

10. REFERENCES

BLAST search www.ncbi.nlm.nih.gov/BLAST

Baylin S.B., Makos M., Wu J.J., Yen R.W.C., de Bustros A., Vertino P., Nelkin B.D. (1991): Abnormal patterns of DNA methylation in human neoplasia: potential consequences for tumor progression. *Cancer Cells*, 3:383-390.

Bergmann L., Miethling C., Maurer U., Brieger J., Karakas T., Weidmann E., Hoelzer D. (1997): High levels of Wilms' tumor gene (WT1) mRNA in acute myeloid leukemias are associated with a worse long term outcome. *Blood*, 90:1217-25.

Bickmore W.A., Oghene K., Little M.H., Seawright A., van Heyningen V., Hastie N.D. (1992): Modulation of DNA binding specificity by alternative splicing of Wilms tumor WT1 gene transcript. *Science*, 257:235-237.

Brieger J., Weidmann E., Fenchel K., Mitrou P.S., Hoelzer D., Bergmann L. (1994): The expression of the Wilms' tumor gene in acute myelocytic leukemia as a possible marker for leukemic blast cells. *Leukemia*, 8: 2138-2143.

Call K.M., Glaser T., Ito C.Y., Buckler A.J., Pelletier J., Haber D.A., Rose E.A., Kral A., Yeger H., Lewis W.H., et al. (1990): Isolation and characterization of zinc finger polypeptide gene at the human chromosome 11 Wilms' tumor locus. *Cell*, 60: 509-20.

Chen C.M., Chen H.L., Hsiao T.H.C., Hsiao A.H.A., Shi H., Brock G.J.R., Wei S.H., Caldwell C.W., Yan P.S., Huang T.H.M. (2003): Methylation Target Array for rapid analysis of CpG Island hypermethylation in Multiple Tissue Genomes. *Am. J. Path.*, 163:37-45.

Chi J.S., Chang H.Y., Wang N.N., Chang D.S., Dunphy N., Brown P.O. (2003): Genomewide view of gene silencing by small interfering RNAs. *PNAS*, 100:6343-6346.

Dahl E., Koseki H., Balling R. (1997): Pax genes in organogenesis. *BioEssays*, 19:755-765.

Davies J.A., Ladomery M., Hohenstein P., Michael L., Shafe A., Spraggon L., Hastie N. (2004): Developement of an siRNA based method for repressing specific genes in renal

organ culture and its use to show that the WT1 tumor suppressor is required for nephron differentiation. *Human Molecular Genetics*, 13:235-246.

Dehbi M., Ghahremani M., Lechner M., Dressler G., Pelletier J. (1996): The Paired-box transcription factor, PAX2, positively modulates expression of the Wilms' tumor suppressor gene (WT1). *Oncogene*, 13: 447-453.

Dehbi M., Pelletier J. (1996): PAX8 mediated activation of the WT1 tumor suppressor gene. *EMBO*, 15:4297-4306.

Deuel T.F., Guan L.S., Wang Z.Y. (1999): Wilms' tumor gene product WT1 arrests macrophage differentiation of HL60 cells through its zinc – finger domain. *Bio. Bio. Res. Comm.*, 254:192-196.

Dey B.R., Sukhatme V.P., Roberts A.B., Sporn M.B., Rauscher F.J. 3rd, Kim S.J., (1994): Repression of the transforming growth factor- β 1 gene by the Wilms' tumor suppressor WT1 gene product. *Mol. Endocrinol.*, 8: 595-602.

Discenza T.M., He S., Lee T.H., Chu L.L., Bolon B., Goodyer P., Eccles M., Pelletier J. (2003): WT1 is a modifier of the Pax2 mutant phenotype: cooperation and interaction between WT1 and Pax2. *Oncogene*, 22:8145-8155.

Discenza MT, Vaz D, Hassell JA, Pelletier J. (2004): Activation of the WT1 tumor suppressor gene promoter by Pea3. *FEBS Letters*, 560:183-191.

Donze O. and Picard D. (2002): RNA interference in mammalian cells using siRNAs synthesized with T7 RNA polymerase. *Nuc. Acid Res.*, 30:e46.

Dressler G.R., Douglas D.C. (1992): Pax-2 is a DNA binding protein expressed in embryonic kidney and Wilms tumor. *PNAS*, 89:1179-1183.

Drummond I.A., Madden S.L., Rohwer-Nutter P., Bell G.I., Sukhatme V.P., Rauscher F.J. 3rd (1992): Repression of the insulin-like growth factor II gene by the Wilms' tumor suppressor WT1. *Science*, 257: 674-678.

