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 towards a potential treatment option for Spinal Muscular Atrophy (SMA). We can confirm the positive results from a pre-specified interim analysis of the CHERISH study in later-onset (consistent with Type 2) SMA. The analysis found that children receiving nusinersen experienced a statistically significant improvement in motor function compared to those who did not receive treatment. Nusinersen demonstrated a favorable safety profile in the trial. With the positive interim analysis, the CHERISH study will be stopped and participants will be able to transition into the SHINE open-label extension study to receive nusinersen.

We conducted detailed scientific analyses using all the available data to identify the earliest point in the CHERISH study when an interim analysis could be conducted to provide us with the well-controlled data needed to help assess the safety and efficacy of our investigational compound. We will make regulators around the globe aware of these data and work with them to identify the most efficient path toward achieving our goal of bringing nusinersen to the SMA community as quickly as possible. Nusinersen has received Accelerated Assessment status from the EMA.

Biogen recently initiated a global expanded access program (EAP) in infantile-onset SMA in October. Given the complexities and operational aspects of opening and EAP in SMA, the initial phase in infantile-onset has taken longer than expected to open. We will continue to explore the ways in which we could broaden the EAP to include patients with Type 2 SMA in geographies where the time to regulatory approval is anticipated to be longer. Obtaining regulatory approval remains our number one priority as we continue to believe it is the best way to provide broad and sustainable access to the SMA community.

Every day, we are spurred on by the stories we hear from the SMA community and are very grateful to all the families participating in our clinical studies as well as the physicians who work tirelessly to care for them day in and day out. We will continue our relentless pursuit of moving nusinersen from an investigational compound to a potential treatment option for the community. We would like to express our deepest appreciation to all involved in this important journey.

Biogen and Ionis Pharmaceuticals.

###