Cures Within Reach
2012-13 Annual Report

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Cures Within Reach
At the Illinois Science + Technology Park
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Skokie, IL 60077
www.cureswithinreach.org
**Cures Within Reach (formerly Partnership for Cures) has been helping patients since 2005 by repurposing drugs and devices to quickly deliver safe and affordable treatments and cures for both common and rare disorders without currently effective treatments.**

Through this work, Cures has emerged as a leader in the Repurposing Revolution, working to transform patient lives through research that looks backward in order to move forward. While patients with catastrophic diseases are suffering, we know that there are immediately usable medical solutions going unnoticed: familiar drugs and devices that have new uses and can improve length and quality of life today. By finding and funding clinical trials with this approach, Cures is creating a powerful new leg of pharmaceutical development, a unique innovation pathway to solve unmet medical needs via repurposing.

Our broad vision at Cures Within Reach is of a world in which the success of medical research is measured by how it has reduced patient suffering; a world in which existing drugs and devices are mined for all utilizations, creating maximum benefit for patients in need.

We are happy to report our progress in 2013 and send a thank you to all individuals and organizations that have joined with us to create results!

**Leadership**

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A Letter from the President

2013 was a year of success and transition for Cures Within Reach! The Patient Impact Initiative research pipeline continued to deliver us a creative, high impact, low cost research portfolio that we are able to match with diverse funders.

Our Partner Research Institutions generate 125-250 new Rediscovery Research proposals each year and our Science Advisors and Executive Board rank and select 20 projects for our PII Portfolio. We solicit patient support groups, foundations and philanthropists to fund these proof of concept trials that cost $25,000-$250,000 over a 12-36 month period. These projects can include:

1) Repurposing FDA approved drugs, devices and nutraceuticals to treat “off label” diseases
2) Testing Pharma and other shelved compounds for new indications
3) Combining drug and non-drug treatment
4) Modifying current treatment protocols to make them work better and help more patients
5) Testing clinical observations from integrative medicine or from other parts of the world

Through this research sourcing process and our partnerships with smaller, disease specific organizations, we were able to initiate nine new research projects, one of which has already completed; receive final results on three past projects; and continue to watch others unfold. Read more about our research progress in the next section.

2013 also brought with it the first Cures Within Reach Honoring Event, which brought together 275 luminaries from the Midwest Bioscience community; a record-breaking fundraising year for the Longest Day of Golf, an expansion of our staff and a name change to greater reflect our commitment to patient impact.

Come join us!

Dr. Bruce E. Bloom
President and Chief Science Officer
Impact of Pet Ownership on Blood Sugar Control in Youth with Type 1 Diabetes Mellitus

Dr. Olga Gupta, University of Texas Southwestern, 12 months, $11,430  Start Date: 08/2012

The researchers aim to discover inexpensive and feasible strategies to improve blood glucose levels in children and teens with type 1 diabetes, a chronic disease of the pancreas that requires vigilant monitoring of blood glucose levels and insulin management. A number of other studies have found that social factors can help in diabetes control. The researchers theorize that the positive effects of owning and caring for a pet, specifically the responsibilities involved in pet care, will also have beneficial results for diabetes management.

This Rediscovery Research project will measure how caring for a pet fish impacts diabetes management in children. The patients will be randomized into two groups, those receiving a fish and all items necessary to care for it, and a control group that receives a photo of a fish. It is hypothesized that the fish care-takers will have fewer hospital/emergency room visits related to diabetes, and that they will show significantly improved control of blood sugar levels.

A large version of this research trial has recently been funded by the NIH and so the funds from Cures Within Reach will enable an additional outcome measure (Quality of Life assessment) to the NIH proposal for patients with Type 1 diabetes mellitus.

Therapeutic Potential Of Inhibiting p38 MAP Kinase In RARS

Dr. Amit Verma, Albert Einstein Medical Center, 48 months, $48,000  Start Date: 06/2009

Dr. Verma’s research team completed the research. While they were unable to prove that repurposed p38 drugs could impact the course of myelodysplastic syndrome, they did uncover several new genes and druggable targets in this disease published in peer reviewed articles.

