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The Procko Family

**Scientist of the Month**

Dr. Kanneboyina Nagaraju

**Upcoming Events**

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Champions to CureDuchenne, Newport Beach Gala, February 9, 2013, Newport Beach, Calif.

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

## Repeat Dosing of SMT C1100 For Treatment of Duchenne Muscular Dystrophy Meets Endpoints in Phase 1 Clinical Trial

Summit, a UK drug discovery company, announced on November 7 that the repeat dosing of the utrophin upregulator SMT C1100 for the treatment of the fatal muscle-wasting disease Duchenne Muscular Dystrophy ('DMD') has successfully met the endpoints as part of a Phase 1 clinical trial in healthy volunteers. The trial evaluated a new formulation of SMT C1100 and the results showed that upon repeat dosing, concentrations of the drug achieved in the blood plasma, stabilized at levels that from preclinical studies are expected to significantly increase utrophin protein production. The new formulation was also shown to be safe and well-tolerated in this Phase 1 trial.

SMT C1100 is a potential disease-modifying, oral small-molecule that works by upregulating (increasing) the amount of a naturally occurring protein called utrophin to maintain the healthy function of muscles. These data strongly support the progression of SMT C1100 into the next stages of development that includes biomarker and long-term safety studies, which will be required before a DMD patient efficacy trial could commence.

"Utrophin upregulation is a unique approach for treating DMD because it could benefit all DMD patients, regardless of their underlying genetic fault," commented Glyn Edwards, Chief Executive Officer of Summit. "We are highly encouraged by these results, as the new formulation achieves blood concentrations that have the potential to significantly increase utrophin levels, with the outcome of maintaining the healthy function of muscles in patients with DMD. The results therefore strongly support continuing clinical evaluation of SMT C1100."

The double blind, placebo-controlled Phase 1 trial examined a new nanoparticle aqueous suspension of SMT C1100 in a total of 48 healthy volunteers. The previously reported results from the single ascending dose cohort showed SMT C1100 to be safe and well-tolerated at all doses. These new data are being reported from the repeat dosing cohort where the volunteers received 100mg/kg twice daily for nine days. These results show that in all volunteers the blood plasma concentration of SMT C1100 stabilized after four days of dosing above the required level expected to increase utrophin protein production by 50% for at least 14 hours a day in a preclinical model. The plasma levels achieved were equivalent to those that gave significant therapeutic benefit in the gold standard disease model of DMD.

The Phase 1 trial has received funding from a group of U.S. DMD foundations: the Muscular Dystrophy Association, Charley's Fund, CureDuchenne, the Foundation to Eradicate Duchenne, Nash Avery Foundation and Parent Project Muscular Dystrophy.

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## Help Us CureDuchenne Now!

CureDuchenne is kicking off our end-of-year campaign: "Help Us CureDuchenne Now." Based on current scientific knowledge, Duchenne could become treatable for this generation of boys. It is urgent to fund critical research projects...Now.

Two companies recently announced meeting major milestones in late-stage clinical trials. One, with an experimental drug by biotech company Sarapeta Therapeutics, appears to preserve and possibly even improve the ability of boys with Duchenne to walk. Second, Dutch biotech company Prosensa has shown positive results in its Phase II trial and has enrolled 180 patients for a final Phase III trial.

CureDuchenne was an early investor in Sarepta's and Prosensa's research and donated a significant amount of the money needed to develop these drugs. The most advanced of the two drugs could receive FDA approval in 2014. It would be a triumph that happens rarely in our lifetimes a cure for a 100 percent fatal disease.

Unfortunately, these drugs will only help about 15 percent of Duchenne boys. CureDuchenne is committed to raising funds to get similar drugs to the other 85 percent of boys who so desperately need it. We can't stand by and watch as some boys receive this therapy and others don't.

