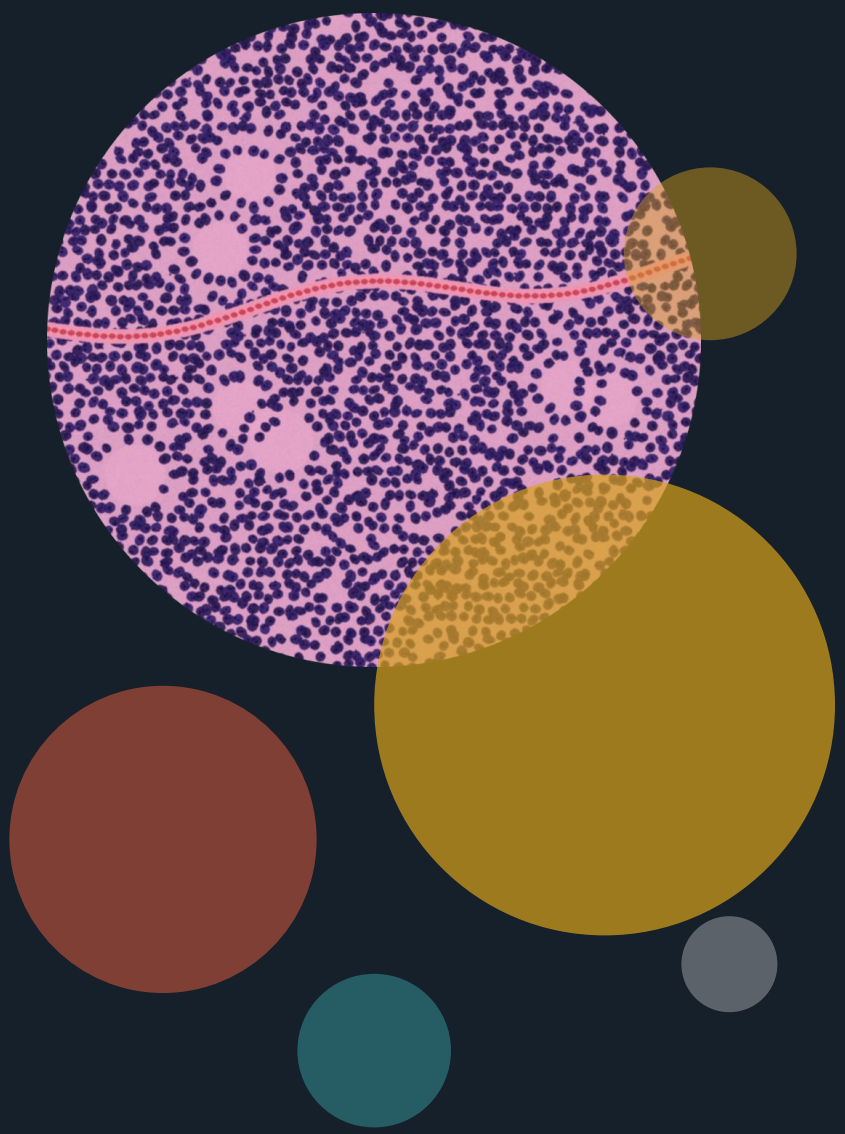
ONCOLOGY NURSING EDUCATION
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New Trends in Pediatric Neuroblastoma



What has changed in treatment, and what it means for nursing care.

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Agenda

01

The basics

Biology, presentation, risk groups

02

Immunotherapy

Anti-GD2 antibodies, CAR-T, NK cells

03

Precision medicine

ALK inhibitors, TERT, germline genetics

04

Keeping remission

DFMO, de-escalation, MIBG therapy

05

Looking ahead

Liquid biopsy, AI, survivorship, quiz

A cancer of early childhood

17 mo
median age at diagnosis

90%
diagnosed before age 5

~15%
of all pediatric cancer deaths

34→83%
5-year survival, ages 1–14 (1975 vs 2020)

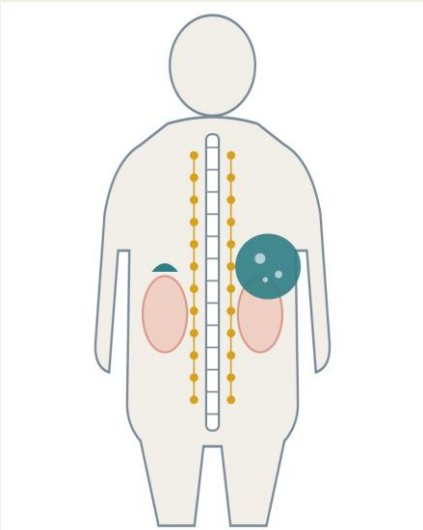
Where it starts

- Embryonal tumor of neural crest cells of the sympathetic nervous system
- Adrenal medulla or paraspinal ganglia anywhere from neck to pelvis
- Most common extracranial solid tumor of childhood: >650 new U.S. cases per year

Sources: NCI Neuroblastoma Treatment PDQ®; Braungart & Losty, World J Pediatr Surg 2026.

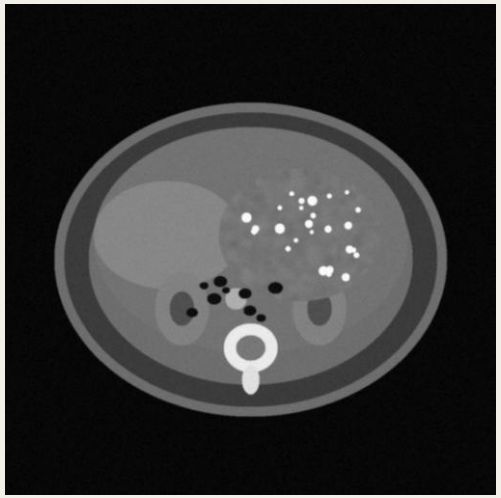
✓ Answers quiz question 1

What neuroblastoma looks like



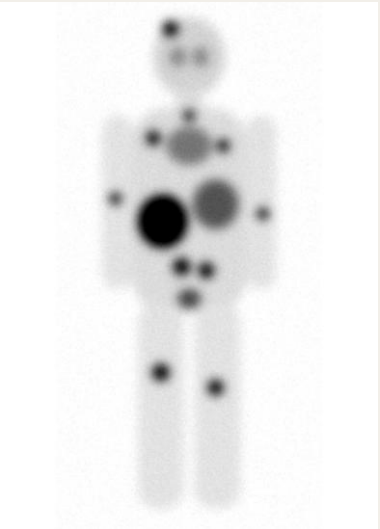
Where it grows

Adrenal gland (here, a left adrenal mass) or the sympathetic chain along the spine



