



FALL '09 EDITION

Together with you, funding our way to a treatment and cure for the CMDs

Recent CMD research updates. To read more, go to www.curecmd.org

Dr. Kevin Campbell receives 2 million dollar NIH grant to find drugs focused on upregulating glycosylation.

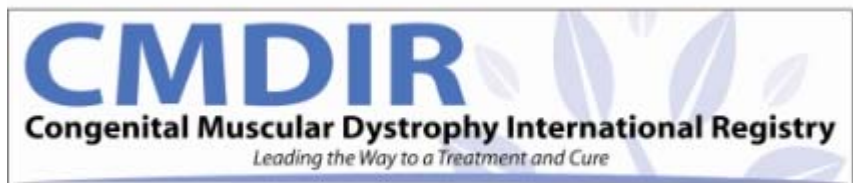
Dr. Brad Hodges, Founder and CSO of Prothelia, a small biotech company, receives a 200,000 NIH grant to determine if laminin 111 has an effect in the dyw mouse model (the mouse model for merosin deficient CMD).

Cure CMD receives 11 scientific grant applications. Cure CMD and SAM have partnered to fund CMD science that focuses on identifying drugs to treat CMD disease.

Drs. Markus Ruegg and Thomas Meier publish paper showing omigapil has an effect on dyw mouse model.



www.curecmd.org



Introduction

Cure CMD continues to make progress towards finding a treatment or cure for Congenital Muscular Dystrophy. Taking the output of the therapeutic conference in July, members of Cure CMD have been busy developing and prioritizing a therapeutic roadmap, identifying and mitigating potential risks and organizing fundraising to fund scientific efforts. Working with leading researchers from around the world, Cure CMD will **fund research grants** that have the highest potential for clinical trials within the shortest period of time possible. Once grants are awarded, Cure CMD will hold researchers accountable to agreed upon objectives and drive progress.

It is important to continue to fund pharmaceutical science that moves drugs forward to clinical trials and then treatments. To that end, Cure CMD depends on our **all volunteer** organization to help in fundraising, volunteering and registering. If you are interested in getting involved, please contact Richard Cloud at rich.cloud@curecmd.org.

Please register if you have not already done so at www.cmdir.org. This will enable us to get data we need for clinical trials as well as identifying our population and providing notification of potential treatments for your specific Congenital Muscular Dystrophy.

We have several news events to share with you and always look forward to your feedback. If you have a story you would like to include in a future newsletter, want further information or would like to provide feedback, please contact Becky May at may4kc@yahoo.com.

Richard Cloud
Chairman, Cure CMD

Cure CMD Reviews Grant Applications

Cure CMD is thrilled to have received 11 outstanding grant applications for CMD science, September 2009. The applications are focused on finding drugs and strategies to slow disease progression in various forms of CMD. Each application is looking for approx 50K funding for 2 years. The grants will be reviewed in October and November of this year by the Cure CMD Scientific and Medical Advisory Board and outside peer review; based on the NIH review process and chaired by Dr. Carsten Bonnemann (see link-<http://curecmd.org/scientists>). Scored recommendations will be presented to the Cure CMD Board for funding with awards announced on December 15th.



We need you to help fund CMD science focused upon identifying drugs to improve the lives of people with CMD. Our target goal is to raise \$200K towards CMD science by October 2010, in order to keep the CMD science projects that are funded this year, funded through 2011.

Consider setting up a recurrent donation of \$25, \$50, or \$150 a month that draws from your bank account. If every family and affected individual who is registered with Cure CMD sets up a recurrent donation we will hit our target.

To set up a recurrent donation, contact Patrick May, patmay@curecmd.com Each quarterly newsletter will let you know how close we are to our goal. Reaching our goal allows us to fund 4 scientific projects. Each dollar you donate goes to research. Cure CMD board and volunteers have covered all expenses associated with running Cure CMD.

You can also help by approaching your company or employer and asking if Cure CMD can be added to the list of sponsored charities. You will be required to provide Cure CMD's EIN number. Contact Patrick May for any questions.