With your support, we can ensure the best research moves forward rapidly and benefits as many Duchenne boys as possible. Just think....25 years ago, Duchenne was a barely understood disease and another generation of Duchenne boys sadly is gone. Today, we are on the brink of a major medical breakthrough that CAN save many of this generation of boys and your gift WILL make it happen.

Please consider making a gift to CureDuchenne. Your generosity can save a child from suffering pain, immobility and the certainty of dying by around the age of 20. NOW is the time to invest in this life changing and lifesaving research! This could be the last generation of Duchenne boys we lose, or the first generation of Duchenne boys who are saved.

Please click [here](#) to help us CureDuchenne Now.

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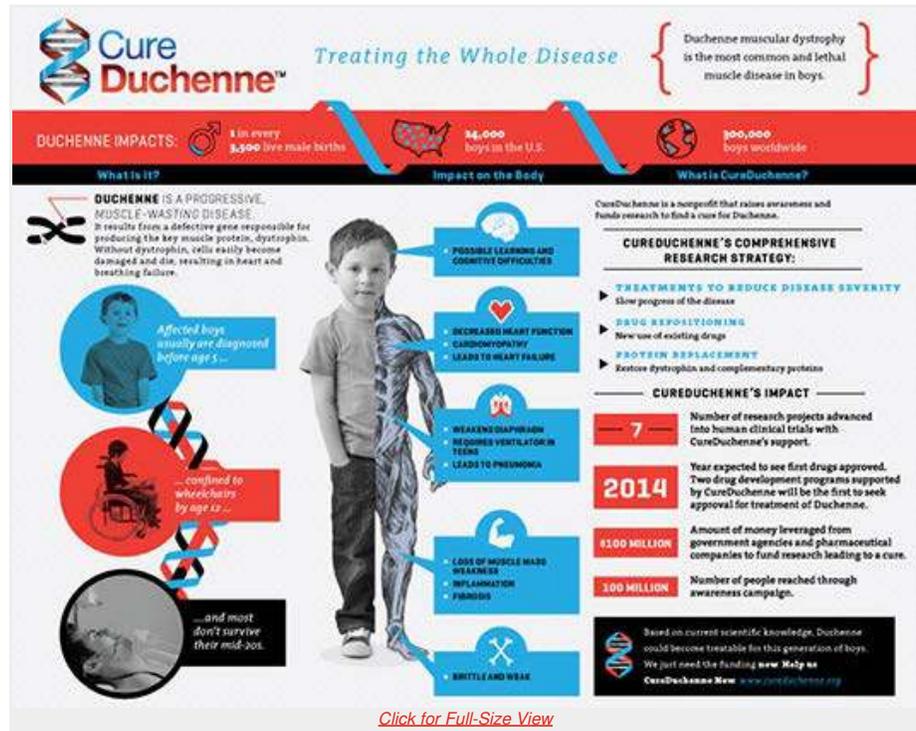
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**New CureDuchenne Infographic**



**CureDuchenne™ Treating the Whole Disease**

Duchenne muscular dystrophy is the most common and lethal muscle disease in boys.

**DUCHENNE IMPACTS:**

- 1 in every 3,500 live male births
- 14,000 boys in the U.S.
- 100,000 boys worldwide

**What is it?**

**DUCHENNE IS A PROGRESSIVE, MUSCLE-WASTING DISEASE.** It results from a defective gene responsible for producing the key muscle protein, dystrophin. Without dystrophin, cells easily become damaged and die, resulting in heart and breathing failure.

**Impact on the Body:**

- POSSIBLE LEARNING AND COGNITIVE DIFFICULTIES
- DECREASED HEART FUNCTION, CARDIOMYOPATHY, LEADS TO HEART FAILURE
- WEAKENS DIAPHRAGM, REQUIRES VENTILATOR IN TEENS, LEADS TO PNEUMONIA
- LOSS OF MUSCLE MASS, WEAKNESS, INFLAMMATION, FIBROSIS
- BRITTLE AND WEAK

**What is CureDuchenne?**

CureDuchenne is a nonprofit that raises awareness and funds research to find a cure for Duchenne.