Stem Cell Transplants for Blood Cancers

Dr. Luis Porrata, Mayo Clinic, $450,000  Start Date: 09/2008

This study is complete. We are waiting for the statistical analysis of the project so that we can gain access to the results.

Repurposing Breath Analysis Devices for Early Diagnosis of Lung Cancer

Dr. Peter Mazzone, Cleveland Clinic, 2 years, $45,000  Start Date: 08/2010

This project is complete. The current work is designed to help develop a breath biomarker assay capable of identifying people with early stage lung cancer. This breath biomarker work may also lead to a novel means to characterize lung cancers, determine their prognosis, and predict and monitor their response to therapy. Though lung cancer is the primary disease this work will impact, the researchers are soon to expand our work to other solid organ malignancies, in particular breast, kidney, and colon cancer. It is likely that unique breath biosignatures exist for these diseases, and that breath testing may be capable of identifying early stage cancers of many types. In a related project the research team will be analyzing the metabolic characteristics of lung tumor tissue, adjacent uninvolved lung tissue, and lung cancer subject blood samples, to increase our understanding of the basis of breath biosignature origins. Finally, they will be using the sensor device to analyze chemicals in blood and urine in an effort to develop tests using these sources.
Updated in 2013

Repurposing a Generic Drug for Pediatric Auto-Immune Diseases
The Use of Drug X for Treatment of Seven Pediatric Autoimmune Diseases
Dr. David Teachey, Children's Hospital of Philadelphia, 36 months, $53,247

Start Date: 09/2011

The funding from the Longest Day of Golf has made a rapid patient impact. In the first 24 months of his work since 2011, 27 patients with five different diseases (ALPS, Evans disease, lupus, autoimmune hemolytic anemia, Idiopathic thrombocytopenic purpura) who had failed every therapy were started on Rapamycin.

Dr. Teachy reports that, “from a study standpoint we are not enrolling as many patients as fast as we had anticipated many physicians who used to refer their patients to us, since they had no effective therapy, are just using rapamycin now. I got emails all the time of rapamycin success stories. So the good news is that since you started this project rapamycin has become the new standard of care for 6 additional diseases! Another important thing is all the responders continue to do great on therapy. Many of them I am starting to wean rapamycin down to lowest possible dose and it is still working great.”

Here is the current breakdown:
Complete Response: 10 ALPS, 3 Evans, 2 Lupus, 2 CVID
Near complete Response: 1 AIHA, 1 ALPS, 1 Lupus
Partial Response: 1 Evans, 1 ITP, 1 ALPS
No response: 2 Evans, 2 ITP
Overall response rate: (CR + PR): 85%

17 of the patients were in complete remission 30 days later-in other words, their symptoms had disappeared with no side effects and very little cost! Imagine the life-changing impact on these young patients and their families! 6 other patients had a partial response-the rapamycin worked better than anything else ever tried! Unfortunately, four of the patients had no response to the rapamycin, but 23 out of 27 patients is a pretty great response! 30-60 children will participate in this study-if the results keep up with the first 27 patients, the published paper will change the standard of care in some or all of these deadly childhood diseases!

Safe and Specific Laser Treatment for Prostate Cancer
Drs. Scott Eggener and Oto Aytekin, University of Chicago, $50,000  Completed: 02/2012

Drs. Eggener and Oto at the University of Chicago are eradicating prostate cancer in its earlier stages using a laser that heats and kills just the cancer cells. The treatment takes just a few hours, is done under a mild sedation, and because the whole process is done under MRI observation, the system reduces the chances of injuring surrounding tissue to nearly zero!

