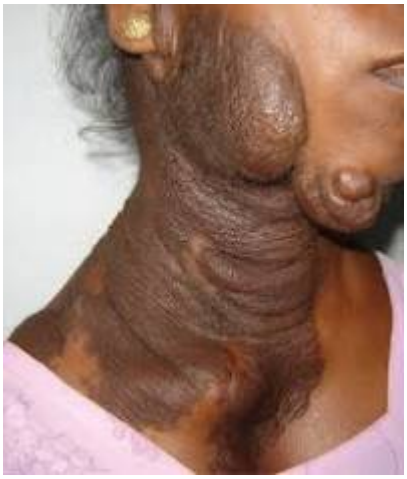
Plexiform neurofibroma treatment



Since plexiform neurofibromas are a major cause of the burden of disease and may also progress to malignancy, many efforts have been undertaken to find a cure for these tumors. However, neither surgery nor medication has so far produced a breakthrough therapeutic success.

Plexiform neurofibromas with sizable intraspinal extensions and resultant spinal cord compromise pose challenging management problems, because these lesions may involve multiple nerves and engulf adjacent vascular and visceral structures [1].

Decisions about surgical treatment and frequency of follow-up must be made judiciously and individualized for each patient [2].

Plexiform neurofibromas arising in the orbito-temporal area pose a greater challenge due to its critical function and cosmetic importance of the face. Such plexiform neurofibromas, separately designated as orbito-temporal plexiform neurofibromas, show complex symptoms such as severe ptosis, ectropion, lacrimal gland dysfunction, and even vision loss [3].

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 NF1 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 our 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 [4].

References

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