

A clinical phase I study reported significant shrinkage of [plexiform neurofibromas](#) following treatment with the MEK inhibitor [selumetinib](#).

Vaassen et al., reported an 11-year-old [Neurofibromatosis type 1](#) patient with a large [plexiform neurofibroma](#) of the neck that had led to a sharp-angled kinking of the cervical spine and subsequent [myelopathy](#). Although surgical stabilization of the [cervical vertebral column](#) was urgently recommended, the vertebral column was inaccessible due to extensive tumor growth. In this situation, treatment with the [MEK](#) inhibitor [trametinib](#) was initiated which resulted in a 22% reduction in tumor volume after 6 months of therapy and finally enabled surgery. These data show that MEK inhibitors may not lead to complete disappearance of NF1-associated plexiform neurofibromas but can be an essential step in a multimodal therapeutic approach for these tumors. The course of this patient suggests that MEK inhibitors are likely to play a significant role in providing a cure for one of the most devastating manifestations of NF1 ¹⁾.

¹⁾

Vaassen P, Dürr N, Röhrig A, Willing R, Rosenbaum T. Trametinib Induces Neurofibroma Shrinkage and Enables Surgery. *Neuropediatrics*. 2019 May 29. doi: 10.1055/s-0039-1691830. [Epub ahead of print] PubMed PMID: 31141829.

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