



2008 Accomplishments—2009 Goals

Thanks to everyone for the great success Cure CMD had in 2008. Due to the commitment of the all volunteer organization, Cure CMD has established itself as the recognized global leader advocating for the CMD's. Accomplishments include :

- Nomination to the US Government Sponsored Medical Dystrophy Committee
- Exceeded First Year Financial Targets
- Established Relationships with other related organizations including the MDA, Treat-NMD, PPMD, NIH, NORD and many others
- Designed Registry for the CMD population
- Established Critically acclaimed Web Site
- Identified research to therapy roadblocks

- Initiated the first research focused conference for the CMD's

In 2009, Cure CMD has set challenging but obtainable Goals

- Increased fund raising targets
- First Research Grant Award
- Launch Registry (Critical for Clinical Trials)
- Host International CMD Research Conference in Atlanta
- Reduce research to trial process roadblocks
- Organize first all-Inclusive CMD Family/Affected Individual Conference
- Utilize international alliances to further

Community Outreach

- Colorado Springs, CO..... The Trojan high school girls volleyball team, coached by Sarah Brockberg, raised over \$1,100 for Cure CMD. Brockberg organized a volleyball Servathon, where each girl made 100 serves for pledges . Ms. Brockberg is mother to Kyra, age 2, with CMD. Thank you team, for supporting Cure CMD!!

- Forest Lake, MN..... A group of neighbors, led by John Barnes and Suzie Stahl, raised over \$700 for Cure CMD in the 1st Annual Light Up the Neighborhood Luminary Project. The neighborhood sold packs of luminaries to neighboring residents, to be displayed on the night of December 24. Barnes and Stahl are close friends of the Cloud family. Thank

CMD research and clinical trials

In order for Cure CMD to be successful we all need to work together, Thanks for your continued support.

Richard Cloud
Chairman, Cure CMD



Rich's Daughter
Ashley, Age 4 with CMD



you, neighbors for supporting Cure CMD!!!

Want to organize a fundraiser? You may be featured on our website and newsletter! Please send your info to: info@curecmd.org.

Special points of interest:

- > First International Scientific CMD Conference set for July 9-11, 2009 in Atlanta, GA
- > First CMD Affected Individual and Parent Conference set for Aug 15-16, CHOPS, Philadelphia, PA
- > CALL TO ACTION—CMD affected individuals and Parents, Register TODAY to help us beat CMD, please visit, www.curecmd.org and click Register
- > Cure CMD selects Scientific and Medical Advisory Board
- > Visit www.curecmd.org For more information including Research, Forum, Fundraising and General Information

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Trojan High School Girls and Kyra, age 2 with CMD



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Research

Dr. Anne Rutkowski, Vice Chairman of Cure CMD



Disease descriptions about congenital muscular dystrophy are now listed on DIRLINE (National Medical Library through NIH), Genetic Alliance and NORD. Improving educational information for parents and affected individuals and assisting with genetic diagnosis are two high priorities for Cure CMD.



**Anne's Daughter
Maia, 10 years old with CMD**

Cure CMD is working with Innolyst to beta test the CMD registry. Innolyst provides solutions to non-profit research foundations to spur collaboration and data-sharing to speed the discovery of treatments for disease

The year 2009 will offer new insight and breakthroughs towards developing therapeutic targets in the CMDs. Santhera announced last year the first clinical trial planned in the CMDs with a drug called omigapil. Santhera has done both preclinical trial testing and secured orphan drug status; first phase clinical trials have been delayed due to economic realities.

Establishing a relationship with NIH (National Institutes of Health) and the MDA, two important funding resources for CMD research has been a top priority. Dr. Anne Rutkowski, Vice Chair of Cure CMD has been nominated to the MDCC (Muscular Dystrophy Coordinating Committee that was created to provide oversight of MD federal funding. Cure CMD has networked with international scientists and pharmaceutical companies pursuing research related to the CMDs.

