About Brain and Spinal Cord Tumors in Children

Overview and Types

If your child has just been diagnosed with brain or spinal cord tumors or you are worried about it, you likely have a lot of questions. Learning some basics is a good place to start.

- What Are Brain and Spinal Cord Tumors in Children?
- Types of Brain and Spinal Cord Tumors in Children

Research and Statistics

See the latest estimates for new cases of brain and spinal cord tumors in children in the US and what research is currently being done.

- Key Statistics for Brain and Spinal Cord Tumors in Children
- What’s New in Research for Childhood Brain and Spinal Cord Tumors?

What Are Brain and Spinal Cord Tumors in Children?

Brain and spinal cord tumors are masses of abnormal cells in the brain or spinal cord that have grown out of control.

Are brain and spinal cord tumors cancer?

In most other parts of the body, there’s an important difference between benign (non-
cancerous) tumors and **malignant** tumors (**cancers**). Benign tumors do not invade nearby tissues or spread to distant areas, and are almost never life threatening in other parts of the body. Malignant tumors (**cancers**) are so dangerous mainly because they can spread throughout the body.

Brain tumors rarely spread to other parts of the body, though many of them are considered malignant because they can spread through the brain and spinal cord tissue. But even so-called benign tumors can press on and destroy normal brain tissue as they grow, which can lead to serious or sometimes even life-threatening damage. Because the difference between benign and malignant tumors isn’t as important in the brain, doctors usually speak of “brain tumors” rather than “brain cancers.”

The main concerns with brain and spinal cord tumors are:

- How fast they grow
- How readily they spread through the rest of the brain or spinal cord
- If they can be removed or treated and not come back

Both benign and malignant tumors can be life threatening.

**Are brain and spinal cord tumors different in children?**

Brain and spinal cord tumors in children tend to be different from **those in adults**. They often form in different places, develop from different cell types, and may have a different treatment and prognosis (outlook).

To learn more about the differences between childhood cancers and adult cancers in general, see **What Are the Differences Between Cancers in Adults in Children?**

**The central nervous system**

To understand brain and spinal cord tumors, it helps to know about the normal structure and function of the central nervous system (CNS), which is the medical name for the brain and spinal cord.

The brain is the center of thought, feeling, memory, speech, vision, hearing, movement, and much more. The spinal cord and special nerves in the head, called **cranial nerves**, carry messages between the brain and the rest of the body. These messages tell our muscles how to move, transmit information gathered by our senses, and help coordinate the functions of our internal organs.
The brain is protected by the skull. Likewise, the spinal cord is protected by the bones (vertebrae) of the spinal column.

The brain and spinal cord are surrounded and cushioned by a liquid called cerebrospinal fluid (CSF). Cerebrospinal fluid is made by the choroid plexus, which is in spaces in the brain called ventricles. The ventricles and the spaces around the brain and spinal cord are filled with CSF.

**Parts of the brain and spinal cord**

The main areas of the brain include the cerebrum, cerebellum, and brain stem. Each area has a special function.

**Cerebrum**: The cerebrum is the large, outer part of the brain. It is made up of 2 hemispheres (halves) and controls reasoning, thought, emotion, and language. It is also responsible for planned (voluntary) muscle movements (throwing a ball, walking,
chewing, etc.) and for taking in and interpreting sensory information such as vision, hearing, smell, touch, and pain.

**Cerebellum:** The cerebellum lies under the cerebrum at the back part of the brain. It helps coordinate movement.

**Brain stem:** The brain stem is the lower part of the brain that connects to the spinal cord. It has bundles of very long nerve fibers that carry signals controlling muscles and sensation or feeling between the cerebrum and the rest of the body. Special centers in the brain stem also help control breathing and the heart beating. In addition, most cranial nerves (described below) start in the brain stem.

The brain stem is divided into 3 main parts: the midbrain, pons, and medulla oblongata.

Because the brain stem is a small area that is so essential for life, it might not be possible to surgically remove tumors in this area.

**Cranial nerves:** The cranial nerves extend directly out of the base of the brain (as opposed to coming out of the spinal cord). These nerves carry signals directly between the brain and the face, eyes, tongue, mouth, and some other areas.

The most common cranial nerve tumors in children are called *optic gliomas*, which are tumors of the optic nerve (the large nerve that runs between the brain and each eye).

