

AMERICAN BRAIN TUMOR ASSOCIATION

# Medulloblastoma



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## ACKNOWLEDGEMENTS

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Founded in 1973, the American Brain Tumor Association (ABTA) was the first national nonprofit organization dedicated solely to brain tumor research. The ABTA has since expanded its mission and now provides comprehensive resources to support the complex needs of brain tumor patients and caregivers, across all ages and tumor types, as well as the critical funding of research in the pursuit of breakthroughs in brain tumor diagnoses, treatments and care.

To learn more, visit **[abta.org](https://www.abta.org)**.

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# Medulloblastoma

## INTRODUCTION

This brochure is about medulloblastoma (pronounced med-you-lo-blas-TOE-muh), which is a primary central nervous system (CNS) tumor, meaning that it begins in the brain or spinal cord. This fast-growing tumor often spreads to other areas of the CNS through cerebrospinal fluid (CSF), which surrounds and protects the brain and spinal cord. Medulloblastomas rarely travel outside the CNS to other parts of the body.<sup>1,2</sup>

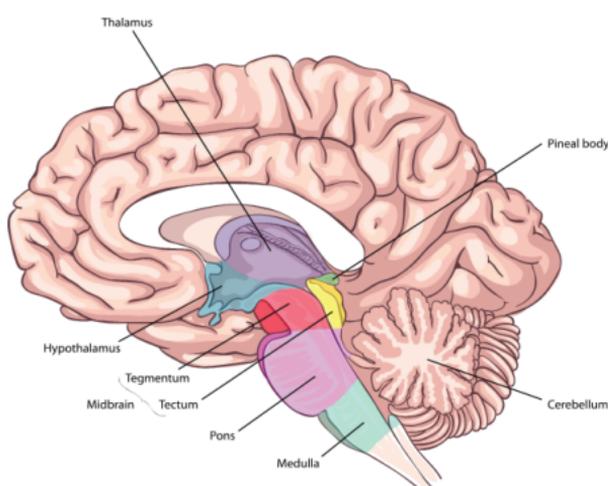


Fig. 1. Brain stem structures.

Medulloblastoma is the most common of the embryonal tumors—tumors that arise from fetal or immature cells that remain after birth.<sup>1,2,3</sup>

While medulloblastomas can occur in both children and adults, they are more common in children. However, medulloblastomas in adults still represent about 30% of all cases diagnosed per year, typically affecting young adults between the ages of 20 and 40.<sup>1</sup>

### **Tumor type**

In the last decade, researchers have learned a lot about medulloblastoma, in large part, due to advances in genetic testing (also referred to as molecular testing).

Medulloblastoma is no longer considered one type of tumor, but rather many subtypes, each with its own genetic make-up.<sup>5</sup> As a result, the World Health Organization (WHO) now divides medulloblastomas into the following genetic subtypes:<sup>2</sup>

- WNT-activated (WNT stands for wingless-related integration site)
- SHH-activated (SHH stands for sonic hedgehog)
- Group 3 (non-WNT/non-SHH)
- Group 4 (non-WNT/non-SHH)

The SHH-activated subtype is further divided into TP53-mutant and TP53-wild type genes.<sup>2</sup> Further research suggests that at least 12 additional medulloblastoma subtypes exist, but they have not yet been incorporated into the WHO classification.<sup>6</sup> The subtypes that occur in children are different than those that occur in adults, which impacts treatment options and outcomes.<sup>7</sup>

In addition, medulloblastomas have four histologic patterns, which refer to what the brain tissue looks like under the microscope. They are:<sup>2</sup>

- The classic pattern—found in most medulloblastomas
- Desmoplastic/nodular medulloblastoma
- Large-cell or anaplastic medulloblastoma
- Medulloblastoma with extensive nodularity

The genetic subtypes are separate from the histologic patterns, but there is some overlap. For example, studies have shown that nearly all WNT-activated tumors have the classic pattern<sup>2</sup> and most of the desmoplastic/nodular medulloblastomas in infants and all of them in adults belong to the SHH-activated subtype. Both the molecular and histologic findings should be used to classify the tumor.

Researchers are learning that medulloblastomas behave differently based on their genetic subtype and histologic patterns when it comes to how fast they grow and if they spread. They also react differently to treatment.

As a result, there is a shift in the treatment of medulloblastoma away from a one therapy fits all model toward a more specific therapy based on the genetic and histological pattern subgroups.<sup>11</sup>

### **Tumor grade**

The WHO uses a grading system with a scale of I to IV for primary brain tumors; grade I is the least malignant (cancerous) and grade IV is the

most malignant. **All medulloblastomas are considered a grade IV tumor.**

Grade IV tumors are made of abnormal looking cells that grow rapidly. Even so, depending on the subgroup, they can have an excellent prognosis (the likely outcome of the disease) with standard treatment approaches.<sup>2</sup>

Tumor grading is important because a tumor's grade influences treatment options. Tumor grades are not the same as tumor staging, the latter of which describes whether the tumor has spread to other parts of the CNS.<sup>12</sup>



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The genetic subtype and histologic patterns of a medulloblastoma help guide treatment decisions.