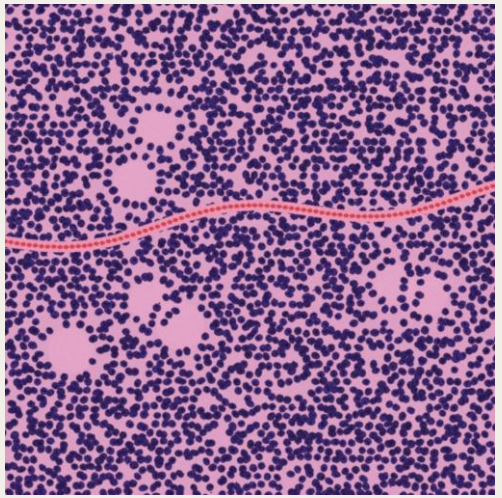
CT or MRI

Large mass with calcifications that can wrap around the aorta and push the kidney



123I-MIBG scan

Dark spots = uptake in the primary tumor and bone metastases



Under the microscope

Sheets of small round blue cells; pink Homer Wright rosettes

Illustrations drawn for this talk to show typical findings; they are not patient images. Findings per NCI PDQ® (diagnosis, MIBG avidity ~90%).

How it shows up on our units

Abdominal mass

The most common presentation

Periorbital bruising

“Raccoon eyes,” proptosis from orbital metastases

Bone pain, limp

Metastatic disease; child refuses to walk

Pallor, bruising

Pancytopenia from marrow involvement

Hypertension

Catecholamine secretion or renal compression

Horner syndrome

Ptosis, miosis, anhidrosis (stellate ganglion)

“Dancing eyes”

Opsoclonus-myoclonus: often favorable biology

Weakness, back pain

Spinal cord compression: an emergency

Source: NCI Neuroblastoma Treatment PDQ®, Clinical Presentation. Diagnosis: imaging + MIBG scan, urine HVA/VMA, biopsy, bone marrow.

One name, very different diseases

Can regress on its own (infants)

Aggressive, metastatic, relapse-prone

Low risk

>90%

5-year survival

Observation or surgery alone. Selected infants with small adrenal masses can be watched without surgery.

Intermediate risk

>90%

5-year survival

Moderate chemotherapy plus surgery, adjusted to tumor biology and response.

High risk

<60%

5-year survival

Intensive multimodal therapy over roughly 18 months, plus maintenance.

Sources: Braungart & Losty, World J Pediatr Surg 2026; NCI PDQ® (COG observation study of infants <6 months).

What decides the risk group

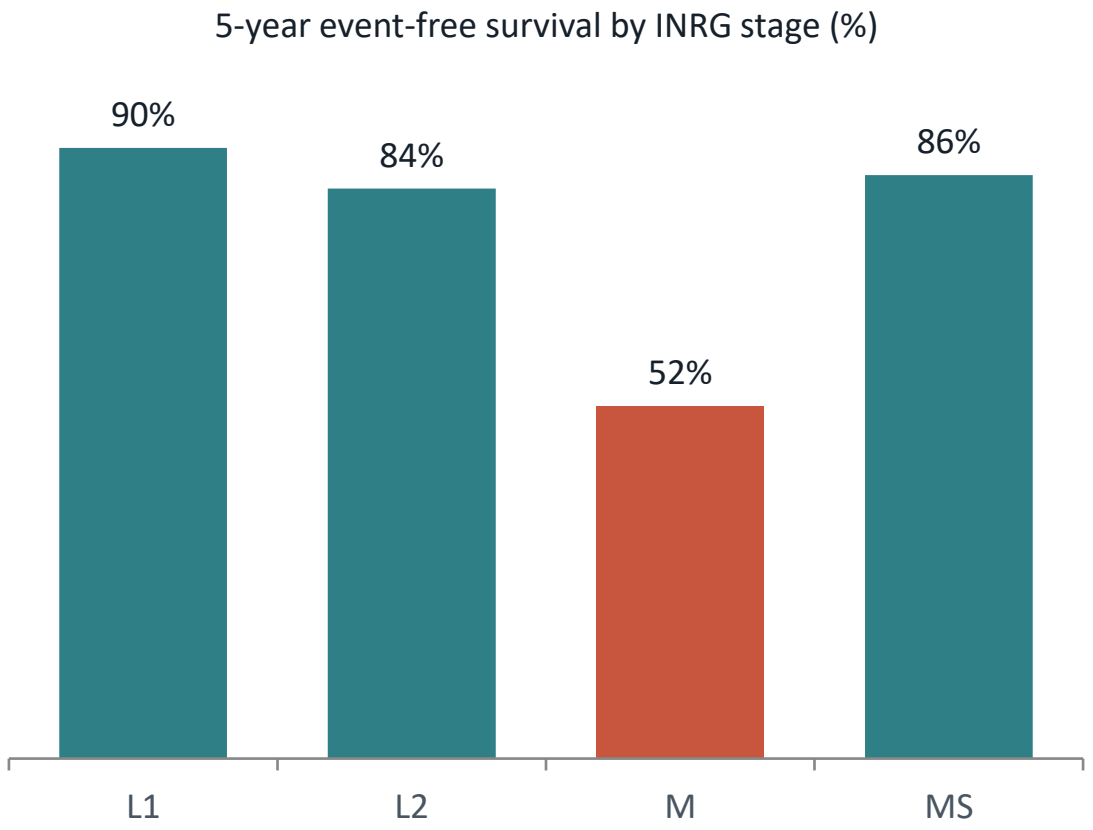
Age
Under vs over 18 months at diagnosis

INRG stage
L1, L2 (image-defined risk factors), M, MS

Histology
Favorable vs unfavorable (INPC)

MYCN amplification
~25% of all tumors; 40–50% of high-risk

Chromosome changes
1p and 11q loss, ploidy, ALK



Data: COG ANBL00B1, n = 4,832 (Irwin et al., J Clin Oncol 2021, via NCI PDQ®). Serum LDH and ferritin also carry independent prognostic weight.