**Spinal cord:** The spinal cord has bundles of very long nerve fibers that carry signals that control muscles, sensation or feeling, and bladder and bowel control.
Types of cells and body tissues in the brain and spinal cord

The brain and spinal cord have many kinds of tissues and cells, which can develop into
**different types of tumors.**

**Neurons (nerve cells):** These are the cells in the brain that help determine thought, memory, emotion, speech, muscle movement, sensation, and just about everything else that the brain and spinal cord do. They do this by transmitting chemical and electric signals through their nerve fibers (axons). Axons in the brain tend to be short, while those in the spinal cord can be as long as several feet.

Unlike many other types of cells that can grow and divide to repair damage from injury or disease, neurons in the brain and spinal cord largely stop dividing about a year after birth (with a few exceptions). Neurons do not usually form tumors, but they can be damaged by tumors that start nearby.

**Glial cells:** Glial cells are the supporting cells of the brain. Most brain and spinal cord tumors develop from glial cells. These tumors are sometimes referred to as a group called *gliomas*.

There are 3 main types of glial cells:

- **Astrocytes** help support and nourish neurons. When the brain is injured, astrocytes form *scar* tissue that helps repair the damage. The main tumors starting in these cells are called *astrocytomas* or *glioblastomas*.

- **Oligodendrocytes** make myelin, a fatty substance that surrounds and insulates the nerve cell axons of the brain and spinal cord. This helps neurons send electric signals through the axons. Tumors starting in these cells are called *oligodendrogliomas*.

- **Ependymal cells** line the ventricles (fluid-filled areas) within the central part of the brain and form part of the pathway through which cerebrospinal fluid (CSF) flows. Tumors starting in these cells are called *ependymomas*.

(A fourth type of cell, called *microglia*, are the infection-fighting cells of the central nervous system. They are part of the immune system and are not truly glial cells.)

**Neuroectodermal cells:** These are very early forms of nervous system cells that are probably involved in brain cell development. They are found throughout the brain. The most common tumors that come from these cells are called *medulloblastomas*, which start in the cerebellum.

**Meninges:** These are layers of tissue that cover and protect the brain and spinal cord. The meninges help form the spaces through which CSF travels. The most common tumors that start in these tissues are called *meningiomas*. 
**Choroid plexus:** The choroid plexus is the area of the brain within the ventricles that makes CSF, which nourishes and protects the brain. Tumors that start here include choroid plexus papillomas and choroid plexus carcinomas.

**Pituitary gland and hypothalamus:** The pituitary is a small gland at the base of the brain. It is connected to a part of the brain called the hypothalamus. Both make hormones that help regulate the activity of several other glands in the body. For example, they control the amount of thyroid hormone made by the thyroid gland, the production and release of milk by the breasts, and the amount of male or female hormones made by the testicles or ovaries. They also make growth hormone, which stimulates body growth, and vasopressin, which regulates water balance by the kidneys.

The growth of tumors in or near the pituitary or hypothalamus, as well as surgery and/or radiation therapy in this area, can affect these functions. For example, tumors starting in the pituitary gland sometimes make too much of a certain hormone, which can cause problems. On the other hand, a child may have low levels of one or more hormones after treatment and may need to take hormones to make up for this.

**Pineal gland:** The pineal gland is not really part of the brain. It is a small endocrine gland that sits between the cerebral hemispheres. It makes melatonin, a hormone that regulates sleep, in response to changes in light. The most common tumors of the pineal gland are called pineoblastomas.

**Blood-brain barrier:** The inner lining of the small blood vessels (capillaries) in the brain and spinal cord creates a very selective barrier between the blood and the tissues of the central nervous system. This barrier normally helps maintain the brain’s metabolic balance and keeps harmful toxins from getting into the brain. Unfortunately, it also keeps out most chemotherapy drugs that are used to kill cancer cells, which in some cases limits their usefulness.

- **References**


  National Cancer Institute Physician Data Query (PDQ). Childhood Brain and Spinal
Types of Brain and Spinal Cord Tumors in Children

Many different types of tumors can occur in the brain and spinal cord. Several factors are important when doctors are trying to figure out how best to treat a tumor and what the prognosis (outlook) is likely to be.

**The type of tumor (based on the type of cell it starts from):** Tumors can form in almost any type of tissue or cell in the brain or spinal cord. Some tumors have a mix of cell types. Different types of tumors tend to start in certain parts of the brain or spinal cord, and tend to grow in certain ways. (The most common types of brain and spinal cord tumors in children are described below.)

