OMSlife Foundation

Volume 5 - May 2016

BREAKING NEWS: OMSLIFE FOUNDATION RECEIVES GRANT FROM NORD

The OMSLife Foundation was recently notified by the National Organization for Rare Diseases (NORD) that it was a finalist to participate in their Natural History Studies. The study is a cooperative effort with the FDA, which will work with twenty NORD organizations to build a patient registry. This is a milestone event on two fronts; it is the first major grant directed to OMSLife, and it accelerates the schedule to build a patient registry.

The grant provides OMSLife with access to technology and resources for five years to build the patient registry. The vision of the patient registry is threefold; provide data for research, show trends and best practices for treating physicians, and provide ample information for OMS caregivers in making critical decisions regarding their OMS warrior's health. This third point is very important, as many OMS caregivers have to rely completely on their doctor's directive since they do not have access to the medical publications on OMS.

The registry will provide data on treatments, therapies, tumors, scans, behavior issues, schooling assistance, treating physicians and hospitals, insurance, and transition to adulthood.

NORD'S
Natural History
Studies Project

For more information on this project, visit our website at:

www.omslifefoundation.org

or visit the NORD website at:
www.rarediseases.org

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OMSLIFE HOSTS CAREGIVER CONFERENCE IN LOS ANGELES

The fifth regional OMSlife Caregiver's Conference was held on March 12 in Los Angeles. Hosted by the OMSLife Foundation, this was the second time the conference has been held at Children's Hospital Los Angeles. Keynote speakers for the conference were two world renowned OMS specialists: Dr. Wendy Mitchell from CHLA, and Dr. Michael Pranzatelli from the National Pediatric Neuroinflammation Center in Orlando, Florida. These two researchers and clinicians have seen almost 500 OMS warriors in their combined 50 years of experience.

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Meet Three OMS Warriors: Cole, Lucy, and Ellie

Cole: Cole was diagnosed with a neuroblastoma and Opsoclonus Myoclonus Syndrome (OMS) in July of 2011 at 15 months old. His symptoms started with a viral infection and fever. Within hours, we began to see neurological changes that continued to progress over the next few days. His initial symptoms consisted of hand tremors, body tremors, and ataxia. He was not walking yet, but he started having difficulty crawling and pulling himself up to a standing position. He was extremely tremulous and irritable. He was initially misdiagnosed with cerebral ataxia due to a viral infection. A week after this diagnosis, I noticed irregular eye movements, staring spells, and sleep abnormalities in addition to his persistent ataxia. These erratic eye movements were what we came to know as opsoclonus. I remember how sad I felt when I realized he had quit babbling in the morning and quit rising up to greet me in his crib.

I remember the world crashing down all around us when we got our final diagnosis. We were told that there had not been much funding or research towards OMS and little was known about the disease or how to treat it. We were also told of the challenges our son and family would face in the future and that the statistics for relapses and long-term disabilities were not in our favor. However, we were blessed that our pediatric oncologist and neurologist had diagnosed and treated OMS before and were able to get us an accurate diagnosis within 3.5 weeks from onset. They were also familiar with Dr. Pranzatelli and his research and the COG study. We were given all the available options for treatment and supported with every decision along the way. I cannot say enough how grateful I am for Dell Children's in Austin and for the team of doctors surrounding us during that time and today.

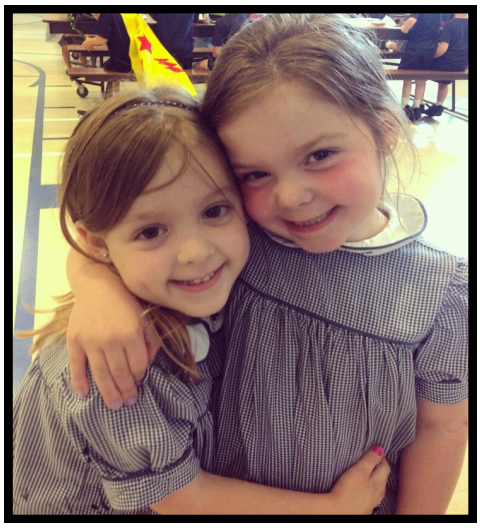
Immediately after resection of the tumor, Cole was started on high dose daily prednisone, IVIG every 4 weeks, and a full cycle of Rituximab (consisting of 4 infusions once per week). We prayed the hardest we had ever prayed and believed the hardest we had ever believed. With every prayer and every dose, we would see significant improvement. Cole was back to baseline within 4 weeks of starting treatment. He did relapse twice after that and each time we re-dosed with Rituximab (4 infusions) and switched from daily prednisone to monthly pulses of dexamethasone. On the last Rituximab re-dose, we weaned him off dexamethasone and finished out that year with IVIG infusions every 4 weeks. Each time we would re-dose with Rituximab, we would see all symptoms disappear. We were able to wean him off IVIG for a full year. During this time, he started Kindergarten and has been doing extremely well. However, during a follow up with our oncologist in December 2015, we discovered he was not making immunoglobulins, possibly due to the multiple doses of Rituximab (this condition is called hypogammaglobulinemia). Since then, we have restarted monthly infusions of IVIG to provide him with some immunity in hopes that he will soon start making his own immunoglobulins. This has been a long journey with many ups and downs. We still hold our breaths if we hear him cough or notice him tripping or notice he is too quiet, but we have never lost hope. With every sad tear I have shed, I have also shed tears of happiness; happiness for watching my son do some of the simplest things in life that many others take for granted. Never lose faith. Never lose hope. *(I tell you therefore: Everything you ask and pray for, believe that you have it already, and it will be yours- Mark 11:22-25)*

