Dear members of the SMA community,

In response to a request from SMA Europe, Biogen and the board members of SMA Europe had a call on Wednesday 11th January, 2017.

The Biogen SMA Lead for Europe opened the call by reiterating our common objective of securing swift approval of nusinersen for the broadest spectrum of SMA patients possible. In response to a further request to share details of the discussion points made throughout the call, Biogen is providing the following update. As you know, there are times we are unable to disclose certain details due to compliance, confidentiality and/ or legal restrictions. However, we are committed to sharing as much information as we are able.

The main points in this update include: the progress being made in Europe to obtain regulatory approval for nusinersen, clarification around recent news about the U.S. price that was released late last year, an update on the Expanded Access Program (EAP) in Europe and finally a response to address the request for information on clinical data. But first, let us reiterate our number one priority is the same as yours - to obtain regulatory approval as soon as we can.

2016 was an historic year for the SMA community. As announced in December, nusinersen received United States FDA regulatory approval for the treatment of all individuals with SMA in the United States. Nusinersen is the first treatment for SMA, demonstrating statistically significant improvements in both motor function and survival.

However, regulatory approval, and pricing pathways in Europe follow a different process, with different timelines, compared to the U.S. The European Medicines Agency, (EMA) accepted our filing in the autumn of 2016. In addition, the EMA's Committee for Medicinal Products for Human Use (CHMP) granted Accelerated Assessment (AA) status to nusinersen, which can reduce the standard review time. An AA status is a regulatory pathway that can be granted by the CHMP that reduces the standard review time for a regulatory filing by at least 60 days (from the standard 210 days to 150). In addition to the time that the CHMP reviews the application, time is added on for the company to respond to questions (termed "clock-stops"). This time can vary depending on the questions asked - and the time it takes for the company to respond. Following a positive opinion by the CHMP, there is another step whereby the European Commission issues the marketing authorization (approval of the product). This process takes up to 67 days for a product under a standard review and has the potential to be reduced slightly for a product under accelerated assessment.

We are providing all the data we have to date. Regulators are reviewing the totality of the data available and will determine approval and the final nusinersen label. In case of approval, the final label will be determined by them and will identify which patients are eligible to be treated with nusinersen. Once approved by the EMA, the next important steps in the process are pricing and reimbursement.

Subject to a positive CHMP opinion and approval by the European Commission, access to the treatment will vary between countries based on the access and reimbursement pathway established in each country. Biogen recognizes that cost and access to treatments are key considerations for patients, providers, payers, and policy makers and Biogen is committed to working with health-care systems and governments to find solutions to achieve nusinersen's access to patients.

Depending on approval and label in the EU, we cannot speculate on the price within European countries. The healthcare systems and pathways to access in the European countries are very different than the US. However, with the recent approval by the FDA and access within the US, we feel it is important to share the principles on which Biogen articulates its approach to prices of our medicines. Consistent with the pricing of the overall Biogen portfolio of therapies, we strive to achieve an appropriate balance among three key Biogen pricing principles – clinical value the therapy provides to patients and families, the impact this new treatment has on the healthcare system, and finally our ability to fulfill our long-term commitment to patients and advancing science through the funding of research and development. We will be using these principles to determine the European price.

We want to assure you that we are committed to providing access to patients who may benefit from nusinersen. We have begun working, where feasible, with health-care systems and governments to discuss solutions to achieve nusinersen's access as quickly as possible, should nusinersen be approved. We will continue to address your requests for more information.

Based on the unmet medical need in SMA and with a great sense of urgency, Biogen initiated one of the largest global expanded access programs (EAP) for eligible patients in infantile-onset SMA (most likely to develop Type 1) in September 2016, before filing with the regulatory agencies. This was initially initiated at existing nusinersen clinical trial sites in countries where EAPs are permitted according to local laws and regulations, where it can be operationalized and where there is a path to long-term availability and reimbursement of nusinersen.

In response to requests from clinicians, we are currently expanding the nusinersen infantile-onset EAP to countries which meet the criteria above. Patients enrolled on the EU EAP program for eligible patients in infantile-onset SMA will continue on this program until reimbursement of nusinersen in their country of origin. The decision to continue enrollment of eligible patients with infantile-onset SMA upon EMA approval has yet to be made.

Given the complexities and operational aspects of opening an EAP in SMA, the initial phase in infantile-onset has taken longer than expected to open. Considering the high unmet medical need and sense of urgency to treat infantile-onset SMA the EAP program will continue to focus on these infants. In parallel, we will continue to evaluate the feasibility to broaden the EAP to include later-onset SMA (consistent with Type 2). Obtaining regulatory approval remains our number one priority, as we believe it is the best way to provide broad and sustainable access to the SMA community.

Finally, we had several questions in relation to clinical data and study results. For any specific, medical scientific questions related to nusinersen please refer to SMA experts in your respective countries. We will be disclosing the details throughout the course of 2017 at medical scientific conferences and on public websites in accordance with local legal and regulatory requirements.

We have greatly appreciated the opportunity to have this important discussion with the board members of SMA Europe and thank them for the opportunity. We will continue to work closely with these patient advocacy organizations so the perspectives of patients and families are reflected in every decision we make. And, of course, we remain committed to supporting the SMA community and partnering with healthcare systems to ensure that patients who may benefit from nusinersen will have access to therapy, once approved in the EU. We continue to understand the urgency for patients with SMA and their families. Each member of our team and the member organizations of SMA Europe are working tirelessly towards nusinersen approval.