**The grade of the tumor:** Some types of brain and spinal cord tumors are more likely to grow into nearby tissues (and to grow quickly) than are others. Brain and spinal cord tumors are typically divided into 4 grades (using Roman numerals I to IV), based largely on how the tumor cells look under a microscope. The higher the grade, the more quickly the tumor is likely to grow:

- **Lower grade (grade I or II) tumors** tend to grow more slowly and are less likely to grow into (invade or infiltrate) nearby tissues.
- **Higher grade (grade III or IV) tumors** tend to grow quickly and are more likely to grow into nearby tissues. These tumors often require more intensive treatment.

**Gene changes in the tumor cells:** Even for a specific type of tumor, the changes in the genes of the tumor cells can be different. For example, many types of tumors are now divided based on whether the cells have mutations in one of the *IDH* genes. For a specific type of tumor, those with *IDH* mutations tend to have a better outlook than those without a mutation. Other gene mutations can also be important for certain types of tumors.
The location of the tumor: Where the tumor is in the brain and spinal cord can affect what symptoms it causes, as well as which treatments might be best. Brain tumors in children are more likely to start in the lower parts of the brain, such as the cerebellum and brain stem, than they are in adults. But they can start in the upper parts of the brain as well.

Gliomas

Gliomas are not a specific type of tumor. Glioma is a general term for a group of tumors that start in glial cells (the supporting cells of the brain). A number of tumors can be considered gliomas, including:

- Astrocytomas (which include glioblastomas)
- Oligodendrogliomas
- Ependymomas
- Brain stem gliomas
- Optic gliomas

About half of all brain and spinal cord tumors in children are gliomas.

Astrocytomas

Astrocytomas are tumors that start in cells called astrocytes, a kind of glial cell that helps support and nourish nerve cells.

Some astrocytomas can spread widely throughout the brain and blend with the normal brain tissue, which can make them hard to remove by surgery. Sometimes they spread along the cerebrospinal fluid (CSF) pathways. It is very rare for them to spread outside of the brain or spinal cord.

As with other brain tumors, astrocytomas are often grouped by grade.

Low-grade (grade I or II) astrocytomas tend to grow slowly and are the most common type in children. Some types, known as non-infiltrating astrocytomas, are grade I tumors that tend to grow very slowly and do not grow into (infiltrate) nearby tissues, so they often have a good prognosis.

- Pilocytic astrocytomas are grade I tumors that tend to grow slowly and rarely grow into nearby tissues. They most commonly occur in the cerebellum but can also begin in the optic nerve, hypothalamus, brain stem, or other areas. They
account for nearly 1 out of 5 brain tumors in children.

- **Subependymal giant cell astrocytomas (SEGAs)** occur in the ventricles (spaces in the brain). They are grade I tumors that tend to grow slowly and rarely grow into nearby tissues. These tumors are almost always linked with an inherited condition called **tuberous sclerosis**.

- **Diffuse astrocytomas** are also slow-growing tumors, but they are grade II tumors that can grow into nearby tissues, which makes them hard to remove with surgery. Though these tumors are thought of as low grade, they tend to become more aggressive and fast growing over time.

- **Pleomorphic xanthoastrocytomas (PXAs)** are grade II tumors that tend to grow slowly, and most can be cured by surgery alone.

- **Optic gliomas** are astrocytomas that start in the optic nerves (the nerves leading from the eyes to the brain). They usually grow slowly, and are often linked with an inherited condition called **neurofibromatosis type 1**. These tumors are rarely fatal, but they may cause vision loss and injury to nearby brain tissue.

**High-grade (grade III or IV) astrocytomas** tend to grow quickly and spread into the surrounding normal brain tissue. These include:

- **Glioblastomas**, which are the fastest growing type of astrocytoma (grade IV).
- **Anaplastic astrocytomas**, which are grade III.

**Oligodendrogliomas**

These tumors start in brain cells called **oligodendrocytes** (a type of glial cell that makes a fatty substance that helps nerve cells send electric signals). These are grade II tumors that tend to grow slowly, but most of them can grow into nearby brain tissue and can’t be removed completely by surgery. Oligodendrogliomas rarely spread along the CSF pathways and even less frequently spread outside the brain or spinal cord. As with astrocytomas, they can become more aggressive over time.