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## INCIDENCE

Incidence refers to how often a disease occurs. **Medulloblastomas are relatively rare**, but they are the second most common cancerous brain tumors in children ages 14 and younger. Most tumors occur in children ages five to nine.<sup>14,15</sup>



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An estimated 357 people are diagnosed with medulloblastomas each year.<sup>1</sup> Of those, 140 are adults ages 15 years and older.

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Medulloblastoma in adults is even less common than it is in children, accounting for less than 1% of all primary brain tumors. In adults, medulloblastoma typically affects younger people between the ages of 20 and 40.<sup>1</sup> It is rare to be diagnosed with medulloblastoma after 40 years of age, but possible.<sup>15</sup> Although these tumors are found in both males and females, they tend to occur more often in males.<sup>16,17</sup>

By dividing medulloblastomas into genetic subtypes, researchers have learned more about their incidence. They have found the following common patterns based on the subtype:

- SHH-activated TP53-wild type tumors occur more often in infants and adults,<sup>10,18</sup> while all other subtypes are more common in childhood.<sup>18</sup>
- TP53-mutant SHH and group 4 tumors have a peak incidence in teens.<sup>10</sup>
- WNT-activated tumors are seen in children and adults, but rarely in infants.<sup>10</sup>
- Group 3 tumors are most often seen in children.<sup>10</sup>



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An estimated 3,840 people are living with this tumor.

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## CAUSES

Like other types of brain tumors, the exact cause of most medulloblastomas is unknown. Scientists have identified abnormalities in the genes of different chromosomes that may play a role in how brain tumors develop. But what causes

normal brain cells to change into abnormal tumor cells is still unclear.<sup>20</sup>

Anything that can increase a person's chance of developing a brain tumor is called a risk factor.<sup>19</sup> Risk factors often influence the development of a brain tumor, but don't directly cause it to develop. Some people with a lot of risk factors never get a brain tumor, while others who don't have any risk factors do develop a brain tumor.

Risk factors that may raise a person's chance of developing medulloblastoma include:<sup>19</sup>

- Gender  
Medulloblastoma is slightly more common in boys than in girls.
- Age  
Medulloblastoma occurs most often in the first 8 years of life, with about half occurring in children younger than 6 years.
- Genetics  
A few inherited cancer syndromes are associated with a higher risk of developing medulloblastoma.

About 5% of children diagnosed with medulloblastoma have a known cancer syndrome that is passed down from generation to generation. Known cancer syndromes include Gorlin syndrome,<sup>8,10</sup> familial adenomatous polyposis (also known as Turcot syndrome), and Li-Fraumeni syndrome.<sup>8,21</sup> These syndromes also can be found in a very small number of adults with medulloblastoma.

The presence of these syndromes differ between subgroups; they are highest for patients with the SHH-activated subtype.

There are no known ways to prevent a brain tumor. People who “inherit” the genetic changes that cause the cancer syndrome do not “inherit” medulloblastoma, meaning the risk of developing the tumor is not passed down to family members.

## SYMPTOMS

As with other primary brain tumors, **symptoms associated with medulloblastoma** often depend on where the tumor is located,<sup>1,8,15</sup> its size, and whether it has spread to other areas inside or outside the CNS.<sup>21</sup> The location is important because it determines which nerves and parts of the brain are affected by the tumor.

Most medulloblastomas form in the cerebellum – the bottom part of the brain at the back of the skull.<sup>1,2</sup> The cerebellum controls muscle coordination and balance.<sup>21</sup>