High-risk therapy, and where the new trends plug in

1

Induction

5–6 cycles of chemotherapy; stem cell collection

2

Local control

Surgery of the primary tumor; radiation

3

Consolidation

High-dose chemotherapy with autologous stem cell rescue

4

Immunotherapy

Anti-GD2 antibody + GM-CSF + isotretinoin

5

Maintenance

DFMO (eflornithine) by mouth for 2 years

TREND Anti-GD2, MIBG and lorlatinib being added

TREND Is transplant always needed?

TREND Backbone of care

TREND New since 2023

Sources: NCI PDQ®; Braungart & Losty 2026; MSK Kids clinical update 2025. Regimens differ between COG (North America) and SIOPEN (Europe).

Outcomes still depend on the risk group

Risk group	Typical patient	Treatment today	Outcome
Low	Localized tumor (L1) without MYCN amplification; infants under 12 months with stage MS and favorable biology	Observation or surgery alone	**5-year EFS 89%, OS 97%**
Intermediate	Unresectable localized tumor (L2) or infant with metastatic disease, MYCN not amplified	2 to 8 cycles of moderate chemotherapy, with or without surgery	**3-year EFS 88%, OS 96%**
High	Metastatic disease at 18 months or older; most MYCN-amplified tumors at any age	Induction, surgery, tandem transplant, radiation, immunotherapy, DFMO	**5-year OS about 62%** **About 20% after relapse**

Low and intermediate risk

The question is how much treatment can be removed.

High risk

The question is what to add, and when.

Sources: NCI Neuroblastoma Treatment PDQ® (Health Professional Version); Irwin et al., J Clin Oncol 2021.

The COG risk classifier was revised in 2021

1. **Staging by imaging, before surgery**
   INRG stages L1, L2, M and MS replace the surgical INSS stage

2. **Tumor genetics count for more**
   MYCN, histology and ploidy, and now segmental chromosome changes such as 1p and 11q loss

3. **Age cut-off of 18 months**
   At 12 to 18 months, metastatic disease is high risk only with an unfavorable feature

4. **What nurses see**
   The final risk group may not be known until biology results return, up to 3 weeks

High risk under the 2021 classifier

- Stage M at 18 months or older
- Stage M under 18 months with MYCN amplification
- Stage MS or L2 with MYCN amplification
- Stage L2, 18 months or older, with unfavorable histology
- Stage M or MS at 12 to 18 months with an unfavorable feature
- Stage L1 with MYCN amplification, not completely resected

Everything else is low or intermediate risk.

Sources: Irwin et al., J Clin Oncol 2021 (COG ANBL00B1, n = 4,832); NCI Neuroblastoma Treatment PDQ®.

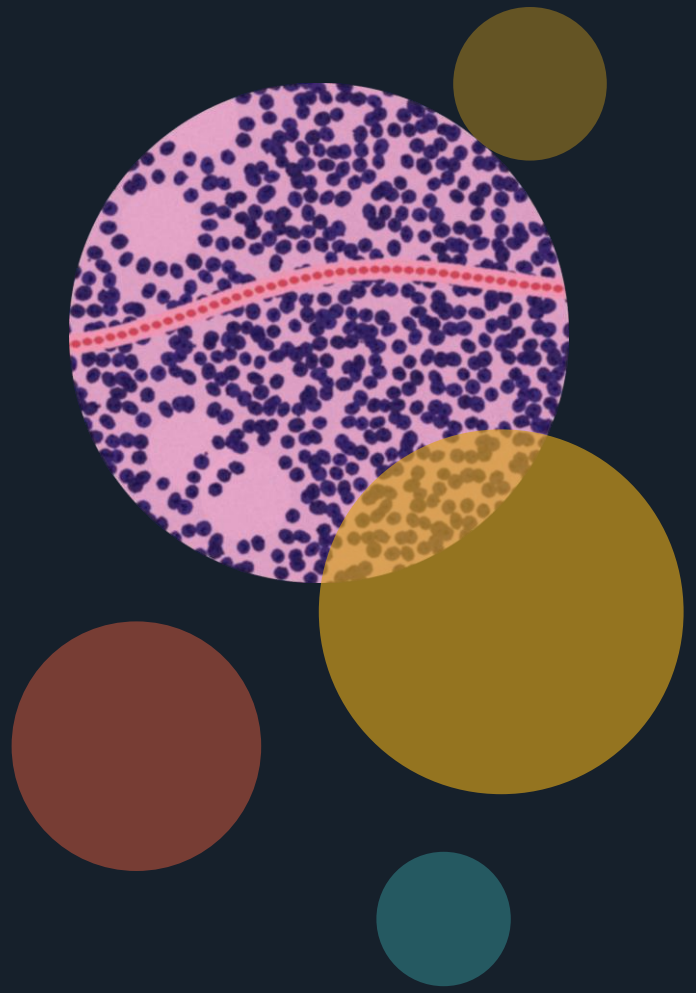
High-risk therapy in 2026: standard and open questions

Phase	Current standard (COG)	What is new or being tested
Induction	5 cycles of multi-agent chemotherapy; stem cell collection	Dinutuximab + GM-CSF with chemotherapy (ANBL2131, phase 3, enrolling) Lorlatinib for ALK-altered tumors (ANBL1531 Arm E) 131I-MIBG (ANBL1531; results pending)
Local control	Surgery of the primary tumor; radiation to the tumor bed	Higher radiation dose for residual tumor (SIOPEN HR-NBL2)
Consolidation	Tandem autologous transplant: 3-year EFS 61.6% vs 48.4% with a single transplant	Single vs tandem transplant re-tested in Europe (HR-NBL2)
Immunotherapy	Dinutuximab + GM-CSF + isotretinoin; IL-2 is no longer used	Humanized antibodies (naxitamab, daretabart)
Maintenance	Eflornithine (DFMO) by mouth for 2 years	FDA approved December 2023; not yet tested in a randomized trial

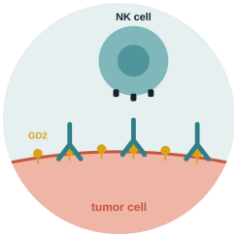
Sources: NCI PDQ®; Park et al., JAMA 2019; Yu et al., Clin Cancer Res 2021; ClinicalTrials.gov NCT06172296, NCT03126916, NCT04221035.

