High-Risk Neuroblastoma

Information for patients and caregivers

This brochure is intended to provide caregivers with basic information about high-risk neuroblastoma. It is not intended to provide medical advice or replace any medical advice provided to you. Please consult your child’s doctor or healthcare team regarding any questions concerning your child’s medical condition.
Learning your child has high-risk neuroblastoma is incredibly difficult. Furthermore, you’ll be faced with decisions that need to be made very quickly, causing the experience to become even more challenging. Making sense of all the information needed to inform these decisions can be overwhelming, and this brochure is designed to help you do that. It includes the following information to help you along the way:

1. An overview of high-risk neuroblastoma
2. Major treatment steps, including critical decision points to discuss with your doctor
3. An explanation of the care team that will be supporting and partnering with you and your child
4. A glossary of commonly used terms

The information in this brochure is provided as introductory background information only and is not meant to replace information provided by your child’s doctor/healthcare team.

Neuroblastoma risk classifications

In many cancers, staging is used to define the extent of the cancer within the body in order to determine treatment. However, in neuroblastoma this is done by risk group.

Risk groups are assigned based on staging and other information, including:

- **Age at time of diagnosis**
- **The shape, function, and structure of the tumor cells**
- **Whether or not there are changes in the genes of the tumor cells**
- **Where in the body the tumor started**
- **What stage the cancer is, meaning how far it has spread in the body**

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**Low-risk** neuroblastoma, tumors are limited to 1 area, are unlikely to spread, and can be removed mostly or completely with surgery, with a low risk of the tumors coming back.

**Intermediate-risk** neuroblastoma, tumors are not easy to remove completely with surgery, and the tumor cells have characteristics that make them more likely to spread.

A **high-risk** designation, which may happen at initial diagnosis, means the cancer is considered aggressive and has been found in multiple places in the body. About half of neuroblastoma patients are considered high-risk at diagnosis.
Treatment for high-risk neuroblastoma: an overview

Different hospitals and children’s cancer centers have specific treatment guidelines or protocols that they follow, which will generally include these main steps shown below. It’s important to remember that, since every child’s disease is different, you and your doctor may make choices that don’t exactly follow these steps.

- **Diagnosis**
  - **Goal:** Remove the tumor by surgery

- **Induction therapy**
  - **Goal:** Reduce tumor size with chemotherapy

- **Local control**
  - **Goal:** Remove the tumor by surgery

- **Consolidation therapy**
  - **Goal:** Eliminate any remaining cancer cells left in the body after previous treatments

- **Maintenance**
  - **Goal:** Prevent cancer from returning

- **Remission and follow-up care**
  - **Goal:** If you have refractory disease
    - Your doctor may decide a different approach is needed, such as a clinical trial or a different approved treatment. It’s important to discuss your options.
  - **Goal:** If you have systemic relapse or a CNS relapse
    - Your doctor may decide a different approach is needed, such as a clinical trial or a different approved treatment. It’s important to discuss your options.

Depending on the burden of disease remaining, there may be different treatment options to consider.
Treatment for high-risk neuroblastoma: an in-depth look

**INDUCTION THERAPY:** For high-risk neuroblastoma, treatment begins with several courses of combined chemotherapy drugs. At this point, you may also choose to take part in one or multiple clinical trials, depending on how well your child responds.

Clinical trials are research studies that test the safety and how well treatment options that are not FDA approved work in patients. Ask your healthcare team about your options or go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and search for neuroblastoma. Your doctor will help you determine the path for your child’s treatment, which can include enrollment in a clinical trial or other treatment options.

*Note: keep in mind that clinical trials may be an option at multiple points in the course of neuroblastoma treatment.*

**REFRACTORY DISEASE:** This means that a patient has partial, minimal, or no response to the initial course of treatment. Different treatment options will be discussed at this point.

**LOCAL CONTROL (SURGERY):** Depending on the size and location of tumors, an operation may be performed to remove as much cancer as possible. In neuroblastoma, surgery can be complicated. Tumors may be located near blood vessels that supply major organs, such as the kidneys, or tumors may press against organs or the spinal cord. Sometimes it may not be possible to remove all of the tumor and more than one surgery will be needed.

**REMAINING DISEASE:** If cancer cells remain in the body after previous treatment, consolidation therapy may be considered to eliminate any remaining disease.

**CONSOLIDATION THERAPY:** Though institutions may differ in their approach, consolidation usually involves several treatments, including high-dose chemotherapy and autologous stem cell transplant (ASCT). ASCT is a procedure in which healthy stem cells (blood-forming cells) are collected, stored, and then given back to the patient after high-dose chemotherapy. It is sometimes done a second time using a different combination of high-dose chemotherapy drugs before the transplant. When it is done twice, it is sometimes referred to as a “tandem” stem cell transplant. Radiation may also be used during consolidation to remove any remaining traces of cancer. Ask your healthcare team about other treatment options that may be appropriate for your child.

**MAINTENANCE:** Treatments used in maintenance have been shown to change immature cancer cells into mature nerve cells. Your care team may consider other therapies that help the body’s immune system fight cancer, such as monoclonal antibody and cytokine treatments. Ask your healthcare team about other treatment options that may be appropriate for your child.

**RELAPSE:** This happens when a patient has been through treatment for high-risk neuroblastoma and the cancer returns. You and your child’s doctor will discuss treatment options and consider things such as the extent of disease or where it’s located. The cancer will most often return in the location of the original tumor, but in some rare cases, the relapse may occur in the central nervous system (CNS), which includes the brain, spinal cord, and their protective tissues and fluid. A CNS relapse requires a specialized treatment plan.