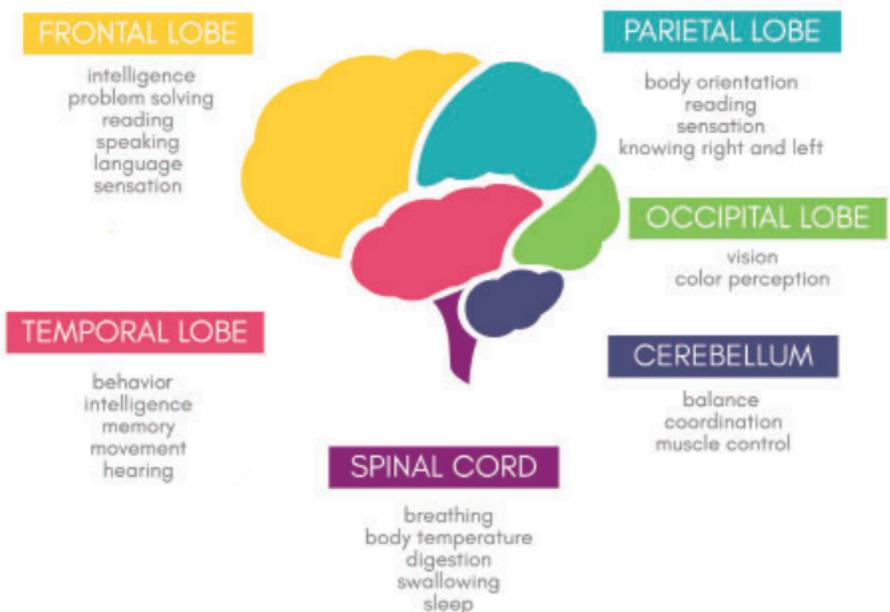


Fig. 2. Functions of the brain.

Individuals with the tumor may appear clumsy or lacking coordination.<sup>1</sup> They may have difficulty walking and frequently fall<sup>15</sup> as well as have problems with handwriting and other motor skills that can worsen over time.<sup>21,25</sup> If the tumor has spread to the spine, it may cause weakness or numbness in the arms and/or legs, a change in normal bowel or bladder habits, and back pain.<sup>1,25</sup>

A small number of medulloblastomas, however, do arise from the brain stem, which is just in front of the cerebellum and connects the brain with the spinal cord.<sup>2</sup> The brain stem controls basic functions such as breathing, heartbeat, blood pressure, control of consciousness, and sleep.

As the tumor grows within the brain, it spreads into normal brain tissue, which may increase pressure on the brain or disrupt connections between normal brain cells. An individual can experience symptoms as a result of this pressure and interference with brain function.<sup>27,28</sup>



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Tell your doctor all of your or your child's symptoms as that can help diagnose the tumor. Relieving symptoms will be a part of the treatment and care plan.

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**Common symptoms** may include headaches, nausea, vomiting, blurred and double vision, fatigue (extreme tiredness

even after sleeping), irritability, and behavioral changes.<sup>1,8,15</sup> Headaches usually occur in the morning and clear up as the day goes on. Eye problems may include an inward turning of one eye. Typically, symptoms evolve over a period of weeks to months.<sup>8,15</sup>

## DIAGNOSIS

Doctors use different types of tests to find, locate, or diagnose a brain tumor. These tests are often done by different specialists who are part of the healthcare team.

### **Different tests**

After getting a thorough medical history from the patient (or caregiver in the case of a young child), the doctor will do a physical examination.<sup>21</sup> Both a neurological exam, which tests a person's vision, hearing, balance, coordination, and reflexes, and neurocognitive testing, which evaluates one's cognitive skills, help determine which part of the brain the tumor is affecting.

Cognitive skills are related to thinking, learning, concentrating, problem-solving, and decision-making. The neurocognitive tests will be done by either a neurologist, a physician trained in the diagnosis and treatment of diseases and disorders of the CNS; a neuro-oncologist, a doctor who specializes in treating patients with brain tumors, and/or the consequences of cancer upon the CNS; or a clinical neuropsychologist, a psychologist who specializes in understanding the relationship between the brain and behavior.

A **magnetic resonance imaging (MRI)** scan of the brain is the most common type of imaging test used to diagnose medulloblastoma.<sup>21</sup> The doctor also may order a **computed tomography (CT)** scan, another form of imaging, if the patient cannot have an MRI. Patients with pacemakers or other artificial metal parts cannot typically have an MRI.

Although an MRI is preferred, a CT scan is quicker. The doctor will likely want to do an MRI of the spine, known as a gadolinium-based MRI, to learn whether the medulloblastoma has spread to the CSF and spine.

The doctor may recommend a **lumbar puncture**, also called a spinal tap, to look for tumor cells within the CSF.<sup>8,21</sup> A lumbar puncture is done only after the pressure in the brain is relieved once the tumor is removed through surgery.



*Fig. 3 Patient being prepped for CT scan.*

Although imaging tests may give the doctor an educated idea of the tumor type, a **biopsy** or surgical resection is needed to be sure of the

diagnosis.<sup>21,29</sup> During a biopsy, the **neurosurgeon** (a doctor who specializes in surgery of the CNS) surgically removes a small piece of the tumor tissue to send to a **neuropathologist** or **pathologist**.

The pathologist examines the sample under a microscope to determine the tumor type and grade, which is included in the pathology report sent to the neurosurgeon and the rest of the healthcare team.<sup>29</sup>

The neurosurgeon may remove a larger part of the tumor to send it for pathology testing. This is known as a surgical resection. A biopsy can be done as a separate procedure or as part of a surgical resection.