Immunotherapy

Teaching the immune system to find GD2, a sugar-lipid that coats nearly every neuroblastoma cell



Anti-GD2 antibodies: still the backbone



Dinutuximab

CHIMERIC ANTIBODY

U.S. standard after transplant, given with GM-CSF and isotretinoin

Dinutuximab beta

CHIMERIC ANTIBODY

European standard; long-term infusion without IL-2 is better tolerated

Naxitamab

HUMANIZED ANTIBODY

FDA accelerated approval 2020 for relapsed/refractory disease in bone or marrow, with GM-CSF

50%

overall response with naxitamab + GM-CSF in relapsed/refractory patients (38% complete). 1-year OS 93%.

Trial 201, n = 52 (Mora et al., Nat Commun 2025)

73.7%

3-year EFS (OS 86%) when anti-GD2 was given with every induction cycle, vs ~50% historically. COG is now testing this in phase 3 (ANBL2131).

St. Jude phase II, n = 64; single center, not randomized

Sources: MSK Kids clinical update 2025; Furman et al., J Clin Oncol 2022; COG ANBL2131 (NCT06172296); NCI PDQ®.

Infusion day: what we watch for



Neuropathic pain

GD2 sits on nerve fibers. Premedicate; IV opioid infusion and gabapentin are common. Slow the rate if severe.

Hypotension & capillary leak

Frequent BP, daily weights, strict I&O, albumin, edema.

Hypersensitivity

Urticaria, bronchospasm, anaphylaxis. Antihistamine premeds; emergency drugs at the bedside.

Fever

Expected cytokine effect, but infection must still be ruled out.

Electrolytes

Hyponatremia and hypokalemia; check labs per protocol.

Eyes & neuro

Pupil changes, blurred vision; report new neurologic signs.

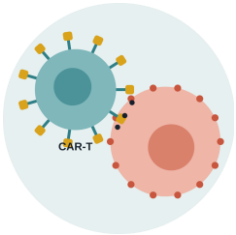
AT THE BEDSIDE

- Naxitamab pain is intense but usually settles within ~15 minutes of the infusion ending
- Many centers staff 1:2 on infusion days
- Prepare families: pain is expected and treated, it is not a sign of harm
- Follow your institutional protocol

Sources: MSK Kids clinical update 2025; dinutuximab prescribing information; CHOC Dinutuximab Care Guideline.

✓ Answers quiz question 3

Cell therapy is reaching a solid tumor



GD2-CART01 · phase 1/2, Rome

Final report 2025: 54 children. Results in the 38 treated with low disease burden at the recommended dose

77%	68%	53%
responded	5-year overall survival	5-year event-free survival

- **Safety switch:** an inducible caspase-9 “suicide gene”; it controlled grade 3 neurotoxicity in 4 children
- **Timing:** better survival when given after 1 or 2 lines of therapy

What comes next

- **B7-H3 CAR-T:** a second target, now in phase 1 trials for relapsed neuroblastoma and sarcomas
- **NK and CAR-NK cells:** possible “off-the-shelf” products with no graft-versus-host risk
- **The challenge:** an immunosuppressive tumor microenvironment; MYCN-amplified tumors are often immunologically “cold”

AT THE BEDSIDE

- Watch for CRS: fever, hypotension, hypoxia; do frequent neuro checks
- Available through clinical trials at specialized centers

Sources: Locatelli et al., Nat Med 2025; Del Bufalo et al., N Engl J Med 2023; NCI trial NCI-2025-00267; Shubina et al., Cancers 2026.

The MSK experience: a vaccine against GD2

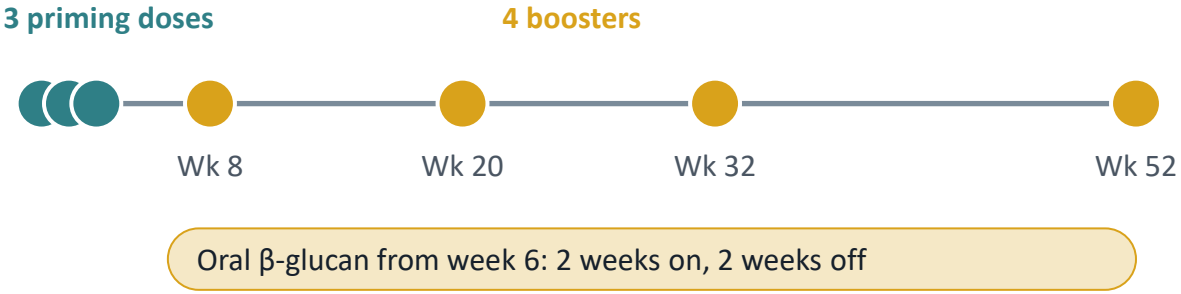


From borrowed antibodies to the child's own

- **Anti-GD2 antibody:** passive. We infuse the antibody; it wears off
- **Ganglioside vaccine:** active. It trains the child's immune system to make its own anti-GD2 (and anti-GD3) antibodies, aiming for long-lasting protection in remission

- **GD2 + GD3** the two gangliosides on neuroblastoma, linked to a KLH carrier protein
- **OPT-821 (QS-21)** plant-derived adjuvant that boosts the immune response
- **Oral β-glucan** yeast-derived supplement by mouth; primes immune cells via the dectin-1 receptor

One year of outpatient shots



AT THE BEDSIDE

- Subcutaneous injections in clinic; no admission needed
- Expect injection-site reactions and low-grade fever (grade 1–2)
- No neuropathic pain or capillary leak, unlike anti-GD2 infusions

Sources: Cheung IY et al., Vaccines 2024 (dosing schedule); Cheung IY et al., J Clin Oncol 2021; MSK Kids clinical update 2025. Available only at MSK (NCT06057948).