Elbashir S.M., Harboth J., Lendeckel W., Yalcin A., Weber K., Tuschl T. (2001): Duplexes of 21 nucleotide RNAs mediate RNA interference in cultured mammalian cells. *Nature*, 411:494-498.

Elbashir S.M., Lendeckel W., Tuschl T. (2001): RNA interference is mediated by 21- and 22-nucleotide RNAs. *Genes Dev.*, 15:188-200.

Elbashir S.M., Martinez J., Patkaniowska A., Lendeckel W., Tuschl T. (2001): Functional anatomy of siRNAs for mediating efficient RNAi in *Drosophila melanogaster* embryo lysate. *EMBO*, 20:6877-6888.

Faisst S., Meyer S. (1992): Compilation of vertebrate encoded transcription factors. *Nucleic Acid Research*, 20:3-26.

Fire A., Xu S., Montgomery M.K., Kostas S.A., Driver S.E., Mello C.C. (1998): Potent and specific genetic interference by double-stranded RNA in *Caenorhabditis elegans*. *Nature*, 391:806-809.

Fraizer G.C., Wu Y.J., Hewitt S.M., Maity T., Ton C.C.T., Huff V., Saunders G.F. (1994): Transcriptional regulation of the human Wilms' tumor gene (WT1). *J. Biol. Chem.* 269:8892-2900.

Gaiger A., Reese V., Disis M.L., Cheever M. (2000): Immunity to WT1 in the animal model and in patients with acute myeloid leukemia. *Blood*, 96:1480-1489.

Gessler M., Poustka A., Cavenee W., Neve R.L., Orkin S.H., Burns G.A. (1990): Homozygous deletion in Wilms tumours of a zinc-finger gene identified by chromosome jumping. *Nature*, 343:774-778.

Gao L., Bellantuono I., Elsasser A., Marley S., Gordon M.Y., Goldman J.M., Stauss H.J. (2000): Selective elimination of leukemic CD34+ progenitor cells by cytotoxic T lymphocytes specific for WT1. *Blood*, 95:2198-2203.

Haber D.A., Sohn R.L., Buckler A.J., Pelletier J., Call K.M., Housman D.E. (1991): Alternative splicing and genomic structure of the Wilms tumor gene WT1. *PNAS*, 88:9618-9622.

Hamilton A.J., Baulcombe D.C. (1999): A species of small antisense RNA in posttranscriptional gene silencing in plants. *Science*, 286:950-952.

Hammond S.M., Bernstein E., Beach D., Hannon G.J. (2000): An RNA-directed nuclease mediates post-transcriptional gene silencing in *Drosophila* cells. *Nature*, 404:293-296.

Hewitt S.M., Hamada S., McDonnell T.J., Rauscher F.J. 3rd, Saunders G.F.(1995): Regulation of the proto-oncogenes bcl-2 and c-myc by the Wilms' tumor suppressor gene WT1. *Cancer Res.*, 55: 5386-5389.

Hofmann W., Royer H.D., Drechsler M., Schneider S., Royer-Pokora B. (1993): Characterization of the transcriptionally regulatory region of the human WT1 gene. *Oncogene*, 8: 3123-3132.

Hosen N., Sonoda Y., Oji Y., Kimura T., Minamiguchi H., Tamaki H., Kwakami M., Asada M., Kanato K., Motomura M., et al. (2002): Very low frequencies of human normal CD34+ haematopoietic progenitor cells express the Wilms' tumor gene WT1 at levels similar to those in leukemia cells. *Brit. J. Haematology*, 116:409-420.

Inoue K., Sugiyama H., Ogawa H., Nakagawa M., Yamagami T., Miwa H., Kita K., Hiraoka A., Masaoka T., Nasu K., et al. (1994): WT1 as a new prognostic factor and a new marker for the detection of minimal residual disease in acute leukemia. *Blood*, 84: 3071-9.

Inoue K., Ogawa H., Sonoda Y., Kimura T., Sakabe H., Oka Y., Miyake S., Tamaki H., Oji Y., Yamagami T., Tatekawa T., Soma T., Kishimoto T., Sugiyama H.(1997): Aberrant overexpression of the Wilms tumor gene (WT1) in human leukemia. *Blood*, 89: 1405-12.

Inoue K., Tamaki H., Ogawa H., Oka Y., Soma T., Tatekawa T., Oji Y., Tsuboi A., Kim E.U., Kawakami M., Akiyama T., Kishimoto T., Sugiyama H. (1998): Wilms' tumor gene (WT1) competes with differentiation – inducing signal in hematopoietic progenitor cells. *Blood*, 91:2969-2976.

