

# CHLD0071: Molecular and Clinical Aspects of Childhood Cancers

View Online



1.  
Weinberg RA. The biology of cancer. 2nd ed. New York: Garland Science; 2014.
2.  
Hanahan D, Weinberg RA. The Hallmarks of Cancer. Cell. 2000 Jan;100(1):57-70.
3.  
Hanahan D, Weinberg RA. Hallmarks of Cancer: The Next Generation. Cell. 2011 Mar;144(5):646-674.
4.  
Lord CJ, Ashworth A. Biology-driven cancer drug development: back to the future. BMC Biology. 2010;8(1).
5.  
Vogelstein B, Papadopoulos N, Velculescu VE, Zhou S, Diaz LA, Kinzler KW. Cancer Genome Landscapes. Science. 2013 Mar 29;339(6127):1546-1558.
6.  
Lee TI, Young RA. Transcriptional Regulation and Its Misregulation in Disease. Cell. 2013 Mar;152(6):1237-1251.

7.

Children's cancer statistics | Cancer Research UK [Internet]. Available from:  
<http://www.cancerresearchuk.org/health-professional/cancer-statistics/childrens-cancers>

8.

International Agency for Research on Cancer. WHO classification of tumours of the central nervous system. Revised 4th edition. Louis DN, Ohgaki H, Wiestler OD, Cavenee WK, editors. Lyon: International Agency for Research on Cancer; 2016.

9.

Taylor, Michael D Northcott, Paul A Korshunov, Andrey Remke, Marc Cho, Yoon-jae. Molecular subgroups of medulloblastoma: the current consensus. *Acta Neuropathologica* [Internet]. 123(3):465–72. Available from:  
[https://search.proquest.com/docview/928783888?rfr\\_id=info%3Axri%2Fsid%3Aprimo](https://search.proquest.com/docview/928783888?rfr_id=info%3Axri%2Fsid%3Aprimo)

10.

Gibson P, Tong Y, Robinson G, Thompson MC, Currie DS, Eden C, Kranenburg TA, Hogg T, Poppleton H, Martin J, Finkelstein D, Pounds S, Weiss A, Patay Z, Scoggins M, Ogg R, Pei Y, Yang ZJ, Brun S, Lee Y, Zindy F, Lindsey JC, Taketo MM, Boop FA, Sanford RA, Gajjar A, Clifford SC, Roussel MF, McKinnon PJ, Gutmann DH, Ellison DW, Wechsler-Reya R, Gilbertson RJ. Subtypes of medulloblastoma have distinct developmental origins. *Nature*. 2010 Dec;468(7327):1095–1099.

11.

Ellison DW, Onilude OE, Lindsey JC, Lusher ME, Weston CL, Taylor RE, Pearson AD, Clifford SC.  $\beta$ -Catenin Status Predicts a Favorable Outcome in Childhood Medulloblastoma: The United Kingdom Children's Cancer Study Group Brain Tumour Committee. *Journal of Clinical Oncology*. 2005 Nov;23(31):7951–7957.

12.

Nataliya Zhukova. Subgroup-Specific Prognostic Implications of TP53 Mutation in Medulloblastoma. *Journal of Clinical Oncology* [Internet]. American Society of Clinical

Oncology; 2013;31(23). Available from:  
<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4878050/>

13.

Stone TJ, Jacques TS. Medulloblastoma: selecting children for reduced treatment. *Neuropathology and Applied Neurobiology*. 2015 Feb;41(2):106–108.

14.

Schwalbe EC, Lindsey JC, Nakjang S, Crosier S, Smith AJ, Hicks D, Rafiee G, Hill RM, Iliasova A, Stone T, Pizer B, Michalski A, Joshi A, Wharton SB, Jacques TS, Bailey S, Williamson D, Clifford SC. Novel molecular subgroups for clinical classification and outcome prediction in childhood medulloblastoma: a cohort study. *The Lancet Oncology*. 2017 Jul;18(7):958–971.

15.