Only about 1% of brain tumors in children are oligodendrogliomas.

**Ependymomas**

About 5% of brain tumors in children are ependymomas. These tumors start in the ependymal cells that line the ventricles or central canal of the spinal cord. They can range from fairly low-grade (slow growing) tumors to grade III (fast growing) tumors, which are called **anaplastic ependymomas**.
Ependymomas may spread along the CSF pathways but do not spread outside the brain or spinal cord. These tumors can block the flow of CSF out of the ventricles, causing the ventricles to become very large – a condition called hydrocephalus.

Unlike astrocytomas and oligodendrogliomas, ependymomas usually do not grow into normal brain tissue. As a result, some (but not all) ependymomas can be removed and cured by surgery. But because they can spread along ependymal surfaces and CSF pathways, treating them can sometimes be difficult.

**Brain stem gliomas**

A brain stem glioma is any type of glioma that starts in the brain stem. This term refers to the location of the tumor, rather than the type of cell it starts in.

- A small number of brain stem gliomas occur as tumors with very distinct edges (called **focal brain stem gliomas**).
- More often, brain stem gliomas grow diffusely throughout the brain stem (where the tumor cells are spread throughout normal tissue), rather than growing as a focal tumor (where the tumor cells are clustered together). These are referred to as **diffuse midline gliomas**. These tumors most often start in the pons, where they are called **diffuse intrinsic pontine gliomas (DIPGs)**. These tumors can be hard to treat.

About 10% to 20% of brain tumors in children are brain stem gliomas. Nearly all of these tumors are some type of astrocytoma.

**Embryonal tumors**

These tumors start in early forms of nerve cells in the central nervous system. About 10% to 20% of brain tumors in children are embryonal tumors. They are more common in younger children than older ones, and are rare in adults. Embryonal tumors tend to grow quickly and often spread throughout the CSF pathways.

**Medulloblastomas** are the most common type of embryonal tumor. These tumors start in the cerebellum. There are several different types of medulloblastomas, based on how the tumor cells look under a microscope, and on which gene mutations the cells have. Some types of medulloblastoma tend to have a better outlook than others, and doctors are now trying to determine how this might affect treatment.

Medulloblastomas can often be treated effectively and tend to have a better outlook
than embryonal tumors in other parts of the brain.

Other, less common types of embryonal tumors include:

- Medulloepithelioma
- Atypical teratoid/rhabdoid tumor (ATRT)
- Embryonal tumor with multilayered rosettes

In the past, many embryonal tumors were referred to as primitive neuroectodermal tumors (PNETs).

**Pineal tumors**

Some types of tumors occur in the pineal gland (a small gland in the middle of the brain). The most common (and fastest growing) of these are called pineoblastomas. These tumors can be hard to treat.

**Germ cell tumors**, which are described below, can also start in the pineal gland.

**Craniopharyngiomas**

These slow-growing tumors start above the pituitary gland but below the brain itself. They account for about 4% of brain tumors in children. These tumors may press on the pituitary gland and the hypothalamus, causing hormone problems. Because craniopharyngiomas start very close to the optic nerves, they can also cause vision problems. This makes them hard to remove completely without damaging the child's vision or hormone balance.

**Mixed glial and neuronal tumors**

Certain tumors that develop in children and young adults (and rarely in older adults) have both glial and neuronal cell components. They tend to have a fairly good outlook.

- Dysembryoplastic neuroepithelial tumors (DNETs) tend to be slow growing (grade II) tumors, and most can be cured by surgery alone.
- Ganglioglioma is a type of grade I tumor that has both mature neurons and glial cells. Most can be cured by surgery alone or surgery combined with radiation therapy.
Choroid plexus tumors

These rare tumors start in the choroid plexus, the area that makes cerebrospinal fluid (CSF) within the ventricles of the brain. Most are benign (choroid plexus papillomas) and can be cured by surgery. However, some are malignant (choroid plexus carcinomas).

Schwannomas (neurilemmomas)

These tumors start in Schwann cells that surround and insulate cranial nerves and other nerves. Schwannomas are usually benign. They often form near the cerebellum on the cranial nerve responsible for hearing and balance, in which case they are called vestibular schwannomas or acoustic neuromas. They may also develop on spinal nerves, just past the point where the nerve leaves the spinal cord. When this is the case, the tumor can press on the spinal cord, causing weakness, sensory loss, and bowel and bladder problems.

