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International Muscular Dystrophy Community Forms Historic Research Partnership

Coalition could serve as model for other disorders

PARIS, Jan. 17, 2007 -- The world's leading muscular dystrophy organizations have launched a historic coalition that will pool knowledge and resources in an attempt to accelerate development of promising treatments and a cure for the most common childhood form of the disease.

At a meeting in Paris, the organizations - The Association Française contre les Myopathies (AFM) of France, the Muscular Dystrophy Association (MDA) and Parent Project Muscular Dystrophy (PPMD), both of the United States, and United Parent Projects Muscular Dystrophy (UPPMD), headquartered in the Netherlands - officially signed a memorandum of understanding to launch the **Duchenne Research Collaborative International (DRCI)**.

"This is a monumental day for the entire Duchenne muscular dystrophy (DMD) community throughout the world," said Lou Kunkel, PhD, Professor of Genetics and Pediatrics at Harvard Medical School, and head of the team that discovered the gene for DMD in 1986. "This historic collaboration among the leading DMD organizations in the world has the potential to provide a real and major boost to the efforts against this devastating and deadly disease."

In addition to being the most prevalent childhood form of muscular dystrophy, affecting about 1 in every 3,500 boys worldwide, Duchenne is also one of the most common lethal genetic disorders diagnosed during childhood.

In forming **DRCI**, the organizations have made a commitment to cooperate, collaborate and communicate openly in support of projects to accelerate translational research into therapeutic options to treat DMD.

Additionally, while focused on DMD right now, the partners have a broader vision to serve as a model for collaboration in the battle against neuromuscular diseases by improving access to information about rare conditions, creating strong public/private partnerships and, ultimately, increasing the amount of funding available for disease research.

"While this is a significant step forward for the Duchenne community, we are confident that the framework we are putting into place can be widely replicated for the benefit of patients with other neuromuscular and rare conditions the world over," said Laurence Tiennot-Herment, President of AFM. "In forming **DRCI**, we are

clearly setting a new direction that hopefully can be used to improve collaboration and accelerate the research agenda for countless other conditions."

The amount of funding being invested in DMD research has grown significantly over the past five years. In the United States, this is due in large part to the enactment of the Muscular Dystrophy Community Assistance, Research, and Education Amendments (MD CARE) Act in 2001.

That law led to an infusion of new research dollars, the establishment by the National Institutes of Health of six research "centers of excellence," and a greater focus on public health education and awareness through the Centers for Disease Control and Prevention.

The European Union (EU) recently approved 10 million euros to aid muscle disease patients. The funding will bring together some of the world's leading doctors and scientists in a network, TREAT NMD, aimed at improving treatment and finding cures for debilitating neuromuscular diseases.

In developing **DRCI**, leaders of the international Duchenne community have established a first series of three near-term goals:

- **A Clearinghouse for Research Investments and Research Resources.** This will provide the Duchenne research and patient community with one central international repository to track research grants and the location and availability of key research resources.
- **A Global Patient Registry.** Through a global registry, smaller local and regional registries can be consolidated into one "super database," providing public health officials and researchers with greater ability to study a much larger proportion of the world's DMD cases. This will help ensure patients from all over the world, especially those in remote locations, have the opportunity to be selected for potentially life-saving treatment and therapy trials. A central database will help researchers locate eligible candidates for clinical trials in a more efficient manner.
- **A Global Clinical Trial Network.** A Global Clinical Trial Network will present a platform of support systems critical to the efficient conduct of clinical research. Through this trial network, the translational research conducted by the global Duchenne research community will be accelerated as researchers draw from shared databases and validated research resources, thus avoiding unnecessary and costly duplication of effort.

The **DRCI** has also created a Professional Ethics Committee which will recommend guidelines for industry and nonprofit organizations that invest time and resources to help patients with Duchenne. The committee is currently developing an ethical platform for investments in translational research in rare disease, using Duchenne as a model.

"These aligned goals represent important steps to serve our global patient community," said Elizabeth Vroom chairman of UPPMD.

Concrete actions have already been taken toward these goals. Just last month, all four organizations - AFM, MDA, PPMD and UPPMD - merged their research grant databases to form a central reference for funded research grants in Duchenne.

"This collaboration is a very big first step that facilitates access to contacts and information throughout the greater patient advocacy community. We clearly realize that we are much stronger when working together as a united force, and we are committed to doing all we can to find treatments and therapies for Duchenne," said Dr. R. Rodney Howell, Chairman of MDA Scientific Advisory Committee and member of the Board of Directors.

While several promising DMD research pathways have already been identified, a number of challenges remain for the **DRCI** to address, including: development of superior animal models; establishment of clearer evidence-based standards of care; greater engagement of industry partners; and reduction in fragmentation of research.

"We have certainly come a long way since my two boys, Christopher and Patrick, were diagnosed with Duchenne 20 years ago," said Pat Furlong, president of PPMD. "I'm confident that, through the resource

sharing and collaboration that is part of this partnership, we will move closer to developing a safe and effective treatment to help the current generation of boys with Duchenne.

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Duchenne Research Collaborative International (DRCI)

Background Information

The Duchenne Research Collaborative International (DRCI) is an international collaboration among four of the world's leading voluntary health organizations who are committed to work together to examine, design, and drive the coordinated execution of projects to accelerate translational research in Duchenne muscular dystrophy. Created in 2006, the DRCI is jointly led by the Association Francaise contre les Myopathies (AFM), Muscular Dystrophy Association (MDA), Parent Project Muscular Dystrophy (PPMD), and United Parent Projects Muscular Dystrophy (UPPMD).

AFM - Created in 1958 by a group of patients and their families, and recognized as being of public utility in 1976, AFM (French Muscular Dystrophy Association) has a single objective: to defeat neuromuscular diseases which are devastating muscle-wasting diseases. It has set itself two missions: curing neuromuscular diseases and reducing the disabilities they cause. For more information, visit: www.afm-france.org.

MDA - The Muscular Dystrophy Association is a voluntary health agency -- a dedicated partnership between scientists and concerned citizens aimed at conquering neuromuscular diseases that affect more than a million Americans. For more information, visit: www.mda.org.

PPMD - Parent Project Muscular Dystrophy's mission is to improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy (DMD) through research, advocacy, education and compassion. For more information, visit: www.parentprojectmd.org.

UPPMD - UPPMD (United Parent Projects Muscular Dystrophy) is an international nonprofit organization set up by Duchenne Parent organizations around the globe. UPPMD is specifically focused on Duchenne -and Becker muscular dystrophy- the most common and most severe of the muscular dystrophies. UPPMD was developed to share aims and goals by working efficiently and collaboratively with the DMD community to accelerate the development of promising treatments and a cure. UPPMD is managed and led by parents under close cooperation with clinicians and researchers. For more information, visit: www.uppmd.org.