PRESS RELEASE

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FOR IMMEDIATE RELEASE

International Myotonic Dystrophy Awareness Day 2022

July 23, 2022, Worldwide: A global alliance of over 50 myotonic dystrophy-focused organizations have, once again, united to celebrate **Myotonic Dystrophy Families Day** on July 23rd, and **International Myotonic Dystrophy Awareness Day** on September 15th.

To improve the quality of life of people living with the disease, it is critical to raise awareness about myotonic dystrophy (DM). This rare, multi-systemic, progressive, and inherited disease affects successive generations but is often misdiagnosed and poorly supported. As many as 1 in 2,100 individuals are at risk of developing the disease or passing it on to the next generation, making myotonic dystrophy the most common form of adult muscular dystrophy. DM is also considered to be one of the most complex and variable of all known conditions, yet there are currently no disease modifying treatments or cures.

After a successful launch of the first International Myotonic Dystrophy Awareness Day in 2021, which engaged multiple policy makers and elected officials from all levels of government, the benefits of the campaign are already bearing fruit:

“In 2021, the efforts of over 50 organizations in the Global Alliance for Myotonic Dystrophy Awareness showed an unprecedented level of collaboration, collective energy, and progress in raising myotonic dystrophy awareness to change the future of the disease,” said Dr. Tanya Stevenson, CEO of the U.S. based Myotonic Dystrophy Foundation. “We saw three government proclamations acknowledging September 15th at the federal and regional levels in the U.S. and Canada,

thousands of social media posts across languages and countries, iconic monuments lit up in green, virtual educational seminars and celebratory programs, a logo contest with participation from over 20 countries, and so much more. We are in awe of the work the Global Alliance has already accomplished, and urge all of our partners to build on these early successes to continue to raise the profile of myotonic dystrophy, and to grow the attention of drug developers, clinical care teams, insurance companies, and government agencies so the devastating generational impact of this disease may receive recognition and families receive the resources they need and deserve."

In addition to the Alliance's core mission of raising myotonic dystrophy awareness among the general population, many members of the Global Alliance for Myotonic Dystrophy Awareness are also leading efforts in two specific areas of focus to maximize our impact in 2022:

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

Myotonic dystrophy awareness and education among clinicians is crucial to delivering the quality of health care that people living with DM require. 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. 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. Physicians may see only one or two patients with DM in their entire practice and therefore may be unfamiliar with DM's 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/or cognitive difficulties, just to name a few.

"Myotonic Dystrophy is a rare and widely variable disease, yet perhaps not as rare as believed, just not identified. Individuals and families living with DM often unfairly bear the burden of educating their clinical care teams about the disease, and the realities of living with it." said Emma-Jayne Ashley, Founding Trustee of Cure DM UK. "Even as self-advocates, it can be difficult to receive appropriate

care and support, and it can take many years to receive a diagnosis in the first place. It is absolutely critical that we increase the level of awareness and education about DM amongst clinicians around the world, and in the meantime, provide families and individuals with the very best resources to be self-advocates. As a Global Alliance, we have a unique opportunity to share knowledge and resources, to improve and standardise care and quality of life for our DM community, worldwide."

Clinical trial readiness for people living with myotonic dystrophy is fundamental to the development of therapies and a cure. This is an exciting time for the DM community with multi-national recruitment for three clinical trials, including one for the congenital form of the disease, and significant new investment into research and technologies focused on identifying treatments. With over 50 companies publicly focused on myotonic dystrophy, the field is constantly learning and 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. But there is more work to be done!

"With the opportunities for participating in DM research and drug trials continuing to grow, it is now more important than ever that we as a patient community engage in these studies and trials," reflected Midori Senoo, Managing Director of the Myotonic Dystrophy Patients' Group of Japan (DM-family). "Individuals living with DM must understand why participation is so important. Because only patients can prove the efficacy of the drug. What only patients can do is advance the world by participating in research. Our organizations can contribute to this goal by educating about opportunities to participate in research, and by working with our research and drug development partners to ensure trials are designed to accommodate the real needs of participants. We have the chance to work together across borders and industries to ensure our global community is aware of and ready to contribute to the future of DM by participating in studies and trials."

