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New Centers Boost Muscular Dystrophy Research

The National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), the National Institute of Neurological Disorders and Stroke (NINDS) and the National Institute of Child Health and Human Development (NICHD), parts of the National Institutes of Health (NIH), have funded three new cooperative research centers for the muscular dystrophies (MD), a group of genetic diseases that result in muscle weakness and wasting. The three institutes will fund the centers at up to $1 million in direct costs per center per year for 5 years. The NIH expects to fund up to two additional centers in future years.

The centers, principal investigators, funding agencies and planned research include:

$ The University of Pittsburgh, Joseph C. Glorioso, Ph.D. (NIAMS). Scientists here will study gene and stem cell therapies to treat muscle disease, in particular Duchenne muscular dystrophy. One project will attempt to deliver and engraft muscle stem cells
into diseased heart tissue without causing an immune response. Researchers will also use herpesvirus vectors in functional genomics studies to discover and characterize factors that guide stem cell maturation into muscle. In collaboration with scientists at the University of Missouri and Ohio State University, adeno-associated viral vectors will be exploited in preclinical studies in a dog MD model seeking the most effective ways to deliver gene therapy. Clinical disease outcomes will be carefully defined in patients with Duchenne and limb-girdle muscular dystrophies for the preparation of phase I gene therapy safety trials.

$ The University of Washington, Seattle, Jeffrey S. Chamberlain, Ph.D. (NICHD). This center will conduct studies to develop new gene therapies for Duchenne muscular dystrophy. Researchers have already achieved some success with viral vectors in delivering genes to mouse muscle. Investigators will use animal models to study several aspects of gene therapy to guide the development of human trials. This center will also conduct translational studies on several other types of muscular dystrophy to accelerate the development of new therapies. The researchers will also create a core resource to make available gene transfer vectors to other researchers.

$ The University of Rochester, New York, Richard T. Moxley, III, M.D. (NINDS). These researchers will look at skeletal muscle at cellular and molecular levels to examine which factors might contribute to problems in the muscular dystrophies, such as muscle wasting. The researchers will focus on myotonic and facioscapulohumeral muscular dystrophies.
The center will also maintain a repository of cell lines, antibodies, tissue and data about gene expression for sharing with other researchers.

The new centers are mandated in the Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001, or the MD-CARE Act (Public Law 107-84), passed by Congress. They will encompass basic, clinical and behavioral research projects. Centers will work individually and collaboratively, and will be overseen by a steering committee.

The Muscular Dystrophy Association is expected to issue an announcement of available supplements to provide up to $500,000 in total costs per center per year for 3 years for additional projects.

Muscular dystrophy is characterized by progressive weakness and degeneration of the skeletal muscles (voluntary muscles that control movement). It can also affect heart muscle, and in some muscular dystrophies can cause problems such as deafness, difficulty with thinking, and other systemic disturbances. Its major forms include myotonic, Duchenne, Becker, Emery-Dreifuss, congenital, limb-girdle and facioscapulohumeral. Duchenne is the most common form affecting children, and myotonic is the most common form affecting adults. MD can affect people of all ages. Although some forms first become apparent in infancy or childhood, others may not appear until middle age or later.
The NIAMS, NICHD and NINDS are part of the Department of Health and Human Services’ National Institutes of Health.

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The mission of the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) is to support research into the causes, treatment, and prevention of arthritis and musculoskeletal and skin diseases, the training of basic and clinical scientists to carry out this research, and the dissemination of information on research progress in these diseases.

The mission of the National Institute of Child Health and Human Development is to ensure that every person is born healthy and wanted, that women suffer no harmful effects from the reproductive process, and that all children have the chance to fulfill their potential for a healthy and productive life, free of disease or disability.

The mission of National Institute of Neurological Disorders and Stroke is to reduce the burden of neurological disease—a burden borne by every age group, by every segment of society, by people all over the world.