

Supplementary Appendix

This appendix has been provided by the author to give readers additional information about his work.

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ON-LINE SUPPLEMENT

MEDICAL PROGRESS

Recent Advances in Neuroblastoma

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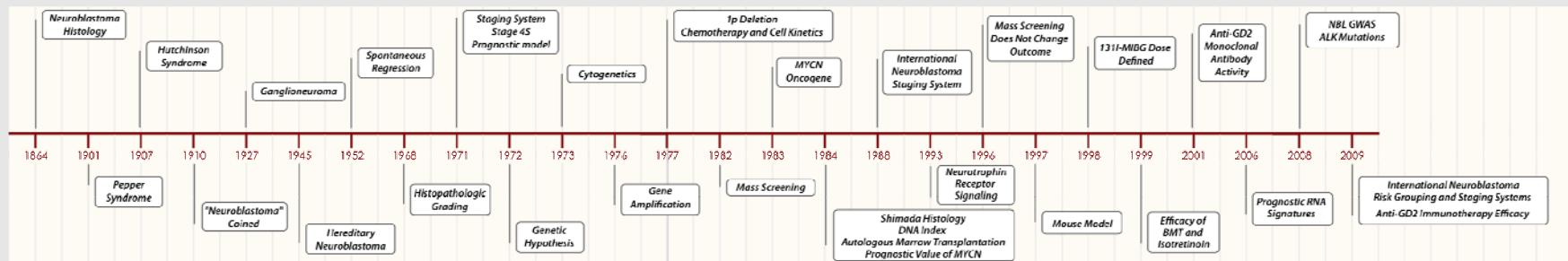
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Timeline of Investigations on Neuroblastoma



See table below for further description and references.

Table of important advances in neuroblastoma.

Year	Investigator(s)	Contribution	Reference
1864	Virchow	First description of neuroblastoma histology	1
1901	Pepper	First description of massive liver involvement associated with suprarenal mass (Pepper syndrome)	2
1907	Hutchinson	First description of neuroblastoma metastases to skull/orbits (Hutchinson syndrome)	3
1910	Wright	Coined term "neuroblastoma" and described bone marrow rosettes	4
1927	Cushing	First description of maturation to ganglioneuroma	5
1945	Dodge	First description of hereditary neuroblastoma	6
1952	Stewart and Everson	First descriptions of spontaneous regression of neuroblastoma	7, 8
1967	Bill and Koop	First international interdisciplinary conference on the biology of neuroblastoma	9
1968	Beckwith	First description of histopathologic grading	10
1971	Evans	First neuroblastoma staging system	11
1971	D'Angio and Evans	Definitive description and definition of the Stage 4S phenotype	12
1971	Breslow	First prognostic model for predicting neuroblastoma outcome	13
1972	Knudson and Strong	Genetic hypothesis for initiation of neuroblastoma tumorigenesis	14
1973	Biedler	First description of neuroblastoma cytogenetics	15
1974	Bolande	Unifying hypothesis for congenital disorders of neural crest including neuroblastoma	16
1976	Biedler	First description of HSRs and DMs as manifestation of gene amplification	17
1977	Brodeur	First description of chromosome arm 1p deletions	18
1977	Hayes	Correlation of cell kinetic and clinical response to chemotherapy in neuroblastoma	19
1982	Sawada	First description of screening for neuroblastoma in newborns	20
1983	Schwab	Discovery of the MYCN oncogene	21
1984	Shimada	Description and implementation of the Shimada histology grading system	22
1984	Look	Demonstration of the prognostic value of DNA index (ploidy)	23
1984	August	First report of autologous transplantation for high-risk neuroblastoma	24
1984	Brodeur and Seeger	Demonstration of the prognostic value of MYCN amplification	25, 26
1988	Brodeur and colleagues	First international neuroblastoma staging system	27
1993	Nakagawara	Demonstration of the central role of neurotrophin receptor signaling	28, 29
1996	Woods and Schilling	Showed that screening for neuroblastoma does not change outcome	30, 31
1997	Weiss and Bishop	Development of a mouse model of neuroblastoma	32
1998	Matthay	Demonstration that targeted radiotherapy with 131I-MIBG has anti-neuroblastoma activity	33
1999	Matthay and Reynolds	Phase III trial showing efficacy of BMT and 13-cis retinoic acid	34
2001	Kushner and Cheung	Demonstration that anti-GD2 monoclonal antibody has anti-neuroblastoma activity	35
2005	Attiyeh	Demonstration of 1p36 and 11q23 LOH as relevant prognostic biomarkers	36
2006	Wei, Ohira, Asgharzadeh and Oberthuer	Demonstration of prognostic RNA signatures	37-40
2008	Maris and colleagues	Discovery of common variations predisposing to sporadic neuroblastoma	41-43
2008	Mosse, Janoueix-Lerosey, George and Chen	Discovery of ALK as the major familial neuroblastoma gene and as a mutated oncogene	44-47
2009	Cohn, Montclair and Pearson	International Neuroblastoma Risk Grouping and Staging Systems	48, 49
2009	Yu	Demonstration that anti-GD2 immunotherapy improves survival	50

