

The UCSD Christini Fund 2015



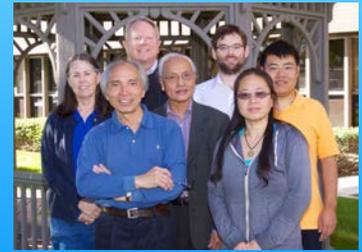
**Debbie Shimizu,
Founder**



Kamran Jamil—Current Harvard student and past Christini Intern & Naviaux Lab grad



MMDC Scientists creating new tests to diagnose and treat mitochondrial disease



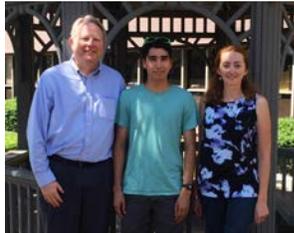
MMDC Scientists in the Naviaux Lab

Seventeen Years of Discovery and Service 1999-2015: The UCSD Christini Fund at Work



Metabolomics

This \$0.5 million machine is helping scientists crack the code of mitochondrial dysfunction and complex disease.



Ethan Coston—MMDC Research Intern

Ethan is a student from Steele Canyon HS. This summer he learned about mitochondria and DNA in the lab with Dr. Cohen.



KUSI Special Report on Mitochondrial Disease

After the 2nd annual "UCSD Mito Walk and Roll 5k" organized by Mrs. Christina Whaley, with help from UCSD and the Christini Fund



The UCSD Suramin Study

Here are some of the nurses, doctors, pharmacists, nutritionists, neuroscientists, and staff who are all part of a study to test a new treatment for autism.

Inspiring Progress in Mitochondrial Disease



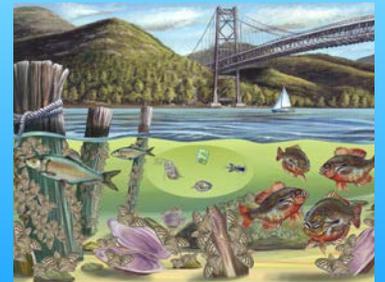
Caylee and Christine Shimizu in 1998



Dr. Naviaux with Madalyn Fandrei, autoimmune disease survivor



Measuring brain waves for the UCSD Autism Study



Showing the connection between environmental health and child health

Accomplishments

Scientific Discovery

The UCSD Christini Fund-- Seventeen Years of Discovery and Service 1999-2015

Medical Education

- Discovery of the cause of Alpers Syndrome (1999)--the oldest known mitochondrial disease (1931)
- The cloning and sequencing of the gene, and development of a DNA test for Alpers syndrome--now the gold standard for diagnosis around the world
- Discovery of the risk of neurological setbacks with common infections in Leigh Syndrome and other mitochondrial disease
- Publication of the first comprehensive reference text for doctors and scientists on Mitochondrial Medicine (*Mitochondrion*, 450 pages, 2004)
- The development of an advanced type of mass spectrometry (FTICR-MS) for automated and rapid screening of mitochondrial DNA mutations
- The development of a new nanolaser technology called the the Biocavity Laser for rapid diagnosis of mitochondrial disease and cancer
- Organization of the first international meetings of the 3 mitochondrial societies in America--The Mitochondrial Medicine Society (MMS), The Mitochondrial Research Society (MRS), and the United Mitochondrial Disease Foundation (UMDF) in 2001, 2002, and 2004
- Organization of the first international meetings to develop Diagnostic Standards for mitochondrial disease (2001, 2002, and 2004)--The International Mitochondrial Standards Initiative
- Discovery of the mitochondrial control of mammalian wound healing and tissue regeneration.
- Discovery that triacetyluridine (TAU) is a promising new treatment for mitochondrial disease
- Discovery of the "New Water MRI" technique for metabolic imaging of mitochondrial function
- Discovery of mutation arrest therapy (MAT) for cancer cells
- Discovery of the largest and most diverse genetic library of life on earth--the DNA of millions of species in a thimble-full of sand on beaches around the world
- Discovery that mitochondria control epigenetic changes in DNA by regulating cell metabolism
- Discovery of "oxidative shielding" and a new understanding of free radical signaling in diabetes and cancer
- Discovery that mitochondria "talk" to neighboring cells with "mitokines"--chemicals like ATP that regulate inflammation, the immune response, lower body temperature, and protect tissues after injury.
- Discovery of a new class of medicines that may revolutionize the treatment of autism, and many other neurologic disorders
- Researchers at the MMDC have published over 90 scientific papers and helped to organize 20 international meetings since 1999. These were all made possible by support from the Christini Fund.



