

Medical Therapies for Brain Tumors

CHEMOTHERAPY | BIOLOGICS | TARGETED THERAPY

Overview

Medical treatment for brain tumors uses chemotherapy, biologics, and targeted therapy. These drugs work alongside surgery and radiation to stop or slow the growth of tumor cells.

Only certain medicines and drugs can reach the brain. This is because of the blood-brain barrier, which prevents harmful things like infections or toxins from entering the brain. Some drugs can pass through the barrier, but many cannot.

About neuro-oncology

Neuro-oncology is a specialty that treats tumors and cancer in the brain and spinal cord. Your care team will design a treatment plan just for you. It is based on your tumor type, where it is located, molecular test results, and your symptoms. Your team may include:

- **Neuro-oncologist or oncologist:** prescribes the best medicines for your tumor type. They also help manage symptoms and side effects.
- **Neurosurgeon:** removes part or all of the tumor with surgery.
- **Radiation oncologist:** treats the tumor with radiation.
- **Nurse practitioner:** guides you through treatment, provides education, manages symptoms, and offers supportive care.
- **Neuropathologist:** studies the tissue cells to determine the tumor type, grade, and molecular profile.
- **Neuroradiologist:** reviews MRI scans to monitor how the tumor responds to treatment.

What is chemotherapy?

Chemotherapy uses anti-cancer drugs to treat some types of malignant brain tumors. Normal cells grow in a controlled way, but cancer cells grow out of control. Chemotherapy disrupts cell division in fast-growing cancer cells. Over time, these cancer cells die, and the tumor may shrink. Chemotherapy can also damage normal cells. However, healthy cells can repair themselves better than cancer cells.

Listed below are drugs used to treat brain tumors.

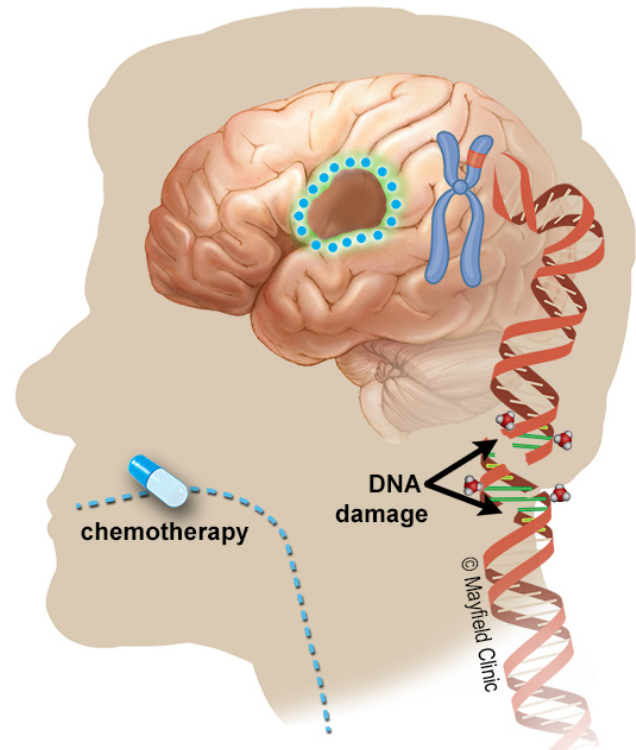


Figure 1. Chemotherapy (Temodar) works by damaging tumor DNA so the cells cannot divide or grow.

Temozolomide (Temodar®)

Temozolomide can pass through the blood-brain barrier. It works by damaging the tumor's DNA. It is often used to treat high-grade astrocytomas and glioblastomas. This drug is taken as a pill by mouth. The frequency depends on the treatment cycle. For example, you may take it every day during radiation therapy, and then once a month for five days in a row after radiation is finished.

Side effects of temozolomide are mild and include:

- **Nausea.** Your doctor will prescribe medicine to help prevent nausea. The nausea is usually tolerable.
- **Low blood counts.** Your doctor will closely monitor your blood counts (white blood cells and platelets). If needed, your dosage may be adjusted or stopped. It is rare (<1% of the time) for low blood counts to be permanent.
- **Infections.** This drug can weaken your immune system, so you will be monitored closely to prevent infections.