The MSK experience: what the vaccine trials show



Phase 1

2014 · 15 patients in ≥2nd remission

80%

relapse-free at 2 years; 12 of 15 made anti-GD2/GD3 antibodies

Phase 2

2021 · 102 patients after prior relapse

71%

5-year OS (PFS 32%); titer ≥150 ng/mL by week 8 → better PFS and OS

Randomized phase 2

2023 · 107 patients

↑ titer

β-glucan from day 1 raised anti-GD2 IgG1 in genetic (dectin-1) responders

Re-vaccination

2024 · 32 patients after relapse on vaccine

81%

OS at 54 months; antibody peaks ~5× higher on the second course

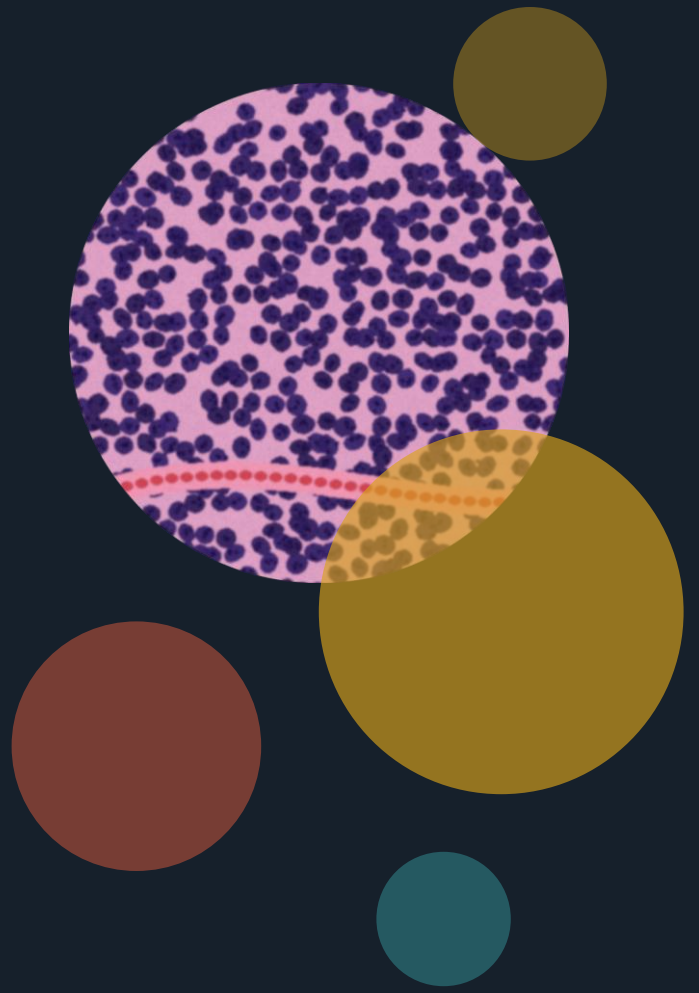
Now enrolling at MSK: phase 2 (NCT06057948, target 94 patients in 1st or 2nd remission) testing the best oral β-glucan schedule to maximize antibody titers.

Keep in mind: single-center, non-randomized for survival; not yet standard of care or FDA approved.

Sources: Kushner BH et al., Clin Cancer Res 2014; Cheung IY et al., J Clin Oncol 2021; Cheung IY et al., JAMA Oncol 2023; Cheung IY et al., Vaccines 2024; MSK Kids clinical update 2025.

Precision medicine

Reading the tumor's DNA to pick the drug, and to spot the patients who need more



ALK: a driver we can now block



2008 ALK identified as the main cause of familial neuroblastoma

2009 Crizotinib tested in children: only ~15% respond in neuroblastoma

2023 Lorlatinib (3rd generation) active in relapsed ALK-driven disease, even after prior ALK inhibitors

Now COG ANBL1531 Arm E: lorlatinib + standard therapy for newly diagnosed patients, then 1 more year

~1 in 4 high-risk tumors carry an ALK mutation (21.5%) or amplification (3.3%) when sequenced with modern NGS

AT THE BEDSIDE

- Oral, daily, at home: teach adherence and storage
- Expect weight gain and high lipids; manage with diet support
- Ask about mood, behavior, sleep and school; the trial tracks neurobehavioral effects

Sources: NCI PDQ®; Goldsmith et al., Nat Med 2023; Berko et al., J Clin Oncol 2025; COG ANBL1531 (NCT03126916).

Genomics is redrawing the risk map



TERT: the “immortality” gene

MSK sequenced more than 600 tumors (MSK-IMPACT + whole genome)

2.8%

TERT promoter mutations

18.4%

TERT rearrangements

Both groups relapsed early (median PFS ~9 months); promoter mutations also had worse overall survival. Investigators propose an “ultra-high-risk” group that may need escalated or TERT-targeted therapy.

Not only the tumor: germline genes

13.9% of 786 patients carried an inherited pathogenic variant (BARD1, ERCC2, CHEK2, MSH3 and others), linked to worse survival.