Jarvis, R.A., Pallota, V., Byrom, M., Brown, D., Ford, L.P. (2002): siRNA-mediated gene silencing in mammalian cells, *Ambion Technical Resources Poster*.

Jackson A.L., Bartz S.R., Schelter J., Kobayashi S.V., Burchard J., Mao M., Li B., Cavet G., Linsley P.S. (2003): Expression profiling reveals off target gene regulation by RNAi. *Nature Biotechnology*, 21:635-637.

Jones P.A., Tatki D. (2001): The role of DNA methylation in mammalian epigenetics. *Science*, 293:1068-1070.

Kleymenova E.V., Yuan X., LaBate M.E., Walker C.L. (1998): Identification of a tumor specific methylation site in the Wilms tumor suppressor gene. *Oncogene*, 16:713-720.

Larsson S.H., Charlieu J.P., Miyagawa K., Engelkamp D., Rassoulzadegan M., Ross A., Cuzin F., van Heyningen V., Hastie N.D. (1995): Subnuclear localization of WT1 in splicing or transcription factor domains is regulated by alternative splicing. *Cell*, 81:391-401.

Laux D.E., Curran E.M., Welshorns W.V., Lubahn D.B., Huang T.H.M. (1999): Hypermethylation of the Wilms' tumor suppressor gene CpG island in human breast carcinomas. *Breast Can. Res. Treat.*, 56:35-43.

Liu Z.J. and Maekawa M. (2003): Polymerase chain reaction-based methods of DNA methylation analysis. *Anal. Bioch.*, 317:259-265.

Lin R.Y., Kubo A., Keller G.M., Davies T.F. (2003): Commiting embryonic stem cells to differentiate into thyrocyte-like cells in vitro. *Endocrinology*, 144:2644-2649.

Loeb D.M., Evron E., Patel C.B., Sharma P.M., Niranjan B., Lakjaya B., Weitzman S.A., Korz D., Sukumar S. (2001): Wilms' Tumor suppressor gene (WT1) is expressed in primary breast tumors despite tumor specific promoter methylation. *Cancer Research*, 61:921-925.

Mailander V., Scheibenbogen C., Thiel E., Letsch A., Blau I.W., Keilholz U. (2004): Complete remission in patient with recurrent acute myeloid leukemia induced by vaccination with WT1 peptide in the absence of hematological and renal toxicity. *Leukemia*, 18:165-166.

Malik K., Salpekar A., Hancock A., Moorwood K., Jackson S., Charles A., Brown K.W. (2000): Identification of differential methylation of the WT1 antisense regulatory region and relaxation of imprinting in Wilms' Tumor. *Cancer Research* 60:2356-2360.

Mares J., Kriz V., Weinhausel A., Vodickova S., Kodet R., Haas O.A., Sedlacek Z., Goetz P. (2001): Methylation changes in promoter and enhancer regions of the WT1 gene in Wilms' tumors. *Cancer Let.*, 166:165-171.

McManus M.T., Haines B.B., Dillon C.P., Whitehurst C.E., van Paris L., Chen J., Sharp P.A. (2002): Small interfering RNA-mediated gene silencing in T lymphocytes. *J. Immunology* 169: 5754-60.

Menssen H.D., Renkl H.J., Rodeck U., Maurer J., Notter M., Schwartz S., Reinhardt R., Thiel E. (1995): Presence of Wilms' tumor gene (wt1) transcripts and WT1 nuclear protein in the majority of human acute leukemia. *Leukemia*, 9: 1060-1067.

Merika M., Orkin S.H. (1995): Functional synergy and physical interaction of the erythroid transcription factor GATA1 with the Kruppel family proteins Sp1 and EKLF. *Mol. Cell. Biol.*, 15:2437-2447.

Milligan, J., Uhlenbeck, O.C. (1987): Synthesis of small RNAs using T7 RNA polymerase. *Methods in Enzymology*, 180: 51-62.

Mitsuya K., Sui H., Meguro M., Kugoh H., Jinno Y., Niikawa N., Oshimura M. (1997): Paternal expression of WT1 in human fibroblasts and lymphocytes. *Human Molecular Genetics*, 6:2243-3346.

Miwa H., Beran M., Saunders G.F. (1992): Expression of the Wilms' tumor gene (WT1) in human leukemias. *Leukemia*, 6: 405-409.

Miyagi T., Ahuja H., Kubota T., Kubonishi I., Koeffler H.P., Miyoshi I. (1993): Expression of the candidate Wilms' tumor gene, WT1, in human leukemia cells. *Leukemia*, 7: 970-977.