**CURE DUCHENNE'S COMPREHENSIVE RESEARCH STRATEGY:**

- TREATMENTS TO REDUCE DISEASE SEVERITY**
  - Slow progress of the disease
- DRUG REPOSITIONING**
  - New use of existing drugs
- PROTEIN REPLACEMENT**
  - Restore dystrophin and complementary proteins

**CURE DUCHENNE'S IMPACT**

- 7** Number of research projects advanced into human clinical trials with CureDuchenne's support.
- 2014** Year expected to see first drugs approved. Two drug development programs supported by CureDuchenne will be the first to seek approval for treatment of Duchenne.
- \$100 MILLION** Amount of money leveraged from government agencies and pharmaceutical companies to fund research leading to a cure.
- 100 MILLION** Number of people reached through awareness campaign.

Based on current scientific knowledge, Duchenne could become treatable for this generation of boys. We just need the funding now. **Help us CureDuchenne Now** [www.cureduchenne.org](http://www.cureduchenne.org)

*Click for Full-Size View*

CureDuchenne is delighted to launch our new infographic. This is a visually compelling way to explain Duchenne muscular dystrophy and the impact it has on the body. The infographic also describes CureDuchenne's comprehensive research strategy and the impact we have had to date in helping to fund Duchenne research.

We hope that this at-a-glance look at Duchenne helps increase awareness and raise funds to help find a cure for Duchenne. We encourage you to share this through social media and ask your family, friends, neighbors and coworkers to help us CureDuchenne Now by making a donation. Click [here](#) to donate.

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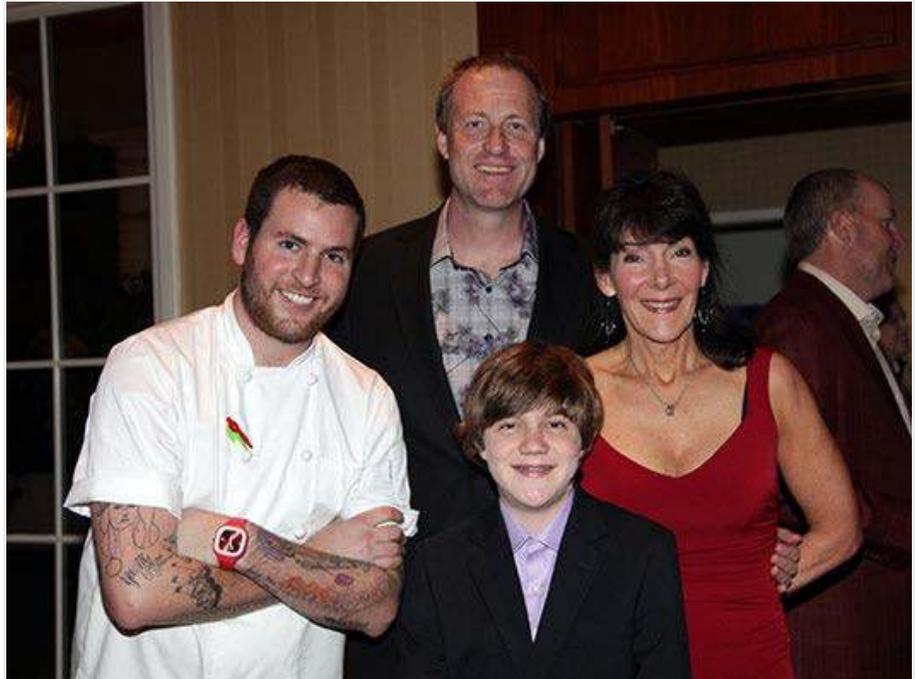
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**Champions to CureDuchenne Newport Beach Gala**

*Paul and Debra Miller, founders of CureDuchenne, and their son Hawken with chef Jason Quinn from the Playground Restaurant during the 2012 Champions to CureDuchenne Newport Beach Gala.*

The 2013 Champions to CureDuchenne Newport Beach Gala will be held Saturday, February 9 at the Balboa Bay Club & Resort in Newport Beach, Calif. This prestigious annual charity event benefits CureDuchenne.

