POSITION STATEMENT ON ACCESS TO THERAPIES FOR SMA

Spinal muscular atrophy (SMA) is a genetic disease that causes muscle weakness and other impairments. It has varying degrees of severity but in all cases, it causes progressive disability and dramatically impacts the lives of those affected and their families.

SMA Europe is the umbrella organisation founded in 2006 to represent the SMA community in Europe. Today, it represents patients across 17 countries in Europe. Our core objectives are to contribute to bring effective therapies to patients in a timely and sustainable way as well as to encourage optimal patient care.

For decades, SMA Europe and its constituent members financed research that has laid the foundation to get to where we are today: a commercially available, meaningful, targeted, therapy to treat SMA and several other promising compounds in clinical trials or in the pipeline.

SMA Europe is a valuable and knowledgeable participant with a genuine and transparent interest in this arena. SMA Europe expects to be respected as an equal stakeholder and as a consequence, to be involved in all aspects of the SMA therapy life-cycle. Our organisation and its members can provide first-hand knowledge and experience of the disease and of the unmet needs of the patient population. We contribute to the education on patients' expectations, on the burden of living with the disease and on the potential economic and societal benefits of stabilising, or improving the health status of the SMA population. SMA Europe is currently working on identifying which aspects of patients' lives are more impacted by SMA. This can provide important information that will help inform which therapy effects are the most meaningful to patients.

SMA Europe also contributes to trials by providing a patient-centric perspective in their design. We provide, when possible, the patients' perspective on the appraisal processes for drug approvals, so that the potential impact of a drug on patients' everyday life and that of their families, is taken into account. ***Ultimately, we strive to form a patient-partnership with the different stakeholders involved in these processes.***

The SMA community finds that currently, there is a gap between the safety and efficacy assessment of drugs and subsequent access to treatments, due to pricing issues. This is the reason why SMA Europe believes there is an urgent need for a more transparent system of how prices are decided upon, and how agreements on reimbursement are reached. ***Pricing and reimbursement schemes of new medicines must include ethical and practical consumer considerations***, especially in the rare disease field.

SMA Europe acknowledges and understands that developing new drugs involves considerable commercial risk and significant investment, and that rare diseases have limited financial returns in market terms, as numbers of potential beneficiaries are low. We also recognise the burden that a very costly drug can have on a health care system. ***Patients need new collaborative***

schemes to ensure that the ultimate purpose of the whole drug development process and approval – patients receiving treatment – is achieved in a timely and efficient manner. This should involve all parties working together across all stages of the process – including the pricing debate.

SMA Europe considers that it can positively contribute to the complicated issues around pricing and reimbursement by being involved in them.

Currently, *insufficient involvement of the community on pricing issues combined with high list prices*, has brought undesired delays and limited access to treatment in key European markets. This has brought a loss of support in the community for new drug initiatives***. If patient representatives could work more closely with other stakeholders, these negative situations would be avoided and the ultimate goal of giving patients the medicines they need would be ensured.***