Cure CMD is assisting Dr. Madhuri Hegde with validation of a CMD chip. Currently, genetic testing requires either a blood sample or muscle biopsy sample and CMD genes are tested in a sequential order based on clinical suspicion or lab protocol. Dr. Hegde's CMD chip technology will test all the CMD genes simultaneously which will not only help expedite testing (faster turnaround) but provide a genetic confirmation more often. Helping people with CMD get a genetic (DNA) diagnosis is critical to building a registry, because a genetic diagnosis confirms the diagnosis and may help decide which therapies in the future apply to any individual with CMD.

Cure CMD has selected a Scientific and Advisory Medical Board. The SMAB will assist the Cure CMD board in awarding research grants on an annual basis by providing peer

review and NIH based score criteria to select most qualified applications. Cure CMD plans to award a CMD research grant by Winter 2009. One research strategy to find a therapy uses high throughput screening to find a drug compound that replaces/increases a deficient CMD protein. Screening for drugs that are already FDA approved would be the fastest way to get a drug to clinical trial. Once drugs are identified, they are tested in muscle cells and mouse models to make sure they have the desired effect, prior to proceeding to clinical trial in humans.

Finally, Cure CMD will push for the development of medical standard of care in the CMDs. We envision developing a document that people with CMD can download to take to their doctor's office and assist in directing medical testing where appropriate.

Submitted by:

Dr. Anne Rutkowski

Registry (Key to finding a Therapy or Cure)

To drive momentum towards clinical trials, Cure CMD has invested heavily in making a CMD patient registry a reality.

understand the disease subtypes, the kind of medical care people are getting and access to diagnostic testing.

A registry is the way a person with CMD will get linked to future clinical trials. A registry will also help answer important questions in CMD that have not been answered, such as, "how many people have CMD in the world?" and "how many people with CMD do not have a genetic diagnosis?". These are important questions that need to be answered to help

Cure CMD is working with Innolyst to develop a "beta test" of the CMD registry. By Jan 30th, 50 individuals with CMD will be invited to test the questions of the CMD registry. The responses will be subsequently reviewed, revisions will be made with the goal to launch the official CMD International Registry (CMDIR) by

Summer 2009. The registry will also allow registered CMD members to search de-identified responses to get feedback on the CMD population regarding questions asked. Cure CMD may invest financially in initial pilot to launch CMDIR. Cure CMD is working with TREAT-NMD, a European group, to make translational research and clinical trials a reality for the muscular dystrophies.

Submitted by:

Dr. Anne Rutkowski

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Cure CMD 2009 Scientific Conferences

Cure CMD is planning the first ever international scientific conference at Emory University, in Atlanta Georgia, on July 9th-11th focused specifically on the Congenital Muscular Dystrophies.

the CMDs. The conference includes representatives from NIH, MDA, TREAT-NMD and the pharmaceutical industry.

We look forward to sharing more details about the conference in the coming months.

This 3 day scientific conference will engage leading CMD experts and international researchers focused on 8 core therapeutic target areas for

The goal is to design a roadmap or investment portfolio with short range, mid range and long-term scientific investments for therapies to slow disease progression and one day find a cure for CMD.

Submitted by: Pat May



Pat May
CFD, Cure CMD

Cure CMD Family and other Conferences

Spreading awareness and knowledge within our community as well as to physicians and genetic counselors are high priorities for Cure CMD.

- b. CMD scientific targets: overview of July conference
- c. CMD medical care
- d. CMD clinical trials/registry

We will also plan to attend the National Society of Genetic Counselors conference to promote improved diagnostic access and increased rates of genetic confirmation. Cure CMD has also participated in Rare Share, a forum for rare genetic diseases.

CMD Family/Affected Person conference, August 15th-16th 2009. The conference will focus on:
a. CMD disease review about the various CMD subtypes

Cure CMD will have a booth at American Academy of Neurology conference, Seattle, April 27th- May 1st to provide feedback, awareness and educational material for neurologists.