Community, business and sports elite will come together for a fun and memorable evening that includes a cocktail reception, a special menu developed by a celebrity chef and Executive Chef Josef Lageder of the Balboa Bay Club, and an honoree presentation. The program will be followed by live music.

Tickets are now available. Please [click here](#) to purchase a table. If you are interested in sponsoring the Newport Beach Gala, please call 949-872-2552.

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## A Stranger's Estate Turns Into Donation for CureDuchenne

Jeannie McGrew, a hard working mother of four, worked as a Vons clerk for more than 35 years. McGrew saw many familiar faces in the store, and listened to shoppers people of all ages, races, and economic status as they shared their life stories. Peggy Bergmann, in her late 60s, shopped at Vons regularly. Bergmann always came alone, took the bus to the store and paid in cash. She was divorced, had no children and was an only child. Her parents had both passed away. These two women struck up a unique friendship.

McGrew did not ask too many questions. Their friendship was about doing things together in the present. Bergmann lived alone in a rented condo and McGrew would invite Bergmann to family holidays at her house. Last November, Bergmann was diagnosed with lung cancer and did not have long to live. She refused any treatment or medication and in the last weeks of her life, McGrew took care of her. Bergmann asked McGrew to go to her safe deposit box and help her get her affairs in order.

McGrew went to the bank, retrieved Bergmann's documents, and took them to an estate attorney. Bergmann had \$15 million of stock certificates sitting in that safe deposit box. Bergmann asked McGrew to help her find charities to give away \$1 million. Bergmann's illness happened very fast and within two weeks she had passed away.



Marc Weiss and Amanda McGrew, Jeannie McGrew's daughter.

McGrew had never been in the position to give much to charity. She and her husband, Brian, worked very hard to support their family, put their children through college and live a modest life in Santa Monica, Calif. McGrew asked her family to help identify charities to support. One of McGrew's children mentioned this story to a coworker, Marc Weiss, who said he had the perfect charity.

As a result of that conversation, a very substantial donation was made to CureDuchenne from Bergmann's estate: in honor of Marc Weiss. The gift was made to help Will Martin, who lives with Duchenne muscular dystrophy. CureDuchenne, and especially the Martin family, would like to acknowledge this extremely generous donation and the love and support given by strangers and family.

Thank you, Marc, for always having Will on your mind and in your heart. Now the McGrew, Weiss and Martin families will grow together, building on the friendship that Bergmann created through her generosity and philanthropic spirit.

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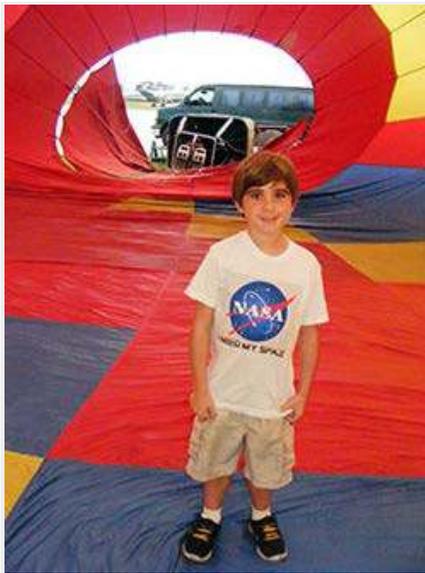
**Family of the Month - The Procko Family**

*Evan Procko (front) with his brother Billy, mom Kim and dad Bill on a camping trip.*

Evan Procko, 8, enjoys cooking, space science, art, music and hanging out with his friends. He doesn't like homework, needles or special attention. "He is a natural musician on the keyboard, no lessons, just discovery and melody coming together," said Kimberly Procko, Evan's mom. "When his music catches my attention and I stare at him, then he stops playing. He can't let it flow if someone is listening."