Please help us provide grants for OMS research. You can send checks to:

The OMSLife Foundation
P.O. BOX 2899
Cypress, TX 77410

Or give an online donation at
www.omslifefoundation.org

The OMSLife Foundation is a 501(c)3 non-profit organization and donations are tax deductible.

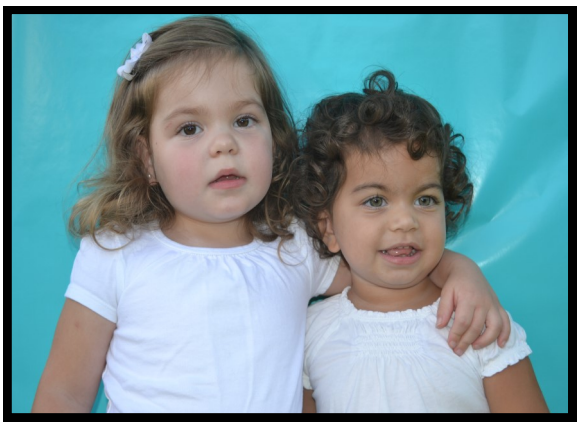


Lucy was diagnosed with OMS in November 2011 at the age of fourteen months. Until that time, Lucy and her twin sister Grace were both developing normally. Lucy went from a typical toddler to an infant literally overnight. She could no longer walk, crawl, sit-up, or hold up her head independently. She had difficulty eating, sleeping and was extremely irritable. She was diagnosed with OMS within just a few days of onset by Dr. Deputy, a Pediatric Neurologist at Children's Hospital New Orleans. Since he had minimal experience with OMS, he quickly put us in contact with Dr. Pranzatelli, who was able to confirm her diagnosis and develop a treatment plan. Within two weeks from onset of symptoms, Lucy began treatment, which consisted of ACTH, four rounds of Rituximab, and monthly IVIg infusions. Lucy also received intensive Physical, Occupational, and Speech therapies.

Lucy began to respond to treatment and was doing quite well for about six months before she had her first relapse. Over the years she has had several relapses which have all coincided with the tapering of medication (primarily ACTH). Finally after almost four years, Lucy was able to successfully taper off of ACTH with the addition of other medications (cyclophosphamide, methotrexate, and dexamethasone pulse therapy). Lucy is currently in the process of tapering dexamethasone and eventually methotrexate and IVIg will follow. She is still receiving speech and occupational therapy. She has come a long way on this journey.

Today Lucy is a happy five year old who loves to swim, run, play and do just about anything with her twin sister Grace. She is enjoying her first year at school (Pre-K 4) and can't wait for summer. She has endured so much in her short life and has amazed us with her courage and strength. She has the determination and stamina to one day reach remission. We feel so blessed to have the OMSLife Foundation as a resource of information and a network to connect with other OMS families. We have connected with many families over the years and it is very heartwarming to be able to reach out so easily. At one time we were very fortunate to have another OMS family (Ellie D'Souza) literally in our neighborhood. The opportunity to form such a close bond with another family with the same rare disease was both reassuring and comforting.

Ellie: Ellie was diagnosed with OMS at 19 months old in January 2013 at Children's Hospital of New Orleans after showing progressive ataxia for a month and then opsoclonus. The neurology group at CHNO was quick to diagnose Ellie once she showed opsoclonus, due to their familiarity with OMS from Lucy. We were so blessed that the doctors were already working with Dr. Pranzatelli to treat to Lucy and we were on our way to see him within a week of dx. Ellie responded well to treatments (IVIG, ATCH and Rituxan) and initially no tumor was found. 10 months after diagnosis, a ganglioneuroblastoma was found during routine scans. We were able to remove the tumor without adding any new treatments. We were truly blessed to be so close to another OMS family and not only have our own amazing support group but friends as well. We were sad to move away from them in Fall 2014 to Houston, Texas. We transferred her care to Dr. Timothy Lotze's team at Texas Children's Hospital and she has been off treatment since March 2015 and is starting physical and occupational therapy to help her catch up with motor skills delays. Ellie is turning 5 in May, will be entering kindergarten in the Fall and loves her dance and gymnastics classes. Throughout our OMS journey, Ellie has remained a positive, happy child!