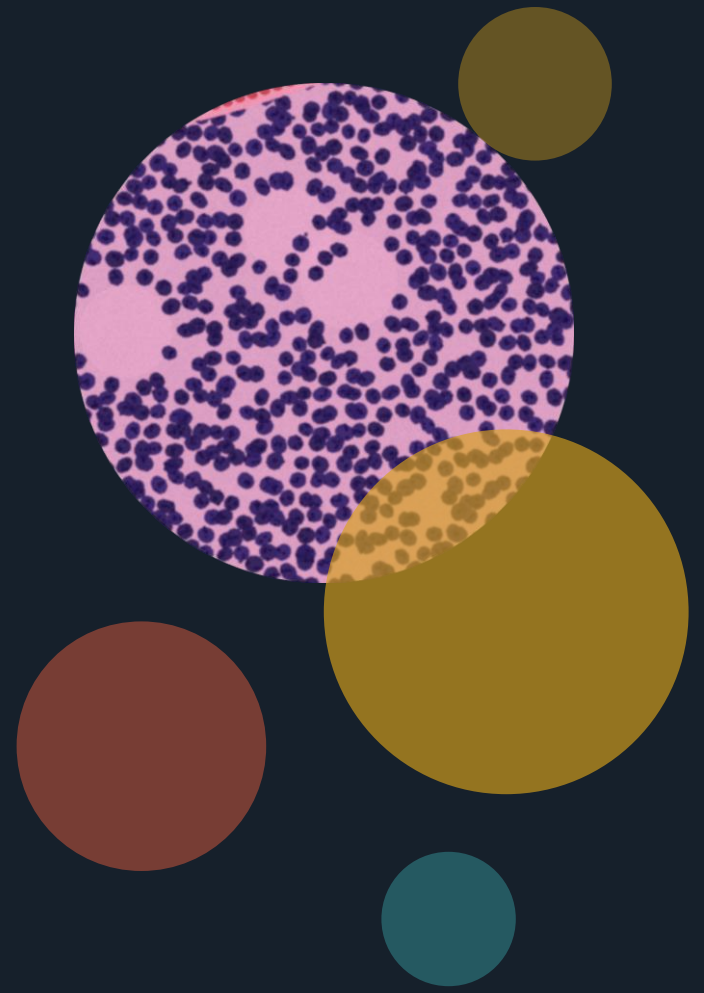
AT THE BEDSIDE

- Families will ask “Is it hereditary?” Refer to genetic counseling
- Children with germline ALK or PHOX2B variants get ultrasound and urine catecholamines every 3 months until age 6

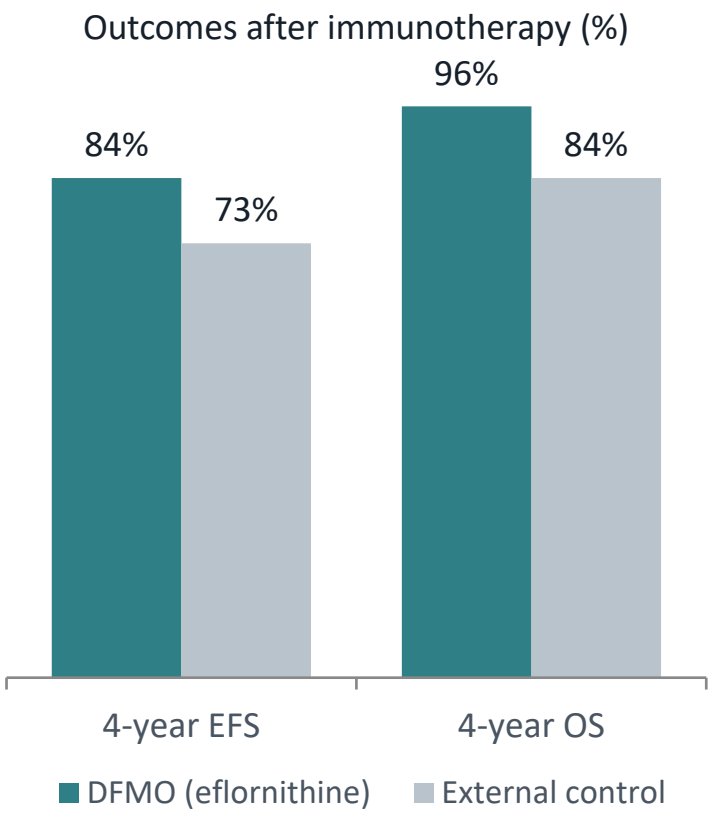
Sources: Persaud et al., JCO Precis Oncol 2025 (MSK); Kim et al., J Natl Cancer Inst 2024; NCI PDQ® (AACR surveillance recommendations).

Keeping remission

Maintenance therapy, doing less when it is safe, and radiation delivered from the inside



DFMO: the first maintenance drug for high-risk disease







Iwilfin
White, round, ~7 mm tablet marked “EFL 192”

Illustration

- **Approved:** FDA, December 2023, after at least a partial response to therapy that included anti-GD2
- **How:** 192 mg tablets by mouth, twice daily, for 2 years
- **Why it works:** blocks ornithine decarboxylase and lowers MYCN and LIN28B signaling
- **Caveat:** single-arm study with an external control; the advisory committee voted 14–6

AT THE BEDSIDE

- Two years at home: adherence is the treatment
- Hearing tests, CBC and liver tests on schedule; contraception counseling for adolescents
- Common: hearing loss, ear infections, fever, pneumonia, diarrhea

Sources: Iwilfin (eflornithine) prescribing information and FDA approval, Dec 13, 2023; MSK Kids clinical update 2025; tablet appearance per Drugs.com pill identifier.

✓ Answers quiz question 2

Less can be more: de-escalation



Skipping the transplant after Rapid COJEC?

MSK, 28 high-risk patients, no myeloablative therapy; anti-GD2 + GM-CSF instead

10 / 10

in complete remission after induction stayed event-free (median 3.7 years)

94%

3-year OS for the 18 with residual metastases (EFS 62%)

Retrospective and single center. COG and SIOPEN protocols still include transplant; this is a question for trials, not yet a new standard.

The same idea elsewhere

- **Infants:** 81% of selected babies under 6 months with small adrenal masses never needed surgery; all alive at 2 years
- **Intermediate risk:** much less chemotherapy with the same excellent outcomes (COG ANBL0531)
- **Goal:** protect hearing, fertility, growth and learning

Sources: Kushner et al., Int J Cancer 2025 (via MSK Kids update); NCI PDQ® (Nuchtern 2012; Twist 2019).

MIBG: radiation delivered from the inside



~90%

of neuroblastomas take up MIBG, a molecule that looks like norepinephrine to the tumor

123I-MIBG to see

Staging scans and response (Curie score)

131I-MIBG to treat

30–40% response in relapsed disease; ANBL1531 tested it in induction

- A same-molecule “see and treat” approach
- Needs stem cell support at high doses
- ANBL1531: the MIBG randomization is closed; results not yet reported

AT THE BEDSIDE

- Shielded room; time, distance and shielding for staff and caregivers
- Thyroid protection (potassium iodide) before and after
- Frequent voiding or catheter to protect the bladder; per radiation safety protocol

Sources: NCI PDQ®; COG ANBL1531 protocol summary; MSK Kids clinical update 2025.