Pfister S, Remke M, Castoldi M, Bai AHC, Muckenthaler MU, Kulozik A, von Deimling A, Pscherer A, Lichter P, Korshunov A. Novel genomic amplification targeting the microRNA cluster at 19q13.42 in a pediatric embryonal tumor with abundant neuropil and true rosettes. *Acta Neuropathologica*. 2009 Apr;117(4):457–464.

16.

Korshunov, Andrey Sturm, Dominik Ryzhova, Marina Hovestadt, Volker Gessi, Marco. Embryonal tumor with abundant neuropil and true rosettes (ETANTR), ependymoblastoma, and medulloepithelioma share molecular similarity and comprise a single clinicopathological entity. *Acta Neuropathologica* [Internet]. 128(8):279–89. Available from: <https://search.proquest.com/docview/1545765655?OpenUrlRefId=info:xri/sid:primo&aaccountid=14511>

17.

Schwalbe EdC, Hayden JT, Rogers HA, Miller S, Lindsey JC, Hill RM, Nicholson SL, Kilday JP, Adamowicz-Brice M, Storer L, Jacques TS, Robson K, Lowe J, Williamson D, Grundy RG, Bailey S, Clifford SC. Histologically defined central nervous system primitive neuro-ectodermal tumours (CNS-PNETs) display heterogeneous DNA methylation profiles and show relationships to other paediatric brain tumour types. *Acta Neuropathologica*. 2013 Dec;126(6):943–946.

18.

Sturm D, Orr BA, Toprak UH, Hovestadt V, Jones DTW, Capper D, Sill M, Buchhalter I, Northcott PA, Leis I, Ryzhova M, Koelsche C, Pfaff E, Allen SJ, Balasubramanian G, Worst BC, Pajtler KW, Brabetz S, Johann PD, Sahm F, Reimand J, Mackay A, Carvalho DM, Remke M, Phillips JJ, Perry A, Cowdrey C, Drissi R, Fouladi M, Giangaspero F, Łastowska M, Grajkowska W, Scheurlen W, Pietsch T, Hagel C, Gojo J, Lötsch D, Berger W, Slavc I, Haberler C, Jouvett A, Holm S, Hofer S, Prinz M, Keohane C, Fried I, Mawrin C, Scheie D, Mobley BC, Schniederjan MJ. New Brain Tumor Entities Emerge from Molecular Classification of CNS-PNETs. *Cell*. 2016 Feb;164(5):1060–1072.

19.

Kirsti Sirkiä, Ulla M. Saarinen-Pihkala, Liisa Hovi, Hannu Sariola. Autopsy in children with cancer who die while in terminal care. *Medical and Pediatric Oncology* [Internet]. Wiley-Blackwell; 1998;30(5):284–289. Available from: [https://onlinelibrary.wiley.com/doi/abs/10.1002/\(SICI\)1096-911X\(199805\)30:5%3C284::AID-MPO4%3E3.0.CO;2-B](https://onlinelibrary.wiley.com/doi/abs/10.1002/(SICI)1096-911X(199805)30:5%3C284::AID-MPO4%3E3.0.CO;2-B)

20.

Buckner T, Blatt J, Smith SV. The Autopsy in Pediatrics and Pediatric Oncology: A Single-Institution Experience. *Pediatric and Developmental Pathology*. 2006 Sep;9(5):374–380.

21.

Bleggi-Torres LF, de Noronha L, Schneider Gugelmin E, Martins Sebastião AP, Werner B, Marques Maggio E, Queiroz Telles JE, Martins Collaço L. Accuracy of the smear technique in the cytological diagnosis of 650 lesions of the central nervous system. *Diagnostic Cytopathology*. 2001 Apr;24(4):293–295.

22.

Hill RM, Kuijper S, Lindsey JC, Petrie K, Schwalbe EC, Barker K, Boulton JKR, Williamson D, Ahmad Z, Hallsworth A, Ryan SL, Poon E, Robinson SP, Ruddle R, Raynaud FI, Howell L, Kwok C, Joshi A, Nicholson SL, Crosier S, Ellison DW, Wharton SB, Robson K, Michalski A, Hargrave D, Jacques TS, Pizer B, Bailey S, Swartling FJ, Weiss WA, Chesler L, Clifford SC. Combined MYC and P53 Defects Emerge at Medulloblastoma Relapse and Define Rapidly Progressive, Therapeutically Targetable Disease. *Cancer Cell*. 2015 Jan;27(1):72–84.