After the treatment, patients walk away with a small Band-Aid and resume their normal activities. The laser device was originally designed for use on breast cancer and brain cancer. Drs. Oto and Eggener approached Cures Within Reach to see if our funders would be interested in supporting the first repurposing of this device for prostate cancer. We contacted one of our funding groups, the organizers of our Longest Day of Golf, and they agreed to raise the $50,000 to cover the costs of the clinical trial over the two year period. In less than 9 months after the initial inquiry from Drs. Oto and Eggener, 9 patients were experiencing the impact of this promising new treatment with excellent initial results and years later, they are continuing to thrive! This pilot clinical trial helped patients directly, and led to the application for a larger clinical trial funded by $500,000 from the NIH. Final Report available on our website.

In 2013, the research team received a $500,000 NIH grant to fund a phase II trial to propel this repurposed technology toward FDA approval and availability to patients worldwide.
Increasing the Effectiveness of Radiation in Breast Cancer Treatment

Dr. Ralph R. Weichselbaum, University of Chicago, 2 years, $100,000  Start Date: 04/2012

Due to important advances in detection and treatment of breast cancer, the majority of patients are diagnosed with early stage cancer, and many are cured. However, too many patients are still diagnosed after their cancer has spread out of the primary site and adjacent lymph nodes to form metastatic tumors in the lung, bone, brain or other sites. For many such patients, even if only one metastasis is detected, their disease will be ultimately fatal despite aggressive treatment with chemotherapy, conventional radiation therapy and/or targeted agents. An effort to discover a cure for metastatic disease offers tremendous potential for impact on the most lethal kinds of cancer.

This research group studied the benefits of directly targeting up to five metastases using a high dose hypofractionated therapy only three times and at two week intervals. When they used the highest tolerated radiation doses on each tumor, nearly all patients benefitted, many survived much longer than expected and a remarkable number appear to have been cured.

They found that treating cancer cells with high radiation doses causes irreversible chromosomal damage, instantly aging the cells so they can no longer grow or reproduce. They think that the combination of inflammation and near dead cells in the irradiated tumor may stimulate the patient's immune system, and the resulting anti-tumor reaction clears the body of cancer.

However, hypofractionated radiation therapy incurs considerable risks due to high individual doses. This limits its use to a few tumor sites and to tissues that tolerate high doses, excluding many patients from treatment. The researchers hypothesize that it may be possible to reduce the radiation doses if they can induce accelerated aging of tumor cells with benign drugs plus lower doses of radiation. Through drug screening they have already discovered nearly 100 candidate radiation enhancers that have the potential to fulfill the key criteria of ready availability, low toxicity and strong enhancement of radiation-induced accelerated cell aging.

They propose to advance several of these leads by testing these drugs in combination with radiation on breast cancer cells and begin the process of qualifying the most promising radiation sensitizing drugs for human clinical studies in breast cancer patients toward achieving high efficacy with lower doses in hypofractionated radiotherapy.

Exome and Next Generation Sequencing to Identify RARS-specific Targetable Defects

Drs Azra Raza & Naomi Galili, Columbia University, 36 months, $1,000,000  Start Date: 08/2012

This research will complete a comprehensive step-wise study of myelodysplastic syndrome (MDS) RARS patients in a two pronged approach. The research team will start by sequencing the genomic DNA of a recent patient, and simultaneously sequence 10 RARS patients from their Tissue Repository. In addition, they will perform RNA and methylation sequencing as well as specific mitochondrial DNA sequencing in order to correlate results from all four sources (exome, RNA, methylation and mitochondrial) to generate a comprehensive molecular portrait of this specific subtype of MDS.

They expect that these results will provide insights into potential therapeutic targets which can be used to develop novel therapies rapidly. Many non-toxic small molecules have already been identified that can block multiple such targets or their pathways including one developed in-house. Such drugs have been routinely discovered through high throughput small molecule screening. If initial analysis does not yield a definitive target, they will proceed to the next 10 RARS patients and so on until a total of 100 have been studied. However, all potential defects identified in the genome of Patient One will be confirmed and a mouse model will be developed for those which seem to be biologically relevant to her disease.

