



Community Statement from Novartis Gene Therapies UK: NHS is Temporarily Pausing Zolgensma Treatment in NHS-Covered Children older than 12 Months

22 Dec 2022

Dear SMA Community,

We acknowledge that NHS England, following discussions with the National MDT (NMDT), decided to make a temporary change to eligibility for Zolgensma[®] ▼ (onasemnogene abeparvovec). The NMDT reported, that recently, a small number of children, particularly those who are older and weighing more than 13.5 kg, have been noted to have drug reactions affecting the liver following the administration of Zolgensma. Hepatotoxicity is a known adverse event associated with the therapy and contained in the product's label. NHS England is temporarily pausing reimbursed use of Zolgensma in children in England older than 12 months of age.

SMA is a devastating genetic disorder and is still one of the leading genetic causes of infant death worldwide. We firmly believe in the overall favourable risk/benefit profile of Zolgensma, which to date has been used to treat more than 2,500 patients worldwide across clinical trials, managed access programs, and in the commercial setting. Over 90 children have received treatment with gene therapy in the United Kingdom.

Novartis is working closely with NHS England to address this temporary change in eligibility for Zolgensma for some NHS-covered SMA patients. We are unwavering in our commitment to the SMA community, given the urgent need to treat patients with SMA.

If you have any questions or have any concerns about this, please contact us.

Sincerely,

Dr Daniel Franks

Medical Director

Novartis Gene Therapies, UK & Ireland

Theodora Weisz

Head Patient Advocacy and Public Affairs

Novartis Gene Therapies, Europe

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See <https://yellowcard.mhra.gov.uk> for how to report side effects.

The current Zolgensma patient information leaflet can be found via the following link <https://www.medicines.org.uk/emc/files/pil.11572.pdf>.

Should you have any additional questions, please contact us at medinfo.gtx@novartis.com, by phone at +44 20 7949 4555 (Monday to Friday, 9AM – 5PM CET), or via our medical information website (<https://medinfo.novartis.com/gene-therapies/>).