

# CMDIR

*Leading the Way to a Treatment and Cure*

## **Congenital Muscle Disease International Registry**

*Congenital Muscular Dystrophy • Congenital Myopathy • Congenital Myasthenic Syndrome*

June, 2014

Greetings!

I would like to introduce you to the Congenital Muscle Disease International Registry (CMDIR). The CMDIR was established in 2009 to identify the global congenital muscle disease population for the purpose of raising awareness, standards of care, clinical trials and in the future treatment or cures. We register through the late onset spectrum of Congenital Muscular Dystrophy, Congenital Myopathy, and Congenital Myasthenic Syndrome. To date, we have almost 1,500 registrants from 68 countries. There is no cost to join the registry.

You do not have to be diagnosed with a specific muscle disorder to register. We welcome everyone with congenital or later onset muscle weakness to register, and can provide information on institutions who perform genetic testing, the names of medical providers who specialize in the diagnosis and treatment of muscle disease, and community support groups to put you in touch with others affected by a muscle disorder.

All data you share with us will remain confidential and will not be shared without your consent. Data is stored in a secure database and maintained in strict accordance with healthcare privacy laws. From time to time, we extract data from surveys and medical records for the purpose of sharing with researchers or pharmaceutical companies in order to advance care standards and possible treatments. All data shared will exclude identifying information and will be in aggregate with other registrants' data.

Your registry profile will include:

- Contact and demographic information
- Healthcare provider information
- An intake survey with questions about diagnosis, progression, and other disorder aspects
- A release to request medical records pertaining to the disorder from your healthcare providers
  - All medical records received will be uploaded to your profile, accessible to you at any time, making the registry a safe and secure record repository
  - Records will be curated for key measures with trend graphics to help you and your providers track disease progression over time

Once your registration is complete and records have been received, we will contact you at regular intervals to:

- Update any contact changes
- Renew your medical release to request subsequent records (expires annually)
- Complete a short annual update survey to explore disease progression over time
- Notify you of clinical studies being conducted in your disease subtype
- Notify you of family or medical conferences and clinics you may be interested in attending
- Update you on the progress of current research and treatment opportunities

1712 Pelican Avenue ♦ San Pedro, CA 90732

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The CMDIR website includes a number of excellent resources to familiarize you with vocabulary, current care guidelines, and research being conducted in the CMDs. We also update the site regularly with information about studies and trials currently recruiting participants.

Simply put, we will not be successful in finding a treatment or cure unless we know who the affected individuals are, what the diagnosis is, and how the disease affects the individual. Your participation in the registry is vital in attaining that goal.

For more information and to register in the CMDIR, please visit [www.cmdir.org](http://www.cmdir.org). If you have any questions, are unsure if you are already registered, or would like assistance registering, please do not hesitate to contact me by phone or email.

Regards,



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