



A NEW DM RESEARCH PROJECT...A RARE opportunity to help!

As most of us are aware, “*Finding the Cure for DM Foundation*” has been contributing to DM research, with the ultimate goal to help treat or cure Canine Degenerative Myelopathy. After many years of research, these projects have not given us much hope for the rapid development of a cure for DM.

The start of the New Year is a perfect time for the Foundation to initiate new efforts and start new research projects designed and specifically intended to help our canine companions. We push forward with even more energy, the progress toward defeating this horrible disease affecting our pets.

While there are millions of dollars invested in ALS research, the ALS society has also invested hundreds of thousands of dollars in the DM Research being conducted at the University of Missouri, along with thousands of dollars contributed by the dog breed associations. However, these research efforts have not provided enough advances on Degenerative Myelopathy, as we continue to struggle for sufficient funds to be truly dedicated only to supporting our pups, with DM research.

While any research conducted is commendable, one of the main focuses of the *Finding the Cure for DM Foundation* is to initiate a new research project, which would be specifically tailored to our DM dogs.

That is what the priority is here at FCDMF...our canine companions.

Now there is “New Hope!”

We have tirelessly searched and prodded for more research and commitment to help eradicate this horrible and heartbreaking disease that stalks our beautiful babies.

We are very excited to tell you now, that we have *finally* found a new team of scientists from Silagene Inc., which is ready and committed to a *new DM study*, and to finding a promising drug therapy intended strictly for Veterinary treatment for our afflicted pets. We offer you now, further information about project “***New Hope.***”

Your support is making a difference!

In order to begin this exciting new project, it is necessary to perform an initial phase of in vitro testing, and the Silagene research team has agreed to accept, as an initial investment, \$7,000.00. We all know that scientific research of any type is extremely costly and lengthy. It typically costs hundreds of thousands of dollars, and takes many, many years, as can be verified from ongoing studies, yet still we have no confirmed, affordable treatment for Degenerative Myelopathy.

In our opinion, \$7,000 is a very small price to pay to begin a project so close to our hearts, and we feel you will agree. This is an extremely good investment for the Foundation, because it allows us to initiate a project of high caliber with very modest funding, which in turn will give us results on which to base future steps of the project in a very short time.

FCDMF had submitted an “RFP” (Request For Proposal) to both Silagene in New Jersey, and Dr. Coates at the University of Missouri. While the University of Missouri did not submit a proposal, Silagene offered a new and innovative proposal. After careful review and consideration, Finding the Cure for DM Foundation feels it to be in the best interest of our DM babies, and the best use of our supporters' collective donation dollars, to contribute to this new, and very promising Silagene study.

While this new research project sounds very exciting and promising, please keep in mind that it may continue several years (as research does) and will require continual funding during this process. Silagene feels that this initial portion of the study will demonstrate that the *U1 Adaptor* is able to silence canine SOD1 *in vitro* and yield results *in approximately 90 days*. Once successful and documented, the next step of the study will be more in depth, and require much more funding. We are hoping at this stage, to be in a position to appeal to larger, more able corporations, to help support this costly project in a way that most of us cannot.

But we won't stop there! Every dollar will help in some way, so no amount is too small. We will continue our Fundraising events, and taking either monetary donations, or contributions through the “Donation Station” on our website. We also hope those of you that initiate other and creative fundraising activities; will continue supporting FCDMF in this manner.

Below, you will find information about the Project Research Team that is so dedicated and committed to researching therapy for our special DM family members, as well as the project description.

Project Research Team:

Silagene Inc. is a Rutgers University "spin-out" company founded by Drs. Samuel Gunderson and Rafal Goracznik, the two inventors of the U1 Adaptor technology. Rutgers University owns the U1 Adaptor patent and has exclusively licensed the technology to Silagene, whose sole mission is to develop U1 Adaptor into a new therapeutic platform able to treat a wide variety of diseases in humans and animals.

Dr. Samuel Gunderson is President and co-founder of Silagene Inc. and Associate Professor of Molecular Biology and Biochemistry at Rutgers University. He holds several gene silencing patents, and has had 16 years of continuous NIH funding to support his research laboratory at Rutgers.

Dr. Rafal Goracznik is currently the Chief Science Officer at Silagene Inc. He co-invented the U1 Adaptor technology while working in Dr. Gunderson's laboratory. Previously, he worked at the Cleveland Clinic Foundation and at UMDNJ. Dr. Goracznik will be the principle investigator responsible for completing the work described in the "New Hope" project.

Project Description:

Treatments that can alter the progressive course of DM disease have been difficult to identify and implement. The pathogenesis of DM is incompletely understood, but widely believed to be driven by mutations in the SOD1 gene. Moreover, delivery of therapeutics to the brain presents special challenges. This study proposes initial testing of a novel therapeutic modality that draws upon a recent innovation in therapeutic gene silencing, using U1 Adaptor oligonucleotides.

Gene silencing has attracted much attention, as the ability to silence a disease-causing gene would offer clinicians and veterinarians, new personalized therapy that targets the root cause of the disease that is the gene. As a therapeutic, gene silencing does not modify or in any way alter the genetic makeup of the patient and is not inheritable. In spite of initial hype and excitement, older gene silencing technologies have struggled to produce effective therapies, largely because the drug molecules are unstable and hard to deliver into the diseased cells, thus requiring high doses to achieve efficacy, but with consequent toxicity.

This study utilizes the novel and patented U1 Adaptor technology that Silagene has the license to. The U1 Adaptor technology offers a solution because as a drug, it is *highly stable and easily deliverable to diseased cells in vivo, without compromising biological activity*. The potential benefits are:

- adjustable suppression** of the target gene,
- capacity to suppress multiple genes** for personalized therapy,
- minimally invasive delivery**, targeted to the brain and brain regions
- rational design methodology that can **shorten by many years, the preclinical development time**.

In this initial phase of the study, Silagene will dedicate 3 months to an intensive laboratory effort to design and synthesize a panel of anti-SOD1 U1 Adaptors. Each U1 Adaptor from this panel will be transfected into canine cells *in vitro*, in order to identify which are able to best silence the mutant SOD1 gene.

The two best anti-SOD1 U1 Adaptors will then be moved forward to a next phase for *in vivo* testing, including in appropriate animal models, a step that will require additional funding and recruitment of a clinical veterinarian into the team. This will enable studies to identify U1 Adaptor formulations able to silence SOD1 in dog brain.

FCDMF is extremely excited, and very optimistic about this new and innovative study, and are so thankful to finally see additional interest in research dedicated to finding a potential therapeutic for our DM companions! We hope that you are as well!

Silagene will work with FCDMF to provide periodic updates, as permitted, and as this study progresses.

We appreciate tremendously, your continued support, and look forward to “**New Hope**” in treating Canine Degenerative Myelopathy in the New Year, 2016!

With New Hope in our hearts,

Cindy, Mindy, Renee, & Debbie

The “Finding the Cure for DM Foundation” Team



www.cure4dm.org