

## Newsletter

# CNM – Together Strong!

## International conference on myotubular and other centronuclear myopathies

Under the motto "together even stronger" the [European Conference for Myotubular and Centronuclear Myopathy](#) took place from May 6-8 2016. It was organised together with the [Myotubular Trust](#).

On this sunny weekend, over 100 participants gathered together near Frankfurt in Germany. There were 45 families represented from 12 countries, and 16 researchers from Germany, France, the Netherlands, UK, US and Canada. The conference was presented in both German and English with simultaneous translation via headsets.

The weekend began with a beautifully colorful spectacle when everyone released a balloon and a wish into the sky. This was followed by a relaxed and happy get together over dinner and drinks.

On Saturday, conference delegates heard about the latest developments in research findings, as well as other studies and initiatives in the field of centro-

nuclear and myotubular myopathy. We were so fortunate to have had such great support from the academic community and their presentations were greatly received. On Saturday afternoon and Sunday morning there was a series of workshops on topics that ranged from Pre-Implantation Genetic Diagnosis, Firefly adapted toys and seating, outcome measures for clinical trials, and updates from Audentes Therapeutics on the clinical studies on gene therapy.

Children and teenagers had a chance to enjoy a program especially organized for them on Friday, Saturday and Sunday. A funny puppeteer made all children laugh and they were also able to act with real actors to play Red Riding Hood. Older children enjoyed playing soccer and going on a treasure hunt.

Sunday was Mother's Day in Germany, and all mothers got a special present: sunflower seeds of [The Big Sunflower Project for Centronuclear and Myotubular Myopathy 2016!](#) We now hope that Sunflowers will grow in all homes of all participants and with them the good hope and good spirit of the conference!



*Some of the participants of the family conference in Niedernhausen near Frankfurt*

## About us:

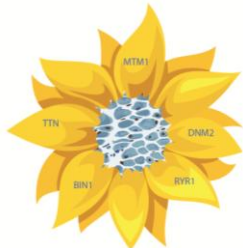
**CNM – Together Strong (ZNM - Zusammen Stark!)** was founded on June 5, 2015 by parents of children with [centronuclear myopathies \(CNM/ZNM\)](#).

**Centronuclear Myopathy** is an umbrella term for a few very rare muscle conditions, e. g. myotubular myopathy. They have in common that the nuclei of the muscle fibres are located in the centre (centronuclear) and not as usual at the edges of the muscles.

**Our goals are to give the families a voice, to share information on CNM and the latest research and to sponsor grants for research on a cure.**

**As these conditions are so rare hardly any public funding for research on a cure exists and before CNM – Together Strong! no registered association represented patients and their families in Germany..**

**We want to change this!**



### Donation to the Myotubular Trust

During our joint conference in May 2016 our association ZNM – Zusammen Stark! was able to hand over a check of **10,000 €** to the English Myotubular Trust. This decision was taken unanimously by the members’ general meeting on the previous day. Thank you so much to all our supporters who have made this possible!

The Myotubular Trust was founded in 2006 with the aim to support research in the field of myotubular and other centronuclear myopathies. For this purpose every year, the Myotubular Trust grants research projects. The [scientific](#)

[advisory board](#) then decides which projects will be funded.

Thus, our donation contributes directly to the research grants of 2017!

We would like to hand over another check by Christmas 2016. For this we rely on your support! We are very happy and grateful if you want to contribute. Every single Euro counts! By the research grants of the past so many has been reached already!

[www.myotubulartrust.com/research\\_programmeandgrants.htm](http://www.myotubulartrust.com/research_programmeandgrants.htm)



*This QR-Code leads you directly to our conference page with programme, photos and films and pdfs of the presentations*



*Frank Schulte, Tracy Ulmer, Melanie Spring, Holger Fischer, Anne Lennox*



*Tracy Ulmer & Hannes Oldenburg  
Vice-President & cash control officer*



*Aart den Hollander & Arlene Wüstner  
ZNM representatives in the steering committee of the Treat NMD patient registry*

### New responsible members in our association

Together with Holger Fischer and Frank Schulte **Tracy Ulmer** is now part of the management board. She was elected unanimously by the members’ general meeting. In the same way, **Hannes Oldenburg**, was elected as cash control officer.