These tumors are rare in children. When schwannomas are found in a child, particularly if there are tumors on both sides of the head, it often means the child has an inherited tumor syndrome such as neurofibromatosis type 2. (See Risk Factors for Brain and Spinal Cord Tumors in Children.)

Other tumors that start in or near the brain

Meningiomas

These tumors begin in the meninges, the layers of tissue that surround the outer part of the brain and spinal cord. Meningiomas cause symptoms by pressing on the brain or spinal cord. They are much less common in children than in adults.

Meningiomas are almost always benign and are usually cured by surgery. Some, however, are located very close to vital structures in the brain and can’t be cured by surgery alone.

Meningiomas are often assigned a grade based on how the tumor cells look.

- Grade I meningiomas, which look most like normal cells, account for most meningiomas.
• **Grade II (atypical) meningiomas** look slightly more abnormal.
• **Grade III (anaplastic or malignant) meningiomas**, which look the most abnormal, make up only about 1% to 3% of meningiomas.

Higher-grade meningiomas are more likely to come back after treatment, and some grade III meningiomas can spread to other parts of the body.

**Chordomas**

These tumors start in the bone at the base of the skull or at the lower end of the spine. Chordomas don’t start in the central nervous system, but they can injure nearby parts of the brain or spinal cord by pressing on them. These tumors tend to come back if they are not removed completely, causing more damage. They usually do not spread to other organs. Chordomas are much more common in adults than in children. For more on these tumors, see Bone Cancer.

**Germ cell tumors**

These rare tumors develop from germ cells, which normally form egg cells in women and sperm cells in men. During normal development before birth, germ cells travel to the ovaries or testicles and develop into egg or sperm cells. But sometimes some germ cells don’t move where they should and end up in abnormal locations such as the brain. They may then develop into germ cell tumors, similar to those that can form in the ovaries or testicles.

Germ cell tumors of the nervous system usually occur in children, most often in the pineal gland or above the pituitary gland. These tumors can sometimes be diagnosed without a biopsy by measuring certain chemicals in the cerebrospinal fluid (CSF) or blood.

Types of germ cell tumors include:

- **Germinomas** (the most common type of CNS germ cell tumor)
- **Choriocarcinomas**
- **Embryonal carcinomas**
- **Teratomas**
- **Yolk sac tumors (endodermal sinus tumors)**

**Neuroblastomas**
These nerve cell tumors are the third most common cancer in children. But neuroblastomas rarely develop in the brain or spinal cord; most develop from nerve cells inside the abdomen or chest. This type of cancer is most common during early infancy. For more information, see Neuroblastoma.

**Lymphomas**

Lymphomas are cancers that start in cells called lymphocytes, which are white blood cells that are part of the immune system. Most lymphomas start in other parts of the body, but a small portion start in the central nervous system (CNS), and are called primary CNS lymphomas. These tumors are rare in children. For more on childhood lymphomas, see Non-Hodgkin Lymphoma in Children.

**Pituitary tumors**

Tumors that start in the pituitary gland are almost always benign (non-cancerous). But they can still cause problems if they grow large enough to press on nearby structures or if they make too much of any kind of hormone. These tumors are more common in teens than in younger children. For more information, see Pituitary Tumors.

**Cancers that spread to the brain from other parts of the body**

Sometimes tumors in the brain are found to have metastasized (spread) there from some other part of the body. Tumors that start in other organs and then spread to the brain are called metastatic or secondary brain tumors (as opposed to primary brain tumors, which start in the brain). This is important because metastatic and primary brain tumors are often treated differently.

In children, metastatic brain tumors are much less common than primary brain tumors. Childhood leukemias can sometimes spread to the CSF around the brain and spinal cord. When this happens, the cancer is still considered a leukemia (the cancer cells in the CSF are leukemia cells), so doctors use treatments directed at the leukemia. For more information, see Childhood Leukemia.

- References
Key Statistics for Brain and Spinal Cord Tumors in Children

Brain and spinal cord tumors are the second most common cancers in children (after leukemia). They account for about 1 out of 4 childhood cancers. More than 4,000 brain and spinal cord tumors are diagnosed each year in children and teens. The incidence rate (number of tumors per 100,000 children) has not changed much in recent years.

Malignant (fast-growing) brain and spinal cord tumors are slightly more common in boys, while non-malignant tumors are slightly more common in girls.