In general, not all of these tests will be used for every patient. The doctor may consider the following factors when choosing which diagnostic tests to use:<sup>29</sup>

- Type of tumor suspected
- Tumor location
- Signs and symptoms
- Age and general health
- Results of other medical tests

Doctors are increasingly using genetic tests to determine the subtype of a medulloblastoma. Testing involves taking a sample of tumor tissue and sometimes also a blood test. After sending the samples to a laboratory that specializes in genetic testing, the doctor will get a report with the test results. Knowing the tumor's genetic and histological pattern subgroup can be helpful when considering treatment options.

## Tumor staging

When all of the diagnostic tests are complete, the doctor can stage the tumor. **Staging** is a way to describe whether the tumor has spread to other parts of the CNS or, less frequently, other parts of the body.<sup>12</sup> Knowing the tumor stage will help determine the best treatment options and can even help determine a patient's prognosis.

Medulloblastoma in children is classified as either standard (average) risk or high risk, depending on the following factors:<sup>12</sup>

- The child's age
- How much tumor remains after surgery
- How much the tumor has spread, if at all

Typically, a standard-risk tumor is located in the cerebellum and has not spread to other areas of the brain or spinal cord. A standard-risk tumor has been almost completely removed during surgery, meaning that less than 1.5 square centimeters (the size of a blueberry) is left (as measured with MRI after surgery). It also does not have any genetic features that are linked with a worse chance of recovery.<sup>12</sup>

In contrast, a high-risk tumor has either spread to other parts of the brain or spine, and/or other parts of the body, or it has not spread but there is more than 1.5 square centimeters remaining after surgery.

Similar for adults, medulloblastoma is classified as either standard or high risk, as generally determined by the amount of tumor remaining

after surgery and the presence or absence of tumor spread.



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Talk to your doctor about conducting genetic tests as it is not yet considered 'standard of care.'

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## TREATMENT

**Current treatment options**, often referred to as "the standard of care," for medulloblastoma are surgery, radiation therapy, and chemotherapy.<sup>8,21,31</sup>

Surgery is typically followed by radiation therapy. Chemotherapy is often part of the treatment plan, depending on the tumor subtype and whether it has spread.<sup>1</sup> Radiation therapy and chemotherapy may be used alone or in combination.<sup>21</sup> Specific treatment approaches will depend on the tumor's size, location, genetic subtype, stage, and progression as well as the patient's age, overall health, and preference.<sup>1,21,31</sup>



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Find a medical center that has doctors who specialize in treating medulloblastoma and access to the latest research.

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## SURGERY

### Having surgery to remove the tumor

is an important first step in treating medulloblastoma.<sup>31</sup> The three goals of surgery are to:<sup>21</sup>

- Remove as much tumor as possible without damaging nearby brain tissue
- Relieve pressure in the brain by draining CSF buildup
- Confirm the diagnosis by getting a tissue sample for biopsy

When the whole tumor is removed, the surgery is called a **gross total resection**.<sup>31</sup> Studies suggest that a patient's prognosis is better when all the medulloblastoma visible on an MRI is removed safely.<sup>21</sup>

While the goal is to remove all the tumor, some medulloblastomas cannot be removed completely because of their location.<sup>31</sup> As an example, in one-third of patients, the tumor grows into the brain stem, making total removal difficult because of potential neurological damage.

Even if the tumor cannot be operated on, the neurosurgeon may still be able to remove some of the tumor and get a tissue sample to confirm the diagnosis. Removing only part of the tumor is called a **subtotal resection**.

Some patients may need a shunt following surgery.<sup>31</sup> A shunt is a long catheter-like tube that drains CSF from the brain and moves it to other parts of the body. In rare cases,

shunting may be recommended before surgery is performed to remove pressure on the brain.<sup>21</sup>

Within days after surgery, an MRI will be done to look at how much tumor, if any, is remaining.<sup>21</sup> The amount of residual or remaining tumor will help the doctor make recommendations for further treatment.

**Common side effects** of surgery include pain, swelling, scarring, headaches, and scalp pain.<sup>20</sup> Rare side effects include infection, major bleeding, blood clots, seizures, and brain damage.

Shortly after surgery, as many as 25% of children develop cerebellar mutism syndrome, also called posterior fossa syndrome.<sup>33,34</sup> This complication is considered rare in adults. Symptoms include loss of speech and balance, muscle stiffness, fatigue, and personality changes. It is unclear why this syndrome occurs. For many children, the symptoms go away quickly, but others can have the symptoms years after surgery. **To learn more about common side effects of surgery, read the ABTA's Surgery brochure.**