Training Physicians to Perform Opportunistic Screening for Melanoma

Dr. June Robinson, Northwestern University, 24 months, $30,000  Start Date: 12/2012

Age-adjusted incidence of melanoma among whites has risen from 7.5 to 21.9 cases per 100,000 representing an increase of nearly 200% over 30 years. Survival is improved with early detection of melanoma and surgical treatment. An estimated 16% of all melanomas are discovered by
physicians. With approximately half of all US physician office visits to the patient's primary care physician, primary care physicians have the opportunity to detect melanoma.

In contrast to other cancers (i.e. colorectal, prostate, cervical), melanoma is detected by non-interventional visual skin. Lack of primary care physician training and low confidence in their examination skills are consistent obstacles identified as barriers to better early detection of melanoma. This lack of self-confidence increases the likelihood that newly graduated primary care trainees will not perform opportunistic screening for melanoma.

Two life-size back simulation models of Ecoflex silicone rubber were created to replicate representative clinically suspicious lesions: melanomas, abnormal nevi, benign nevi. The melanoma simulation models and threshold rules for management afford medical students the opportunity to learn visual assessment, management, and counseling skills. The goal is biopsy of 100% of melanomas with 20% of benign lesions either watched for change or referred to a dermatologist for assessment. During the Primary Care Clerkship, 81% of third year medical students achieved the goal of biopsying melanomas and 14% chose to watch the lesions for change in 3 months; however, 50% of benign lesions were watched for change or referred to the dermatologist.

By adding rules for dermoscopic (magnified) evaluation of the pigmented lesions to the skills training process, it is expected that fewer benign lesions will be watched for change or referred to a dermatologist and the detection of melanoma at an earlier phase, melanoma in situ, will improve.

Human Clinical Trial Using Gene Therapy for Batten Disease
Ronald G. Crystal, MD, Weill Cornell Medical College, 36 months, $750,000 Start Date: 06/2010

Batten Diseases comprise a group of genetic diseases that affect children. Children with these gene defects are missing key enzymes that help to break down waste products in brain cells. These waste products build up, and eventually destroy the brain cells. This causes a loss of function in these children, and eventually death.

This research team is undertaking a pilot clinical trial determining the impact of putting a working copy of the gene into a virus that can be injected into the brain. Six billion copies of the virus will be injected into the brains for children with Batten Disease in the hopes that they will spread out and infect brain cells, and deliver a working copy of the gene into the cells. These brain cells can then produce the missing enzyme, allowing the brain cells and the children to thrive.

If this study can prove that this specific virus and delivery method can deliver the gene to the cells and then cells can incorporate the gene to produce the enzyme, we will be have a life-saving treatment for children with Batten Disease.

To date, six children have undergone this procedure without serious side effects. These children will be monitored to determine whether their disease slows, stops or reverses.

Biomarkers for Rapid Assessment of Experimental Treatments in LINCL Batten Disease
Ronald G. Crystal, MD, Weill Cornell Medical College, 36 months, $47,000 Start Date: 04/2012

Batten Diseases comprise a group of genetic diseases that affect children. Children with these gene defects are missing key enzymes that help to break down waste products in brain cells. These waste products build up, and eventually destroy the brain cells. This causes a loss of function in these children, and eventually death.

This research team is helping to find Batten Disease biomarkers in the blood and other easily accessed fluids and cells in the human body. Biomarkers are molecules that help researchers and clinicians check to see how a disease is progressing, and if a treatment is working. Having a biomarker to check a disease is like having a water sample to check the chlorine content in a pool— you take a small sample and test it to check the overall status. If this study can find these biomarkers, we will have a powerful tool that will help us find new life saving treatments for children with Batten Disease.
This project is screening biomarkers in the cerebrospinal fluids (CSF) of Batten Diseases children compared to CSF from non-Batten children. The techniques used in this study enable the simultaneous detection of thousands of metabolites in a modest quantity of CSF fluid (<5 μL), with an accuracy that enables definite assignment of molecular formulae and quantitative changes in expression levels.