Christine



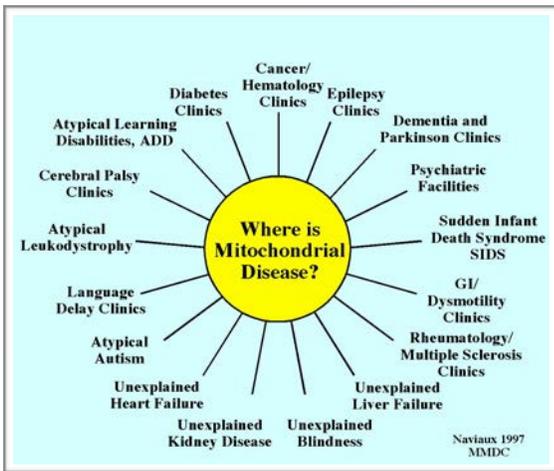
Beau

The UCSD Christini Fund-- 17 Years of Service

We have been overwhelmed with the generosity of the people of San Diego who have come out each year to help support research for mitochondrial disease. The panel above lists just a few of the accomplishments that have been made in the past 17 years. These have ranged from a new gene test that has become the gold standard for diagnosing Alpers syndrome, to the discovery of a new class of medicines to treat mitochondrial dysfunction in autism and several other neurological disorders. Physician education has been a major goal in the first decade, to help doctors learn to recognize mitochondrial disease. In 1994, the average child with mitochondrial disease was seen by an average of 12 different doctors before they were referred for expert care at the MMDC. By 2004, after some 10 international medical meetings organized by Dr. Naviaux and his colleagues, a child with mitochondrial disease was seen by just 2-3 other doctors before it was recognized the child might have a mitochondrial disease.



**Mrs. Debbie
Shimizu, Founder**



only caused rare childhood diseases like Leigh or Alpers syndrome.



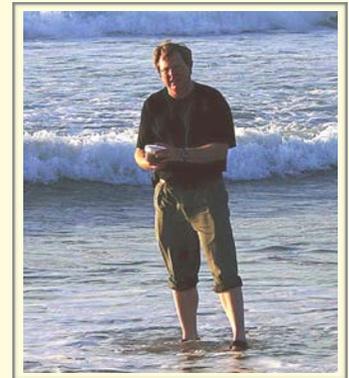
Back in those early days, Dr. Naviaux drew up the diagram on the left, illustrating how mitochondrial dysfunction can play a role in many common disorders as well, from diabetes, to cancer, from autism to chronic pain syndromes, and to autoimmune diseases. The exciting new field of mitochondrial medicine is changing how doctors think about both the cause and treatment many of the toughest diseases known.

More than Just a Childhood Disease

In 1994-1997, most doctors had never heard of mitochondrial disease, and those who had believed it



about the connections between human health and the environment.



Mitochondria in Autism, Chronic Fatigue Syndrome, PTSD, TBI, Autoimmune Disease, & Cancer

