PICO framework : Neuroblastoma Treatment Overview

Neuroblastoma is an embryonal cancer that begins in immature nerve cells called neuroblasts, typically found in unborn babies and newborns. Normally, these neuroblasts mature into healthy nerve cells, but in some children, they start growing uncontrollably and form a tumor - this is neuroblastoma. Since neuro blasts usually mature by age 5, the cancer is rare in older children and adults. Most cases are diagnosed before age 5, and about 90% occur in children under 10, making it the most common cancer in babies under 1 year.

Population

Children with neuroblastoma, categorized by risk level:

- Low-risk: Localized disease without MYCN amplification
- Intermediate-risk: Stage L2 or M (<18 months), favorable biology (DI >1, no segmental chromosomal changes)
- High-risk: MYCN amplification, older age, unfavorable histology, or advanced stage (M or MS ≥12 months)

Intervention

Risk-adapted multimodal treatment based on classification :

Low - Risk :

Observation or Surgery

Imaging follow-up (e.g., ultrasound, MIBG)

Intermediate - Risk :

Chemotherapy (2–8 cycles)

Surgery + Surveillance (regular scans)

High - Risk :

Induction: Multi-agent chemo + stem cell collection

Surgery: If safe/needed

Consolidation: High-dose chemo + stem cell rescue + Radiation

Post-Consolidation: Anti-GD2 immunotherapy (e.g., dinutuximab) + Sargramostim + Isotretinoin ± Continuation Therapy: Eflornithine (optional)

Comparator

Comparator varies by risk group. For low-risk patients, treatment options like observation are compared to surgical removal to assess whether intervention is necessary. In intermediate-risk, shorter versus longer courses of chemotherapy, or chemo with/without surgery, are compared to balance effectiveness and side effects. For high-risk neuroblastoma, the standard multimodal approach—including chemotherapy, surgery, stem cell transplant, radiation, and immunotherapy—is compared with alternative strategies such as single vs. tandem transplants, bridging therapy, or experimental treatments in clinical trials like newer immunotherapies or continuation therapy (e.g., eflornithine).

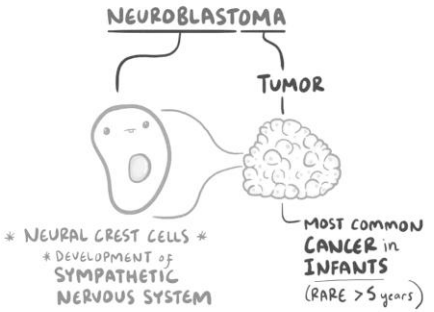
Outcomes

Primary Outcomes:

- *Event-Free Survival (EFS)* :
  Time a patient stays free from cancer events (like recurrence, progression, or death).
- *Overall Survival (OS)* :
  How long patients live after diagnosis or treatment - regardless of relapse.

Secondary Outcomes:

- *Response to Treatment* :
  Tumor shrinkage or disappearance after chemo/surgery.
- *Recurrence Rate* :
  How often the cancer comes back after treatment.
- *Treatment-Related Toxicities* :
  Side effects like:
  - Hearing loss (due to platinum drugs)
  - Heart damage (from anthracyclines)
  - Fertility issues (from alkylating agents)
- *Long-Term Effects*:
  Growth problems, learning delays, chronic organ damage



Abbreviations

MYCN - Myelocytomatosis Neuroblastoma ; DI - DNA Index ; MS - Stage of Neuro Blastoma ; L2 - Tumor Stage in Neuroblastoma

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