Medical Center

30 December 2011

Dear BallouSkies Team,

Greetings from The Ohio State University, where 2011 has been nothing short of a remarkable year thanks to your support in progressing towards our goals of: 1) improving detection of heart muscle disease in individuals with muscular dystrophies, 2) refining understanding through research how muscle can be protected from damage, 3) translating 'benchtop' research progress to clinical trials and 4) educating doctors and scientists so that our advances can benefit patients all around the world.

Our initial work in mice with genetic changes that cause similar muscle weakening seen in boys with muscular dystrophy showed that old drugs used to treat high blood pressure and advanced heart failure appeared to have a protective effect on both heart and skeletal muscle. Since this work was published in August, we have received a flood of interest from patients, families, physicians and scientists from around the world. There are now multiple efforts well underway to translate these findings to a meticulously-designed clinical trial: such a trial is *essential* to provide patients, families and doctors solid evidence that these medications work as we think they should based on our mouse studies to prevent muscle damage and reduce death and disability.

As I mentioned in the last update, we have teamed up with one of the largest clinics for Duchenne muscular dystrophy patients in the country to execute such a clinical trial. We have achieved the following milestones in the last few months: i) secured a supply of study drug from the pharmaceutical manufacturer, ii) identified a study pharmacy that can take these tablets and manufacture uniform capsules that look identical but contain either the study medicine or placebo to be assigned randomly to patients enrolled in the trial, iii) obtained approval from each institution's Human Subject's Research Committee, also called Institutional Review Boards, comprised of independent clinicians, scientists and lay people that make sure our study is conducted as safely as possible, iv) secured an exemption from the Food and Drug Administration that allows us to test existing drugs for a new use i.e. treatment of muscular dystrophy. We are also conducting in parallel additional preclinical studies that allow us to answer questions like: does either drug work just as well alone as in combination? how do the drugs work? can treatment be improved based on these results? We anticipate that enrollment in the clinical trial will begin in February, 2012, we will complete baseline enrollment over the next 6 months and complete all 1-year follow-up over the ensuing 12 months. We have set an ambitious timetable targeting presentation of preliminary results by the end of 2013 with appropriate benchmarks along the way.

With the continued support of BallouSkies, we are confident that our dedicated team of researchers and physicians will ultimately generate 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.

Here's wishing you good health, good friends and good cheer in 2012,

Subha V. Raman

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