23.

Blümcke I, Aronica E, Becker A, Capper D, Coras R, Honavar M, Jacques TS, Kobow K, Miyata H, Mühlebner A, Pimentel J, Söylemezoğlu F, Thom M. Low-grade epilepsy-associated neuroepithelial tumours — the 2016 WHO classification. *Nature Reviews Neurology*. 2016 Dec;12(12):732–740.

24.

Chhabda S, Carney O, D'Arco F, Jacques TS, Mankad K. The 2016 World Health Organization Classification of tumours of the Central Nervous System: what the paediatric neuroradiologist needs to know. *Quantitative Imaging in Medicine and Surgery*. 2016 Oct;6(5):486–489.

25.

Schwartzentruber J, Korshunov A, Liu XY, Jones DTW, Pfaff E, Jacob K, Sturm D, Fontebasso AM, Quang DAK, Tönjes M, Hovestadt V, Albrecht S, Kool M, Nantel A, Konermann C, Lindroth A, Jäger N, Rausch T, Ryzhova M, Korbel JO, Hielscher T, Hauser P, Garami M, Klekner A, Bognar L, Ebinger M, Schuhmann MU, Scheurlen W, Pekrun A, Frühwald MC, Roggendorf W, Kramm C, Dürken M, Atkinson J, Lepage P, Montpetit A, Zakrzewska M, Zakrzewski K, Liberski PP, Dong Z, Siegel P, Kulozik AE, Zapatka M, Guha A, Malkin D, Felsberg J, Reifenberger G, von Deimling A, Ichimura K, Collins VP. Driver mutations in histone H3.3 and chromatin remodelling genes in paediatric glioblastoma. *Nature*. 2012 Feb;482(7384):226–231.

26.

Sturm D, Witt H, Hovestadt V, Khuong-Quang DA, Jones DTW, Konermann C, Pfaff E, Tönjes M, Sill M, Bender S, Kool M, Zapatka M, Becker N, Zucknick M, Hielscher T, Liu XY, Fontebasso AM, Ryzhova M, Albrecht S, Jacob K, Wolter M, Ebinger M, Schuhmann MU, van Meter T, Frühwald MC, Hauch H, Pekrun A, Radlwimmer B, Niehues T, von Komorowski G, Dürken M, Kulozik AE, Madden J, Donson A, Foreman NK, Drissi R, Fouladi M, Scheurlen W, von Deimling A, Monoranu C, Roggendorf W, Herold-Mende C, Unterberg A, Kramm CM, Felsberg J, Hartmann C, Wiestler B, Wick W, Milde T, Witt O. Hotspot Mutations in H3F3A and IDH1 Define Distinct Epigenetic and Biological Subgroups of Glioblastoma. *Cancer Cell*. 2012 Oct;22(4):425–437.

27.

Lewis PW, Muller MM, Koletsky MS, Cordero F, Lin S, Banaszynski LA, Garcia BA, Muir TW,

Becher OJ, Allis CD. Inhibition of PRC2 Activity by a Gain-of-Function H3 Mutation Found in Pediatric Glioblastoma. *Science*. 2013 May 17;340(6134):857–861.

28.

Bender S, Tang Y, Lindroth AM, Hovestadt V, Jones DTW, Kool M, Zapatka M, Northcott PA, Sturm D, Wang W, Radlwimmer B, Højfeldt JW, Truffaux N, Castel D, Schubert S, Ryzhova M, Şeker-Cin H, Gronych J, Johann PD, Stark S, Meyer J, Milde T, Schuhmann M, Ebinger M, Monoranu CM, Ponnuswami A, Chen S, Jones C, Witt O, Collins VP, von Deimling A, Jabado N, Puget S, Grill J, Helin K, Korshunov A, Lichter P, Monje M, Plass C, Cho YJ, Pfister SM. Reduced H3K27me3 and DNA Hypomethylation Are Major Drivers of Gene Expression in K27M Mutant Pediatric High-Grade Gliomas. *Cancer Cell*. 2013 Nov;24(5):660–672.