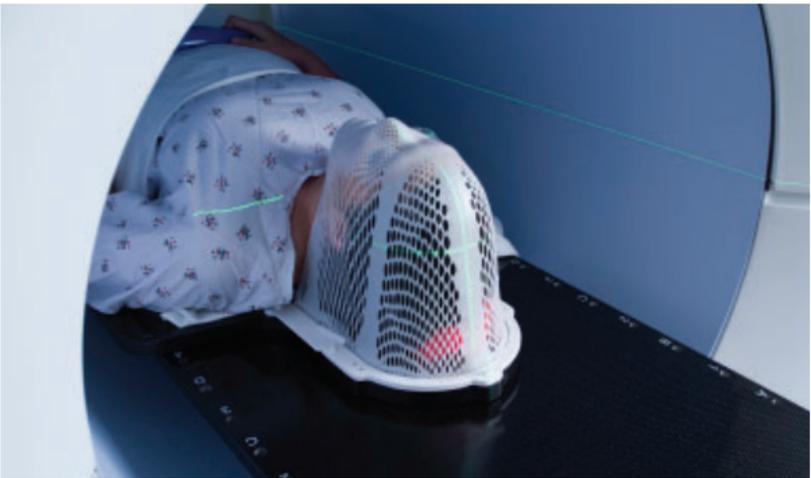
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Discuss your goals for treatment and quality of life with your doctor, weighing the benefits and risks of surgery as well as other treatment options.

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## RADIATION

Once the tumor is removed surgically (or after a biopsy if it cannot be removed), the doctor will recommend radiation therapy to slow or stop the medulloblastoma from growing and spreading in the CNS.<sup>31</sup> Radiation therapy uses very focused, high-energy rays (either photons or protons) to kill the smallest of tumor cells that remain in the brain. A doctor who specializes in giving radiation therapy is called a **radiation oncologist**.



*Fig. 4. Patient receiving targeted radiation therapy while wearing a customized mask for head stabilization during treatment.*

The most common type of radiation used to treat medulloblastomas is known as **external beam radiation therapy**, or **EBRT**.

There are different EBRT methods, such as stereotactic radiotherapy, intensity-modulated radiation therapy, and proton therapy, but all of them involve using a machine to deliver the radiation through the skin directly to the tumor. They also deliver a high targeted dose of radiation to the tumor, while limiting the amount of radiation to nearby healthy brain tissue and other body parts.<sup>31,33</sup> Reducing

exposure to the spine, heart, lungs, inner ear, and thyroid, among other areas, could result in fewer side effects. Radiation directed at the whole brain and spine, which is known as craniospinal radiation therapy, is typically recommended.<sup>8,33</sup>

Radiation therapy is usually started two to four weeks after surgery to give the body time to heal.<sup>21</sup> Generally, radiation therapy is given in a series of treatments called fractions over several weeks. Spacing the treatments out allows enough radiation to get into the body to kill the tumor, while giving the healthy cells time to recover.

Since irradiating the brain and CNS can interfere with a child's growth and development, doctors may choose a different treatment plan for children, especially very young ones.<sup>31</sup>

For children younger than 3 years of age, radiation therapy may be delayed or not even considered.<sup>33,34</sup> If radiation therapy is recommended, how much radiation will be given and what type will be based on the child's age and tumor features.

- For children older than 3 years of age and adults, radiation therapy is given to the entire brain and spine. Then a boost of radiation is aimed directly at the tumor and the surrounding area or the cerebellum.
- For children younger than 3 years of age, radiation therapy may be directed only to the cerebellum or the tumor and the surrounding area after surgery and chemotherapy.

Different people experience different side effects from radiation therapy. **Common side effects** include fatigue, mild skin reactions, vomiting, and loss of appetite.<sup>31</sup> Most of these side effects stop once treatment is finished.

But some people also have long-term side effects that may include problems with growth, low hormone levels, and learning disabilities or cognitive problems. This is likely because the parts of the body that control these functions, which are still developing in children, are in the path of the radiation beam.

As a result, the treatment may interfere with normal function. For example, long-term survivors of medulloblastoma experience moderate to severe hearing loss.<sup>33</sup> Detecting hearing loss early on may minimize its severity. **To learn more about radiation therapy, read the ABTA's brochures on Conventional Radiation Therapy, Proton Therapy and Stereotactic Radiosurgery.**



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Talk to your doctor about the different types of radiation therapy and which one might be right for you or your child.

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## CHEMOTHERAPY

Chemotherapy uses powerful chemicals, or drugs, to stop or slow the growth of tumor cells.<sup>31</sup> It can be given through an intravenous tube, or IV, or a pill. **Chemotherapy is used to kill any remaining tumor cells** following surgery and/or radiation.

