

Dear BallouSkies Family and Friends,

We are working diligently on multiple fronts to combat heart muscle disease in Duchenne muscular dystrophy (DMD), a genetic process that causes heart damage at some point in life of virtually all boys with DMD. One effort is an initial clinical study in boys with DMD of a drug called eplerenone; we have completed our enrollment goal of 40 subjects in a very carefully designed trial. As of November 2013, more than half of the individuals in the trial have completed all 1-year follow-up visits. These visits include MRI scans of the heart that allow us to measure the heart's function and extent of muscle damage. We expect that all 1-year follow up for the double-blind part of this study will be completed by mid-2014. It then takes a few months for statisticians to analyze the results so that we can then submit our findings to the rigorous process of independent scientific review. If this goes quickly, we expect to be able to present the results of this study by the beginning of 2015.

Also going on in parallel are continued experiments in a mouse model of DMD that are testing different types and combinations of medications to prevent muscle damage. This critical basic research is also designed to help us figure out exactly how the medicines affect different types of muscle that, in turn, will guide further refinement of treatment approaches and considerations of new strategies to keep muscles healthy over time.

Thanks to BallouSkies, the research team was successful in securing competitive funding from the National Institutes of Health. We started working on this in 2011, and our revised proposal was funded in September of 2013. This grant helps support both the clinical studies as well as the basic research, particularly covering personnel and overhead costs so that our work towards a cure for heart disease in DMD can continue uninterrupted.

We are grateful this Thanksgiving for the tremendous support and encouragement from not only BallouSkies but also the inspiring young men with DMD and their families who have come from so far to be part of the research studies. Here's to our collective good health so we may continue to make forward progress together!

BallouSkies Research Team
November 2013