29.

Hashizume R, Andor N, Ihara Y, Lerner R, Gan H, Chen X, Fang D, Huang X, Tom MW, Ngo V, Solomon D, Mueller S, Paris PL, Zhang Z, Petritsch C, Gupta N, Waldman TA, James CD. Pharmacologic inhibition of histone demethylation as a therapy for pediatric brainstem glioma. *Nature Medicine*. 2014 Dec;20(12):1394–1396.

30.

Pathania M, De Jay N, Maestro N, Harutyunyan AS, Nitarska J, Pahlavan P, Henderson S, Mikael LG, Richard-Londt A, Zhang Y, Costa JR, Hébert S, Khazaei S, Ibrahim NS, Herrero J, Riccio A, Albrecht S, Ketteler R, Brandner S, Kleinman CL, Jabado N, Salomoni P. H3.3K27M Cooperates with Trp53 Loss and PDGFRA Gain in Mouse Embryonic Neural Progenitor Cells to Induce Invasive High-Grade Gliomas. *Cancer Cell*. 2017 Nov;32(5):684–700.e9.

31.

Larson JD, Kasper LH, Paugh BS, Jin H, Wu G, Kwon CH, Fan Y, Shaw TI, Silveira AB, Qu C, Xu R, Zhu X, Zhang J, Russell HR, Peters JL, Finkelstein D, Xu B, Lin T, Tinkle CL, Patay Z, Onar-Thomas A, Pounds SB, McKinnon PJ, Ellison DW, Zhang J, Baker SJ. Histone H3.3 K27M Accelerates Spontaneous Brainstem Glioma and Drives Restricted Changes in Bivalent Gene Expression. *Cancer Cell*. 2018 Dec;

32.

Martinez-Barbera JP, Andoniadou CL. Concise Review: Paracrine Role of Stem Cells in Pituitary Tumors: A Focus on Adamantinomatous Craniopharyngioma. *STEM CELLS*. 2016 Feb;34(2):268–276.

33.

Gump JM, Donson AM, Birks DK, Amani VM, Rao KK, Griesinger AM, Kleinschmidt-DeMasters BK, Johnston JM, Anderson RCE, Rosenfeld A, Handler M, Gore L, Foreman N, Hankinson TC. Identification of targets for rational pharmacological therapy in childhood craniopharyngioma. *Acta Neuropathologica Communications*. 2015 Dec;3(1).

34.

Martinez-Barbera JP, Buslei R. Adamantinomatous craniopharyngioma: pathology, molecular genetics and mouse models. *Journal of Pediatric Endocrinology and Metabolism*. 2015 Jan 1;28(1-2).

35.

Goschzik T, Gessi M, Dreschmann V, Gebhardt U, Wang L, Yamaguchi S, Wheeler DA, Lauriola L, Lau CC, Müller HL, Pietsch T. Genomic Alterations of Adamantinomatous and Papillary Craniopharyngioma. *Journal of Neuropathology & Experimental Neurology*. 2017 Jan 9;

36.

Azarova AM, Gautam G, George RE. Emerging importance of ALK in neuroblastoma. *Seminars in Cancer Biology*. 2011 Oct;21(4):267-275.

37.

Beierle EA. MYCN, Neuroblastoma and Focal Adhesion Kinase (FAK). *Frontiers in bioscience (Elite edition)* [Internet]. NIH Public Access; 3. Available from: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3171213/>

38.

Bell E, Chen L, Liu T, Marshall GM, Lunec J, Tweddle DA. MYCN oncoprotein targets and their therapeutic potential. *Cancer Letters*. 2010 Jul;293(2):144-157.

39.

Berry T, Luther W, Bhatnagar N, Jamin Y, Poon E, Sanda T, Pei D, Sharma B, Vetharoy WR, Hallsworth A, Ahmad Z, Barker K, Moreau L, Webber H, Wang W, Liu Q, Perez-Atayde A, Rodig S, Cheung NK, Raynaud F, Hallberg B, Robinson SP, Gray NS, Pearson ADJ, Eccles SA, Chesler L, George RE. The ALKF1174L Mutation Potentiates the Oncogenic Activity of MYCN in Neuroblastoma. *Cancer Cell*. 2012 Jul;22(1):117–130.

