Dear members of the SMA community,

In response to your requests for information, we are providing an update on the progress made in moving the nusinersen development program forward. On October 7th, we completed the submission of our marketing authorisation application (MAA) to the European Medicines Agency (EMA), which marks a critical step in the pathway to a possible approval of nusinersen.

As soon as the EMA receives an application, a validation or review period begins, in which they will review the submission to ensure the application is complete and sufficient to proceed. In the EU, companies are notified that the submission has been validated approximately 30 days after submission and then the review period starts. However, the actual timing may vary depending upon the specifics of the validation.

Regulatory review varies but, in general, standard review in the EU averages between 13-15 months. Recently, the EMA's Committee for Medicinal Products for Human Use (CHMP) granted Accelerated Assessment to nusinersen, which can reduce the standard review time from 210 days to 150 days. However, the actual timing may vary depending upon the specifics of a submission's review. The timeline is largely driven by the questions regulators have for us once they look at the data and our ability to provide them with the information they need.

We are providing all the data we have to date and are seeking a broad label for the treatment of SMA. The product label will instruct physicians on the use of nusinersen, if approved. Regulators review the totality of the data provided, and if approved, the content and breadth of the final label is decided by the agency based on their assessment of the data we provide to them.

In addition, we continue to be encouraged by the data from a number of nusinersen studies recently presented in late-breaking session at the 2016 World Muscle Society Congress in Granada, Spain.

We appreciate the questions we have received from the SMA community over the past several weeks and are pleased to share this progress for the nusinersen program. Please know we understand every day counts for patients with SMA and their families. Each member of our team is working tirelessly and exploring every option to speed the development, and hopefully, approval of nusinersen.

Sincerely,

Biogen and Ionis