Title: Phase I Clinical Trial: Targeted Therapy for Ewing’s Sarcoma Patients
Institution: Mary Crowley Cancer Research Centers
Name of Researcher: Dr. Maurizio Ghisoli, M.D.

Mary Crowley Cancer Research Centers leases a 10,000 square foot, out-patient, biosafety level II approved, translational clinic from Medical City Hospital in Dallas, Texas. This facility is experienced in administration of vaccines, oncolytic viruses, and other gene therapeutics under Phase I and II FDA approved clinical trials. The clinical staff is routinely involved in pharmacokinetics, pharmacodynamic and immune evaluation protocols, as well as assessment of adverse events for participants in research trials. Our team provides expertise in clinical trial design in compliance with the FDA.

The mission of Mary Crowley Cancer Research is to expand treatment options for all cancer patients through investigational vaccine, gene, and cellular therapies. We achieve this mission by conducting clinical trials that provide hope to cancer patients today, while advancing science for patients tomorrow. Mary Crowley is now bringing this hope to one of the most overlooked demographics in cancer—children. Development of new cancer drugs for pediatric patients is an afterthought to those for adults. Less than 16,000 children are diagnosed with cancer annually in the U.S., compared to 1.6 million adults. The high cost of drug development makes it economically infeasible for pharmaceutical companies to dedicate research money to such a small product market. Therefore, without philanthropic funding of clinical research, new treatments will never become available to children with cancers such as Ewing’s Sarcoma.

Ewing’s Sarcoma is an aggressive malignant bone and soft tissue cancer most commonly diagnosed in children and adolescents. Only 30% of patients with Ewing’s survive. The five-year survival of children who recur within two years of initial diagnosis is only 7%. New treatments, therefore, are desperately needed. However, there are very few clinical trials dedicated to Ewing’s Sarcoma; therefore, our work with Ewing’s is highly critical. With aid from private foundations, individuals, and families of Ewing’s patients, Mary Crowley has helped fund over $1 million for preclinical research to bring an innovative Ewing’s therapy from the laboratory to a clinical trial, for which we now seek funding. This targeted therapy opened as a Phase I clinical trial at Mary Crowley in early October. We respectfully request funds to support patient enrollment onto this targeted therapy clinical trial.

Our Phase I clinical trial will enroll patients with advanced Ewing’s Sarcoma whose prior treatments have failed. Ewing’s has a well-defined genetic abnormality—a fusion of two specific genes, known as a driver gene. This distinctive fusion enables a targeted therapy to precisely attack this mutated driver gene. Our preclinical research revealed over 90% knockdown of this driver gene, resulting in significant tumor growth reduction. Due to the complexity of this protocol and the amount of oversight and safety assessment required for a first-in-human trial for children, enrollment of each patient will cost approximately $25,000 for one cycle of treatment. A grant of $25,000, therefore, will give hope to a child and allow them to participate in this clinical trial.

While this targeted therapy trial will help children who have the worst expected outcomes and the fewest effective treatment options, the findings will also expedite research in other pediatric cancers, bringing about treatments that are more precise and less toxic than traditional therapies like chemotherapy. Thank you for your consideration of this request.