-------- Weitergeleitete Nachricht --------

Betreff: Ask EMA - (ASK-61575) Letter dated 7 October 2019 on the approval of Zolgensema manufactured by Novartis

Datum: Tue, 22 Oct 2019 16:17:00 +0200 (CEST)

Von: AskEMA <askema-no_reply@ema.europa.eu>

Antwort an: askema-no_reply@ema.europa.eu

An: info@dgm.org

Re: EMA request reference ASK-61575

Dear Dr. iur. Perschke, dear Dr. Schwersenz,

Thank you for writing to us about Zolgensma.

EMA is fully aware of the needs of patients with SMA and how difficult it is waiting for development and authorisation of safe and effective medicines. We wish to assure you that we do all that we can to assess medicines as efficiently and promptly as possible, while maintaining our responsibility to ensure that a medicine's benefits outweigh its risks before it is authorised.

As you are aware, the evaluation for Zolgensma started in November 2018 and is ongoing. EMA's scientific Committees for Advanced Therapies (CAT) and for Medicinal Products for Human Use (CHMP) are both currently assessing Zolgensma. AveXis is currently preparing its responses to a second round of questions from the two Committees.

When EMA makes its recommendation, this will be published on our website as part of that month's highlights from the CHMP, the scientific committee responsible for human medicines (https://www.ema.europa.eu/en/committees/chmp/chmp-agendas-minutes-highlights).

Please be aware that if EMA makes a positive recommendation there will be a few more steps before Zolgensma can be made available to patients. Firstly, the recommendation will be sent to the European Commission, which is the only body with the legal authority to authorise the medicine for marketing across the EU. Once the Commission authorises a medicine, it is then up to the company to decide in which EU countries it will first market the medicine, and to negotiate with the national authorities of those countries on prices and reimbursement. This may mean that it takes a little while before the first patients can be treated.

Unfortunately, availability of a medicine under a compassionate use programme is outside the EMA scope of responsibility, as these are managed by the national competent authority (BfArM in Germany https://www.bfarm.de/DE/Home/home_node.html). We can only advise to contact the company (Avexis Netherlands BV, email: MedInfo@avexis.com) on this topic.

I hope this information is of some help. I wish to express again how mindful EMA staff and committees are of the needs of patients in this evaluation and all of our work.

Best regards,
Elizabeth Scanlan
Stakeholders and Communication Division
Please help us to improve our service by giving us your feedback – https://ec.europa.eu/eusurvey/runner/AskEMA

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