SMA Europe's Statement
Dec 26, 2019

AVXS-101 (Zolgensma®) to be made available globally through a controversial programme

The biotechnology company Avexis, a wholly owned subsidiary of Novartis, has recently announced the launch of a global managed-access programme (MAP) for its gene therapy product AVXS-101.

Under the programme, Avexis intends to make a limited number of AVXS-101 packages available at no cost for use in countries where the drug has not yet received regulatory approval. Further details can be found **on the company's website**.

SMA Europe has received the proposed programme with mixed feelings. Given the high unmet need of a significant part of the SMA community, we fully appreciate the fact that the programme will offer access to this promising therapeutic option to a number of infants across the globe. Every saved life is a gift.

At the same time, we find it hard to support the manner in which the program proposes to establish patient eligibility and carry out patient selection. We believe that a health lottery is an inappropriate way of addressing the unmet medical need in this severe disease in a fair and equitable way. The approach adopted by Avexis raises a number of ethical questions.

We are also concerned about the lack of involvement, at the programme's design and implementation stages, of recognised neuromuscular experts as well as European SMA patient organisations. We consider it unacceptable that decisions on the medical treatment will be effectively undertaken not by the treating clinician but by a pharmaceutical company, whilst proper clinical assessment and triaging will be replaced with a blind draw. We are alarmed that the programme will make thousands of SMA babies compete with each other for a life-saving treatment, splitting tightly knit communities and turning this experimental drug into a coveted prize.

Of concern are also the far-reaching ramifications of operating such a programme when the manufacturer has not carried out sufficient medical education work about their product among clinicians, authorities and patients themselves.

Finally, given the short history of AVXS-101 and limited safety and efficacy data, we believe that long-term follow-up must be provided for all patients who receive this experimental gene therapy drug, be it as standalone therapy or in combination with other drugs. It is crucial to systematically capture all data generated through the AVXS-101 programme so as to gain a better understanding on the drug's safety, efficacy and the real value for the patient.

SMA Europe, supported by its Scientific Advisory Board, has reached out to Avexis and offered to work jointly on improving the design of the AVXS-101 managed-access programme so that this new therapeutic option is made available in a clinically justified, safe and fair manner.

As this programme unfolds, we stress that parents of potentially eligible children discuss all the available therapeutic options with their clinicians, so as not to delay the start of treatment. Early introduction of treatment is crucial in spinal muscular atrophy.

The goal of SMA Europe is to support fair and equal access to effective therapies for spinal muscular atrophy. Working with healthcare professionals, public authorities and the pharmaceutical industry, we strive that people affected by spinal muscular atrophy have access to all the therapeutic options they could benefit from. Our long-stated position is that therapies for spinal muscular atrophy should be priced in a way that will allow wide and equitable access to treatment and ensure long-term sustainability of healthcare systems in our countries.

SMA Europe is committed to a better future for all people affected by spinal muscular atrophy and their near and dear ones.