April, 2016

BioMarin Update to the Duchenne Community
Welcome to BioMarin's latest update to the Duchenne Community. Here is the latest information regarding BioMarin's products in development for the treatment of DMD.

EU Marketing Authorization Application (MAA) for drisapersen
The EU drisapersen marketing application remains on target for a potential CHMP opinion in the first half of 2016.

Extension studies of drisapersen, including IV route of administration as an option
Maintenance of dosing for chronic disease through a regulatory approval has been a hallmark of BioMarin's programs to date. However, where possible, we are continuing to make drisapersen available as an investigational therapy in previously treated boys and young men. We have three separate clinical trials providing extension treatment of drisapersen for those who previously participated in clinical trials.

In addition to the standard subcutaneous route of administration, we have introduced an option of IV administration in each of these studies for patients who may not be able to tolerate continued subcutaneous dosing.

Study Number	Countries	Eligibility	Study Purpose	Status
DMD114673 (initiated via protocol amendment)	Belgium & Sweden	Prior DMD114673 subjects	Assess effect, safety & tolerability of long-term treatment and safety, tolerability & PK of IV administration	Enrollment complete Study Ongoing
DMD115501 (initiated via protocol amendment)	United States, Canada	Prior DMD114876 subjects Prior DMD114349 subjects from US & Canada	Assess long-term safety, tolerability and efficacy	Enrollment complete. Study Ongoing; IV option implemented at 2 sites in Canada and 1 in the US
BMN051-302 (new protocol)	10 sites in 5 countries	Prior DMD114349 subjects	Assess effect, safety & tolerability of long-term treatment with subcutaneous administration and safety,	Currently recruiting

Study Number	Countries	Eligibility	Study Purpose	Status
		Prior DMD114117 subjects	tolerability & PK of IV administration	

Additional DMD Programs
Clinical trials for other exon-skipping oligonucleotides, BMN 044, BMN 045 and BMN 053, are ongoing.

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.

We are grateful for the continued support from the Duchenne community.

Kind regards,

BioMarin Patient Advocacy

For patient and family group leaders, please contact BioMarin Patient Advocacy at patientadvocacy@bmrn.com or, in the North America, BioMarin Medical Affairs at medinfo@bmrn.com. In Europe, Middle East, Africa, or Asia Pacific, please contact paul.humphrey@bmrn.com.

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