About 3 out of 4 children with brain tumors (all types combined) survive at least 5 years after being diagnosed. But the outlook can vary a great deal based on the type of tumor, where it is, and other factors. For survival information on some particular tumor types, see Survival Rates for Selected Childhood Brain and Spinal Cord Tumors.

Visit the American Cancer Society’s Cancer Statistics Center for more key statistics.

- References


What’s New in Research for Childhood Brain and Spinal Cord Tumors?

There is always research going on in the area of brain and spinal cord tumors. Scientists and doctors are looking for causes and ways to prevent them, better tests to help characterize these tumors, and better ways to treat them.

Finding and testing for gene changes in brain tumors

In recent years, researchers have found some changes in genes, chromosomes, and proteins inside brain tumor cells that can be used to help predict a child’s outlook (prognosis) or help guide treatment. Some examples of changes that can now be tested for include:

- *IDH1* or *IDH2* gene mutations
- Chromosomal 1p19q co-deletions
- MGMT promoter methylation

For children with medulloblastomas, doctors can now also test for other gene changes that can help show if they are likely to have a better outlook (and therefore might require less intensive treatment).

For more on these tests, see "Lab tests of biopsy specimens" in Tests for Brain and Spinal Cord Tumors in Children.
Researchers are also looking for other changes in tumor cells that might help guide treatment.

**Imaging and surgery techniques**

Recent advances have made surgery for brain tumors much safer and more successful. Some of these newer techniques include:

- **Magnetic resonance spectroscopy (MRS).** In this approach, described in Tests for Brain and Spinal Cord Tumors in Children, specially processed MRS information is used to make a map of important chemicals involved in tumor metabolism. This can help surgeons direct their biopsies to the most abnormal areas in the tumor. It can also help doctors direct radiation to the right areas and evaluate the effects of chemotherapy or targeted therapy.

- **Diffusion tensor imaging (DTI), also known as tractography.** This is a type of MRI test that can show the major pathways (tracts) of white matter in the brain. This information can be used by surgeons to help avoid these important parts of the brain when removing tumors.

- **Fluorescence-guided surgery.** For this approach, the patient drinks a special dye a few hours before surgery. The dye is taken up mainly by the tumor, which then glows when the surgeon looks at it under special lighting from the operating microscope. This lets the surgeon better separate tumor from normal brain tissue. Researchers are now looking to improve on the dyes currently in use.

- **Newer surgical approaches** for some types of tumors. For example, a newer approach to treat some tumors in or near the pituitary (such as some craniopharyngiomas) is to use an endoscope, a thin tube with a tiny video camera lens at the tip. The endoscope is passed through a hole made in the back of the nose, which allows the surgeon to operate through the nasal passages and limits the potential damage to the brain. A similar technique can be used for some tumors in the ventricles, where a small opening in the skull near the hairline serves as the point of endoscope insertion. The use of this technique is limited by the tumor’s size, shape, position, and by how many blood vessels it contains.

**Radiation therapy**

Children's brains are very sensitive to radiation, which can lead to side effects if normal brain tissue receives a large dose, especially if the child is very young. Several newer
types of radiation therapy now let doctors aim radiation more precisely at the tumor, which helps spare normal brain tissue from getting too much radiation. Newer techniques such as stereotactic radiosurgery, 3-dimensional conformal radiation therapy (3D-CRT), intensity modulated radiation therapy (IMRT), and proton beam therapy are described in Radiation Therapy for Brain and Spinal Cord Tumors in Children.

Clinical trials have shown that in some situations, using chemotherapy can let doctors use lower doses of radiation therapy without lowering the chance that treatment will be effective. Doctors are now trying to determine if even lower doses of radiation can be used and still give the same results.

**Chemotherapy**

New approaches may help make chemotherapy (chemo) more useful against brain and spinal cord tumors.

**Adjuvant chemotherapy**

In some children and infants with brain tumors, chemo is given right after surgery to either delay radiation therapy (particularly in infants) or to decrease the radiation dose needed to treat the tumor. This is known as adjuvant chemotherapy. Some studies are looking at whether giving prolonged chemo can help avoid the need for radiation therapy at all in certain cases.

**High-dose chemotherapy and stem cell transplant**

One of the main factors that limits the doses of chemo that can be given safely is its effects on the bone marrow, where new blood cells are normally made. A stem cell transplant allows higher doses of chemo to be given than would normally be possible. First, blood stem cells are removed from either the child's blood or the bone marrow and are stored in a deep freeze. The child is then treated with very high doses of chemo. The blood stem cells are then thawed and infused back into the body, where they settle in the bone marrow and start making new blood cells.

