PRESS RELEASE

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FOR IMMEDIATE RELEASE

Global Alliance of 57+ Myotonic Dystrophy Focused Organisations Unite to Raise Awareness on Rare Disease Day 2023

February 28, 2023, Worldwide: On Rare Disease Day 2023, the Global Alliance for Myotonic Dystrophy Awareness proudly stands together to celebrate the remarkable strength and resilience of the myotonic dystrophy community. With over 57 organisations focused on advancing the understanding of and care for this rare genetic disorder, the Alliance continues to be a beacon of hope for people living with myotonic dystrophy (DM), their families, and healthcare professionals around the globe.

The Global Alliance for Myotonic Dystrophy Awareness invites individuals, organisations, and policymakers around the world to join us in raising awareness of myotonic dystrophy and advocating for improved care and research for those living with the disease.

Myotonic dystrophy (DM) is a rare and complex disease affecting muscle strength and wasting, respiratory systems, heart function, cognitive abilities, and more. DM is an inherited disease that affects successive generations and is often misdiagnosed and poorly supported. With as many as 1 in 2,100 individuals at risk of developing the disease or passing it on to the next generation, myotonic dystrophy is the most common form of adult-onset muscular dystrophy. Despite its prevalence and complexity, there are currently no disease-modifying treatments or cures available for this debilitating condition.

While myotonic dystrophy affects people all around the world, possibly including people in your local community, it remains a relatively unknown disorder. By joining forces to raise awareness, the Global Alliance aims to shed light on the challenges faced by those living with myotonic dystrophy and to increase awareness and understanding of the disease across the globe.

“With thousands of people in the US affected by this relatively unknown disease, we will continue to focus on the urgent need for improved diagnosis, treatment, and research to better serve the myotonic dystrophy community,” said Dr. Sharon Hesterlee, Chief Research Officer at the Muscular Dystrophy Association. "By working together, we can improve the lives of those affected by DM and create a more supportive and inclusive society for all.”

As part of the Global Alliance’s commitment to achieving positive change for individuals and families affected by DM, its members will direct their energies toward two primary areas of focus in 2023:

1. Awareness and education among clinical care teams,
2. Clinical trial readiness for participants.

The first area of focus is raising awareness and education among clinical care teams. This will involve working with medical professionals to increase their understanding of DM, including its symptoms, diagnosis, and available treatments. By doing so, the Global Alliance hopes to improve the quality of care that individuals with DM receive, reduce the time to diagnosis, and ultimately improve their quality of life.

“The importance of clinicians’ awareness and knowledge of myotonic dystrophy cannot be overstated. Despite its prevalence, myotonic dystrophy is often overlooked due to the variability of the condition and the symptoms overlapping with other diseases. Although it is now confirmable with a simple genetic test, a diagnosis can still be difficult even when DM is suspected,” said Dr. Homira Osman of Muscular Dystrophy Canada. “It is common for older family members to only be diagnosed after the birth of a congenitally affected child, which means misdiagnoses can persist for decades and delayed diagnosis is very common. Physicians may see only one or two patients with DM in their entire practice and

therefore may be unfamiliar with the varied and complex symptoms that can appear in nearly every system of the body, from locking muscles (myotonia), to heart, breathing, digestive, hormonal, early onset cataracts, and cognitive difficulties, just to name a few."

The second area of focus will be clinical trial readiness for participants. With no existing cure for DM, clinical trials are crucial to the development of new treatments and therapies. However, recruiting participants for clinical trials can be challenging, and the Global Alliance will work to improve access to information about trials and support for those who wish to participate. The Global Alliance will also work to ensure that clinical trials are designed to be inclusive, accessible, and accommodating for all individuals with DM.

The Global Alliance for Myotonic Dystrophy Awareness recognizes the importance of clinical trial readiness in the quest for therapies and ultimately, a cure for DM. "We must be ready to participate in clinical trials. The goal has to be to provide the greatest potential for finding a cure in the shortest possible time frame for the many people that are suffering, and even at risk of dying," said Jorg van Gent, Founding Chair of MD Nederland. "With over 50 companies and institutions offering significant new investment into research and technologies focused on identifying treatments for myotonic dystrophy, there is hope for improved quality of life, reduced disability, and increased life expectancy for individuals living with this disease within the next few years. These new technologies also hold great promise for individuals with other muscular dystrophies and for conditions with a similar genetic base, such as ALS, Fragile X syndrome and Huntington's disease."

The Global Alliance encourages and invites all members of the DM community to actively participate in Rare Disease Day on February 28th, DM Families Day on July 29th, and International Myotonic Dystrophy Awareness Day on September 15th. These important days present an opportunity for individuals and organizations to come together and raise awareness of myotonic dystrophy through various activities. These may include sharing personal experiences with friends and family, writing letters to, or meeting with, policy makers to advocate for DM research funding and provision of care, sharing DM clinical care guidelines with healthcare providers, organizing fundraising events, lighting up monuments and landmarks in green, and more!