OMS Warriors Lucy and Ellie

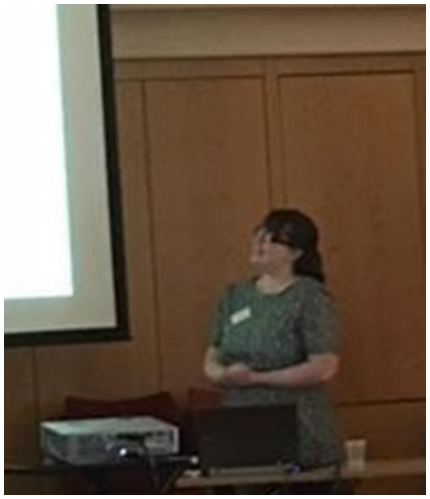
OMSLIFE CO-SPONSORS EIGHTH INTERNATIONAL OMS MEDICAL CONFERENCE

OMSLife had the honor in April of co-hosting the eighth International Workshop on Opsoclonus Myoclonus Syndrome Clinical and Basic Science Conference held in Abingdon, England. The conference brings the best medical authorities in OMS research and clinical studies together. This year, doctors and caregivers from nine countries were present to spend three days in discussions on OMS research trends. Sessions included clinical trials, current grants investigating OMS, education guides for caregivers, and potential new treatments.

This medical conference is unique in that it is hosted by foundations in the UK and US run by OMS caregivers. The steering committee is comprised of caregivers and medical staff.

Over the years, this conference has brought research results to light, allowed the group to identify critical research needs, and openly discuss opportunities for research funding. As a result, OMS research funding has increased over the years and collaborative research is being done.

Thank you to the Dancing Eyes Syndrome Support Trust in England for beginning this conference fifteen years ago and for allowing OMSLife to co-host the 2016 event.



Nichola Ejaz of DES Trust presenting at the OMS Conference

Eighth International OMS Medical Conference Attendees—April 2016

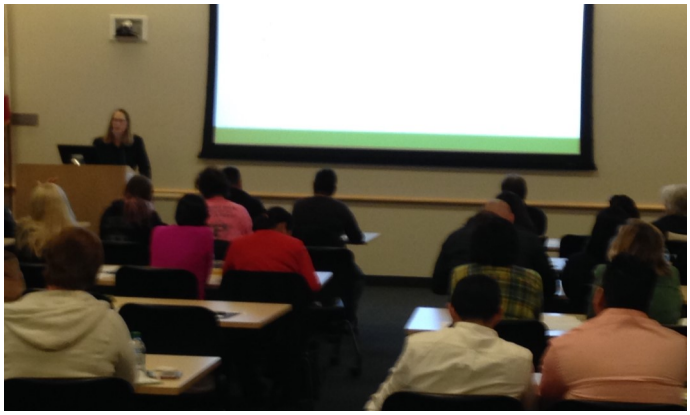
Caregiver Conference-continued from p.1

Dr. Mitchell stepped the audience through the progression of OMS from making the diagnosis, to initial treatments, weaning medications, relapses, and quality of life. Dr. Pranzatelli spent much of his discussion on relapses including causes and treatment when they occur, therapies, and patient registries.

Roughly seventy caregivers attended the conference from thirteen states plus Canada. Other speakers for the event included Liz Tate from National Pediatric Neuroinflammation Center, Dr. Mark Borchert and Dr. Araz Marachelian; both from CHLA.