In addition, **Aart den Hollander** and **Arlene Wüstner** will represent our association in the steering committee of the NMD patient registry ([mtmcmregistry.org](http://mtmcmregistry.org)) [www.treat-nmd.eu/](http://www.treat-nmd.eu/)

Thank you for your commitment!



# Research news

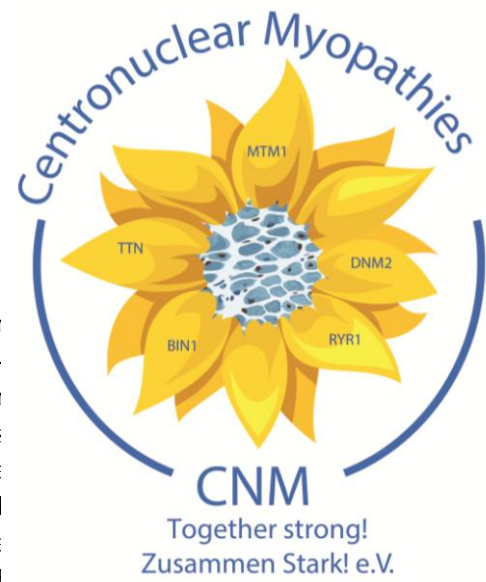
## New therapy to cure several CNM?

Myotubular myopathy (MTM) is caused by a mutation in the MTM1 gene, which is responsible for the production of a protein called myotubularin. People affected by MTM do not produce this protein and have severe muscle weakness. Other centronuclear myopathies (CNM) are caused for instance by an alteration of the DNM2 gene, which produces the protein dynamin. In people affected by CNM linked to dynamin, we believe dynamin is present in the muscle but probably acting in an uncontrolled manner. Consequently, either a lack of myotubularin, or dynamin going 'wild', will lead to a muscle disease.

Some therapies to cure MTM rely on the addition of the missing myotubularin in the patients' muscles. In our group we follow a different approach: we want to keep dynamin under control. We do not yet fully understand how myotubularin and dynamin interact in muscles. But we do know that the balance or equilibrium between myotubularin and dynamin in muscle cells is very important. In MTM patients, this balance is disrupted as more

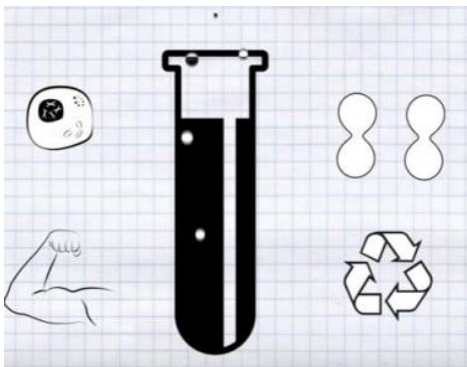
Dynamin is found in the muscles than in the ones of healthy people. In our experiments, we reduced dynamin in mice models with MTM. The results were very promising: the MTM mice with a reduced amount of dynamin had more strength than before and survive as long as healthy mice. This allowed the MTM mice to walk around the cage and even swing hanging from a wire supporting their whole body weight. This improvement on the muscle strength was also obvious on the cell structure of the MTM mice with reduced dynamin. The cell nuclei moved from the center, typical location in MTM patients, to the normal position in healthy muscles, at edge of the cell. Additionally, MTM mice with reduced dynamin do not show negative effects on other body organs.

Because of these great results with mouse models, we have now founded a start up together with industrial partners. The company Dynacure is dedicated towards developing a human drug to test it in clinical trials with patients in the near future.



[Dr. Belinda Cowling](#), Pathophysiology of neuromuscular diseases, department translational Medicine and Neurogenetics, [IGBMC, Illkirch, France](#)

## ZNM celebrates the first birthday with a film



Screen shot of the short film

For our first anniversary on June 5 we thought to do something special. For this reason we have published a short film with the title: [„What is myotubular myopathy? What is centronuclear myopathy?“](#)

Thank you to all who have contributed to this project without charging anything: speaker René Wagner, scientific advisor Johann Böhm, IGBMC and Clemens Maucksch, filmmaker.

