



Here is our update of funding-research partnerships as of 8-1-2010:

### **New Initiatives:**

**Patient Impact Initiative:** We have completed our pro bono consulting engagement with McKinsey. A final report will be issued by August 24. We are currently in discussions with Baylor, Harvard, Cleveland Clinic, Mayo Clinic, Johns Hopkins and the Universities of Chicago, Iowa and California to become Charter Research Institution Partners.

**Cravat Foundation-** We are working with Dr. Guy Weinberg at University of Illinois Chicago to create a funding partnership to support a very interesting project in a rare disease. Dr. Weinberg was referred to us by our funded researcher Dr. Doug Feinstein. Dr. Weinberg discovered that epithelioid hemangioendothelioma (EHE), a rare blood vessel tumor with a fatal outcome, might have an infectious cause. It behaves less like a typical cancer (it doesn't respond to either chemotherapy or radiation; metastases don't seem to affect outcome) and more like a chronic infection such as TB. He did some research to determine that EHE might be the cause by the bacteria Bartonella, which causes a similar looking disease in the HIV patients. This hypothesis is both testable and suggests a new, potential treatment: antibiotics. **This is a perfect example of digging through the literature to find a potential safe, rapid and economical cure for a deadly disease-Rediscovery Research™!**

**Rockford Chapter-** We are still working with our Rockford volunteer leaders to set up a Rockford clinical trial location for the non-invasive brain stimulating MS work we supported at UW Madison. We were close to closing a deal, but the economy got in the way. A location in Rockford would allow more Rockford and Chicago MS patients to participate as subjects in MS trials and help to build Rockford as a location for the UWM medical research in other diseases.

**Rare Disease Research-Leukemia-Henry Schueler 41 & 9 Foundation-**Drs. Charles Mullighan at St. Jude Children's Research Center and Steve Hunger at Denver Children's Hospital to use the Children's Oncology Network have now started the first ever gene review of the rare hypodiploid acute lymphocytic leukemia (HALL), the disease Hank Schueler had when he died. **In the first two quarters of work Dr. Mullighan and his team are making excellent progress. They have increased the sample size by soliciting samples from other institutions, and early analysis shows that two different tumor suppressor genes within HALL cells are frequently (up to 50%) deleted or otherwise not functioning. This insight could be a step towards figuring out how to treat the diseases.**

**Dogs for Cures Foundation-**we have a new agreement to start a "Dogs for Cures" fund at Partnership for Cures to raise funds to support research into the use of medical service dogs for patients with Type I Diabetes and other diseases. This is a Seattle based group started by Lisa Kelly and dog trainer Ron Pace. Lisa's son Liam has a diabetic alert dog named Max who is with Liam 24 hours a day-at school and at home, awake or asleep. Max knows when Liam's blood sugar is too high or low and alerts Liam or his parents. He's much better than a glucose meter, and a lot more fun to play catch with! The goal would be to raise enough

funds to train 12 dogs and their owner/patient partners and conduct a clinical study comparing the dogs to the standard glucose management.

**Rare Disease Research-Batten Disease-Jasper Against Batten Fund**-May 1, 2009 we created a new funding partnership to raise funds for research to support a clinical trial for Batten Disease, a fatal nerve disease of infants and children. **SINCE THEN WE HAVE RAISED OVER \$750,000 TOWARD THIS CRITICAL MISSION!**

**We are funding a project in the UK with Dr Jonathan D. Cooper in the Department of Neuroscience at King's College, London to develop a mouse model of CLN2 Batten Disease.**

Cornell Medical Center in NYC received approval for a clinical trial for Batten Disease. **We have been selected by Cornell as the non-profit through which all of the private philanthropic research funds would be provided to Cornell if and when a privately funded protocol or expanded access program is approved for patients that do not fit the NIH clinical trial criteria.**

In October we agreed to fund a joint project at University of Iowa and Rush University Medical Center testing the last stages of a gene vector therapy that would then be ready to move towards a human clinical trial. **The research complete by end of August.**

Researcher Dr. David Sullivan at Johns Hopkins Medical Center is screening a library of over 2500 already approved drugs that might help Batten patients. **We are hopeful that we will have candidate drugs that could be used by physicians treating Batten patients in October 2010.**

We just agreed to fund gene therapy research at UNC that could be easily delivered to Batten patients to replace their missing enzyme. We are hoping to receive final research results within the next two months.

**Prostate Cancer-Our Chicago Longest Day of Golf 2009 generated enough funding for two new direct patient impact clinical projects. Two Longest Day of Golf Prostate Cancer projects began in October 2009:**

Dr. Scott Eggener's human clinical trial, approved by the FDA, will test a treatment that can eliminate prostate cancer cells without damaging the surrounding tissues. **He has treated 5 patients, all successful and back to normal life the next day!** There are 5 more patients to be treated, and their 6 month reevaluations will be critical.

**Dr. Russell Szmulewitz has completed the first two quarters of work on time and budget.** The project is finding markers in the blood that will help find new drugs for hormone resistant prostate cancer.

**Lung Cancer Research-LUNGeVity Foundation and the Judy Hirsch Foundation**-We received over \$200,000 from our LUNGeVity funding partners to support the second year of three research projects, two at Rush University Medical center here in Chicago and one at Mass General in Boston. **The Mass General project has created a clinical trial that is enrolling lung cancer patients in less than 1 year since we started-THAT IS IMPACT!** Because of our ability to locate and validate high patient impact projects, LUNGeVity has approved another \$100,000 of research funding for 2009, and we have received 2 new projects that have been validated and underway at Johns Hopkins (improving the way radiation is used to kill lung

cancer cells) and the Cleveland Clinic (designing a “sniffer machine” that can detect lung cancer in high risk patients.)