Metabolite profiling was performed on the CSF from 2 groups: (1) normal (7 subjects), and (2) Batten Disease (18 subjects). Five μl of CSF was analyzed from each subject. 1042 distinct metabolites were identified and 60 metabolites were found to have > 2-fold difference between LINCL vs. normal subjects.

This preliminary data is very interesting and supportive of our hypothesis that the CSF of Batten children have different metabolites from non-Batten children.

**Repurposing BCG as a Low-Cost Treatment for Type 1 Diabetes - Phase II Human Clinical Trial**
*Denise Faustman, MD, PhD, Harvard and Massachusetts General Hospital, 36 months, $25.6M*

**Start Date: 01/2011**
This project is awaiting additional funding to complete the $27.1M required to support the study.

**Initiated in 2013**
*Nine new projects began in 2013*

**Screening Drug Libraries for Efficacy in Stimulating Mutated CLN2 Genes (Batten Disease)**
*David A. Pearce, PhD, Sanford School of Medicine, 12 months, $45,000  Start Date: 04/2012*

We propose a project to screen drug libraries for agents that show efficacy in stimulating the activity of mutated CLN2 genes in a defined cell assay.

An outline of this project is:
- We will use standard fibroblasts for screening assays, supplemented by CLN2 affected fibroblasts with the required mutations we received through an MTA with General Hospital Corporation.
- We will set up and optimize the assay in a 96-well plate format. Fluorescent detection will be used to obtain the primary data. The assay is summarized as follows:
  - Cells will be grown in 96 well plates, rinsed and lysed with a triton X-100 lysis buffer. Plates will be incubated in the cold for 1 hour at 40°C on a shaking platform, and then a substrate will be added. The plates will be warmed and the fluorescence read over time on a 96-well plate reader.
- The Sanford Library, supplemented by other approved drugs, will be screened using the cell-based assay. The library will be screened at several drug concentrations, usually 10 mM and 100 mM.
- Drugs that appear to increase LINCL activity will be further studied using conventional dose ranging to determine effective doses and establish the reproducibility of individual drugs and combinations of drugs.

Any drugs, compounds and combinations that appear to up- or down-regulate the production of TPP-1 will be further studied to provide further insight into mechanisms of the disease and pathways for abrogating the negative effects of the mutation.

**Reformulating a European hospital drug to meet an urgent need for ambulatory patients**
*LAT Pharma LLC, $65,000 Started: 09/2013*

LAT Pharma LLC is an early-stage company focused on developing a novel outpatient therapy to treat ascites due to liver cirrhosis. More than 50,000 Americans suffer from advanced ascites, requiring frequent hospitalization and often progressing to death. The company and its Seattle-based research partner, PharmaIN Corp., have developed a promising new agent by reformulating a drug.
used in European hospitals for more than 20 years to treat related conditions. Recognizing the urgent need for a novel therapeutic approach, the FDA granted Orphan Drug Designation to the company’s lead compound to encourage clinical development.

Published data on the European IV version of the active agent, terlipressin, confirm the potential for LAT Pharma’s formulation to treat ascites, as do animal studies conducted by PharmAlN. The compound is patent-protected in the US, and the novel drug delivery system, called Protected Graft Co-Polymer (PGC™) is covered by a separate intellectual property (IP) estate. The company hopes to commence Phase I human clinical studies of the new drug candidate in late 2014.

Upregulation of Cln2 by fibrate drugs
Dr. Kalipada Pahan, Rush University Medical Center, 24 months, $50,000 Start Date: May 2013

Batten Diseases comprise a group of genetic diseases that affect children. Children with these gene defects are missing key enzymes that help to break down waste products in brain cells. These waste products build up, and eventually destroy the brain cells. This causes a loss of function in these children, and eventually death.

This research team recently discovered that several already available and safe drugs can help brain cells produce more of the enzyme missing in the brain cells of children with Batten Disease. They have evidence that three commonly used drugs can cause healthy human and mice cells to produce more of that enzyme. The next step is to test these drugs in cells taken from actual Batten Disease patients, to see if it can have the same enzyme increasing effect.