Although some children with certain brain or spinal cord tumors (such as medulloblastomas) have responded well to this very intensive treatment, it can have serious side effects, and it is not yet known if it is effective enough to become a standard treatment. For now, most doctors consider this treatment experimental for brain and spinal tumors. Clinical trials are being done to determine how useful it is.
Improving chemotherapy drugs

Many chemo drugs are limited in their effectiveness because the tightly controlled openings in the brain capillaries, sometimes referred to as the blood-brain barrier, prevents the drugs from getting from the bloodstream to some parts of the brain tumor. Researchers are now trying to modify some of these drugs by coating them with tiny layers of fat (liposomes) or attaching them to molecules that normally cross the blood-brain barrier, to help them work better. This is an area of active research.

Getting chemotherapy directly to tumors

Some newer approaches might help doctors get chemo directly to brain and spinal cord tumors.

For example, in one method called convection enhanced delivery, small tubes are placed into the tumor in the brain through a small hole in the skull during surgery. The tubing extends through the scalp and is connected to an infusion pump, through which chemo drugs can be given. This can be done for hours or days and might be repeated more than once, depending on the drug used. This technique can also be used to get other, newer types of drugs into the tumor. This is still an investigational method, and studies are continuing.

Researchers are also looking at the possibility of using lasers or other means to disrupt the blood-brain barrier and allow drugs to more readily reach brain tumors.

Other new treatments

Researchers are also testing some newer approaches to treatment that may help doctors target tumors more precisely. The hope is to develop more effective treatments that cause fewer side effects. Although these treatment approaches are promising, most are still experimental at this time and are only available through clinical trials.

Targeted drugs

As researchers have learned more about the gene changes in tumor cells that help them grow, they have developed newer drugs that target these changes. These targeted drugs work differently from standard chemo drugs. Here are some examples of targeted drugs now being studied or in use:

- Everolimus (Afinitor) is a drug that targets mTOR, a protein involved in cell growth.
This drug may shrink or slow the growth of **subependymal giant cell astrocytomas (SEGAs)** that can’t be removed with surgery.

- A small portion of **low-grade gliomas** have been found to have changes in the *BRAF* gene, which can help them grow. Early research has shown that drugs that target *BRAF* might be helpful in treating these tumors if other treatments are no longer working. Some of these drugs, such as dabrafenib (Tanfinlar) and vemurafenib (Zelboraf), are now being tested in larger studies.

- Some types of **medulloblastomas** tend to have mutations (changes) in genes that are part of a cell signaling route called the sonic hedgehog (SHH) pathway. This pathway is crucial for the development of the embryo and fetus, but it can be overactive in some medulloblastoma cells. Drugs that target proteins in this pathway are now being tested against medulloblastoma in clinical trials.

Many other targeted drugs are already being used to treat other types of cancer, and some are being studied to see if they will work for brain tumors as well.

### Angiogenesis inhibitors

Tumors have to create new blood vessels (a process called *angiogenesis*) to keep their cells nourished. Targeted drugs that attack these blood vessels are used to help treat some cancers, including some brain tumors in adults. Several drugs that impair blood vessel growth are now being studied for use against brain tumors in children.

### Hypoxic cell sensitizers

Some drugs increase the oxygen content in the tumor, which makes tumor cells more likely to be killed by radiation therapy if the drugs are given before treatment. Studies are now looking to see if this affects treatment outcomes.

### Immunotherapy

The goal of **immunotherapy** is to help the body’s own immune system fight the tumor.

Several types of vaccines are being developed against brain tumor cells. Unlike vaccines against infectious diseases, these vaccines are meant to help treat the disease instead of prevent it. The goal of the vaccines is to stimulate the body’s immune system to attack the brain tumor cells.

Early study results of some of these vaccines have shown promise, but more research is needed to determine how effective they are. At this time, brain tumor vaccines are
available only through clinical trials.

Other types of drugs that affect the immune system are also being studied.

**Therapeutic viruses**

Researchers have done a great deal of lab work with viruses that reproduce only within brain tumor cells and then cause those cells to die, while leaving normal cells alone. Research using these viruses in humans with brain tumors is still in very early stages.

- **References**


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