40.

Brodeur GM. Neuroblastoma: biological insights into a clinical enigma. *Nature Reviews Cancer*. 2003 Mar;3(3):203–216.

41.

Brodeur GM, Bagatell R. Mechanisms of neuroblastoma regression. *Nature Reviews Clinical Oncology*. 2014 Dec;11(12):704–713.

42.

Garrett M, Brodeur, Robert C, Seeger, Manfred Schwab, Harold E. Varmus and J. Michael Bishop. Amplification of N-myc in Untreated Human Neuroblastomas Correlates with Advanced Disease Stage. *Science* [Internet]. American Association for the Advancement of Science; 1984;224(4653):1121–1124. Available from: <http://www.jstor.org/stable/1692440>

43.

Burkhart CA, Cheng AJ, Madafiglio J, Kavallaris M, Mili M, Marshall GM, Weiss WA, Khachigian LM, Norris MD, Haber M. Effects of MYCN Antisense Oligonucleotide Administration on Tumorigenesis in a Murine Model of Neuroblastoma. *JNCI Journal of the National Cancer Institute*. 2003 Sep 17;95(18):1394–1403.

44.

Chen L, Iraci N, Gherardi S, Gamble LD, Wood KM, Perini G, Lunec J, Tweddle DA. p53 Is a Direct Transcriptional Target of MYCN in Neuroblastoma. *Cancer Research*. 2010 Feb 15;70(4):1377–1388.

45.

Cossu I, Bottoni G, Loi M, Emionite L, Bartolini A, Di Paolo D, Brignole C, Piaggio F, Perri P, Sacchi A, Curnis F, Gagliani MC, Bruno S, Marini C, Gori A, Longhi R, Murgia D, Sementa AR, Cilli M, Tacchetti C, Corti A, Sambuceti G, Marchiò S, Ponzoni M, Pastorino F. Neuroblastoma-targeted nanocarriers improve drug delivery and penetration, delay tumor growth and abrogate metastatic diffusion. *Biomaterials*. 2015 Nov;68:89-99.

46.

Evans AE, Baum E, Chard R. Do infants with stage IV-S neuroblastoma need treatment? *Archives of Disease in Childhood*. 1981 Apr 1;56(4):271-274.

47.

Guglielmi L, Cinnella C, Nardella M, Maresca G, Valentini A, Mercanti D, Felsani A, D'Agnano I. MYCN gene expression is required for the onset of the differentiation programme in neuroblastoma cells. *Cell Death & Disease*. 2014 Feb;5(2):e1081-e1081.

48.

Huang M, Weiss WA. Neuroblastoma and MYCN. *Cold Spring Harbor Perspectives in Medicine*. 2013 Oct 1;3(10):a014415-a014415.

49.

Huber K, Kalcheim C, Unsicker K. The development of the chromaffin cell lineage from the neural crest. *Autonomic Neuroscience*. 2009 Nov;151(1):10-16.

50.

Liu Z, Thiele CJ. ALK and MYCN: When Two Oncogenes Are Better than One. *Cancer Cell*. 2012 Mar;21(3):325-326.

51.

Marabelle A, Sapin V, Rousseau R, Periquet B, Demeocq F, Kanold J. Hypercalcemia and 13-  
-retinoic acid in post-consolidation therapy of neuroblastoma.

Pediatric Blood & Cancer. 2009 Feb;52(2):280–283.

52.

Matthay KK, Villablanca JG, Seeger RC, Stram DO, Harris RE, Ramsay NK, Swift P, Shimada H, Black CT, Brodeur GM, Gerbing RB, Reynolds CP. Treatment of High-Risk Neuroblastoma with Intensive Chemotherapy, Radiotherapy, Autologous Bone Marrow Transplantation, and 13-*cis*-Retinoic Acid. *New England Journal of Medicine*. 1999 Oct 14;341(16):1165–1173.

53.

