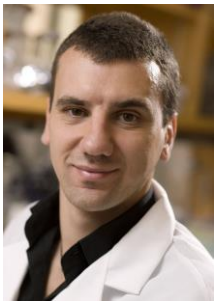
August 15, 2014

Beyond Batten Disease Foundation partners with NCL Foundation to award $344,742 to research team of Dr. Marco Sardiello, Baylor College of Medicine, Houston

As part of a larger $1.75M grant, BBDF continues to support Dr. Marco Sardiello's research, investigating the role of CLN3 the juvenile Batten disease gene, CLN3, in Juvenile Neuronal Ceroid Lipofuscinosis (JNCL).

Recognizing the promise of BBDF-funded research, NCL Foundation in Hamburg, Germany, awarded the 5th NCL Research Award to Dr. Marco Sardiello and his team at Baylor College of Medicine in Houston, Texas. The award, which amounts to $133,942, will finance a postdoctoral fellow, Dr. Alberto diRonza, whose work will be dedicated to unraveling the primary role of the normal CLN3 protein and the lysosomal defects that result in Batten disease. NCL's funding is in conjunction with BBDF's funding of $210,800 to Dr. Marco Sardiello and his team. With these combined funds, the researchers hope to gain insight into the role of *CLN3*.



Dr. Marco Sardiello and his team are dedicated to the research and development of innovative therapies to treat lysosomal diseases (LDs), including juvenile Batten disease. LDs are the most common childhood neurodegenerative diseases. The majority of LDs are caused by defects in one of over 60 known soluble lysosomal enzymes or 25 transmembrane proteins in the outer layer of lysosomes. Lysosomes are cellular organelles that play a key role in the degradation and recycling of cellular organelles, proteins, lipids and other substances. Therefore, defects in genes that encode lysosomal proteins result in excessive accumulation and devastating effects.

Complicating treatment, the normal *CLN3* gene (when mutated, causes juvenile Batten disease) encodes a transmembrane protein. Therefore, candidate therapies targeting soluble enzyme deficiencies, such as enzyme replacement therapy, bone marrow transplantation, or gene therapy, are generally not considered for treating juvenile Batten.

Dr. Sardiello and his team are investigating transcription factor EB (TFEB). Dr. Sardiello was part of a team which discovered TFEB is a master gene in the network regulating the biogenesis and activity of lysosomes and that inducing TFEB can increase lysosomal clearance in certain disorders. This discovery and its continued study show potential in the treatment of juvenile Batten disease.

About Beyond Batten Disease Foundation
Beyond Batten Disease Foundation (BBDF) is the world's largest organization dedicated to funding research for a treatment or cure for juvenile Batten disease. Batten disease is a rare, inherited pediatric neurological disorder, which begins with vision loss and seizures, followed by cognitive and motor impairment, and ultimately death by the late teens or 20s. Since its inception in 2008, BBDF has raised over $14.6 million for research through donations, co-funding, leveraging and partnerships. BBDF is spearheading a unique, cohesive strategy, incorporating independent scientific resources and collaboration with related organizations to drive research in Batten Disease. For more information, visit www.beyondbatten.org.

About NCL Foundation
The NCL Foundation was founded 2002 by Dr. Frank Husemann, after his then 6-year-old son Tim was diagnosed with JNCL. Neuronal Ceroid Lipofuscinosis is a rare metabolic disorder which leads to a progressive loss of nerve cells. It is the most common form of childhood dementia. Affected children suffer from neurodegeneration affecting different types of neurons, also in the retina, and this results in early blindness, mental deterioration, loss of motor function and the development of epileptic seizures. Many NCL patients do not survive past their third decade. www.ncl-foundation.com

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