Chemotherapy drugs currently being used to treat medulloblastoma include vincristine, lomustine, cyclophosphamide, cisplatin, carboplatin, and etoposide.<sup>21</sup>



*Fig. 5. Patient receiving chemotherapy.*

Typically, more than one chemotherapy is recommended because they attack the tumor in different ways and may be more effective when used together. As an example, a combination of cisplatin, lomustine, and vincristine have been used to successfully treat medulloblastoma as have cisplatin, cyclophosphamide, and vincristine.<sup>34</sup>

Researchers continue to evaluate chemotherapy drugs used to treat brain tumors to develop new ones or new approaches to using them.<sup>31</sup>

Because medulloblastomas are rare in adults, treatment plans, including the dose of radiation and the role of chemotherapy, are not as clear for adults as they are for children.<sup>21,33</sup> Overall, the chemotherapy drugs that have been used to treat children have worse side effects in

adults.<sup>33</sup> However, recent studies suggest that chemotherapy can be beneficial for treating adults. Treatment decisions for adults must balance the possible benefits of chemotherapy on long-term survival against the risk of severe side effects.

Researchers are studying different ways to use chemotherapy before, during, or after radiation therapy because it appears that combining the treatments is more effective.<sup>31</sup>

Higher doses of chemotherapy seem to work best when there is only a little bit of tumor left after surgery. For children, when and how chemotherapy is given is based on a child's age. High-dose chemotherapy may be used before or instead of radiation therapy for children younger than 3 years of age.

Several cycles of chemotherapy may be used after radiation therapy in children who are older than 3 years of age. Chemotherapy is usually scheduled for a specific number of cycles given over a set period of time.<sup>31</sup>

**Common side effects** of chemotherapy include fatigue, risk of infection, nausea and vomiting, hair loss, loss of appetite, and diarrhea.<sup>31</sup> Side effects, which can depend on the dose of chemotherapy given, usually stop after treatment is finished.

But some side effects, such as peripheral neuropathy (damage to the nerves that cause pain, numbness, tingling, swelling or muscle weakness that starts in the hands and feet and worsens over time) in adult patients treated with vincristine, can be long-term. About

40% to 60% of long-term survivors of childhood medulloblastoma experience moderate to severe hearing loss associated with cisplatin chemotherapy.<sup>33</sup>

**To learn more about common side effects of chemotherapy, read the ABTA's Chemotherapy brochure.**



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Tell your doctor about prescription medications, over-the-counter drugs, and supplements that you or your child are taking because they can interact with chemotherapy.<sup>31</sup>

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## PALLIATIVE CARE

Medulloblastoma and its treatments cause physical symptoms and side effects.<sup>31</sup> Relieving these symptoms and side effects is an important part of supportive care, sometimes referred to as palliative care.

Palliative care is for anyone, regardless of their age, or type and stage of disease. It should be started right after a diagnosis for best results. As an example, corticosteroids may be used to reduce edema (swelling in the brain near the tumor) that is sometimes caused by the brain tumor itself or therapies to treat it.<sup>33</sup>

**People who receive palliative care often have less severe symptoms, better quality of life, and are more satisfied with treatment.** Palliative care may include medication, nutritional changes,

relaxation techniques, and emotional and spiritual support, among others.<sup>31</sup>



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Ask your doctor which therapies can be used to treat symptoms and side effects.<sup>31</sup>

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## CLINICAL TRIALS

**Clinical trials offer individuals the chance to use new or experimental tests and treatments**

(meaning they have not yet been proven) before they become the standard of care. More than 60% of children with cancer are treated in clinical trials.<sup>31</sup> Availability of clinical trials for adult patients with medulloblastoma is more limited, but efforts are underway to increase their participation.

Among the new drugs being tested for the treatment of medulloblastoma in clinical trials are targeted therapies and immunotherapies.<sup>1,39</sup>

**Targeted therapies** refer to treatments that target certain proteins that help cause the medulloblastoma to grow and survive. Unlike chemotherapy that can kill all cells, targeted therapy is more precise in killing specific tumor cells with a specific abnormal protein.

**Immunotherapies** refer to drugs that enlist the body's own immune system to fight the tumor.<sup>1,21</sup> Other clinical trials focus on new ways to relieve symptoms and side effects.<sup>33,41</sup>

People who want to join a clinical trial volunteer and must meet certain rules called eligibility criteria, such as having a specific type of tumor or not having been treated with a certain therapy. Most clinical trials cover treatment costs.

**To learn more, read the ABTA's Clinical Trials brochure.**



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Some treatments being studied may not work or come with severe side effects.<sup>41</sup> Talk to your doctor to see if a clinical trial is right for you or your child.

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## FOLLOW-UP CARE

Following treatment for medulloblastoma, **patients should meet routinely with their doctor** to:

- Manage side effects that continue after treatment is finished
- Check if the tumor has returned
- Monitor overall health<sup>33,42</sup>

Typically, patients will be seen every three months for the first one to two years following treatment then every six to 12 months after that.<sup>33</sup> Follow-up visits may include a history and physical examination, an MRI of the brain and spine, and other medical tests.

