PRESS RELEASE

Canada Contact Name: Homira Osman
Canada Contact Email: Homira.osman@muscle.ca

Japan Contact Name: Midori Senoo
Japan Contact Email: international@dm-family.net

UK Contact Names: Stephen Uncles
UK Contact Emails: stephen.uncles@sky.com

US Contact Name: Tanya Stevenson
US Contact Email: Tanya.Stevenson@myotonic.org

FOR IMMEDIATE RELEASE

Global Alliance of 60+ Myotonic Dystrophy Organizations Unites for Rare Disease Day 2025

February 28, 2025, Worldwide – The Global Alliance for Myotonic Dystrophy Awareness (Global Alliance) proudly joins the global rare disease community in commemorating Rare Disease Day on February 28th, 2025. With over 60 organizations collaborating worldwide, the Alliance continues to advance understanding, care, and research for myotonic dystrophy (DM), one of the most prevalent rare genetic disorders.

Myotonic dystrophy (DM) is a progressive, inherited disorder that impacts muscle strength, respiratory systems, heart function, and cognitive abilities, among other areas. Despite its prevalence, DM remains underdiagnosed and misunderstood, with no current cure or disease-modifying treatments available. The Global Alliance is committed to addressing these gaps through collaboration and education.

In 2025, the Global Alliance reaffirms its commitment to two joint areas of focus:

1. **Raising awareness and providing education among clinical care teams.**
2. **Enhancing clinical trial readiness for participants.**

These priorities address the critical challenges faced by individuals and families affected by myotonic dystrophy.

In line with its renewed priorities, the Global Alliance will continue working with healthcare providers to enhance awareness and understanding of DM, its symptoms, and available care guidelines. The aim of this education is to reduce delays in diagnosis and improve the quality of care provided to individuals affected by the disease.

Muscular Dystrophy Canada, member of the Global Alliance, recently launched an initiative to improve access to genetic testing for individuals impacted by myotonic dystrophy. This groundbreaking effort aims to address one of the key barriers to timely diagnosis and participation in research. "Genetic testing is the cornerstone of understanding and managing myotonic dystrophy, and improving access will empower individuals and families with critical information," said Dr. Homira Osman, Vice-President of Research and Public Policy at Muscular Dystrophy Canada. "By ensuring

equitable access to testing, we can pave the way for earlier diagnoses, better care, and greater inclusion in clinical trials. Together, we're making strides toward meaningful change for the DM community." Muscular Dystrophy Canada's new initiative embodies the Global Alliance's shared areas of focus by increasing awareness among clinical care teams while also helping those living with DM to get necessary information about clinical trials.

The Global Alliance continues work to prepare participants around the globe for clinical trials, which are crucial research studies testing treatments and therapies for myotonic dystrophy. The Global Alliance is dedicated to improving access to trial information and providing support for those interested in participating, while also advocating for inclusive and accessible trial designs that accommodate the diverse needs of the DM community.

"Myotonic dystrophy registries play an essential role in bridging the gap between patients and clinical trials," said Helen Walker, Registry Curator for the UK DM Patient Registry. "By collecting and maintaining accurate data shared by patients, registries not only help researchers understand the disease better but also ensure that patients are aware of and prepared for opportunities to participate in trials and other research opportunities. This is a vital step toward developing effective treatments and improving quality of life for the DM community."

Dr. Andy Berglund, Director of the RNA Institute at the University at Albany, highlighted the growing momentum in DM research: "The strides being made in myotonic dystrophy research would not be possible without the collective efforts of patients, clinicians, and advocacy groups. The renewed focus on clinical trial readiness ensures that the DM community is well-positioned to benefit from emerging therapies. At the RNA Institute, we are honored to be part of the Global Alliance to drive progress and create hope for those living with DM."

The Global Alliance invites everyone—individuals living with DM, families, healthcare professionals, industry, and researchers—to join in raising awareness and advocating for those living with myotonic dystrophy on Rare Disease Day. This day provides a unique opportunity to unite and amplify the voices of those impacted by DM. Activities can include sharing personal stories with loved ones, engaging policymakers to advocate for research funding and improved care, providing clinical care guidelines to healthcare providers, or even hosting fundraising events.

By working together, we can build a brighter future for the DM community.

For more information, visit Global Alliance for Myotonic Dystrophy Awareness.

The Global Alliance for Myotonic Dystrophy Awareness

To learn more and apply to join the Global Alliance, please visit www.myotonic.org/international-dm-day

ASMNM

Muscular Dystrophy Australia

ABMM Téléthon.be

NMD4C

Muscular Dystrophy Canada / Dystrophie musculaire Canada

Muskelsvindfonden plads til forskelle!

Euro-Dyma

AFM Téléthon Innover pour guérir

DGM Deutsche Gesellschaft für Muskelkranke e.V.

MDA Ελλας

Centro Clinico Nemo

fm

DM family

Maastricht UMC+

MD Nederland

Spierziekten Nederland

The University of Auckland New Zealand

FB

ARTHEx biotech

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INCLIVA | VLC

Universitat de València

ASRIMM

Lupin Neurosciences

AMO Pharma

Cure DM Research ♥ Support ♥ Awareness

Myotonic Dystrophy Support Group

Muscular Dystrophy UK Fighting muscle-wasting conditions

NHS St George's University Hospitals NHS Foundation Trust Outstanding care every time

TREAT-NMD Neuromuscular Network

UK Myotonic Dystrophy Patient Registry
Newcastle University
John Walton Muscular Dystrophy Research Centre

University College London Hospitals NHS Foundation Trust

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Houston Methodist Leading Medicine

Juvena Therapeutics Decoding Secreted Proteins

K-State

School of Medicine Department of Neurology

The Jackson Laboratory Leading the search for tomorrow's cures

MDA Muscular Dystrophy Association

Myotonic Dystrophy Foundation

Myotonic Dystrophy Family Registry

Connecting people with researchers

Paul D. Wellstone Muscular Dystrophy Research Centers

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Vertex

VCU School of Medicine

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