If this study shows that Batten Disease cells will produce more of the enzyme when they come in contact with one or more of these drugs, we might finally have a safe, cost effective life-saving option to treat children with Batten Disease.

Lysosomal Membrane Permeability in NCL Disease: Pathophysiology and Therapeutic Potential
Dr. Matthew C. Micsenyi, PhD - Postdoctoral Fellow, Dr. Steven U. Walkley, DVM, PhD - Laboratory Head, Albert Einstein College of Medicine, 12 months, $70,000, Start Date: March 2013

The research team has done extensive studies that show that certain protective structures inside the brain cells of children with various Batten Diseases become leaky, releasing dangerous molecules into the brain cells. These molecules cause the brain cells to die. The team will study whether a drug developed for ALS can be repurposed to reduce the leakiness of these protective structures inside the brain cells, and therefore reduce the brain cell death in these children stricken with Batten Diseases. This is a one year study on mice models of two kinds of Batten Disease. The results could lead directly to the use of this repurposed drug in a clinical trial on Batten Disease patients. The study will be completed at the Albert Einstein College of Medicine in Bronx, New York. The budget is $70,000.00, part of which is supplied by the Hope 4 Bridget Fund at Cures Within Reach.

Global Gene Transfer for Batten Disease
Dr. Steven Gray, University of North Carolina, $75,000 Start Date: 04/2013

Principal Investigator Steven Gray, Ph.D. is a member of the research team at the Gene Therapy Center at UNC. He is leading the Global Gene Transfer for Batten Disease project, which is modeled after a project at UNC that is focused on the ultra-rare childhood disease Giant Axonal Neuropathy (GAN) and is a parallel study in infantile (INCL, CLN1 mutation) and late infantile (LINCL, CLN2 mutation) Batten disease.

The project aims to further test a successful global gene delivery platform in mouse models for INCL and LINCL, to see if it imparts a therapeutic benefit over previous gene therapy approaches. If these studies are successful, existing large animal biodistribution studies would pave a clear path forward for human translation following appropriate toxicology studies.
For human applications, the AAV-based intrathecal gene delivery platform would provide these benefits:
- A minimally-invasive injection route
- A moderate dose that could be readily manufactured at clinical grade by the Gene Therapy Center
- Efficient expression of the therapeutic gene throughout the entire brain and spinal cord volume that can be scaled to humans

**Early Treatment with an FDA Approved Drug Attenuates Cardiomyopathy in Duchenne Muscular Dystrophy**

Dr. Subha Raman, Ohio State University, 3 years, $92,000, Start Date: June 2013
Selected for funding by both the NIH and Cures Within Reach

Duchenne muscular dystrophy (DMD) is a deadly X-linked disease affecting 1 in 3,500 males. DMD patients suffer significant disability due to muscle issues, including to their heart muscle. Current guidelines advocate initiating early heart protective treatment, yet this treatment paradigm has not improved survival much beyond the third decade of life. Potentially promising approaches like gene therapy will take considerable time to improve outcomes.

This research group has recently shown in a DMD mouse model that certain existing drugs typically reserved for advanced heart failure patients can preserve cardiac muscle function at 80% of normal and near-completely prevents fibrosis development. This research group plans to execute a randomized, controlled clinical trial of one of these drugs plus the current standard of care vs. the current standard of care alone in patients with DMD. The treatment group is expected to show significant delays in heart disease using highly reproducible imaging biomarkers selected for efficient sample size design.