Mossé YP, Laudenslager M, Longo L, Cole KA, Wood A, Attiyeh EF, Laquaglia MJ, Sennett R, Lynch JE, Perri P, Laureys G, Speleman F, Kim C, Hou C, Hakonarson H, Torkamani A, Schork NJ, Brodeur GM, Tonini GP, Rappaport E, Devoto M, Maris JM. Identification of ALK as a major familial neuroblastoma predisposition gene. *Nature*. 2008 Oct;455(7215):930–935.

54.

Pastorino F, Marimpietri D, Brignole C, Paolo D, Pagnan G, Daga A, Piccardi F, Cilli M, Allen T, Ponzoni M. Ligand-Targeted Liposomal Therapies of Neuroblastoma. *Current Medicinal Chemistry*. 2007 Dec 1;14(29):3070–3078.

55.

Qiao J, Paul P, Lee S, Qiao L, Josifi E, Tiao JR, Chung DH. PI3K/AKT and ERK regulate retinoic acid-induced neuroblastoma cellular differentiation. *Biochemical and Biophysical Research Communications*. 2012 Aug;424(3):421–426.

56.

Reynolds CP, Matthay KK, Villablanca JG, Maurer BJ. Retinoid therapy of high-risk neuroblastoma. *Cancer Letters*. 2003 Jul;197(1-2):185–192.

57.

Schwab M. MYCN in neuronal tumours. *Cancer Letters*. 2004 Feb 20;204(2):179–187.

58.

Sidell N. Retinoic Acid-Induced Growth Inhibition and Morphologic Differentiation of Human Neuroblastoma Cells In Vitro. *JNCI: Journal of the National Cancer Institute*. 1982;

59.

Vogelstein B, Papadopoulos N, Velculescu VE, Zhou S, Diaz LA, Kinzler KW. Cancer Genome Landscapes. *Science*. 2013 Mar 29;339(6127):1546-1558.

60.

Wright JH. NEUROCYTOMA OR NEUROBLASTOMA, A KIND OF TUMOR NOT GENERALLY RECOGNIZED. *The Journal of Experimental Medicine*. The Rockefeller University Press; 1910;12(4).

61.

Yang, LiqunKe, Xiao-XueXuan, FanTan, JuanHou, Jianbing. PHOX2B Is Associated with Neuroblastoma Cell Differentiation. *Cancer Biotherapy & Radiopharmaceuticals* [Internet]. 31:44-51. Available from:  
[https://search.proquest.com/docview/1776665507?rfr\\_id=info%3Axri%2Fsid%3Aprim](https://search.proquest.com/docview/1776665507?rfr_id=info%3Axri%2Fsid%3Aprim)

62.

Zhu S, Lee JS, Guo F, Shin J, Perez-Atayde AR, Kutok JL, Rodig SJ, Neuberg DS, Helman D, Feng H, Stewart RA, Wang W, George RE, Kanki JP, Look AT. Activated ALK Collaborates with MYCN in Neuroblastoma Pathogenesis. *Cancer Cell*. 2012 Mar;21(3):362-373.

63.

Hasle H, Niemeyer CM. Advances in the prognostication and management of advanced MDS in children. *British Journal of Haematology*. 2011 Jul;154(2):185-195.

64.

Niemeyer CM, Kratz CP. Paediatric myelodysplastic syndromes and juvenile

myelomonocytic leukaemia: molecular classification and treatment options. *British Journal of Haematology*. 2008 Mar;140(6):610–624.

65.

Wegman-Ostrosky T, Savage SA. The genomics of inherited bone marrow failure: from mechanism to the clinic. *British Journal of Haematology*. 2017 May;177(4):526–542.

66.

Strebhardt K, Ullrich A. Paul Ehrlich's magic bullet concept: 100 years of progress. *Nature Reviews Cancer*. 2008 Jun;8(6):473–480.

67.

Koebel CM, Vermi W, Swann JB, Zerafa N, Rodig SJ, Old LJ, Smyth MJ, Schreiber RD. Adaptive immunity maintains occult cancer in an equilibrium state. *Nature*. 2007 Dec;450(7171):903–907.

68.

