to the health care system, feel overwhelmed and lost. I am there to listen, to help them stay calm, and to help them have a better day overall." ▲

may be required. Whatever treatment is necessary, performers shouldn't rush to get back to practice or rehearsal too soon. And when returning from a period of inactivity, they should gradually work up to their previous activity level.

If you dance or play an instrument, or if you are just learning, here's some advice:

- Make sure you're using proper posture and technique.
- Use the appropriate equipment, such as an instrument that is the right size for you, or proper shoes. If you play an instrument, a different chin rest or built-up keys may help prevent problems.
- Build up your endurance gradually to condition yourself for longer or more difficult pieces.
- Always stretch and warm up before you begin practicing or performing, and stretch and cool down when you're finished.
- Take frequent breaks to give your muscles a chance to relax and your joints and ligaments time to rest.

Contact Susan Bettendorf at 301-451-4831 or bettends@mail.nih.gov for a list of Web sites that dancers or musicians may find useful.

Facts and suggestions were derived from these helpful articles: "Musculoskeletal Injuries in Performing Artists" by Charlotte LoBuono in *Patient Care*, April 30, 2001; and "Musculoskeletal Problems in Musicians," a 1988 Center for Safety in the Arts publication by Miriam Daum. ▲

NIAMS Research Updates: Promising Muscle Cell Research

A team of researchers working in the NIAMS Laboratory of Muscle Biology have discovered a way to encourage developing muscle cells to become muscle fibers. These developing cells are immature cells that have not differentiated, which means they have not yet begun to change from their original form into a specialized form. The discovery may be used one day to correct problems that occur during muscle development. The finding may also help researchers encourage muscle growth and repair in adults who have diseases, such as muscular dystrophy, that cause the muscles to waste away.

Simona Iezzi, Ph.D., and Vittorio Sartorelli, M.D., along with colleagues in California and Italy, used human and mouse muscle cells in their experiments. The researchers have known for some time that a certain enzyme holds back muscle building by preventing the body from creating the necessary immature muscle cells. The investigators decided to try blocking the enzyme at different stages of a muscle cell's development.

The researchers wanted to see if they could get the immature cells to differentiate more efficiently; that is, to get more cells to mature into muscle fibers, instead of simply reproducing. Many of the immature cells would eventually turn into muscle fibers, but the researchers' goal was to see if they could encourage more of the cells to mature into muscle fibers than normally would. They exposed the dividing immature cells to an enzyme blocker and then put them into a solution that stimulates cells to change. Under these conditions, the researchers succeeded in getting the immature cells to turn into muscle fibers more efficiently.

Dr. Sartorelli's group continues to investigate how the immature cells are stimulated to become muscle fibers. The group will also see if the cells that have been encouraged to form muscle fibers will restore muscle function when transplanted into mice with muscular dystrophy. In addition, the researchers plan to expose muscle stem cells from adult mice to enzyme blockers to better understand stem cell biology and the blockers' potential use as therapeutic tools. ▲

CLINICAL STUDIES, *continued from page 2*

Clinical Trials Phases

Phase I studies look at how safe a new drug or vaccine is, and the best way and how often to give it. Because little is known about the possible risks and benefits, Phase I studies usually include a small number of participants who receive different doses of the drug.

Phase II studies involve a treatment for which the dose and side effects have been studied in a Phase I trial. Many more research participants are tested in this phase to define side effects and to learn the extent to which the treatment has the effect that will help the condition under

study. Some research participants may benefit from a Phase II study.

Phase III studies compare the new treatment against a standard treatment. (Phase II and III studies may compare the treatment to a placebo, such as a dummy pill, in certain cases or when no standard treatment is available.) Phase III studies may include hundreds or thousands of people, some of whom receive the new treatment and others the standard treatment. The studies are designed to find where the new drug fits in managing a particular condition.

Research participants can change their minds, however, and decide to withdraw from the study at any time. Participation is voluntary.

Third, the NIH provides a patient representative who helps ensure that research participants are informed of their rights and responsibilities, and that they

understand what the clinical center is, what it can offer and how it operates. Representatives also help to answer questions and resolve matters related to the study.

Finally, research participants at the clinical center are protected by a "Bill of Rights," a set of guidelines ensuring their care, privacy, confidentiality and access

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NIAMS Has Free Health Information

NIAMS has free health information (some in Spanish) available to the public, health professionals and organizations. Information is available on arthritis, lupus and other rheumatic diseases, skin disorders, joint problems and musculoskeletal diseases.

Contact the NIAMS at 1-877-22-NIAMS (free call), TTY: 301-565-2966. Check our Web site at www.niams.nih.gov/hi/. Many of our publications can be printed directly from our site.

Free information on osteoporosis, Paget's disease of bone, osteogenesis imperfecta, primary hyperparathyroidism, and other metabolic bone diseases and disorders is also available from the NIH Osteoporosis and Related Bone Diseases~National Resource Center (NIH ORBD~NRC). Contact the NIH ORBD~NRC at 1-800-624-BONE, TTY: 202-466-4315, or at www.osteoporosis.nih.gov. ▲

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to medical records. The Bill of Rights was developed by the American Hospital Association for use in all hospitals in the country.

IRPartners: What does it cost to participate in a clinical study?

DC: The NIH is a Federal agency supported by taxpayer dollars. All study-related medical care is provided at no cost to the participant. Clinical studies in other medical facilities may or may not charge patients for care.

IRPartners: How can I find out about joining a study?

DC: There are two ways to find out about studies at NIH. You can contact the clinical center by phone at 1-800-411-1222 or visit the clinical center's "Find a Study" Web site at <http://clinicalstudies.info.nih.gov>. For studies taking place at other research facilities across the country, you can visit the *ClinicalTrials.gov* Web site, which lists studies funded by the Federal government, drug companies and private and nonprofit organizations.

The clinical center is the Federal government's premier medical research hospital, investigating 22 areas of diseases, including arthritis and musculoskeletal and skin diseases. Made up of inpatient care units, outpatient clinics and research laboratories, the clinical center supports approximately 900 clinical studies and a vast number of basic research studies. More than 9,500 new patients visit the clinical center each year, and together with researchers, nurses and allied health-care professionals, the center helps to find causes, treatments, preventions and cures for diseases. ▲

NIAMS Community Health Center

The NIAMS has set up the NIAMS Community Health Center to help doctors and scientists understand the causes of rheumatic diseases and why many of these diseases occur more often and more severely in certain minority communities. With this information, we can find better ways to treat and prevent these diseases. There are no experimental treatments or medications being used at the CHC. Call 202-673-0000 for information.