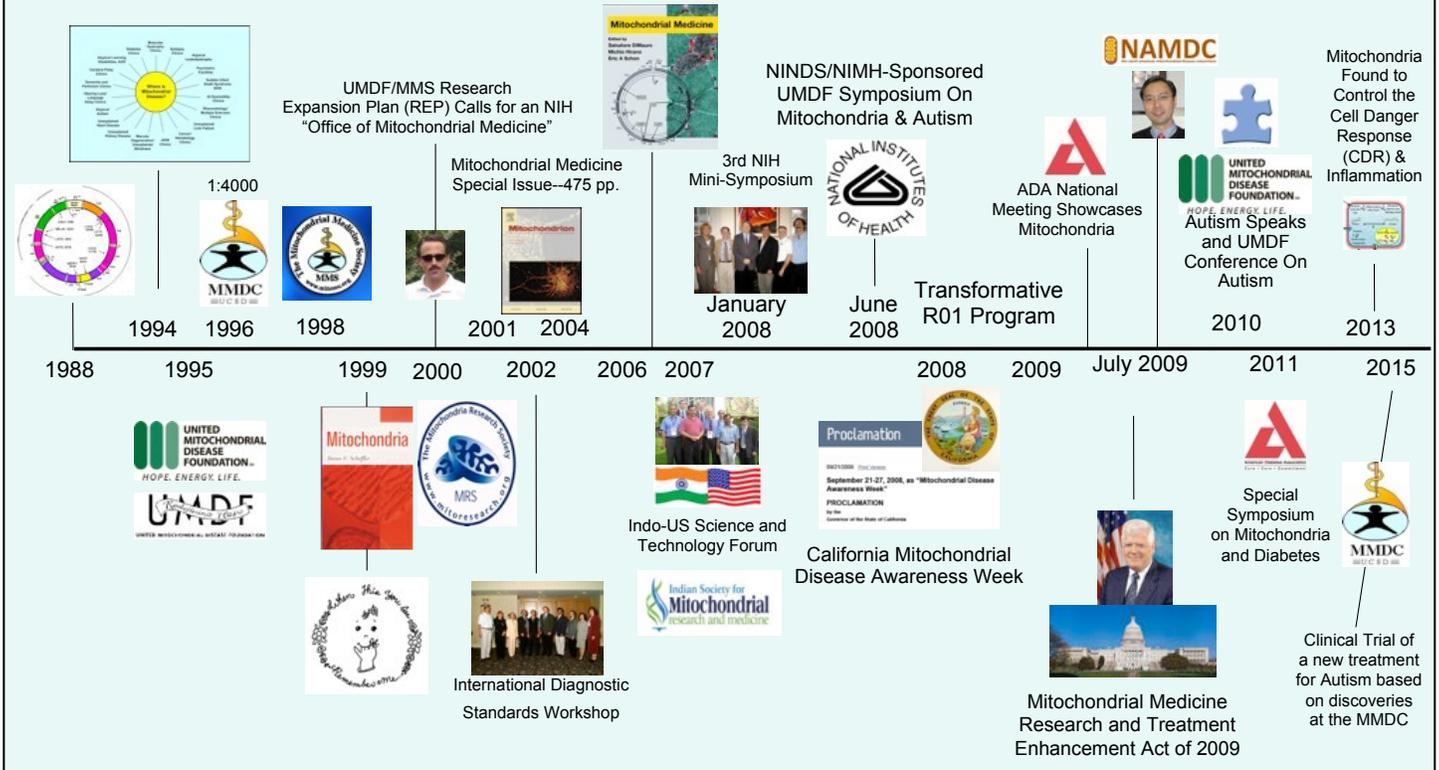
Over the past 5 years, great excitement has developed among scientists regarding the link between mitochondria, autism, diabetes, PTSD, autoimmune disease, and cancer. Surprisingly, mitochondrial function is abnormal in each of these diseases. Dr. Naviaux's discoveries have led to a new clinical trial this year to test a completely new class of medicines in autism. If successful, these discoveries will transform how we treat many chronic disorders, and change how doctors think

Scientific Exploration can lead scientists in unexpected directions. Sometimes to cure a childhood disease, we need to look to the sea, or the rain forests. The answers are waiting for us to discover in threads that connect us to all life on Earth. Dr. Naviaux's team at UCSD has a track record of innovation and discovery that has meant real progress for children with mitochondrial disease for over 20 years. If you are interested in learning more about the research at the MMDC, please go to www.christini.org, or contact UCSD Health Sciences Development, at 858-534-6223, or by email: nbelt@ucsd.edu.

To Donate, go to: www.christini.org

The Beginnings of a New Medical Discipline

Mitochondrial Medicine—The First 30 Years



The UCSD Christini Fund

Christine Shimizu lived only to her 2nd birthday, but her memory lives on today, bringing hope and smiles to children around the world. Her life stands today, as it did 17 years ago, as a lighthouse and guiding light, leading the way to hope and a better life for children and families touched by mitochondrial disease.



Footprints on the Moon and Mitochondria

In 1969, Neil Armstrong left this footprint on the moon. This iconic image bears a striking resemblance to a mitochondrion. It is fitting that the greatest scientific exploration effort ever launched--the landing of a man on the moon--has left us with a reminder to inspire us to explore the next frontier in medicine; the mitochondria in our cells. Mitochondria are the future of medicine. They help us to move and think, to love and play. Your support has helped us to make a difference.

Thank You!