BioMarin Update on Duchenne Muscular Dystrophy Program April 2015

We would like to update the Duchenne community on developments in our exon skipping program for exon 51. We remain committed to redosing all of the boys who were previously receiving the investigational drug under this program where this is possible. It is important for the understanding of the disease and the future development of therapies that we continue to collect medical information from boys who are redosed.

BioMarin are pleased to announce that we will recommence dosing as part of a new clinical trial with a new approved protocol. Boys will be assessed at their original study sites as part of the enrolment process. Ambulation will not be an inclusion or exclusion criterion for enrolment, but boys will need to have been previously dosed as part of a clinical trial.

We are now talking with the investigators at the study sites to provide them with more information about the new trial. There will need to be some local regulatory approvals prior to the start of the study and in some cases this could take several months. More detailed patient information will be available to families once it is approved by the local Ethics Committees.

We are also working with the study sites to see how we might redose boys nearer to their homes, while retaining close contact with the study sites. We hope that this will significantly reduce the overall burden and make this study easier for boys and their families.

Your treating doctors and medical teams remain the best source of advice for the care of your sons. Families are encouraged to stay in close contact with their medical teams and also with the patient associations for regular updates about our Duchenne programs.

For patient and family group leaders, please contact Celia Economides or Paul Humphrey from BioMarin Patient Advocacy

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