In the meantime this film is available in English. More languages will follow, on our ZNM youtube-channel:

<https://youtu.be/2HrUeetT25s>



On June 5 we have celebrated our first birthday

## Our association:

ZNM – Zusammen Stark! e. V.

(CNM - Together Strong!)

Founded on 05 June 2015

**Members:** 126, of these 33 families with people with CNM (October 2016)

### Address:

Käte Hamburger Weg 8,  
70569 Stuttgart

### Management board:

President:

Dr. Holger Fischer  
[holfischer@gmail.com](mailto:holfischer@gmail.com)

Vice President:

Tracy Ulmer

Treasurer:

Frank Schulte

Our association is registered at the county court in Stuttgart.

We are officially recognised charity (tax number: 99018/60576)

### Bank Account:

IBAN :  
DE33 2789 3760 2051 0918 00

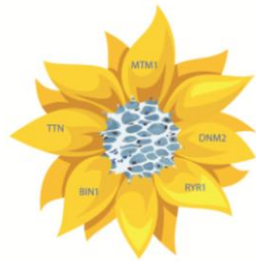
BIC: GENODEF1SES

### Visit our Homepage:

[www.znm-zusammenstark.org](http://www.znm-zusammenstark.org)

Like us on Facebook:

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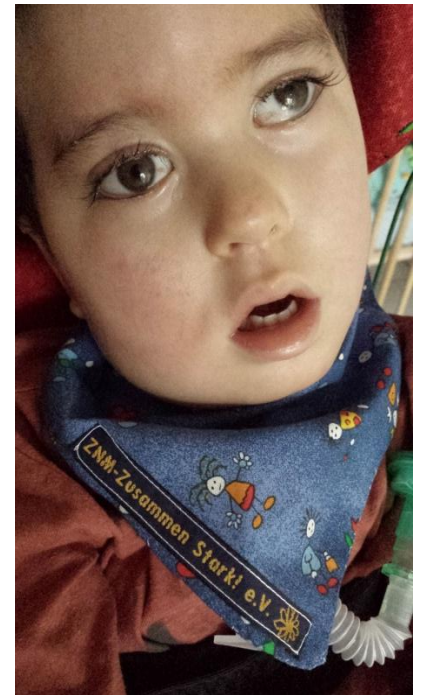
Association for Centronuclear Myopathies

## Emil in our hearts

Emil, a sweet 2-year-old boy from Stuttgart unfortunately lost his battle against MTM this May. Emil inspired his parents, Jen and Holger, to found this association and will continue to inspire us all to continue fighting for children with MTM and CNM. Emil, you will always remain alive in our thoughts and our hearts, like all other children with MTM and CNM, who had to leave way too early! We miss you so much, but at the same time we are endlessly grateful for your life and for showing us, what is important in life: to love and be loved in return!

*„Emil felt marvelously relieved. It is true, bad luck remains bad luck. However, to have a few buddies who voluntary join you and help you, this is a wonderful feeling.“*

*(Erich Kästner, Emil and the Detectives)*



More on Emil's adventures you can read in his blog:  
[emil-augustin.blogspot.de](http://emil-augustin.blogspot.de)

## Our plans for 2017:

In May 2017, we will organize again a family conference. We will invite therapists, who will show us how to better care for our family members with MTM/CNM or ourselves. For the children and teenagers, we will organize a very nice program.

Moreover, we will continue supporting various research efforts from our scientific community devoted to find a cure or a treatment for patients with MTM/CNM.

We thank you in advance for your support financing these activities!

## How you can help:

We are so happy, if you are willing to support us. - Every single Euro counts!

There are different ways for every budget to help:

- Without extra costs you can support us through the online platform [good-ing.de](http://good-ing.de) - just visit the site before you do your online shopping and ZNM gets a small bonus.

- you can also make a direct donation by bank transfer (account no. on the left) or by using the platform [betterplace.org](http://betterplace.org) - this also works from abroad, by credit card or PayPal.

- or do you want to support us on a regular basis? Then, you might want to become a sustaining member of ZNM. The application form can be found on [our homepage](http://our.homepage).