- **Constipation.** You will be given stool softeners and/or laxatives to help keep your bowels moving.
- **Fatigue.** This is a common side effect that your care team will help you manage. Try to set a good sleep schedule and rest when needed.

PCV regimen

The PCV regimen is a combination of three drugs: procarbazine, lomustine (CCNU), and vincristine. It is often used for high-grade oligodendrogliomas and recurrent gliomas. Treatment is given in cycles that repeat every 6 weeks:

- Day 1: take lomustine (CCNU) by mouth.
- Days 8-21: take procarbazine by mouth daily.
- Days 8 and 29: receive vincristine through an IV infusion into a vein.

Side effects of PCV include:

- **Bone marrow toxicity.** PCV can slow how your bone marrow works. Over time, it can profoundly lower your red and white blood cell counts, as well as platelets. Blood tests are taken to check your counts before starting a new cycle.
- **Fatigue.** This is a common side effect that your care team will help you manage. Try to set a good sleep schedule and rest when needed.
- **Nausea, vomiting.** Your doctor will prescribe medication to help prevent nausea.
- **Lung toxicity.** This is a rare but serious side effect producing a dry, persistent cough, shortness of breath, or fever.
- **Neuropathy.** This is a numbness and tingling feeling in the hands and feet.

What are biologics?

Biologic medicines are made using living cells, not chemical drugs. They can target specific proteins or molecules that control tumor growth.

Bevacizumab (Avastin®)

Brain tumors need a blood supply to grow and spread. Bevacizumab blocks a protein called VEGF and prevents new tumor-feeding blood vessels. It works by starving the tumor of food and oxygen. Bevacizumab is given through an IV (into a vein) or a port, once every two weeks.

Bevacizumab is used to treat brain tumors that are actively swelling and have not responded to other treatments.

Side effects of bevacizumab are related to how it affects blood vessels.

- **Hypertension.** Avastin can raise existing high blood pressure. Your doctor may prescribe

medicine to control it or adjust what you already take.

- **Proteinuria.** This can happen if the kidneys leak protein in the urine. Your doctor will check your kidney function. If needed, the doctor may prescribe an anti-hypertensive medication or pause your therapy. This drug rarely (<1% of the time) leads to kidney damage.
- **Bleeding.** This drug can cause bleeding in places such as your stomach or intestines. Tell your doctor if you take blood thinners or have any problems with your stomach or intestines.
- **Blood clots.** This drug can increase the risk of clots (including heart attack, stroke, leg clots, and lung emboli). Tell your doctor if you have a history of these problems and/or if you are having calf pain and swelling.

What is targeted therapy?

Targeted therapy uses precision medicines designed to block specific genes, proteins, or the tissue environment that allow tumor cells to grow and survive. They can spare healthy brain tissue and often cause fewer side effects.

Vorasidenib (Voranigo®)

Vorasidenib is a targeted therapy that delays tumor growth. It works by blocking mutated IDH1 and IDH2 enzymes. It is a pill given to treat low-grade gliomas with an IDH-mutation.

Side effects of vorasidenib include:

- **Fatigue.** This is a common side effect that your care team will help you manage. Try to set a good sleep schedule and rest when needed.
- **Diarrhea.** This can be managed through a regimen of hydration, diet changes, and over-the-counter medication.
- **Constipation.** You will be given stool softeners and/or laxatives to help keep your bowels moving.
- **Abdominal pain.** This can be caused by nausea or constipation. In rare cases, it can be a sign of acute liver injury.
- **Muscle pain.** If you begin feeling aches, stiffness, or pain in the arms and legs, your care team will help you manage this.
- **Elevated liver enzymes.** Your doctor will check your liver with blood tests and may pause or reduce medication if needed.