**Some patients experience side effects from treatments long after the therapy has stopped.**<sup>42</sup>

Also, other side effects called **late effects** may start months or even years after active treatment is done. Late effects are not limited to the brain or CNS. They may include heart and lung problems, growth and hormone issues, and the rare occurrence of a second type of cancer. Emotional problems, such as anxiety and depression, and a decline in cognitive skills have been known to occur.

Treating late effects may require seeing other specialists, such as an endocrinologist, who is a doctor trained in treating growth or hormone imbalances; an oncologist or neuro-oncologist, who is trained in treating cancer, particularly with chemotherapy drugs; and/or a neuropsychologist, a psychologist who understands the relationship between the brain and behavior. Some patients may benefit from rehabilitation, ranging from physical therapy, family or individual counseling, nutritional planning, and/or educational help.

**Keeping a personal health record** is important for patients with medulloblastoma, who should be monitored for the rest of their lives for recurrence and side effects.<sup>42</sup>

Some patients will continue to see their oncologist, while others will go back to their family doctor. At some point in the person's life, it is likely that a doctor who was not directly involved in his or her care will be leading the patient's follow-up care.



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Talk to your doctor about the ongoing medical care and monitoring you or your child will need after treatment.

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When a patient no longer has any symptoms and the tumor cannot be found in the brain or spinal cord, it is referred to as being in remission.<sup>31</sup> Many remissions are permanent, but it's still important to understand the risk of recurrence, that is, the chance of the tumor returning.

## RECURRENCE

**Despite improved prognosis for patients with medulloblastoma, for about 20% to 30% of them the tumor will return.**<sup>33</sup> A recurring tumor either comes back in the same area where it was first found or it travels to the brain or spine.

In children, most recurrences happen within the first three years after their initial diagnosis. Adults tend to have relapses after longer periods of time, and the tumor also can come back in other parts of the body, such as the bone or bone marrow.

If a tumor does return, the doctor will perform a new round of tests to learn as much about the recurrent tumor as possible to help figure out the best treatment options.<sup>31</sup> These tests may include blood tests, imaging scans, and spinal taps. The doctor will recommend testing based

on such factors as tumor type, location of the recurrent tumor, and the type of treatment given previously.<sup>42</sup>

In general, **treatment for a recurrent tumor may include** additional surgery, radiation therapy (depending on whether or how much radiation was given after the original diagnosis), and chemotherapy.<sup>31</sup> These treatments may be used in a different combination or given on a different schedule than used with the first tumor. Some clinical trials focus on new medications and treatment combinations specifically for patients who have a recurrent tumor.<sup>11,31</sup>

High-dose chemotherapy may be given in the hope of providing a cure or prolonging the patient's life. High-dose chemotherapy combined with radiation therapy can sometimes result in longer tumor-free survival, especially for infants and young children.

High-dose chemotherapy may be followed up with a stem cell transplant.<sup>31</sup> This medical procedure involves replacing tumor cells in the bone marrow with very special cells called hematopoietic stem cells that develop into healthy bone marrow. This procedure used to be called a bone marrow transplant. The doctor will consider the type of tumor, results of previous treatment, and a patient's age and general health before recommending a stem cell transplant.

This approach has been studied in small groups and resulted in longer tumor-free survival for

some children who have not received radiation therapy. However, studies showing the benefit of using this approach are very limited.<sup>37</sup> The most serious side effect of stem cell transplant is risk of infection, most of which can be treated with antibiotics. Other side effects from stem cell transplant are similar to those from chemotherapy.



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Talk to your doctor about the possibility of the tumor recurring and treatment options available.<sup>31</sup>

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## PROGNOSIS

Prognosis refers to the chance of recovery or survival from a disease. A prognosis is based on statistics that look at a large group of people with the same disease over time. Keep in mind that **statistics on survival rates are estimates and cannot accurately predict what will happen to each person.** Typically, they are measured every five years, so the latest estimates may not include the most current methods of diagnosing and treating medulloblastoma.<sup>47</sup>

A patient's prognosis should take into account the following factors:

- **How much tumor remains in the brain after surgery**

The prognosis is better when the entire tumor can be surgically removed.<sup>48</sup>

- **Whether the tumor has spread at the time of diagnosis**

The prognosis tends to be better if the tumor has not spread when first diagnosed.<sup>33</sup>

- **Whether the tumor has spread to other parts of the body**

While spreading to other parts of the body is linked with a poorer prognosis,<sup>48</sup> medulloblastomas tend not to spread outside the CNS.<sup>1,2</sup>

- **Tumor genetic subtype**

WNT-activated tumors are associated with the most favorable prognosis of the four subtypes in children,<sup>7,15</sup> with a five-year survival of more than 95%.<sup>10</sup>

- **Tumor staging**

Standard-risk disease is associated with long-term survival rates of about 85%; high-risk disease is associated with long-term survival rates of about 70%.<sup>8</sup>