Cure CMD Family Conference

This August, another great Congenital Muscular Dystrophy family conference was held at Children's Hospital of Philadelphia. Over 40 families attended, representing a variety of CMD subtypes. The conference also had participation from several CMD experts. Topics included a summary of the July Therapeutic Targets in CMD Conference, presentations by representatives from the MDA and National Institutes of Health (John Porter, NINDS), and information on lung, bone, nutrition and heart health of those with CMD. Break-out sessions were held, in which families could choose from several topics to get more intimate and specific information. Some of the breakout sessions consisted of: transitioning to independent living, fathers of children with CMD, and visiting with disability equipment vendors. Additionally, children with CMD, and their siblings, enjoyed fun and supervised activities, with the help from Child Life experts and students from the University. The kids were able to play wheelchair hockey, do arts and crafts and watch movies; plus get a visit from a local fire department and a performance from a children's singing group. All of the families seemed to enjoy gaining such valuable information and having the opportunity to share knowledge, experiences, and fun with other families with CMD. Cure CMD together with CHOP will post an online survey by October 5th to plan next year's Family conference. An email will be sent to all participants and a link provided on the Cure CMD forum.

CMD Standard of Care Conference

This November in Brussels, a conference on CMD care standards will take place. The CMD Standard of Care conference will bring together 45 international physicians with backgrounds in pulmonology, cardiology, gastroenterology, palliative care, diagnostics, neurology and orthopedics. The goal is to draft a CMD Standard of Care (SOC) guideline to be published in 2010. A SOC guideline will improve the lives of people with CMD by guiding physicians who are not familiar with CMD in how to treat and manage CMD. A Standard of Care is necessary to launch clinical trials to ensure that all people with CMD who participate in future clinical trials are receiving the same standard of medical care, allowing conclusions to be drawn about a drug or intervention's effect. This conference was possible because of donations from Treat-NMD, the Italian Telethon, AFM and Cure CMD; as well as input from CMD families through the CMD Standards of Care survey. If you or your family member has Congenital Muscular Dystrophy, and you have not already done so, please log onto www.curecmd.org, go to second Research News post and fill out the Standards of Care survey to help provide information for this important initiative.



Cure CMD Golf Tournament Raises Awareness and Funds

Cure CMD's Golf Tournament in Forest Lake, MN on September 13th raised over \$12,000 that will go towards finding a treatment and cure for Congenital Muscular Dystrophy.

On Sunday, September 13, 2009 over 71 golfers enjoyed a beautiful afternoon of golf, dinner, prizes and silent auction. Ashley Cloud (5), Isabelle White (6) and Katie Nelson (18), children affected by Congenital Muscular Dystrophy, greeted golfers at registration. Golfers enjoyed their interactions with these greeters who chased several of them in their power chairs playing "freeze tag."

With a shotgun start, the scramble golf event began with a mood of excitement and anticipation. Mike Belisk and Will Lessard both won the longest putt game and graciously donated their cash prizes back to Cure CMD. Although there were no winners for the hole in one Sebring convertible prize, several golfers came dangerously close. Joey Browner, former six time pro bowl MN Viking defensive back won the longest drive contest.

After golf, Ann Cloud presented prizes to the first, second and fifth place foursome winners as well as over 41 raffle prizes that included gift cards, golf club, golf balls and shirts. The largest golf prize was awarded to the fifth place foursome winner and included \$200 gift certificate at Tannersbrook Golf Course and four \$25 Blue Heron restaurant gift cards.

Cure CMD's Alliance with S.A.M.

Cure CMD is thrilled to have teamed up with advocacy group **Struggle Against Muscular Dystrophy (S.A.M.)**. SAM is a registered charity based in Northern Ireland, whose vision is to ultimately improve the treatment, quality of life and long term outlook for persons affected by Muscular Dystrophy through awareness, research, education, and advocacy. Cure CMD and SAM's ultimate goals are the same: to find effective treatments and eventually a cure for Congenital Muscular Dystrophy. To date, SAM has raised approximately \$200K in total. Through our alliance, SAM has provided financial support for the set up and maintenance of the Congenital Muscular Dystrophy International Registry. Additionally, SAM has allocated \$100K for a S.A.M. CMD Translational Research Grant to be awarded in 2010; made possible through Cure CMD and SAM collaboration and the Cure CMD research grant funding mechanism.