Bill owns and operates a health club in Branford, Florida, where Kimberly teaches kindergarten. Evan has an older brother Billy, 11, who does not have Duchenne. Billy is Evan's guardian. He helps carry Evan when needed and is always watching out for him. Kim and Bill do their best to keep life as normal as possible for their family. They spend each summer adventuring and living on the road in their camper van, traveling from Florida to Alaska and Canada. Bill has modified a jogging stroller, so they can all hike together into remote areas of wilderness. Their family motto is to "Live IN the Minute." They believe that confidence and positive thoughts are necessary in any survival situation.

Evan is one of 12 boys in the United States, participating in Sarepta's exon skipping clinical trial. Evan has endured needles, blood draws, infusions and special attention, as part of the trial. He and his dad, Bill, flew from Gainesville, Florida, to Columbus, Ohio, every week for 10 months to participate in the phase II trial. Evan has even been featured in the newspaper and on local TV stations to promote Sarepta's clinical trial.



*Evan Procko gets ready for a hot air balloon ride that his aunts and uncles gave to him after he had a third biopsy.*

Every Tuesday, Bill would pick up Evan from school, they would stop at a restaurant to pick up food to bring on the plane and then fly to Columbus. After doctor visits and infusions, they would fly home on Wednesdays. Evan made friends with TSA staff in the Gainesville, Atlanta and Columbus airports as well as restaurant workers, cab drivers and hotel staff on his weekly trips as they learned Evan's story about living with Duchenne muscular dystrophy. Evan was diagnosed with Duchenne at age 4.

The weekly trips were difficult on the tight knit Procko family. It was hard for Evan to be separated from his mom and brother. It was heartbreaking for Kim to see the tears he was holding back as she hugged him goodbye. Many times Bill had to pull Evan out of class and leave a prized project undone on his desk while his classmates were still working.

Now Evan is able to get his weekly infusions locally at the University of Florida. Evan was originally on placebo but is now on the actual drug and showing signs of improvement in his upper body strength.

"Duchenne is working against you at the same time the drug is trying to counteract the results, but we remain confident that this drug will work," said Bill. "When Evan was first diagnosed there was no chance for a treatment; now Duchenne is not as much as a sentence but more of a race against time."

"Being accepted in the Sarepta trial was an answer to our prayers," said Kim. "We believe with all our hearts that funding scientific research can save Evan's life and the lives of little boys like him all over the world. It has been a difficult journey for Evan. He is so sensitive to pulling a band aid off his body, much less enduring I.V.'s, blood draws and muscle biopsies. However, all that pales in comparison to viewing the slides showing new dystrophin in his body. It is nothing short of amazing."

When the Procko's first learned about Evan's diagnosis they were looking for resources and found CureDuchenne. They liked CureDuchenne's sole focus on raising money for research to find a cure.

"We had an immediate connection with CureDuchenne," said Bill. "CureDuchenne looks at what can help immediately and are always looking forward on what will work, what is the next step. This impressed us. With CureDuchenne we found hope. When we ask people in our community to donate their hard earned money we know that CureDuchenne will put the money to good use. They have proved it with the Sarepta trial."

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**Scientist of the Month - Dr. Kanneboyina Nagaraju**

Kanneboyina Nagaraju, PhD, DVM, an immunologist with an expertise in molecular mechanisms of target tissue injury in muscle disease, is a principal investigator at Children's Research Institute Center for Genetic Medicine Research at Children's National Medical Center and a tenured Professor of Integrative Systems Biology and Pediatrics. One of the main focuses of Dr. Nagaraju's laboratory is to perform preclinical drug testing on neuromuscular disease models especially in the mdx mouse model of Duchenne muscular dystrophy.