References

1. Virchow R. Hyperplasie der Zirbel und der Nebennieren. Die Krankhaften Geschwulste 1864;11:149-50.
2. Pepper W. A study of congenital sarcoma of the liver and suprarenal with the report of a case. . Am J Med Sci 1901;121:287-99.
3. Hutchinson R. Suprarenal sarcoma in children with metastases to skull. Quart J Med 1907;1:33-8.
4. Wright JH. Neurocytoma or neuroblastoma, a kind of tumor not generally recognized. . Journal of Experimental Medicine 1910;12:556-61.
5. Cushing H, Wolbach SB. The Transformation of a Malignant Paravertebral Sympathicoblastoma into a Benign Ganglioneuroma. Am J Pathol 1927;3:203-16.
6. Dodge HJ, Beuner MC. Neuroblastoma of the adrenal medulla in siblings. Rocky Mt Med 1945;42:35-8.
7. Stewart FW. Experiences in spontaneous regression of neoplastic disease in man [Bertner lecture]. Texas Rep Biol & Med 1952;10:239-53.
8. Everson T, Cole W. Spontaneous regression of cancer: Preliminary report. Annals of Surgery 1956;144:366.
9. Bill AH, Koop CE. Conference on The Biology of Neuroblastoma. In; 1967: Journal of Pediatric Surgery; 1967.
10. Beckwith JB, Martin RF. Observations on the histopathology of neuroblastomas. J Pediatr Surg 1968;3:106-10.
11. Evans AE, D'Angio GJ, Randolph J. A proposed staging for children with neuroblastoma. Children's cancer study group A. Cancer 1971;27:374-8.
12. D'Angio GJ, Evans AE, Koop CE. Special pattern of widespread neuroblastoma with a favourable prognosis. Lancet 1971;1:1046-9.
13. Breslow N, McCann B. Statistical estimation of prognosis for children with neuroblastoma. Cancer Research 1971;31:2098-103.
14. Knudson AGJ, Strong LC. Mutation and cancer: Neuroblastoma and pheochromocytoma. American Journal of Human Genetics 1972;24:514-22.
15. Biedler JL, Helson L, Spengler BA. Morphology and growth, tumorigenicity, and cytogenetics of human neuroblastoma cells in continuous culture. Cancer Research 1973;33:2643-52.
16. Bolande RP. The neurocristopathies: A unifying concept of disease arising in neural crest maldevelopment. Human Pathology 1974;5:409-29.
17. Biedler JL, Spengler BA. A novel chromosome abnormality in human neuroblastoma and antifolate-resistant Chinese hamster cell lines in culture. J Natl Cancer Inst 1976;57:683-95.
18. Brodeur GM, Sekhon GS, Goldstein MN. Chromosomal aberrations in human neuroblastomas. Cancer 1977;40:2256-63.

19. Hayes FA, Green AA, Mauer AM. Correlation of cell kinetic and clinical response to chemotherapy in disseminated neuroblastoma. *Cancer Res* 1977;37:3766-70.
20. Sawada T, Todo S, Fujita K, Iino S, Imashuku S, Kusunoki T. Mass screening of neuroblastoma in infancy. *Am J Dis Child* 1982;136:710-2.
21. Schwab M, Alitalo K, Klempnauer KH, et al. Amplified DNA with limited homology to myc cellular oncogene is shared by human neuroblastoma cell lines and a neuroblastoma tumour. *Nature* 1983;305:245-8.
22. Shimada H, Chatten J, Newton WA, Jr., et al. Histopathologic prognostic factors in neuroblastic tumors: Definition of subtypes of ganglioneuroblastoma and an age-linked classification of neuroblastomas. *Journal of the National Cancer Institute* 1984;73:405-13.
23. Look AT, Hayes FA, Nitschke R, McWilliams NB, Green AA. Cellular DNA content as a predictor of response to chemotherapy in infants with unresectable neuroblastoma. *New England Journal Medicine* 1984;311:231-5.
24. August CS, Serota FT, Koch PA, et al. Treatment of advanced neuroblastoma with supralethal chemotherapy, radiation and allogeneic or autologous marrow reconstitution. *Journal of Clinical Oncology* 1984;2:609-16.
25. Brodeur G, Seeger RC, Schwab M, Varmus HE, Bishop JM. Amplification of N-myc in untreated human neuroblastomas correlates with advanced disease stage. *Science* 1984;224:1121-4.
26. Seeger RC, Brodeur GM, Sather H, et al. Association of multiple copies of the N-myc oncogene with rapid progression of neuroblastomas. *New England Journal of Medicine* 1985;313:1111-6.
27. Brodeur GM, Seeger RC, Barrett A, et al. International criteria for diagnosis, staging, and response to treatment in patients with neuroblastoma. *J Clin Oncol* 1988;6:1874-81.
28. Nakagawara A, Arima-Nakagawara M, Scavarda NJ, Azar CG, Cantor AB, Brodeur GM. Association between high levels of expression of the TRK gene and favorable outcome in human neuroblastoma. *New England Journal of Medicine* 1993;328:847-54.
29. Nakagawara A, Azar CG, Scavarda NJ, Brodeur GM. Expression and function of TRK-B and BDNF in human neuroblastomas. *Molecular and Cellular Biology* 1994;14:759-67.
30. Woods WG, Tuchman M, Robison LL, et al. A population-based study of the usefulness of screening for neuroblastoma. *Lancet* 1996;348:1682-7.
31. Schilling FH, Spix C, Berthold F, et al. Neuroblastoma screening at one year of age. *N Engl J Med* 2002;346:1047-53.
32. Weiss WA, Aldape K, Mohapatra G, Feuerstein BG, Bishop JM. Targeted expression of MYCN causes neuroblastoma in transgenic mice. *EMBO Journal* 1997;16:2985-95.
33. Matthay KK, DeSantes K, Hasegawa B, et al. Phase I dose escalation of 131I-metiodobenzylguanidine with autologous bone marrow support in refractory neuroblastoma. *Journal of Clinical Oncology* 1998;16:229-36.