Supportive medications

During treatment, you may have symptoms that can be managed with medicines, such as:

- **Dexamethasone:** This is a steroid that helps reduce swelling in the brain and relieve nausea. Side effects may include trouble sleeping, irritability, upset stomach, high blood sugar, ulcers, and slow wound healing. It is

important to follow instructions for tapering the steroid dosage. Do not abruptly stop taking steroids.

- **Ondansetron (Zofran®):** Helps reduce nausea and vomiting.
- **Stool softeners/laxatives:** Helps prevent and reduce constipation from treatment.
- **Anti-epileptic medications:** Helps prevent or control seizures caused by a brain tumor.

Clinical trials

Clinical trials are research studies in which new treatments—drugs, diagnostics, procedures, and other therapies—are tested in people to see if they are safe and effective. Research is always being conducted to improve the standard of medical care. Information about current clinical trials is online at clinicaltrials.gov.

How is tumor treatment monitored?

Your care team will monitor your progress through regular exams, blood tests, and scans.

How a tumor responds to treatment or recurs depends on the tumor type and its molecular makeup. For example, tumors with the biomarker called MGMT (promoter methylation) are more likely to respond better to temozolomide.

Your care team will also watch for any signs or symptoms that the tumor is coming back or growing.

Since it is hard to predict if or when a tumor may recur, monitoring with MRI or CT scans is essential. Follow-up scans are done on a schedule based on your tumor type.

Sources & links

If you have more questions, please contact Mayfield Brain & Spine at 513-221-1100.

Sources

1. Stupp R, et al. Radiotherapy plus Concomitant and Adjuvant Temozolomide for Glioblastoma. *N Engl J Med* 352:987-996, 2005
2. Buckner JC, et al. Radiation plus Procarbazine, CCNU, and Vincristine in Low-Grade Glioma. *N Engl J Med* 374:1344-1355, 2016
3. Kim KJ, et al. Inhibition of vascular endothelial growth factor-induced angiogenesis suppresses tumor growth in vivo. *Nature* 362:841-844, 1993

4. Mellinghoff IK, et al. Vorasidenib in IDH1- or IDH2-Mutant Low-Grade Glioma. *N Engl J Med* 389:589-601, 2023

Support

Support groups provide an opportunity for patients and their families to share experiences, receive support, and learn about new treatments. Local support groups in the Cincinnati area include:

Jewish Hospital Brain Tumor Support Group (monthly in-person meeting), 513-467-7609

Cancer Support Community of Greater Cincinnati-Northern Kentucky, 513-791-4060

Links

American Brain Tumor Association www.abta.org

National Brain Tumor Society www.braintumor.org

mytumorID.org

Glossary

anemia: low levels of red blood cells to carry oxygen to your body tissues.

benign: does not invade nearby tissues or spread; not cancerous.

biologics: medications made using living organisms, unlike standard chemically synthesized drugs.

blood-brain barrier: a filtering mechanism made up of blood vessels and glial cells that protect the brain by keeping out many harmful substances. Only certain types of chemotherapy can cross this barrier to reach a brain tumor.

chemotherapy: treatment with toxic chemicals

malignant: having the properties of invasive growth and ability to spread to other areas.

metastasis: the spreading of malignant cells.

metastatic: cancerous tumor that has spread from its original source through the blood or lymph systems.

methylation: a biochemical process where a small chemical tag (a methyl group) is added to DNA. It acts as an "on/off" switch for the gene that repairs DNA damage.

neutropenia: low levels of neutrophils (a type of white blood cell that defends against fungal and bacterial infections).

targeted therapy: medications designed to pinpoint specific genes, proteins, or the tissue environment that allow tumor cells to grow and survive.

thrombocytopenia: low levels of platelets that are required to clot blood.



updated > 5.2026

reviewed by > Richard Curry, MD, Stephanie Green, NP, Mayfield Clinic, Cincinnati, Ohio

Mayfield Certified Health Info materials are written and developed by the Mayfield Clinic. We comply with the HONcode standard for trustworthy health information. This information is not intended to replace the medical advice of your health care provider. © Mayfield Clinic 1998-2026.