*Kanneboyina Nagaraju, PhD, DVM*

The FDA generally recommends therapeutic interventions to be tested on animal models to demonstrate the safety and effectiveness of the drugs before allowing the initiation of human clinical trials. The Duchenne mouse model is called the mdx mouse.

Dr. Nagaraju, in collaboration with TREAT-NMD and the muscular dystrophy community, helped to develop standard operating procedures (SOP) for preclinical assessments in muscular dystrophy animal models (mice and dogs). These SOPs are focused on methodologies to measure skeletal muscle, heart and respiratory function and has created a rigorous testing system that has become the gold standard for testing therapeutics in mouse models. These SOPs are available freely for other researchers around the world to follow so that these techniques are done right and results are more predictable and reproducible.

Dr. Nagaraju established preclinical drug testing facility after joining Children's National Medical Center in 2006. He had tested more than 50 drugs and majority (more than 90 percent) didn't show efficacy in the mdx mouse model. The disease severity in mdx mice is mild. There are obvious differences when testing mice compared to humans. The skeletal muscles of mice are different than humans such as walking on four legs vs. two legs. Mice that live in cages are restricted in their activity and can't run freely as they do in wild. To compensate for some of these variables, mice are run on a treadmill twice a week for 30 min at 12meters/min. When this is done one can unmask the mild disease phenotype in these mice, thus making this a better model to test drugs. Human patients are genetically heterogeneous and have different mutations with significant differences in disease progression. Therefore, it is impractical to make a mouse model that represents highly variable disease progression that we see in patients.

Mdx mice develop disease peaks muscle disease by 6 weeks and subside around 12 weeks of age and progresses sub-clinically until 9 months age. Cardiac and respiratory dysfunctions are detectable after 9 months age. Mdx mice have slightly lower life span than normal mice. Researchers can learn a lot by studying these mice. One needs to take some of this information into consideration to meaningfully interpret the usefulness of mouse data to human clinical trials.

"It is through efforts by CureDuchenne and other Duchenne organizations that have helped facilitate the advancement of drug testing in preclinical clinical trials," said Dr. Nagaraju. "It is group effort of the entire community that allows us to continue to test drugs to help find a cure for Duchenne and other rare diseases."





*"Together we can CureDuchenne"*

November 2012

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## Tinker Bell Half Marathon, January 18-20, 2013, Anaheim, Calif.

CureDuchenne is proud to be an official charity of the Tinker Bell Half Marathon. The Tinker Bell Half Marathon weekend is January 18-20, 2013.

Disneyland® Resort becomes Never Land for a magical weekend. It starts with a Family 5K and a Kids' Races and culminates with 13.1 mile run that weaves through Disneyland® Resort. To support a participant, [click here](#). For more information call CureDuchenne at 949-872-2552.

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**Champions to CureDuchenne, Newport Beach Gala,  
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Save the date for the Champions to CureDuchenne Newport Beach Gala on February 9, 2013 at the Balboa Bay Beach Club in Newport Beach, Calif. The gala will feature a celebrity chef, live music, dinner and drinks and a live auction. More details to come.

- Individual Tickets - \$350
- Table Sponsor (Table of 8) - \$2,500
- VIP Table (Table of 8) - \$5,000

[Click here](#) to register.

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## Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Join Dallas Cowboys and Texas Rangers players for a Bowling to CureDuchenne event on Sunday, February 10 from 2 p.m. to 6 p.m. at Lewisville Lanes AMF in Lewisville, Texas. There will be a celebrity bowling on each lane. There will be games, food, raffles and a silent auction. All proceeds from the event will benefit CureDuchenne.

- Bowler (child) - \$50
- Bowler (adult) - \$100
- Sponsor a team - \$400
- Sponsor a lane - \$1,000

[Click here](#) to register.