January 18th 2016

BioMarin Update to the Duchenne Community

As you are likely aware, last week, we announced that the United States Food and Drug Administration (FDA) issued a Complete Response Letter to BioMarin for our new drug application for drisapersen for the treatment of Duchenne muscular dystrophy amenable to exon 51 skipping. We are disappointed to let you know that the FDA has concluded that the standard of substantial evidence of effectiveness has not been met. We are reviewing the Complete Response Letter and will work with the FDA to determine the appropriate next steps regarding this application.

While we are disappointed in the FDA's decision, we continue to believe that drisapersen provides a meaningful benefit to boys with Duchenne amenable to exon 51 skipping. The ongoing drisapersen extension studies will continue, as will the ongoing clinical trials for other exon-skipping oligonucleotides, BMN 044, BMN 045 and BMN 053, while BioMarin is exploring next steps for this application. Patients currently receiving drisapersen, BMN 044, BMN 045 and BMN 053 will remain on therapy.

This is without question disappointing and heartbreaking, especially for those that could potentially benefit from a drug like drisapersen. We are incredibly grateful to the Duchenne community for its unwavering support during this process and will continue to work with you as we move forward. The patients and families that we serve are extraordinary, and we appreciate their participation in the clinical trials. The optimism, resiliency and thoughtfulness of our patients motivate us every single day.

Kind regards,

BioMarin Patient Advocacy