Liquid biopsy and AI: less poking, more information



What is measured

Tumor DNA in blood (ctDNA), tumor cells, tumor mRNA, exosomes

Why it matters

Tracks minimal residual disease and clonal evolution in real time; sensitive blood/marrow RT-qPCR tracked outcome in a prospective study

Personalized markers

Patient-specific TERT breakpoint assays monitor disease by digital PCR

Artificial intelligence

Reads imaging and pathology; in the lab, machine learning told neuroblastoma cells from monocytes 97.9% of the time

AT THE BEDSIDE

- Mostly research for now, not routine care
- Expect extra research tubes at set time points
- Help families understand consent and why samples are drawn
- Could one day mean fewer bone marrow procedures

Sources: Sharma et al., Mol Neurobiol 2025; van Zogchel et al., J Exp Clin Cancer Res 2024; Pirone et al., APL Bioeng 2023; Serbena et al., Curr Oncol Rep 2026.

Survival is not the only endpoint



Hearing

Cisplatin, and now DFMO

Fertility

Alkylators and transplant

Growth & endocrine

Thyroid, growth, puberty

Learning

Cognition, school support

Emotional health

Child, siblings and parents

Second cancers

MDS and thyroid cancer reported

"As more children survive, fertility, hearing, endocrine health, cognition and emotional well-being need lifelong follow-up."

Paraphrasing Braungart & Losty, 2026

Sources: Braungart & Losty, World J Pediatr Surg 2026; MSK Kids clinical update 2025.

QUICK CHECK

True or false?

1

Neuroblastoma arises from neural crest cells of the sympathetic nervous system and is the most common extracranial solid tumor of childhood.



2

DFMO (eflornithine) is an IV drug given in the hospital during the anti-GD2 immunotherapy cycles.



3

Severe neuropathic pain is an expected effect of anti-GD2 antibodies, so patients need premedication and a pain plan, often with IV opioids.



QUICK CHECK

Answers

1

Neuroblastoma arises from neural crest cells of the sympathetic nervous system and is the most common extracranial solid tumor of childhood.

TRUE

Most children are diagnosed before age 5; urine HVA/VMA support the diagnosis.

2

DFMO (eflornithine) is an IV drug given in the hospital during the anti-GD2 immunotherapy cycles.

FALSE

It is an oral tablet taken at home twice daily for up to 2 years, after immunotherapy. Monitor hearing, blood counts and liver tests.

3

Severe neuropathic pain is an expected effect of anti-GD2 antibodies, so patients need premedication and a pain plan, often with IV opioids.

TRUE

GD2 is also expressed on peripheral nerve fibers. Also watch for hypotension, capillary leak and hypersensitivity.

TAKE-HOME

Five things to bring back to the unit

01 **Biology drives the plan.** The same diagnosis can mean watchful waiting or 18 months of therapy.

02 **Anti-GD2 is the backbone, and it is moving earlier.** Pain and capillary leak are nursing-sensitive toxicities.

03 **Targeted and cell therapies are entering frontline trials.** Lorlatinib for ALK-driven disease; CAR-T for relapse.

04 **Maintenance now lives at home.** Two years of DFMO: adherence and hearing checks are ours to protect.

05 **The future is less invasive and less toxic.** Liquid biopsy, de-escalation and lifelong survivorship care.

References (1 of 2)

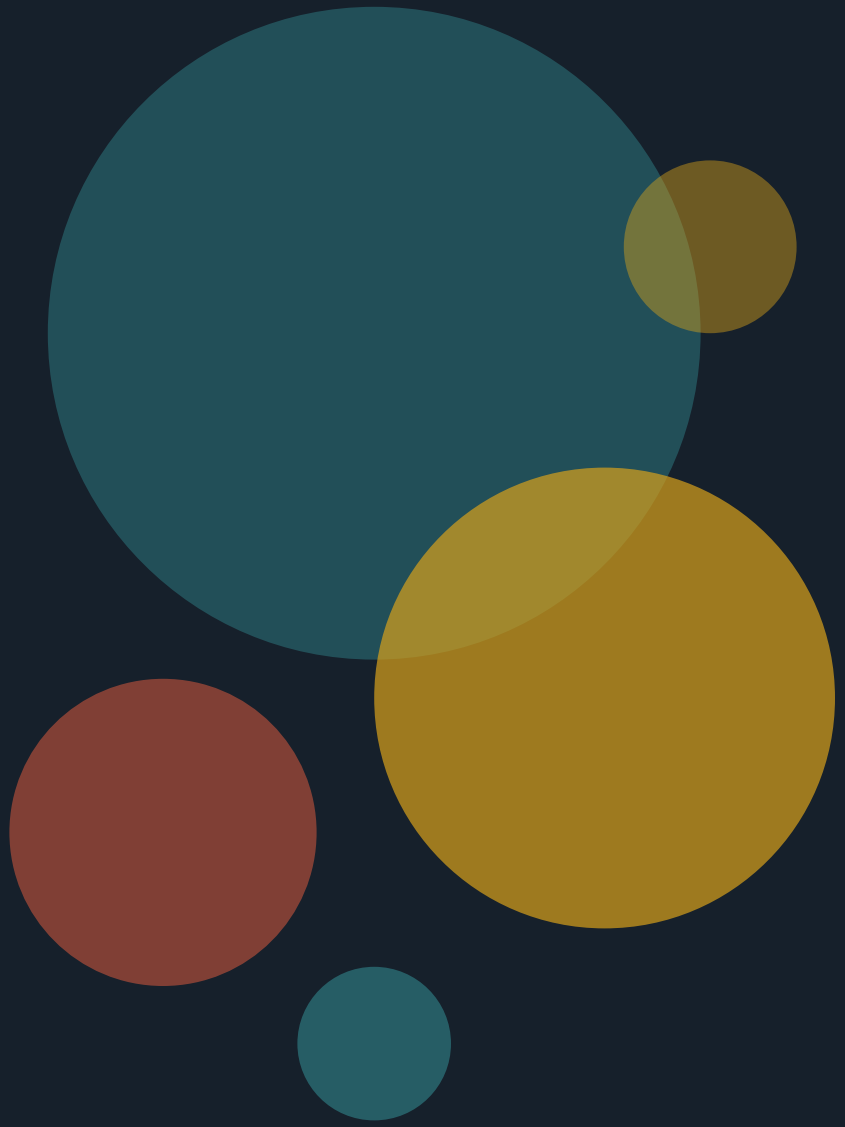
1. National Cancer Institute. Neuroblastoma Treatment (PDQ®)–Health Professional Version. https://www.cancer.gov/types/neuroblastoma/hp/neuroblastoma-treatment-pdq (accessed November 2026).
2. Braungart S, Losty PD. Diagnosis and management of neuroblastoma in children. World J Pediatr Surg. 2026;9(1):e001127. doi:10.1136/wjps-2025-001127 (PMC12778254)
3. Sharma R, Yadav J, Bhat SA, et al. Emerging trends in neuroblastoma diagnosis, therapeutics, and research. Mol Neurobiol. 2025;62(5):6423-6466. doi:10.1007/s12035-024-04680-w
4. Memorial Sloan Kettering Cancer Center. MSK Kids: recent research advances for high-risk neuroblastoma. Clinical update, September 25, 2025.
5. Kushner BH, Basu EM, Cardenas FI, Kramer K, Modak S. Rapid COJEC without myeloablative therapy for high-risk neuroblastoma. Int J Cancer. 2025 (online ahead of print).
6. Mora J, Chan GCF, Morgenstern DA, et al. Naxitamab plus GM-CSF for relapsed or refractory high-risk neuroblastoma: a phase 2 clinical trial. Nat Commun. 2025;16(1):1636.
7. Persaud N, Gundem G, Kushner BH, et al. Clinical significance of TERT promoter mutations in neuroblastoma. JCO Precis Oncol. 2025;9:e2500074.
8. Del Bufalo F, De Angelis B, Caruana I, et al. GD2-CART01 for relapsed or refractory high-risk neuroblastoma. N Engl J Med. 2023;388(14):1284-1295.
9. Goldsmith KC, Park JR, Kayser K, et al. Lorlatinib with or without chemotherapy in ALK-driven refractory/relapsed neuroblastoma: phase 1 trial results. Nat Med. 2023;29(5):1092-1102.
10. Berko et al. J Clin Oncol. 2025;43:1673-1684 (incidence and prognostic impact of ALK and other driver alterations at diagnosis, COG ANBL0532 cohort).
11. Furman WL, McCarville B, Shulkin BL, et al. Improved outcome in children with newly diagnosed high-risk neuroblastoma treated with chemoimmunotherapy: updated results of a phase II study using hu14.18K322A. J Clin Oncol. 2022;40(4):335-344.
12. US WorldMeds. IWILFIN (eflornithine) tablets, prescribing information; FDA approval December 13, 2023.
13. Kim J, Vaksman Z, Egolf LE, et al. Germline pathogenic variants in neuroblastoma patients are enriched in BARD1 and predict worse survival. J Natl Cancer Inst. 2024;116(1):149-159.