Submitted by:
Dr. Anne Rutkowski

The staff of Cure CMD would like to give a big **THANKS** to Dr. Carsten Bonnemann whose support and tireless efforts for all people with CMD's will make the possibility of a cure or treatment attainable.

Pat's Daughter
Aubrey, 2 years old with CMD



Cure CMD depends on donations both small and large to enable the realization of our shared goals.

Cure CMD Financials

As we close 2008, I'm happy to report that CureCMD's financial position at the end of the year far outpaced our original goals. Our plan was to begin to build a cash balance during 2008 that would allow the organization to award its first research grant during 2009. As the year closed out, CureCMD has built its cash balance to over \$45,000.

effort to target areas that can provide much needed funding. Many of you have organized local fundraisers which have helped increase both CureCMD's awareness and revenue. We continue to encourage everyone to organize a fundraiser at their local level. Every dollar raised helps!

can be utilized more directly on funding research efforts. We appear well on our way to meeting the financial goal of awarding our first grant during 2009.

During 2009 we are making a more concerted

CureCMD is completely operated on a volunteer basis. As there are no payroll costs to overcome, those donations received

Submitted by: Pat May

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Please Visit us at
www.curecmd.org

On November 18-19 of 2008, a core working group of congenital muscular dystrophy (CMD) experts met with Indiana University registry experts and Cure CMD staff in a workshop aimed at setting up a patient registry for the CMDs. A representative of TREAT-NMD participated in the workshop to harmonize efforts in the US and Europe.

You can read about the workshop in the most recent TREAT NMD newsletter: <http://www.treat-nmd.eu/patients/news/news/393/>



Participants: (pictured)

standing: Dr. Anna Sarkozy (Treat NMD), Dr. Steven Moore (U of Iowa, neuropathologist), Jackie Johnson (Indiana University, Bioinformatics/registry design), Dr. Anne Connolly (Wash Univ, neurologist), Dr. Tom Winder, (Prevention Genetics), Dr. Susan Sparks (CNMC, genetics), Dr. Tatiana Foroud (Indiana University, genetics/bioinformatics)

Seated: Dr. Anne Rutkowski (Cure CMD), Rich Cloud (Cure CMD)

Not pictured: Dr. Carsten Bonnemann (CHOP, neurologist), Dr. Kathy Mathews (U of Iowa, neurologist), Dr. Susan Iannaccone (UT Southwestern, neurologist).

Cure CMD

Finding a cure or treatment for the
Congenital Muscular Dystrophies

We are interested in your
feedback, opinions and
contributions to our
newsletter. You can email
us at info@curecmd.org



Owen, 5 years old with CMD

How to help

There are many ways you can help Cure CMD further finding a cure or treatment for Congenital Muscular Dystrophy.

Probably the easiest way to help is to go to our website and/or mail a contribution to Cure CMD. As a wholly volunteer organization you can be assured that your donations are going where they will have the biggest impact to our mission.

You can also volunteer.

There are currently immediate need for a Fundraising Director, Newsletter Director, Web Guru, and Fundraising Volunteers. There are numerous tasks that we could use help with, let us know if you are interested at info@curecmd.com.

Another way you can help would be to network with others affected with a CMD and have both you and them register. A registry gives us the ability

to work with researchers with defined populations and mutations in finding a cure or treatment.

Lastly, you can send us feedback on how we can improve or provide more value.

I would like say a special thank you to all the families, affected individuals, medical resources that have contributed time and money so far.

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Cure CMD has put together a flyer on the CMDs to be distributed to physician and genetic counselor offices. Please assist us in this effort and email your physician/genetic counselor's address to info@curecmd.com.

To assist people with CMD, a pdf of certain key CMD review articles can be found on the Cure CMD website under Medical Overview. Please print a copy and take with you to your physician if your physician does not currently have CMD expertise.

This Newsletter and previous issues can be found on Cure CMD's website under the tab "Newsletter".

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