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## About Neuroblastoma

Get an overview of neuroblastoma and the latest key statistics in the US.

### Overview

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

- [What Is Neuroblastoma?](#)

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## What Is Neuroblastoma?



chemicals (hormones). This is important, because neuroblastoma cells often release certain chemicals that can cause symptoms (see [Signs and Symptoms of Neuroblastoma](#)<sup>3</sup>).

## Neuroblastomas

Neuroblastomas are cancers that start in early nerve cells (called **neuroblasts**) of the sympathetic nervous system, so they can be found anywhere along this system.

- Most neuroblastomas begin in the abdomen, either in an adrenal gland or in sympathetic nerve ganglia.
- Most of the rest start in sympathetic ganglia near the spine in the chest or neck, or in the pelvis.
- Rarely, a neuroblastoma has spread so widely by the time it is found that doctors can't tell exactly where it started.

Some neuroblastomas grow and spread quickly, while others grow slowly. Sometimes, in very young children, the cancer cells die for no reason and the tumor goes away on its own. In other cases, the cells sometimes mature on their own into normal ganglion cells and stop dividing (which makes the tumor a benign ganglioneuroma - see below).

## Other autonomic nervous system tumors in children

Not all childhood autonomic nervous system tumors are malignant (cancerous). Some tumors are benign (non-cancerous), and some can have both benign and cancer cells within the same tumor.

- **Ganglioneuroma** is a benign (non-cancerous) tumor made up of mature ganglion and nerve sheath cells.
- **Ganglioneuroblastoma** is a tumor that has both malignant and benign parts. It contains neuroblasts (immature nerve cells) that can grow and spread abnormally, similar to neuroblastoma, as well as areas of more mature tissue that are similar to ganglioneuroma.

If a child is thought to have one of these tumors, it is usually removed by surgery and looked at carefully with a microscope to see if it has areas of cancer cells (which would make it a ganglioneuroblastoma). If the final diagnosis is ganglioneuroma, no other treatment is needed. If it's found to be a ganglioneuroblastoma, it's treated the same way as a neuroblastoma.

## Hyperlinks

1. [www.cancer.org/cancer/understanding-cancer/what-is-cancer.html](http://www.cancer.org/cancer/understanding-cancer/what-is-cancer.html)
2. [www.cancer.org/cancer/types/cancer-in-children/differences-adults-children.html](http://www.cancer.org/cancer/types/cancer-in-children/differences-adults-children.html)
3. [www.cancer.org/cancer/types/neuroblastoma/detection-diagnosis-staging/signs-and-symptoms.html](http://www.cancer.org/cancer/types/neuroblastoma/detection-diagnosis-staging/signs-and-symptoms.html)

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## Key Statistics About Neuroblastoma

Neuroblastoma is by far the most common cancer in infants (younger than 1 year old). There are about 700 to 800 new cases of neuroblastoma each year in the United States. This number has remained about the same for many years.

The average age of children when they are diagnosed is about 1 to 2 years. Rarely, neuroblastoma is detected by ultrasound even before birth. About 9 out of 10 neuroblastomas are diagnosed by age 5. It is rare in people over the age of 10 years.

Statistics related to survival are discussed in [Survival Rates for Neuroblastoma Based on Risk Groups](#)<sup>1</sup>.

Visit the [American Cancer Society's Cancer Statistics Center](#)<sup>2</sup> for more key cancer statistics.

## Hyperlinks

1. [www.cancer.org/cancer/types/neuroblastoma/detection-diagnosis-staging/survival-rates.html](http://www.cancer.org/cancer/types/neuroblastoma/detection-diagnosis-staging/survival-rates.html)
2. [cancerstatisticscenter.cancer.org/](http://cancerstatisticscenter.cancer.org/)

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American Cancer Society. *Cancer Facts & Figures 2020*. Atlanta, Ga: American Cancer Society; 2020.

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# What's New in Neuroblastoma Research?

- [Genetics of neuroblastomas](#)
- [Treatment](#)

Important research into neuroblastoma is being done right now in many university hospitals, medical centers, and other research institutions around the world. Each year, scientists find out more about what causes the disease and how to improve treatment.

## Genetics of neuroblastomas

Researchers now have better tests to look for changes in the genes of neuroblastoma cells. Researchers might know that a change has happened on a certain chromosome (a strand of DNA inside the cell, which contains its genes), but they still need to learn more about that gene or what part of a gene has been affected. There are a few different ways that genes change in neuroblastoma cells:

- Sometimes there are extra copies of the same gene (called **amplification**) on a chromosome.
- Sometimes a chromosome can have missing pieces of DNA (called **deletions**) or extra pieces of DNA (called **gains** or **additions**), which can affect which genes the chromosome has.
- Sometimes genes have **mutations** (changes in the DNA) or other changes that can turn the gene on or off, which can affect how the cell grows and divides.

Understanding the gene changes in neuroblastoma helps researchers understand which neuroblastomas are likely to be cured with less intense treatment, and which will need more aggressive treatment. Some of these gene changes are being used now to help cancer care teams determine a child's neuroblastoma [stage](#)<sup>1</sup> and [risk group](#)<sup>2</sup>. Other gene changes might help researchers find new treatments that work on certain types of neuroblastoma cells.