SAM & Hillsborough Oyster Festival Most recently, SAM had the honor of being chosen as the featured charity for the 17th Hillsborough Oyster Festival, the second largest event on the Northern Irish calendar. The festivities spanned four action-packed days and nights, opening with a Fashion Extravaganza in the festival marquee showcasing ladies and menswear from local boutiques. On Friday evening the fabulous Oyster Festival Masquerade Ball took place which this year raised a record amount via donations and an exclusive charity auction. On Saturday the whole village came alive with a gourmet market, carnival and the epic Soapbox Derby which this year was held on cardiac hill (otherwise known as Hillsborough Main Street) - a great laugh for the 12,000 visitors and locals who packed the streets of our picturesque Georgian village for the day! On Sunday hundreds of car enthusiasts young and old braved the rain to enjoy Super Dreamcar Sunday for an afternoon of motoring exotica, which included Ferraris, Porsches, Aston Martins, rally cars, and a selection of 'superduper cars'. In all, the Oyster Festival events raised a whopping \$35,000 and this money will form a substantial part of a second SAM research grant, which will be awarded in early 2010. We are so incredibly grateful for such terrific support from our local community and would like to thank everyone involved in the Oyster Festival for their dedication and hard work!

2009 - 2010 Calendar of Events

What	Event	When	Where	Who
Medical Conference	National Society for Genetic Counselors	Nov 11, 2009	Atlanta, GA	Cure CMD staff will exhibit information about the FIRST and ONLY CMD International Patient Registry (CMDIR). The goal is to drive genetic counselor awareness about the CMDs, the need for genetic confirmation and referral of people with CMD to the CMDIR. We'd like to thank Mike Newton, Joey Bower, Jeff Moore and Judy Moore for their investment in attending the NSGC, staffing the Cure CMD booth and driving CMD awareness.
Conference	CMD Standard of Care Workshop	Nov 14, 2009	Brussels, Belgium	Cure CMD has led this initiative with Dr. Ching Wang and Dr. Thomas Sejersen towards development of an 80 member international CMD SOC committee and an online professional survey of CMD care issues. This initiative will culminate in this Nov conference, bringing together 39 international physicians to draft a CMD Diagnostic Review and CMD Care Guideline (professional/lay person). This conference is generously supported by TREAT-NMD, AFM, FTELE and Cure CMD.
Conference	TREAT-NMD/NIH Translational Conference	Nov 17, 2009	Brussels, Belgium	This conference seeks to address barriers to developing translational scientific approaches and identifying drugs to treat rare neuromuscular diseases. Cure CMD's Anne Rutkowski has been invited to present Cure CMD's accomplishments in the last year with the broad support of the CMD community and the international physician, scientist, advocacy and government communities. Anne will discuss the CMDIR, the CMD SOC initiative, the Cure CMD grant award mechanism and conferences co-organized
Meeting	Muscular Dystrophy Coordinating Committee	Nov 30, 2009	Bethesda, Maryland	Muscular Dystrophy Coordinating Committee is the oversight committee mandated by the MD Care Act. This committee brings together the program directors of each NIH institute involved in funding muscular dystrophy research and the CDC and Department of Defense. This meeting is open to the public (if you would like to attend, please email anne.rutkowski@curecmd.com to send you a registration form). Cure CMD's Anne Rutkowski has been nominated and selected to sit on the MDCC committee. Nov 30th, Hyatt Regency Bethesda, Bethesda, Maryland (7:30am- 5pm).
Fundraising	Pedal 4 Pierce	Dec 12, 2009	Canberra, Australia	This terrific Aussie Fundraising event was the brainchild of a 10 year old boy who decided to do something to raise funds for his friend, Pierce's disease (CMD).
Fundraising	Cure CMD Team runs/walks NJ 1/2 Marathon	May 2, 2010	Long Branch, NJ	Cure CMD has teamed up with Cecilia Kodoma to organize the NJ 1/2 marathon fundraising event. Each team member has pledged to raise \$500 and roll/run/walk in support of CMD awareness! The event will kick off with a pre-1/2 marathon spaghetti dinner. We hope you will consider supporting a Cure CMD team member by pledging in their name or joining the Cure CMD Team. Look for 2 other 1/2 marathon events in the upcoming year.
Fundraising	Kyra's Krusaders Fundraising Family Event	June 26, 2010	Pipestone, Minnesota	Emily Ludolph is organizing the second annual Mud Volleyball and Family Event on June 26th at Swanny's Bar and Grill in Pipestone, Minnesota. If you are a local family or have ties to friends and family in the Minnesota area, reserve the date and come out and show your support!

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