Mackall CL, Merchant MS, Fry TJ. Immune-based therapies for childhood cancer. *Nature Reviews Clinical Oncology*. 2014 Dec;11(12):693–703.

69.

Klebanoff CA, Rosenberg SA, Restifo NP. Prospects for gene-engineered T cell immunotherapy for solid cancers. *Nature Medicine*. 2016 Jan;22(1):26–36.

70.

*Nature Reviews Immunology*. 2012;12(4). Available from:  
<https://www.nature.com/nri/volumes/12/issues/4>

71.

Majzner RG, Heitzeneder S, Mackall CL. Harnessing the Immunotherapy Revolution for the

Treatment of Childhood Cancers. *Cancer Cell*. 2017 Apr;31(4):476–485.

72.

Greaves MF, Wiemels J. Origins of chromosome translocations in childhood leukaemia. *Nature Reviews Cancer*. 2003 Sep;3(9):639–649.

73.

Zelent A, Greaves M, Enver T. Role of the TEL-AML1 fusion gene in the molecular pathogenesis of childhood acute lymphoblastic leukaemia. *Oncogene*. 2004 May;23(24):4275–4283.

74.

Slany RK. The molecular mechanics of mixed lineage leukemia. *Oncogene*. 2016 Oct;35(40):5215–5223.

75.

Milne TA. Mouse models of MLL leukemia: recapitulating the human disease. *Blood*. 2017 Apr 20;129(16):2217–2223.

76.

Sadelain M, Rivière I, Riddell S. Therapeutic T cell engineering. *Nature*. 2017 May 24;545(7655):423–431.

77.

Johnson LA, June CH. Driving gene-engineered T cell immunotherapy of cancer. *Cell Research*. 2017 Jan;27(1):38–58.

78.

Yong CSM, Dardalhon V, Devaud C, Taylor N, Darcy PK, Kershaw MH. CAR T-cell therapy of solid tumors. *Immunology and Cell Biology*. 2017 Apr;95(4):356–363.

79.

Fisher J, Abramowski P, Wisidagamage Don ND, Flutter B, Capsomidis A, Cheung GWK, Gustafsson K, Anderson J. Avoidance of On-Target Off-Tumor Activation Using a Co-stimulation-Only Chimeric Antigen Receptor. *Molecular Therapy*. 2017 May;25(5):1234–1247.

80.

Brown CE, Alizadeh D, Starr R, Weng L, Wagner JR, Naranjo A, Ostberg JR, Blanchard MS, Kilpatrick J, Simpson J, Kurien A, Priceman SJ, Wang X, Harshbarger TL, D'Apuzzo M, Ressler JA, Jensen MC, Barish ME, Chen M, Portnow J, Forman SJ, Badie B. Regression of Glioblastoma after Chimeric Antigen Receptor T-Cell Therapy. *New England Journal of Medicine*. 2016 Dec 29;375(26):2561–2569.

81.

Morsut L, Roybal KT, Xiong X, Gordley RM, Coyle SM, Thomson M, Lim WA. Engineering Customized Cell Sensing and Response Behaviors Using Synthetic Notch Receptors. *Cell*. 2016 Feb;164(4):780–791.

82.

Rasaiyaah J, Georgiadis C, Preece R, Mock U, Qasim W. TCR $\alpha\beta$ /CD3 disruption enables CD3-specific antileukemic T cell immunotherapy. *JCI Insight*. 2018 Jul 12;3(13).

83.

Ghorashian S, Amrolia P, Veys P. Open access? Widening access to chimeric antigen receptor (CAR) therapy for ALL. *Experimental Hematology*. 2018 Oct;66:5–16.

84.

Qasim W, Zhan H, Samarasinghe S, Adams S, Amrolia P, Stafford S, Butler K, Rivat C, Wright G, Somana K, Ghorashian S, Pinner D, Ahsan G, Gilmour K, Lucchini G, Inglott S, Mifsud W, Chiesa R, Peggs KS, Chan L, Farzaneh F, Thrasher AJ, Vora A, Pule M, Veys P. Molecular remission of infant B-ALL after infusion of universal TALEN gene-edited CAR T cells. *Science Translational Medicine*. 2017 Jan 25;9(374).

85.