- **Tumor histologic patterns**

Patients with medulloblastoma with extensive nodularity and desmoplastic/nodular medulloblastoma have been shown to have a good prognosis when treated with standard treatment approaches.<sup>2,8,31</sup>

- **Age at diagnosis**

Generally, older children tend to have a better prognosis.<sup>15,33</sup> Reducing or eliminating radiation therapy in very young children because of the possible severe side effects to their developing CNS may play a role in this poorer prognosis.<sup>33</sup>

The outcome for patients diagnosed with medulloblastoma has improved dramatically over the past several decades.<sup>15,33</sup> **Most patients with medulloblastoma can be treated successfully.**<sup>31</sup> However, it is important to understand that these statistics do not reflect differences in outcome between standard-risk and high-risk groups (since high-risk groups may not do as well), and the tumor's genetic subtype and histologic patterns as well as the patient's characteristics and responses to treatment.

The following are the 5-year and 10-year survival rates for individuals with medulloblastoma: Overall, the Central Brain Tumor Registry of the United States reports that 65% of individuals with medulloblastoma are alive 10 years after being diagnosed.<sup>49</sup>

Tumor Type	Age Group	5-Year Survival Rates	10-Year Survival Rates
Medulloblastoma	Children (0-14)	73%	65%
	Adults 15-39	75%	68%
	Adults 40+	69%	48%

Source: CBTRUS 2018

Additionally, 10-year survival means that the patients were followed for only 10 years; how well they did beyond that length of time is not known.



Talk to your doctor about expected outcomes to get a more individualized prognosis.

## CHALLENGES AND FUTURE DIRECTIONS

The growing knowledge of genes and their role in brain tumor development has allowed researchers to further categorize medulloblastomas in ways that are having a significant impact on both treatment and survival.<sup>8</sup> But there is much more work to be done.

Knowing the genetic make-up of the tumor is an important first step in developing drugs that target and kill the cancer cells.<sup>5</sup> Future research is focused on such efforts as:

- Designing therapies that call on the body's own immune system to fight the tumor<sup>21</sup>
- Using modified viruses that kill only tumor cells and spare healthy cells<sup>21</sup>
- Testing drugs, which are approved for treating certain skin cancers, that attack the SHH pathway tumors<sup>33</sup>

Research efforts also are attempting to decrease the side effects of treatment therapies, while keeping high cure rates for patients with medulloblastoma.<sup>33</sup> The goal is to improve survival and quality of life after therapy.

The hope is that this better understanding will lead to improved and more precise treatment. Together, the medical and scientific communities, supporting organizations, and the patients and their families are building on past successes toward a better cure for all persons diagnosed with medulloblastoma.



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## AMERICAN BRAIN TUMOR ASSOCIATION INFORMATION, RESOURCES AND SUPPORT

### BROCHURES

Educational brochures are available on our website or can be requested in hard copy format for free by calling the ABTA. Most brochures are available in Spanish, with exceptions marked with an asterisk.

### GENERAL INFORMATION

About Brain Tumors: A Primer for Patients and Caregivers

Brain Tumor Dictionary\*

Brain Tumors: A Handbook for the Newly Diagnosed\*

Caregiver Handbook\*

### TUMOR TYPES

Ependymoma

Glioblastoma and Anaplastic Astrocytoma

Medulloblastoma

Meningioma

Metastatic Brain Tumors

Oligodendroglioma and Oligoastrocytoma

Pituitary Tumors

### TREATMENT

Chemotherapy

Clinical Trials

Conventional Radiation Therapy

Proton Therapy

Stereotactic Radiosurgery\*

Steroids

Surgery

# AMERICAN BRAIN TUMOR ASSOCIATION INFORMATION, RESOURCES AND SUPPORT

## INFORMATION

ABTA WEBSITE | [ABTA.ORG](https://abta.org)

Offers more than 200 pages of information, programs, support services and resources, including: brain tumor treatment center and support group locators, caregiver resources, research updates and tumor type and treatment information across all ages and tumor types.

## EDUCATION & SUPPORT

- **ABTA Educational Meetings & Webinars**  
In-person and virtual educational meetings led by nationally-recognized medical professionals.
- **ABTA Peer-to-Peer Mentor Program**  
Connect with a trained patient or caregiver mentor to help navigate a brain tumor diagnosis.
- **ABTA Connections Community**  
An online support and discussion community of more than 25,000 members.
- **ABTA CareLine**  
For personalized information and resources, call 800-886-ABTA (2282) or email [abtacares@abta.org](mailto:abtacares@abta.org) to connect with a CareLine staff member.

## GET INVOLVED

- Join an ABTA fundraising event.
- Donate by visiting [abta.org/donate](https://abta.org/donate).

## CONTACT THE ABTA

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