Eflornithine (DFMO) in the treatment of high-risk neuroblastoma patients

Information for patients and families

on behalf of
UK Neuroblastoma Clinical Trials Group & CCLG Neuroblastoma Special Interest Group
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Eflornithine (Difluoromethylornithine or DFMO) is an oral drug, which has been investigated for its effects in neuroblastoma. In December 2023 it was approved by the FDA (Food and Drug Administration) in the US for patients with high-risk neuroblastoma who have had a response to prior multimodal treatment including anti-GD2 immunotherapy. This approval was mainly based on data comparing[1] a single arm Phase 2 trial study with US frontline treatment on the COG ANBL0032 trial[2].

In the trial, DFMO was given orally for two years after completion of treatment for high-risk neuroblastoma. According to the analysis[3], Four-year Event Free Survival from the end of immunotherapy was 84% in the DFMO group versus 72% in the non-DFMO group. Data from 360 patients was assessed for toxicity and safety; the most common adverse reactions were hearing loss, ear infection, fever, pneumonia, and diarrhoea. New or worsening hearing loss requiring new use of hearing aids occurred in 7% of patients.

There are significant limitations to the data, and ideally a randomised controlled trial (RCT) would have been done some years ago to produce robust scientific evidence. However, due to the existing data and FDA approval, a randomised trial is not now feasible. Data on the efficacy and safety of DFMO added to the end of standard SIOPEN multimodal therapy is lacking.

Following the FDA approval of DFMO, guidelines for treatment of neuroblastoma in the US were updated, with a recommendation[4] that doctors discuss DFMO as a continuation therapy option with patients and families, and it is now being widely prescribed in the US.

In Europe, an application for approval of DFMO use for high-risk neuroblastoma with the MHRA (Medicines and Healthcare products Regulatory Agency) and EMA (The European Medicines Agency) was made by the pharmaceutical company, Norgine. In the meantime, in October 2024, Norgine launched an Expanded Access Program (EAP) in the UK, to allow access to DFMO while approval and funding was sought. The program provided free of charge DFMO to patients, via their normal NHS oncology service, on a named patient basis. In April 2026 the application for approval was withdrawn and the EAP closed.

In light of the above, SIOPEN, the European Neuroblastoma Research Network, issued a statement, acknowledging use of DFMO in front-line treatment of high-risk neuroblastoma (Statements — SIOPEN Research Network).

DFMO is not therefore a standard part of neuroblastoma treatment in Europe, and there are no current approved or funded pathway for patients in the UK to receive it.

Families and patients who are considering DFMO as a maintenance therapy should see This update on the Solving Kids Cancer website for more information
www.solvingkidscancer.org.uk/dfmo-update-2026

References:

1. The Event-Free Survival hazard ratio (HR) was 0.48 (95% CI, 0.27 to 0.85) and Overall Survival HR was 0.32 (95% CI, 0.15 to 0.70) in favour of treatment with DFMO compared to no post-maintenance treatment.
2. Oesterheld J, Ferguson W, Kraveka JM, et al. Eflornithine as Postimmunotherapy Maintenance in High-Risk Neuroblastoma: Externally Controlled, Propensity Score-Matched Survival Outcome Comparisons. J Clin Oncol 2024; 42(1): 90-102.
3. Data from 91 patients on the trial was matched to 270 patients who had had the same front-line treatment regime on the COG ANBL0031 trial.
4. Bagatell R, Park JR, Acharya S, et al. Neuroblastoma, Version 2.2024, NCCN Clinical Practice Guidelines in Oncology. J Natl Compr Canc Netw 2024; 22(6): 413-33.