Hubert CG, Rivera M, Spangler LC, Wu Q, Mack SC, Prager BC, Couce M, McLendon RE, Sloan AE, Rich JN. A Three-Dimensional Organoid Culture System Derived from Human Glioblastomas Recapitulates the Hypoxic Gradients and Cancer Stem Cell Heterogeneity of Tumors Found. *Cancer Research*. 2016 Apr 15;76(8):2465–2477.

86.

Richmond A, Su Y. Mouse xenograft models vs GEM models for human cancer therapeutics. *Disease Models and Mechanisms*. 2008 Sep 1;1(2–3):78–82.

87.

Phoenix TN, Patmore DM, Boop S, Boulos N, Jacus MO, Patel YT, Roussel MF, Finkelstein D, Goumnerova L, Perreault S, Wadhwa E, Cho YJ, Stewart CF, Gilbertson RJ. Medulloblastoma Genotype Dictates Blood Brain Barrier Phenotype. *Cancer Cell*. 2016 Apr;29(4):508–522.

88.

Lu B, Green B, Farr J, Lopes F, Van Raay T. Wnt Drug Discovery: Weaving Through the Screens, Patents and Clinical Trials. *Cancers*. 2016 Sep 1;8(9).

89.

Northcott PA, Korshunov A, Pfister SM, Taylor MD. The clinical implications of medulloblastoma subgroups. *Nature Reviews Neurology*. 2012 Jun;8(6):340–351.

90.

Niklison-Chirou MV, Erngren I, Engskog M, Haglöf J, Picard D, Remke M, McPolin PHR, Selby M, Williamson D, Clifford SC, Michod D, Hadjiandreou M, Arvidsson T, Pettersson C, Melino G, Marino S. TAp73 is a marker of glutamine addiction in medulloblastoma. *Genes & Development*. 2017 Sep 1;31(17):1738–1753.

91.

Hourigan CS, Karp JE. Minimal residual disease in acute myeloid leukaemia. *Nature Reviews Clinical Oncology*. 2013 Aug;10(8):460–471.

92.

Kotrova M, Trka J, Kneba M, Brüggemann M. Is Next-Generation Sequencing the way to go for Residual Disease Monitoring in Acute Lymphoblastic Leukemia? *Molecular Diagnosis & Therapy*. 2017 Oct;21(5):481–492.

93.

O'Connor D, Enshaei A, Bartram J, Hancock J, Harrison CJ, Hough R, Samarasinghe S, Schwab C, Vora A, Wade R, Moppett J, Moorman AV, Goulden N. Genotype-Specific Minimal Residual Disease Interpretation Improves Stratification in Pediatric Acute Lymphoblastic Leukemia. *Journal of Clinical Oncology*. 2018 Jan;36(1):34–43.

94.

Vora A, Goulden N, Wade R, Mitchell C, Hancock J, Hough R, Rowntree C, Richards S. Treatment reduction for children and young adults with low-risk acute lymphoblastic leukaemia defined by minimal residual disease (UKALL 2003): a randomised controlled trial. *The Lancet Oncology*. 2013 Mar;14(3):199–209.

95.

Vora A, Goulden N, Mitchell C, Hancock J, Hough R, Rowntree C, Moorman AV, Wade R. Augmented post-remission therapy for a minimal residual disease-defined high-risk subgroup of children and young people with clinical standard-risk and intermediate-risk acute lymphoblastic leukaemia (UKALL 2003): a randomised controlled trial. *The Lancet Oncology*. 2014 Jul;15(8):809–818.

96.

Hunger SP, Mullighan CG. Acute Lymphoblastic Leukemia in Children. *New England Journal of Medicine*. 2015 Oct 15;373(16):1541–1552.

97.

O'Connor D, Enshaei A, Bartram J, Hancock J, Harrison CJ, Hough R, Samarasinghe S,

Schwab C, Vora A, Wade R, Moppett J, Moorman AV, Goulden N. Genotype-Specific Minimal Residual Disease Interpretation Improves Stratification in Pediatric Acute Lymphoblastic Leukemia. *Journal of Clinical Oncology*. 2018 Jan;36(1):34–43.