**Rare Disease Research-Birt Hogg Dube Syndrome-Myrovlytis Trust-** We are working with John Solly, the Charity Director of this Great Britain not-for-profit. We just completed our reviews of proposals for \$100,000 in grants over 1-2 years. 3 grants were selected for funding and began on October 1, 2009. This partnership creates our first International Partnership, with our Great Britain funding partner and our three researchers from Netherland, Canada and Wales! In addition, we will have a second grant cycle later this summer, and **expect to add BHD to our list of drug screening programs this year!**

#### **Ongoing Initiatives:**

**Lymphoma Research-Brinson Foundation, the Plunkett Family Foundation and our Rockford Group** have supported this Mayo Clinic blood cancer transplant human clinical trial. After 17 months over 80 patients have been enrolled. **Early data from this project indicates that it was successful in increasing the success of these transplants from 45% TO 80%! We will continue to learn more as time passes, but this research is making an impact less than two years after the research started!**

**Multiple Sclerosis Research-Rockford Chapter of PFC-**Our funded human clinical trial at the world renowned TCNL research center has now demonstrated that patients can quickly regain walking, balance, vision, hearing, sleep and other body functions with this non-invasive stimulation therapy. **ALL PATIENTS IN THIS STUDY RECOVERED SIGNIFICANT FUNCTION.** The first prototype PoNS portable devices are now being used by patients with the same success as the lab basic therapy. With significant support from Partnership for Cures, we have directed another \$400,000 to the TCNL for the next 10 patient clinical trial that includes home use of the PoNS, stage two of PoNS development, documentation of the training program and training of a physical therapist. **We are talking with a number of Rockford Hospitals who are very interested in helping raise funds and providing staff, resources and facilities to creating research locations for this work in Rockford.**

**CureforMS.org research at Johns Hopkins:** Our Newsweek feature landed us a new group that wants to fund MS research with us. Scott Rothrock and Ted Smith signed an agreement so that we can begin working together. Their goal is to raise \$1.5M or more over the next 24 months to fund a phase III clinical trial repurposing cyclophosphamide and Copaxone for relapsing remitting MS to be conducted at Johns Hopkins and other medical centers. See their website at [www.CureforMS.org](http://www.CureforMS.org).

**Parkinson ’s Disease:** The first two test patients have been treated at the TCNL at UW Madison to help develop a Parkinson’s Disease Clinical Trial as the next step in the development of the CN-NINM therapy. The data from these two initial patients will be used to create a clinical trial that should be approved at the UW Madison in the second quarter of next year.

**Diabetes Research-Friend United for Juvenile Diabetes Research-**We are currently in the fourth quarter of two projects at Harvard supported by our funding partners at FUJDR, both clinical trials involving patients. **Friends United gave us another grant which is already in use testing a drug to regenerate pancreas cells!** Partnership for Cures made a this grant to Exsulin Corporation, with funds provided by Friends United for Juvenile Diabetes Research and the Miller Diabetes Fund, to support development of a novel breakthrough therapy aimed at the underlying cause of insulin-dependent Type 1 and Type 2 diabetes. The therapy, branded as Exsulin™, uses a naturally-occurring peptide that induces regeneration of pancreatic insulin-

producing islet cells which could lead to reversal of the effects of diabetes. Exsulin Corporation is the sponsor of a Phase II clinical trial currently enrolling patients with established Type 1 diabetes (also known as juvenile diabetes) at the Mayo Clinic and McGill University. Exsulin is the first and only islet regeneration therapy in Phase II development. We are also waiting for a proposal from a company called Smart Insulin for presentation to Friends United.

**Myelodysplastic Syndrome (MDS) Research Initiative-Private Funding Group-**Three patient impact research projects began in June 2009. One project is testing a repurposed drug at Albert Einstein Medical center in NYC. One project at St. Vincent's Cancer Center in NYC is looking at the genetics of MDS patient samples to determine what repurposed drugs might help. The third project at Northwestern is adding an existing non-MDS drug to patient treatment to see if it helps, as well as starting the first MDS Clinic here in Chicago. **TWO OF THESE PROJECTS SHOULD YIELD RESULTS IN ONE YEAR AND THE OTHER IN TWO YEARS!** This funding group has pledged \$1,000,000 over the next 4 years to fund MDS research. **We just received an additional grant from the Grant Healthcare Foundation to fund this research.**

**Various Disease Research-Grant Healthcare Foundation-**In December 2008 the GHF Board of Directors approved and sent us a check for a Pilot Grant at the University of Illinois studying repurposing drugs for multiple sclerosis. The project is complete on budget and ahead of schedule. **The results were published in the Journal of Neuroimmune Pharmacology and the research team applied for a \$250,000 clinical track grant from the National MS Society. Also, we received a 2010 grant from Grant Healthcare Foundation for MDS research, and Joan Ridell at GHF asked us to put together proposals for pulmonary fibrosis research in late 2010.**

**Training Physician Scientists in Translational Research-Rockefeller Brothers Fund-** We continue to support the nine Culpeper Scholars through this \$5.5 M five year grant from the Rockefeller Brothers Fund.