**REMISSION AND FOLLOW-UP CARE:** Remission means that there has been a decrease in or disappearance of signs and symptoms of cancer.

- **In partial remission,** some, but not all, signs and symptoms of cancer have disappeared.
- **In complete remission,** all signs and symptoms of cancer have disappeared, although cancer still may be in the body.
- **When remission is achieved with treatment,** follow-up care continues. Regular testing, including laboratory tests and scans, will be done periodically and then less frequently as time goes on.
- **Long-term follow-up care** will continue so that patients stay as healthy as possible. This is tailored to the individual patient and takes into consideration which treatments were given.
The Neuroblastoma Care Team

A team of healthcare and other professionals will guide you every step of the way.

YOUR HEALTHCARE TEAM CAN VARY. IT MAY INCLUDE:

**Pediatric oncologist**
A pediatric oncologist specializes in diagnosing and treating cancer in children. A child with neuroblastoma may have a team of oncologists led by an expert who specializes in treating neuroblastoma.

**Physician assistant (PA)**
PAs work with oncologists and may give physical examinations, recommend tests, read test results, help with surgery, provide treatments, and manage side effects. They may also prescribe medicines, administer chemotherapy, provide information and counseling, and perform procedures.

**Oncology nurse practitioner (NP)**
NPs may perform physical examinations, diagnose and treat patients, recommend tests, read test results, prescribe medicines, administer and manage treatments, manage side effects, provide information and counseling, and perform procedures.

**Oncology nurse**
Oncology nurses serve in many roles depending on their experience, advanced education, and specialized certification. They may perform physical examinations, administer medicines and treatments, coordinate care with the larger team, and provide information and counseling.

**Pediatric surgeon**
A pediatric surgeon specializes in performing surgery for pediatric patients. In the case of neuroblastomas, they will attempt to surgically remove the tumor and any known cancer cells.

**Child life specialist**
A child life specialist assists patients with procedural activities, such as distracting them from pain.

**Oncology social worker**
An oncology social worker is an expert in coordinating and providing help to cancer patients and their families. A social worker provides counseling, helps manage financial problems, and assists with housing or childcare issues when treatments are given at a facility far from home.

**Patient navigator**
A navigator helps guide patients and families through their journeys from diagnosis through treatment and beyond. The navigator can find and coordinate counseling, and can advise on financial and other support services. A patient navigator may be a nurse, social worker, or even a volunteer, depending on the hospital.

**Radiation oncologist**
A radiation oncologist is a medical doctor who reviews the results of various imaging tests to diagnose disease.

**Research nurse**
Research nurses are trained to identify specific problems that may arise for patients participating in a clinical trial.

**TALK TO YOUR CHILD’S HEALTHCARE TEAM ABOUT NEXT STEPS AND TREATMENT OPTIONS**
Understanding what comes next and knowing your options, including how treatments work, will help you collaborate effectively with your child’s healthcare team.
Glossary

Here are some common terms you may hear when your child is diagnosed with neuroblastoma. Your care team is a great resource. You can always rely on them for additional information.

- **Biopsy**
  The removal of cells or tissue for examination in a laboratory.

- **Bone marrow**
  The soft, sponge-like tissue in the center of most bones that produces blood cells.

- **Central nervous system (CNS)**
  The brain and spinal cord, including their protective tissues and fluid.

- **Cycle of treatment or treatment cycle**
  A period of treatment followed by a period of rest that is repeated on a schedule. When a cycle is repeated multiple times on a regular schedule, it is called a course of treatment.

- **Cytokine**
  A cancer treatment made in laboratories that mimics the way certain proteins naturally affect the immune system. Cytokines are often given in combination with other therapies. Some cytokines slow the immune system, while others speed it up.

- **Immune system**
  The network of cells, tissues, organs, and the substances they make that help the body fight infections and other diseases.

- **Lymph nodes**
  Lymph nodes are small vessels located throughout the body that filter fluid from tissues and trap viruses and bacteria, as well as cancer cells. During surgery, doctors remove and examine lymph nodes under a microscope to check for cancer cells.

- **Antibody therapy**
  Antibodies are proteins made by certain white blood cells in response to antigens, substances that trigger a response by the body's immune system. Antibodies can bind to antigens on cancer cells to help destroy them. A monoclonal antibody is engineered in a lab. They are used in some treatments to bind to specific substances on cancer cells. They can be used alone or to carry drugs or radioactive substances directly to cancer cells.

- **Autologous stem cell transplant (ASCT)**
  ASCT is a procedure in which healthy stem cells (blood-forming cells) are collected, stored, and then given back to the patient. In high-risk neuroblastoma, healthy stem cells used for the transplant are taken from the child's own body instead of from a donor. This makes it an autologous (self-donating) stem cell transplant. Healthy stem cells are usually taken and stored early during induction therapy. High-dose chemotherapy is given, and after that, the child's healthy stem cells are infused back into the body to create healthy new blood cells.
  Stem cell transplants are done in the hospital, and after the procedure, a stay in an isolation room is required for a few weeks to help prevent infections, while the transplanted stem cells are making new blood cells. Full recovery can take several months.

- **Preparation for treatment**
  Before the child begins treatment, doctors will give the child certain medicines to help prepare the body for treatment. This can include medications to help prevent infections, reduce the risk of bleeding, and harden the bone where chemotherapy is given.
THE Y-MABS® COMMITMENT

Y-mAbs is committed to developing innovative therapies to help treat pediatric cancer. In addition to sponsoring clinical trials, Y-mAbs offers a patient support program that provides information and resources to patients and families on accessing Y-mAbs products.

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