34. Matthay KK, Villablanca JG, Seeger RC, et al. Treatment of high-risk neuroblastoma with intensive chemotherapy, radiotherapy, autologous bone marrow transplantation, and 13-cis-retinoic acid. Children's Cancer Group. *New England Journal of Medicine* 1999;341:1165-73.
35. Kushner BH, Kramer K, Cheung NK. Phase II trial of the anti-GD2 monoclonal antibody 3F8 and granulocyte-macrophage colony-stimulating factor for neuroblastoma. *J Clin Oncol* 2001;19:4189-94.
36. Attiyeh EF, London WB, Mosse YP, et al. Chromosome 1p and 11q deletions and outcome in neuroblastoma. *N Engl J Med* 2005;353:2243-53.
37. Asgharzadeh S, Pique-Regi R, Sposto R, et al. Prognostic significance of gene expression profiles of metastatic neuroblastomas lacking MYCN gene amplification. *J Natl Cancer Inst* 2006;98:1193-203.
38. Oberthuer A, Berthold F, Warnat P, et al. Customized oligonucleotide microarray gene expression-based classification of neuroblastoma patients outperforms current clinical risk stratification. *J Clin Oncol* 2006;24:5070-8.
39. Ohira M, Oba S, Nakamura Y, et al. Expression profiling using a tumor-specific cDNA microarray predicts the prognosis of intermediate risk neuroblastomas. *Cancer Cell* 2005;7:337-50.
40. Wei JS, Greer BT, Westermann F, et al. Prediction of clinical outcome using gene expression profiling and artificial neural networks for patients with neuroblastoma. *Cancer Res* 2004;64:6883-91.
41. Maris JM, Mosse YP, Bradfield JP, et al. Chromosome 6p22 locus associated with clinically aggressive neuroblastoma. *N Engl J Med* 2008;358:2585-93.
42. Capasso M, Devoto M, Hou C, et al. Common variations in BARD1 influence susceptibility to high-risk neuroblastoma. *Nat Genet* 2009.
43. Diskin SJ, Hou C, Glessner JT, et al. Copy number variation at 1q21.1 associated with neuroblastoma. *Nature* 2009;459:987-91.
44. Chen Y, Takita J, Choi YL, et al. Oncogenic mutations of ALK kinase in neuroblastoma. *Nature* 2008;455:971-4.
45. George RE, Sanda T, Hanna M, et al. Activating mutations in ALK provide a therapeutic target in neuroblastoma. *Nature* 2008;455:975-8.
46. Janoueix-Lerosey I, Lequin D, Brugieres L, et al. Somatic and germline activating mutations of the ALK kinase receptor in neuroblastoma. *Nature* 2008;455:967-70.
47. Mosse YP, Laudenslager M, Longo L, et al. Identification of ALK as a major familial neuroblastoma predisposition gene. *Nature* 2008;455:930-5.
48. Cohn SL, Pearson AD, London WB, et al. The International Neuroblastoma Risk Group (INRG) Classification System: An INRG Task Force Report. *J Clin Oncol* 2008.
49. Monclair T, Brodeur GM, Ambros PF, et al. The International Neuroblastoma Risk Group (INRG) Staging System: An INRG Task Force Report. *J Clin Oncol* 2008.
50. Yu AL, Gilman AL, Ozkaynak MF, et al. A phase III randomized trial of the chimeric anti-GD2 antibody ch14.18 with GM-CSF and IL2 as immunotherapy following dose intensive chemotherapy for high-risk

neuroblastoma: Children's Oncology Group (COG) study ANBL0032. *Journal of Clinical Oncology* 2009;27:Abstr 10067z.