References (2 of 2)

14. van Zogchel LMJ, Decarolis B, van Wezel EM, et al. Sensitive liquid biopsy monitoring correlates with outcome in the prospective international GPOH-DCOG high-risk neuroblastoma RT-qPCR validation study. J Exp Clin Cancer Res. 2024;43(1):331.
15. Irwin MS, Naranjo A, Zhang FF, et al. Revised neuroblastoma risk classification system: a report from the Children's Oncology Group. J Clin Oncol. 2021;39(29):3229-3241.
16. Children's Oncology Group. ANBL1531: 131I-MIBG or lorlatinib added to intensive therapy for newly diagnosed high-risk neuroblastoma. ClinicalTrials.gov NCT03126916.
17. Shubina IZ, Bui CB, et al. Tumor microenvironment in neuroblastoma and immunotherapeutic approaches: towards more effective treatment. Cancers. 2026;18(7):1079.
18. Pirone D, Montella A, Sirico DG, et al. Phenotyping neuroblastoma cells through intelligent scrutiny of stain-free biomarkers in holographic flow cytometry. APL Bioeng. 2023;7(3):036118.
19. Serbena DR, et al. Artificial intelligence for diagnosis, risk stratification, and prognosis of neuroblastoma: a systematic review and meta-analysis. Curr Oncol Rep. 2026. doi:10.1007/s11912-026-01824-0
20. Children's Hospital of Orange County (CHOC). Dinutuximab (Unituxin®) Care Guideline.
21. Kushner BH, Cheung IY, Modak S, Kramer K, Ragupathi G, Cheung NK. Phase I trial of a bivalent gangliosides vaccine in combination with β-glucan for high-risk neuroblastoma in second or later remission. Clin Cancer Res. 2014;20(5):1375-1382.
22. Cheung IY, Cheung NK, Modak S, et al. Survival impact of anti-GD2 antibody response in a phase II ganglioside vaccine trial among patients with high-risk neuroblastoma with prior disease progression. J Clin Oncol. 2021;39(3):215-226.
23. Cheung IY, Mauguen A, Modak S, et al. Effect of oral β-glucan on antibody response to ganglioside vaccine in patients with high-risk neuroblastoma: a phase 2 randomized clinical trial. JAMA Oncol. 2023;9(2):242-250.
24. Cheung IY, Mauguen A, Modak S, et al. Long prime–boost interval and heightened anti-GD2 antibody response to carbohydrate cancer vaccine. Vaccines. 2024;12(6):587.
25. Locatelli F, Pagliara D, De Ioris MA, et al. GD2-targeting CAR T cells in high-risk neuroblastoma: a phase 1/2 trial. Nat Med. 2025;31(11):3689-3699.
26. Park JR, Kreissman SG, London WB, et al. Effect of tandem autologous stem cell transplant vs single transplant on event-free survival in patients with high-risk neuroblastoma. JAMA. 2019;322(8):746-755.
27. Yu AL, Gilman AL, Ozkaynak MF, et al. Long-term follow-up of a phase III study of ch14.18 (dinutuximab) + cytokine immunotherapy in children with high-risk neuroblastoma: COG study ANBL0032. Clin Cancer Res. 2021;27(8):2179-2189.
28. Children's Oncology Group. ANBL2131: dinutuximab with chemotherapy, surgery and stem cell transplantation for newly diagnosed high-risk neuroblastoma. ClinicalTrials.gov NCT06172296.
29. Additional resources provided: Biomedicine & Pharmacotherapy 2026 (sciencedirect.com/science/article/pii/S0753332226000521); News-Medical, “Risk-adapted frameworks reshape pediatric neuroblastoma care,” Aug 6, 2026; v.org, “New approaches to neuroblastoma”; Pediatric neuroblastoma treatment market outlook (Yahoo Finance); educational videos (YouTube).

THANK YOU

Questions?



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