**The use of antiplatelet agents to prevent tumor metastasis: a preclinical study**

Dr. Michael Bezuhly, IWK Health Centre, 2 years, $55,000, Start Date March 2013

The goal of the proposed study is to determine whether two repurposed drugs can prevent cancer metastasis in well-established mouse models and by what mechanisms this beneficial effect, if present, is mediated. Specific objectives are:

1. To examine the role of one drug in inhibiting platelet-induced cancer cell proliferation and expression of genes implicated in cancer spread.
2. To determine the effect of that same drug on cancer cell adhesion to and transmigration across endothelial cells, the cells that line the inner wall of blood vessels.
3. To examine the role of the two repurposed drug sin disrupting cancer cell-platelet interactions and improving the ability of immune cells called natural killer cells to kill cancer cells.
4. To evaluate the effectiveness of the two repurposed drugs in reducing metastases and improving survival in established mouse experimental metastasis models.
5. To elucidate the role of the two repurposed drugs in inhibition of new blood vessel growth, known as angiogenesis, in metastases.
6. To publish the data in a high-impact peer-reviewed journal (eg, Blood).
7. To use this preclinical data to apply for further funding to enroll human subjects in a multicenter randomized controlled clinical trial

**The DEM-CHILD database-network - offering an online NCL patient registry for clinicians, researchers and parents**

Dr. Angela Schulz, MD, University Medical Center Hamburg-Eppendorf, 24 months, $154,000 State Date May 2013

Neuronal ceroid lipofuscinoses (NCLs) form the most frequent group of neurodegenerative diseases in childhood. They are characterized by dementia, visual loss, epilepsy, motor decline, and premature death. No cure is currently available, and palliative treatments are frequently
controversial. To date, the genes for up to fourteen NCL-forms have been characterized. The clinical course and its variability in the different NCL forms are still relatively unknown, which makes the evaluation of therapies (experimental and traditional palliative) difficult. The project will implement a novel network of the experienced NCL clinicians and geneticists by connecting the European Commission funded project DEM-CHILD (Germany, UK, Finland, Italy, India) with NCL experts from countries around the world such as US, Brazil, Argentina, Turkey, France, Norway and Denmark. The aim is to collect the world’s largest, clinically and genetically best characterized, set of NCL patients. Data will be collected in the already developed DEM-CHILD patient database. This database is online accessible and password protected. It does not only contain both retrospective and prospective clinical data but also information about available biospecimen from each patient in the database and multiple statistical tools to directly evaluate one patient’s disease progression compared to all patients in the database with the same NCL-form or even same mutation background. In addition, the database will also collect data on quality of life (QoL) from patients. Therefore it will also be accessible to parents, patients and caregivers to give them the opportunity to fill out QoL questionnaires on a regular basis. Therefore, as result of the project, a statistically valid body of detailed quantitative information on natural histories of genetically characterized NCL forms will serve as an indispensable tool for the evaluation and validation of new therapies and will have a significant impact on the validation and improvement of palliative care of NCL patients.

Lysosomal Biomarkers in Neuronal Ceroid Lipofuscinosis

Dr. David Sleat, Robert Wood Johnson Medical School 12 Months, $100,000, Start Date: 04/2013

Clinical trials for Batten disease include gene therapy, stem-cell transplant therapy, and small molecule therapies. Clinical trials are also planned for enzyme replacement therapy. The success of these trials is dependent on the ability to measure response to treatment to make sure patients are getting better on the right dose of therapy. Measuring response quickly will also accelerate approval of effective treatments and allow trials of ineffective treatments to be terminated in a timely manner.

The aim of this research is to identify effective biological molecules, called biomarkers, that will help measure the success of Batten research using mouse models. These biomarkers will show alterations in the brain that happen because of the Batten disease. Correction of the Batten disease will result in normalization of these brain changes. These studies will eventually move from the lab to the clinic.

Our Organizational Partners

The following groups have partnered with us in 2013 for mutual benefit. We have provided support in the form of 501(c)3 status, donation processing, and helped with sourcing and/or vetting research funding opportunities. Working with multiple Batten Disease groups has helped us to support research progress in a rare, pediatric disease area with so few patients that the research tends to be sparse.
Some of our Research Institutional Partners

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<td>Sanford Burnham Medical Research Institute</td>
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2012-13 Financial Documents are available on our website at: 

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