Here are some specific DNA and gene changes currently being studied:

- DNA changes on the short arm of chromosome 6 (6p22) are more likely to be seen in neuroblastomas that grow more aggressively.
- Neuroblastoma cells in older children are more likely to have changes in the *ATRX* tumor suppressor gene. Tumors with this gene change tend to grow more slowly, but they are also harder to cure. This may help explain why older children tend to have high-risk neuroblastomas (which are often harder to treat), while younger children tend to have low- or intermediate-risk neuroblastomas (which are often easier to treat).
- Changes in or having more than one copy (amplification) of the *ALK* and *MYCN* genes are features used to help decide a child's risk group. Some drugs that target cells with *ALK* gene changes are already used to treat other types of cancer, and they are now being studied for use against neuroblastomas with *ALK* gene changes (see below). Some scientists also are studying how *ALK* gene changes might be related to extra copies of the *MYCN* gene in neuroblastoma cells.

## Treatment

Survival rates for neuroblastoma have gotten better as doctors have found ways to improve on current treatments, but survival rates for children with high-risk neuroblastoma are not as good as they are for children with low- or intermediate-risk disease.

Most clinical trials of high-risk neuroblastoma (more aggressive and hard-to-treat tumors) are focused on finding the best combinations of chemotherapy (chemo) drugs, stem cell transplant regimens, immunotherapies and other new treatments to try to cure more children. Current studies of low- and intermediate-risk neuroblastoma are trying to figure out if children can get less treatment and still do as well.

## Chemotherapy

The search continues for the best combinations of chemo drugs to treat neuroblastoma.

Several chemo drugs that are already used to treat other cancers, such as topotecan, irinotecan, and temozolomide, are now being studied in combination with other kinds of therapies for use against high-risk neuroblastoma or neuroblastoma that has come back.

Other studies are looking to see if some children with low- or intermediate-risk neuroblastoma can be treated with less (or even no) chemotherapy. The goal is to still

have the same good results, but with fewer side effects from treatment.

## Stem cell transplants

Doctors are also trying to improve the success rate for children with aggressive neuroblastoma with high-dose chemotherapy and [stem cell transplants](#)<sup>3</sup>, using different combinations of [chemotherapy](#)<sup>4</sup>, [radiation therapy](#)<sup>5</sup>, [retinoids](#)<sup>6</sup>, [immunotherapy](#)<sup>7</sup>, and other treatments. For example:

- Recent research has suggested that giving two stem cell transplants (**tandem transplants**) to children with high-risk neuroblastoma works better than giving just one stem cell transplant.
- Doctors are studying whether using different combinations of chemo drugs, such as **busulfan and melphalan**, might work better than the chemo combinations currently being used before a stem cell transplant.
- Other studies are looking at whether using stem cells donated from another person (an **allogeneic stem cell transplant**), instead of from the patient (an **autologous stem cell transplant**), might help some children with hard-to-treat tumors.

## Retinoids

Retinoids such as 13-*cis*-retinoic acid (isotretinoin) can reduce the risk of recurrence after treatment in children with high-risk neuroblastoma, especially when they are given with certain immunotherapy treatments. Giving 13-*cis*-retinoic acid in combination with different types of chemotherapy drugs, immunotherapies called monoclonal antibodies, and targeted drugs is being studied in a number of clinical trials to help determine the combinations that might work best.

## Targeted drugs

Knowing what makes neuroblastoma cells different from normal cells might lead to new approaches to treating this disease. Newer drugs that target neuroblastoma cells more specifically than standard chemo drugs are now being studied in clinical trials. For example:

In some neuroblastomas, the cells have changes in the *ALK* gene that help them grow. Drugs that target cells with changes in the *ALK* gene, such as crizotinib (Xalkori), have already been shown to be helpful in treating some other types of cancer. Crizotinib is now being studied as part of the treatment for children with high-risk neuroblastoma



when the cells have *ALK* gene changes. Other drugs that target cells with *ALK* changes, such as lorlatinib (Lorbrena) and ceritinib (Zykadia), are also being studied.

In some neuroblastomas, the cells have an overactive Aurora A kinase signaling pathway, which helps the cells grow. Drugs that target the Aurora A kinase pathway, such as LY3295668 erbumine, are now being studied in clinical trials for use against neuroblastoma.

Doctors are also looking more closely at the gene changes inside neuroblastoma cells to see if other targeted drugs might be helpful. Some of the targeted drugs being studied in this way include sorafenib, dasatinib, and vorinostat.

Many other targeted drugs are now being studied for use against neuroblastoma as well.

## **Immunotherapy**

Immunotherapy is the use of medicines to help a patient's own immune system fight cancer. Several different kinds of immunotherapy are now being tested or used against neuroblastoma.

### ***Anti-GD2 monoclonal antibodies***

The [monoclonal antibodies](#)<sup>8</sup> dinutuximab (Unituxin) and naxitamab (Danyelza), which target GD2 on neuroblastoma cells, are now being used in the United States for some children with high-risk neuroblastoma, to help immune system cells find and destroy the cancer cells. These antibodies are typically used after a stem cell transplant, but studies are now being done to see if they might be helpful earlier in the course of treatment as well.

Other antibodies that target GD2 are also being studied. For example, Hu14.18K322A is a modified antibody that might work as well as other GD2 antibodies without some of the side effects. And dinutuximab beta (Qarziba), which is similar to dinutuximab, is now being used in Europe.

### ***Vaccines***

Several cancer vaccines are also being studied for use against neuroblastoma. For these vaccines, injections of modified neuroblastoma cells or other substances are given to try to get the child's own immune system to attack cancer cells. These treatments are still in the early stages of clinical trials.



[highlights.html](#)

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