The Ohio State University Medical Center

30 August, 2011

Dear BallouSkies Team,

Greetings from The Ohio State University, where your support continues to fuel advances in tackling the devastating consequences of muscular dystrophy. This letter summarizes our remarkable progress in just the last few months. Our goals remain: 1) to improve diagnosis of heart muscle disease for individuals with muscular dystrophies with noninvasive, high-resolution heart imaging so that beneficial treatment can be started sooner, 2) to better understand through research how muscle can be protected from damage to guide development of better treatments, and 3) to educate doctors and scientists so that our advances can benefit patients all around the world.

A huge accomplishment was publication this month of our dramatic study in a mouse model of muscular dystrophy in the American Heart Association's journal *Circulation*, the #1-rated scientific journal in cardiovascular medicine worldwide. In mice with genetic changes that cause similar muscle weakening seen in boys with muscular dystrophy, old drugs traditionally used to treat high blood pressure and advanced heart failure had a remarkably protective effect on both heart and skeletal muscle. This paper was published in print on August 2, 2011, and we have been getting feedback from patients, families, physicians and scientists from around the world ever since then. One example is an email I received from a father in Finland who also works in their ministry of health. We are now working together to find the right people who can champion better coordination of care between muscle disease specialists (neurologists) and heart disease specialists (cardiologists) in that country.

We have been working literally day and night to translate these promising findings from the lab to a clinical trial – a rigorous, double-blind study in patients is required to give affected individuals, their families and their doctors the necessary evidence to change practice. We've teamed up with one of the largest clinics for Duchenne muscular dystrophy patients in the country to execute a meticulous study in patients affected by this deadly disease, but as you can imagine the resources needed to do this are considerable. We have a tremendous amount of volunteer time provided by expert physicians and scientists but we also need to support the people who are working very hard on regulatory documents, screening patients for eligibility in the trial and arranging all aspects of follow-up visits and coordination of care for study participants. Additionally, there are costs associated with the tests performed over the course of the trial that noninvasively evaluate heart and muscle so that we can objectively measure how well the medicine works to improve health and outcomes.

With the continued support of BallouSkies, we are confident that our dedicated team of researchers and physicians will complete this trial over the course of the next year, generating much-needed information to improve the lives of people with muscular dystrophies. We thank you for your commitment to helping us achieve these goals and we renew our pledge to be careful stewards of your support.

Wishing you good health, good friends and good cheer in 2011,

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