



Cold Spring Harbor Laboratory

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Dear Friends of T.J.,

I am writing to update you about exciting new developments in rhabdomyosarcoma research at Cold Spring Harbor Laboratory (CSHL). We have made significant progress in a very short amount of time – progress that is possible because of your generous contributions.

As you know, in May 2014 we hosted the first Rhabdomyosarcoma Symposium, bringing together researchers and clinicians from around the world. This meeting crystallized the most promising avenues for future research and outlined the path necessary to advance research and move into the clinic. It allowed us to identify clear directions that have become the catalyst for a new Sarcoma Research Project.

This project is a directed effort to understand how sarcomas arise and to identify new therapeutic strategies. The first stage of the project focuses on rhabdomyosarcoma. With the help of local supporters like you, as well as the Christina Renna Foundation and the Michelle Paternoster Memorial Foundation and internal CSHL support, we have created a program to understand rhabdomyosarcoma and rapidly move toward the identification of new therapeutic targets for drug development.

CSHL scientist Chris Vakoc M.D, Ph.D. is spearheading the initial research. He has developed a powerful approach that pinpoints the genetic weaknesses within a population of cancer cells. Vakoc's strategy has already been successful in developing a drug for acute myeloid leukemia, with this treatment now in Phase 1 clinical trials.

Vakoc is working in close collaboration with Charles Keller M.D., a physician and professor at Oregon Health and Science University, who is one of the world leaders in rhabdomyosarcoma research. Keller offers tremendous insight into the disease and has already created tools that will be instrumental in moving rhabdomyosarcoma research forward.

Their plan is already in action. Keller visited CSHL in September and presented a seminar to summarize the current state of rhabdomyosarcoma research. We are hosting one of Keller's senior research associates for a three-week visit to CSHL. While she is here, she is training Vakoc's scientists to work with the tools that the Keller lab has created. At the same time, Vakoc will teach her how to apply his technology so that she can bring the knowledge back to Keller's lab. The goal will be to collaborate over the next couple of years and beyond to identify new therapy strategies for treating rhabdomyosarcoma.

It is your support that has made all of this possible. The partnership between the Friends of T.J. and CSHL presents us all with the unique opportunity not only to improve lives, but to save them.

With my sincere thanks,