Through these efforts, the Global Alliance hopes to foster wider recognition and a greater understanding of the disease and to ultimately improve the lives of those affected by it.

Together, we are changing the future of myotonic dystrophy.

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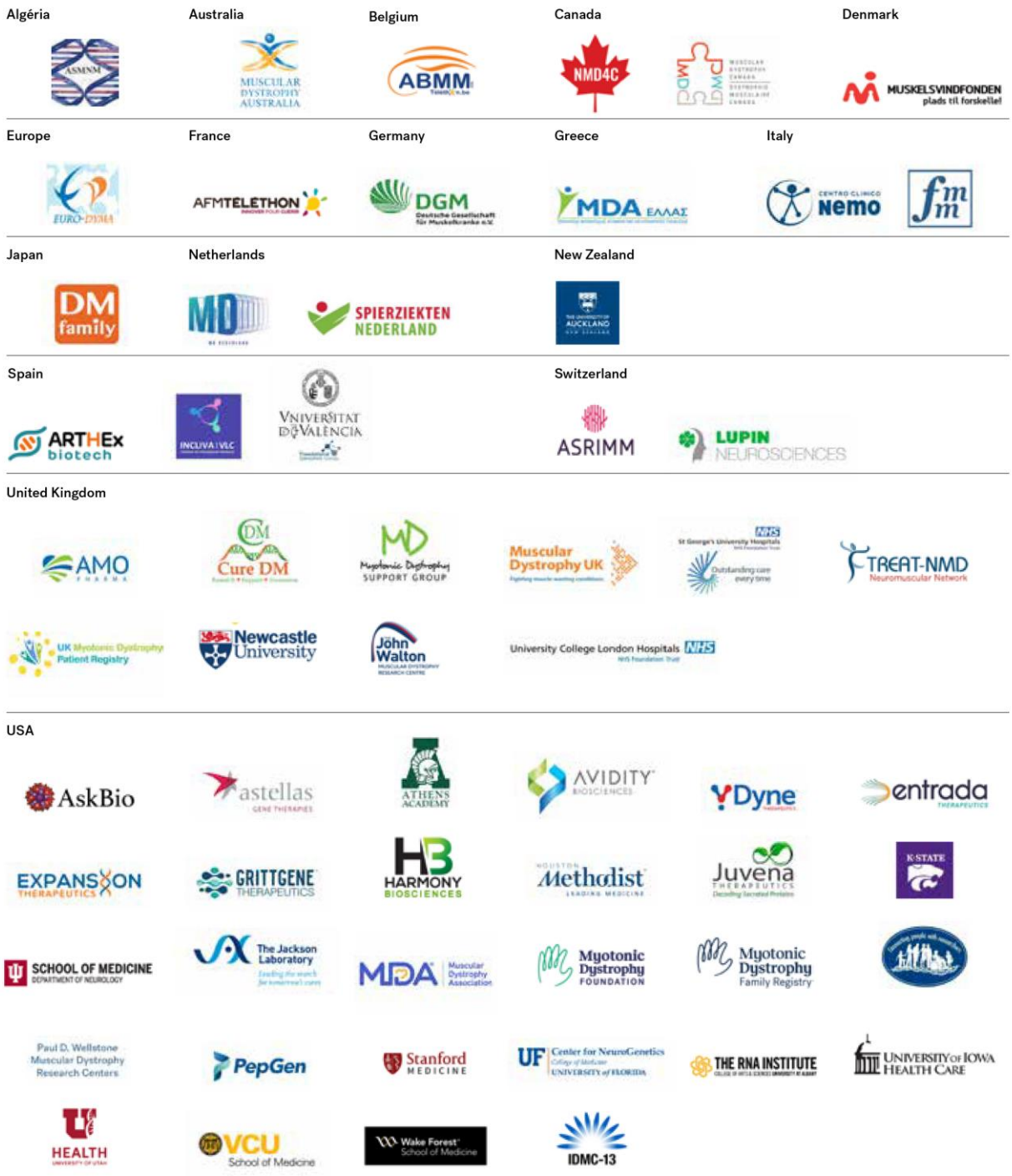
Established in 2021, the Global Alliance for Myotonic Dystrophy Awareness now includes over 57 international nonprofit organisations, academic and research institutions, biotechnology and pharmaceutical companies, patient advocacy groups, and others working together to raise myotonic dystrophy awareness. Discover DM resources and learn how you can join the movement at: https://www.myotonic.org/international-dm-day

The Global Alliance for Myotonic Dystrophy Awareness



To join the Global Alliance, visit https://www.myotonic.org/international-dm-day.