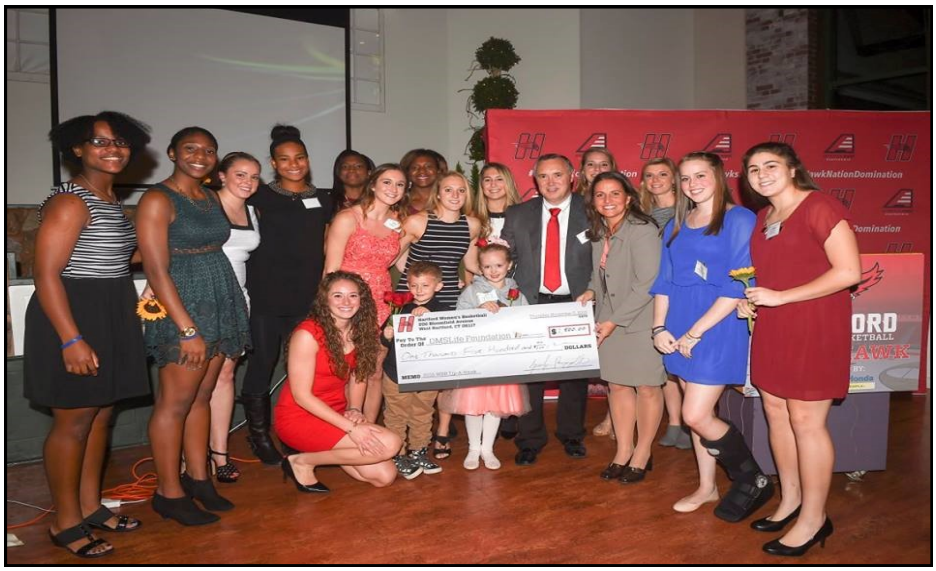
That evening, the caregivers and their families took an opportunity to meet for a reception at the Embassy Suites. This was a chance for OMS warriors to meet each other and for caregivers to compare notes in their battle of the disease.

This conference was the first in which we invited specialists outside of the region to participate. It was such a success that we are now making plans for a national gathering in 2017 in Houston. Our hopes are that we can invite the top specialists in the country to participate in the event with our caregivers. More details to come soon!



Liz Tate presents at the OMSLife Caregivers Conference in Los Angeles

MEET ONE OF OUR SPONSORS - HARTFORD HAWKS WOMENS BASKETBALL TEAM



OMSlife Foundation president Mike Michaelis and OMS warrior Zoe receive a check from UH Women's Basketball Team

The University of Hartford Women's Basketball team is no ordinary division 1 team. Members of the team are learning skills on and off of the court by serving those less fortunate. Such is the case when they aligned with Team Impact, an organization that matches children battling life threatening and chronic illnesses with college athletic teams.

The Hartford Hawks Women's team brought seven year old OMS warrior Zoe on to their team. Zoe attended practices and games with the team. Even when not at practice, the team was with Zoe, whether baking cookies at her house or bowling with her family. She was one of the team, and the team became one of the family.

The team invited OMSLife Foundation president, Mike Michaelis to speak at their annual Tip-A-Hawk fundraiser for the team. This is an annual event to raise money for team expenses, but part of the proceeds goes to a charity. During the festivities, the team presented OMSLife with a check in honor of honorary teammate Zoe.

Later in the season, the team hosted a second event during one of their home games. They raised awareness of OMS through video clips, collected teddy bears for kids at the local children's hospital and raised money for OMSLife. Thank you to the Hartford Hawks Womens Basketball team for being such a tremendous teammate and role model to Zoe!

OMSlife president Mike Michaelis speaks at the annual Tip-A-Hawk fundraiser.

OMSLIFE JOINS NORD

The OMSLife Foundation was recently accepted as a member of the National Organization for Rare Disorders (NORD). NORD is the leader in providing support and services for those people afflicted with rare diseases. Their goal is to improve the lives of those individuals and families afflicted by rare diseases. They do this in a number of ways such as patient advocacy, education, and research. They also pioneered the patient assistance program to help patients unable to get the medications required to battle their disease. For more information on NORD, visit their web site at **www.rarediseases.org** .

THE MISSION OF THE OMSLIFE FOUNDATION IS TO RAISE AWARENESS OF OMS, DEVELOP A SUPPORT NETWORK FOR CAREGIVERS, AND FUND RESEARCH FOR A CURE

MEET THE OMSLIFE BOARD OF DIRECTORS

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The OMSLife Foundation
P.O. BOX 2899
Cypress, TX 77410

OMSLIFE FOUNDATION—REVIEW OF RECENT ACTIVITIES

- **Caregiver Conferences** Hosted events in Los Angeles, Houston, Cincinnati, and Boston that connected OMS caregivers
- **2015 Fundraiser** Raised over $40,000 for OMS research and awareness
- **Web Site and Facebook Page** Connecting new OMS warriors with OMS specialists, and raising awareness of OMS
- **New members** Have added over 300 new warriors to our forum since 2014.
- **Surveys** Beginning pilot program to collect OMS data for researchers and clinicians
- **Patient Registry (NEW!)** - In development stages. Should be deployed in late 2016.
- **2016 International Medical Conference** - Co-hosted event in April for medical professionals around the world.

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HELP US BEAT OMS!!!! I want to help raise awareness of OMS and help in research funding! Enclosed is my donation.

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$100 ☐
$200 ☐
OTHER ☐

☐

Check the box if you like to receive our e-newsletter.

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The OMSLife Foundation is a 501(c)(3) nonprofit organization founded in 2012.