Miyagishi M., Hayashi M., Taira K., (2003): Comparison of the suppressive effects of antisense oligonucleotides and siRNAs directed against the same targets in mammalian cells. *Antisense and Nucleic Acid Drug Dev.*, 13:1-7.

Miyoshi Y., Ando A., Egawa C., Taguchi T., Tamaki Y., Tamaki H., Sugiyama H. Noguchi S. (2002): High expression of Wilms' tumor suppressor gene predicts poor prognosis in breast cancer patients *Clin. Cancer Res.*, 8:1167-1171.

Morris J.F., Madden S.L., Tournay O.E., Cook D.M., Sukhatme V.P., Rauscher F.J., 3rd (1991): Characterization of the zinc finger protein encoded by the WT1 Wilms' tumor locus. *Oncogene*, 12:2339-48.

Mouthon M.A, Bernard O., Mitjavila M.T., Romeo P.H., Vainchenker W., Mathieu-Mahul D., (1993): Expression of tal-1 and GATA-binding proteins during human hematopoiesis. *Blood*, 81:647-655.

Muratovska A., Zhou C., Shuji He., Goodyer P., Eccles M.R. (2003): Paired-Box genes are frequently expressed in cancer and often required for cancer cell survival. *Oncogene*, 22:7989-7997.

Nass S.J., Herman G.J., Gabrielson E., Iversen P.W., Parl F.F., Davidson N.E. et al. (2000): Aberrant methylation of the estrogen receptor and E-cadherin 5' CpG island increases with malignant progression in human breast cancer. *Cancer Res.*, 60:4346-4348.

Oji Y., Ogawa H., Tamaki H., Oka Y., Tsuboi A., Kim E.H., Soma T., Tatekawa T., Kawakami M., Asada M., Kishimoto T., Sugiyama H. (1999): Expression of the Wilms' tumor gene WT1 in solid tumors and its involvement in tumor cell growth. *Jpn. J. Cancer Res.*, 90: 194-204.

Oji Y., Miyoshi S., Maeda H., Hayashi S., Tamaki H., Nakatsuka S.I., Yao M., Takahashi E., et al., (2002): Overexpression of the Wilms' tumor gene WT1 in *de novo* lung cancers. *Int. J. Cancer*, 100:297-303.

Oji Y., Miyoshi S., Koga S., Nakano Y., Ando A., Nakatsuka S.I., Ikeba A. et al. (2003): Overexpression of the Wilms' tumor gene WT1 in primary thyroid cancer. *Cancer Sci.*, 94:606-611.

Oka Y., Elisseeva O.A., Tsuboi A., Ogawa H., Tamaki H., Li H., Oji Y., Kim E.H. et al. (2000): Human cytotoxic T-lymphocyte responses for peptides of the wild-type Wilms' tumor gene (WT1) product. *Immunogenetics*, 51:99-107.

Osaka M., Koami K., Sugiyama T. (1997): WT1 contributes to leukemogenesis: expression patterns in 7,12-Dimethylbenz(a)anthracene (DMBA)- induced leukemia. *Int. J. Cancer*, 72:696-699.

Pavletich N.M. and Pabo C.O. (1991): Zinc finger-DNA recognition: crystal structure of a Zif268-DNA complex at 2.1 Å. *Science*, 252: 809-817.

Palmer R.E., Kotsianti A., Cadman B., Boyd T., Gerald W., Haber D.A. (2001): WT1 regulates the expression of the major glomerular podocytes membrane protein Podocalyxin. *Curr. Biol.*, 11:1805-1809.

Patmasiriwat P., Fraizer G., Kantarjian H., Saunders G.F. (1999): WT1 expression in myelodysplastic syndrome and acute leukemia. *Leukemia*, 13:891-900.

Ryan G., Steele-Perkins V., Morris J.F., Rauscher F.J. 3rd, Dressler G.R. (1995): Repression of Pax-2 by WT1 during normal kidney development. *Development*, 121: 867-875.

Scheibenbogen C., Letsch A., Thiel E., Schmittel A., Mailaender V., Baerwolf S., Nagorsen D., Keilholz U. (2002): CD8 T - cell responses to Wilms' tumor gene product WT1 and proteinase 3 in patients with acute myeloid leukemia. *Blood*, 100:2132-2137.

Shivdasani R.A., Fujiwara Y., McDevitt M.A., Orkin S.H. (1997): A lineage selective knockout establishes the critical role of transcription factor GATA1 in megakaryocyte growth and platelet development. *EMBO*, 16:3965-3973.

