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sowie

Initiative „Forschung und Therapie“ für SMA
im Förderverein in der DGM e.V.

Freiburg, 7.10.2019

Approval of Onasemnogene Abeparvovec-xioi (Zolgensma®) manufactured by Novartis;

To whom it may concern

Dear Sir, Dear Madam,

let us first thank you for your work making it possible that safe and effective drugs reach the patients in Europe in due time. We approach you now because of the following issue.
It frequently happens that innovative pharmaceutical products are approved in the US by the FDA earlier than they are approved by EMA. This is usually a minor problem because many companies apply for Compassionate Use in Europe to make sure that patients in need can be provided with the new product as early as possible. Currently, however, we have great problems in Germany with the innovative product onasemnogene abeparvovec-xioi (Zolgensma®, Novartis) which was approved in the US on May 24th this year for the treatment of Spinal Muscular Atrophy in children up to the age of two years. Novartis has no intention to apply for Compassionate Use in Germany, amongst others, because Nusinersen (Spinraza®) manufactured by Biogen is available for the same indication.

Currently the case of a 13 months old boy with SMA has attracted great attention in Germany. The boy was treated with Nusinersen and in the opinion of the parents did not respond well enough to the treatment. The parents tried to collect money for a treatment with Zolgensma® in the US by a nationwide fundraising campaign. Before they reached their goal their health insurance agreed to finance the therapy in Germany.

Unfortunately, there have been reports in the media and on TV that even children who have already symptoms of SMA could be completely cured with onasemnogene abeparvovec-xioi (Zolgensma®). We certainly know that this is not the case and that the treatment effect depends, amongst others, on the stage of the disease and that an optimal drug effect can be expected mainly in presymptomatic children.

This case has put an enormous emotional pressure on parents in the same situation who have no access to this drug and has raised great anxiety. The parents develop the strong feeling not to doing everything humanly possible for their own child. There is also increasing pressure on the physicians to apply for "named patient import" which has strong limitations in the German health care system.
In this very difficult situation for the patients and their parents we, the German Patient Advocacy Group for this patients, approach you and ask you to come to a timely decision on the approval of onasemnogene abeparvovec-xioi.

Yours sincerely

Dr. iur. Stefan Perschke
chairman - DGM e.V.

Dr. Inge Schwersenz
chairwoman – Initiative SMA