The Global Alliance invites all members of the DM community to participate in International Myotonic Dystrophy Awareness Day on September 15th by sharing

their experiences with friends and family, writing letters to or meeting with policy makers, advocating for DM research funding and provision of care, sharing DM clinical care guidelines with healthcare providers, raising funds for research, and other activities that will help foster a greater understanding about the disease.

"As we take stock of our accomplishments and prepare for the future, we must continue to work together to raise awareness of myotonic dystrophy amongst the general population, the medical community, researchers and drug developers, insurance companies, and government agencies," said Joachim Spross, General Secretary of the German Society for Muscle Diseases (DGM). "As support groups, DM advocates and patients ourselves, we feel it is our duty to respond to the generational impact of this disease with efforts to amass more resources to reduce the time to a diagnosis, improve quality of life, provide appropriate and early clinical care, encourage trial participation, and find effective treatments. **Together, we can and will change the future of myotonic dystrophy.**"

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Established in 2021, the Global Alliance for Myotonic Dystrophy Awareness includes over 50 international nonprofit organisations, academic and research institutions, biotechnology and pharmaceutical companies, patient advocacy groups, and others working together to raise awareness about myotonic dystrophy. Learn more about how to join the movement and access myotonic dystrophy resources at:
https://www.myotonic.org/international-dm-day

The Global Alliance for Myotonic Dystrophy Awareness

Members listed alphabetically by country. An * indicates a founding member of the Global Alliance for Myotonic Dystrophy Awareness.

MDA Australia*
(Australia)

Association Belge contre les Maladies neuro-Musculaires*
(Belgium)

The Neuromuscular Disease Network for Canada*
(Canada)

Muscular Dystrophy Canada
(Canada)

Muskelsvindfonden*
(Denmark)

Euro-DyMA*
(Europe)

AFM-Téléthon*
(France)

Deutschen Gesellschaft für Muskelkranke e.V.*
(Germany)

Muscular Dystrophy Association HELLAS*
(Greece)

Centro Clinico NeMO
(Italy)

FMM - Fondazione Malattie Miotoniche*
(Italy)

Myotonic Dystrophy Patients' Group of Japan*
(Japan)

Stichting MD Nederland*
(Netherlands)

Spierziekten Nederland*
(Netherlands)

Centre for Brain Research, Neurogenetic Clinic, University of Auckland
(New Zealand)

INCLIVA. Health Research Institute
(Spain)

Universitat de València
(Spain)

Association Suisse Romande Intervenant contre les Maladies neuro-Musculaires*
(Switzerland)

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Lupin Neurosciences
(Switzerland)

AMO Pharma Ltd
(United Kingdom)

Cure DM Myotonic Dystrophy UK*
(United Kingdom)

Myotonic Dystrophy Support Group*
(United Kingdom)

Muscular Dystrophy UK*
(United Kingdom)

St. George's University Hospitals NHS Foundation Trust
(United Kingdom)

TREAT-NMD
(United Kingdom)

UK Myotonic Dystrophy Patient Registry*
(United Kingdom)

University College London Hospitals NHS Foundation Trust
(United Kingdom)

AskBio
(United States)

Astellas Gene Therapies
(United States)

Astellas Gene Therapies
(United States)

Avidity Biosciences
(United States)

Dyne Therapeutics
(United States)

Entrada Therapeutics
(United States)

Expansion Therapeutics
(United States)

GrittGene Therapeutics
(United States)

Harmony Biosciences
(United States)

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Houston Methodist
Department of Neurology
(United States)

Juvena Therapeutics, Inc.
(United States)

Kansas State University
Wildcat Extension
(United States)

Indiana University School of
Medicine, Dept of Neurology
(United States)

The Jackson Laboratory (JAX)
(United States)

Muscular Dystrophy
Association USA*
(United States)

Myotonic Dystrophy Foundation*
(United States)

Myotonic Dystrophy Family Registry*
(United States)

National Registry for Myotonic Dystrophy
& Facioscapulohumeral Dystrophy
(United States)

Wellstone Muscular Dystrophy
Cooperative Research Center (MDCRC)
(USA)

PepGen
(USA)

Stanford University
(USA)

University of Florida Center
for Neurogenetics
(USA)

The RNA Institute, University
at Albany, SUNY
(USA)

University of Iowa Health Care
(USA)

University of Utah
(USA)

Virginia Commonwealth
University
(USA)

Wake Forest University,
Department of Neurology
(USA)

International Myotonic
Dystrophy Consortium
(Worldwide)

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