Siehl J.M., Thiel E., Heufelder K., Snarski E., Schwartz S., Mailänder V., Keilholz U. (2003): Possible regulation of Wilms' Tumour Gene 1 (WT1) expression by the paired box genes PAX2 and PAX8 and by the haematopoietic transcription factor GATA-1 in human acute myeloid leukemias, *Br. J. Hematology*, 123:235-242.

Silberstein G.B., Dressler G.R., Van Horn K. (2002): Expression of the PAX2 oncogene in human breast cancer and its role in progesterone-dependent mammary growth. *Oncogene*, 21:1009-1016.

Singer-Sam J., Yang T.P., Mori N., Tanguay R.L., LeBon J.M., Flores J.C., Riggs A.D. (1989) Nucleic Acid Methylation. Alan R. Liss, New York, Vol. 128, 185-298.

Siu L.L.P., Chan J.K.C., Wong K.F., Choy C., Kwong Y.L. (2003): Aberrant promoter CpG methylation as a molecular marker for disease monitoring in natural killer cell lymphomas. *Br. J. Haemat.*, 122:70-77.

Sposi N.M., Zon L.I., Care A., Valtieri M., Testa U., Gabbianelli M., Mariani G., Bottero L., Mather C., Orkin S.H. et al. (1992): Cycle dependent initiation and lineage-dependent abrogation of GATA1 expression in pure differentiating hematopoietic progenitors. *PNAS*, 89:6353-6357.

Stayner C.K., Cunliffe H.E., Ward T.A., Eccles M.R. (1998): Cloning and characterization of the human PAX2 promoter. *J. Biol. Chem.*, 273:25472-25470.

Svedberg H., Chylicki K., Baldetorp B., Rauscher F.J. 3rd, Gullberg V. (1998): Constitutive expression of the Wilms' tumor gene (WT1) in the leukemic cell line U937 blocks parts of the differentiation program. *Oncogene*, 19:925-932.

Takayashi S., Onodera K., Motohashi H., Suwabe N., Hayashi N., Yanai N., Nabesima Y., Yamamoto M (1997): Arrest in primitive erythroid cell development caused by promoter specific disruption of the GATA1 gene. *J. Biol. Chem.*, 272:12611-12615.

Tuschl T., The siRNA user guide 2001. www.mpibpc.gwdg.de/ateilungen/100/105/sirna.html

Wang Z.Y., Qiu Q.Q., Enger K.T., Deuel T.F. (1993): A second transcriptionally active DNA-binding site for the Wilms tumor gene product WT1. *PNAS*, 90:8896-8900.

Walters D.K., Jelinek D.F. (2002): The effectiveness of double stranded short inhibitory RNAs (siRNA) may depend on the method of transfection. *Antisense Nuc. Acid Dev.*, 12:411-418.

Weiss M.J., Keller G., Orkin S.H. (1994): Novel insights into erythroid development revealed through in vitro differentiation of GATA1 embryonic stem cells. *Genes Dev.* 8:1184-1197.

Weiss M.J., Orkin S.H. (1995): GATA transcription factors: key regulators of hematopoiesis. *Exp. Hematol.*, 23:99-107.

Weiss M.J., Orkin S.H. (1995): Transcription factor GATA1 permits survival and maturation of erythroid precursors by preventing apoptosis. *PNAS*, 92:9623-9627.

Werner H., Re G.G., Drummond I.A., Sukhatme V.P., Rauscher F.J. 3rd, Sens D.A., Garvin A.J., LeRoith D., Roberts C.T. (1993): Increased expression of the insulin-like growth factor I

receptor gene, IGF1R, in Wilms' tumor is correlated with modulation of IGF1R promoter activity by the WT1 tumor gene product. *PNAS*, 90: 5828-32.

Whyatt D., Lindeboom F., Karis A., Ferreira R., Milot E., Hendriks R., de Brujin M., Langeveld A., Gribnau J., Gosveld F., Philipsen S. (200) *An intrinsic but cell-nonautonomous defect in GATA1 overexpressing mouse erythroid cells.* *Nature*, 406:519-524.

Worm J., Aggerholm A., Guldberg P. (2001) *In-tube DNA methylation profiling by fluorescence melting curve analysis.* *Clin. Chem.*, 47:1183-1189.

Wu X., Wang Y., Pei X. (1999) Effect of WT1 antisense oligonucleotide on proliferation and apoptosis of human leukemia cells. *Zhonghua Xue Ye Xue Za Zhi*. 20: 631-3.

Wu Y. J., Fraizer G. C., Saunders G.F. (1995) GATA-1transactivates the WT1 hematopoietic specific enhancer. *J. Biol. Chem.*, 270: 5944-5949.

Yamagami T., