

# Melphalan

Melphalan (trade name Alkeran, in former USSR also known as Sarcolysin) is a [chemotherapy](#) drug belonging to the class of nitrogen mustard [alkylating agents](#).

Otherwise known as L-phenylalanine mustard, or L-PAM, melphalan is a phenylalanine derivative of mechlorethamine.

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Intra-arterial Melphalan for Neurologic [Non-Langerhans Cell Histiocytosis](#) <sup>1)</sup>.

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A phase II study explored the therapeutic gain obtained when exposing these patients to a combination of intra-arterially administered carboplatin and melphalan at first or second relapse as a salvage treatment in Glioblastoma recurrence. Fifty-one consecutive patients diagnosed with glioblastoma were accrued and offered this treatment at first or second relapse. A Karnofsky score of  $\geq 60$  was required, and when appropriate, patients were first reoperated prior to accrual. Patients enrolled were treated every 4 weeks (1 cycle) for up to 12 cycles. Progression was evaluated by Macdonald criteria. Primary end point surrogates were overall survival from diagnosis and study entry. Median survival from diagnosis and study entry was 23 and 11 months, respectively. The median time to progression was 5.2 months. All patients enrolled were treated for a minimum of 2 cycles. Hematologic toxicity was manageable, with an 8 % of grade II neutropenia, 12 % of grade II thrombocytopenia and 7 % of grade III thrombocytopenia. This therapeutic strategy represents an adequate option in the second-line treatment of Glioblastoma recurrence. The adjunction of an osmotic permeabilization could be considered to further expand delivery, and hopefully improve survival in these patients <sup>2)</sup>.

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Patsalides et al describe their initial experience with a novel therapeutic approach that consists of intraarterial (IA) infusion of chemotherapy to treat progressive [spinal metastases](#).

The main inclusion criterion was the presence of progressive, metastatic epidural disease to the spine causing spinal canal compromise in patients who were not candidates for the standard treatments of [radiation therapy](#) and/or surgery.

All tumor histological types were eligible for this trial. Using the transfemoral arterial approach and standard neurointerventional techniques, all patients were treated with IA infusion of [melphalan](#) in the arteries supplying the epidural tumor. The protocol allowed for up to 3 procedures repeated at 3- to 6-week intervals. Outcome measures included physiological measures:

- 1) Periprocedural complications according to the National Cancer Institute's Common Terminology Criteria for [Adverse Events](#)
- 2) MRI to assess for tumor response.

Nine patients with progressive spinal metastatic disease and cord compression were enrolled in a

Phase I clinical trial of selective IA chemotherapy. All patients had metastatic disease from solid organs and were not candidates for further radiation therapy or surgery. A total of 19 spinal intraarterial chemotherapy (SIAC) procedures were performed, and the follow-up period ranged from 1 to 7 months (median 3 months). There was 1 serious adverse event (febrile neutropenia). Local tumor control was seen in 8 of 9 patients, whereas tumor progression at the treated level was seen in 1 patient.

These preliminary results support the hypothesis that SIAC is feasible and safe <sup>3)</sup>

## Melphalan for Retinoblastoma

### Melphalan for Retinoblastoma

<sup>1)</sup>

Francis JH, Gobin YP, Nasany RA, Knopman J, Ulaner GA, Panageas K, Hatzoglou V, Salvaggio K, Abramson DH, Patsalides A, Diamond EL. Intra-arterial Melphalan for Neurologic [Non-Langerhans Cell Histiocytosis](#). *Neurology*. 2021 May 12;10.1212/WNL.0000000000012070. doi: 10.1212/WNL.0000000000012070. Epub ahead of print. PMID: 33980709.

<sup>2)</sup>

Fortin D, Morin PA, Belzile F, Mathieu D, Paré FM. Intra-arterial carboplatin as a salvage strategy in the treatment of Glioblastoma recurrence multiforme. *J Neurooncol*. 2014 Sep;119(2):397-403. doi: 10.1007/s11060-014-1504-4. Epub 2014 Jun 20. PubMed PMID: 24947313.

<sup>3)</sup>

Patsalides A, Yamada Y, Bilsky M, Lis E, Laufer I, Gobin YP. Spinal intraarterial chemotherapy: interim results of a Phase I clinical trial. *J Neurosurg Spine*. 2015 Oct 23;1-6. [Epub ahead of print] PubMed PMID: 26496162.

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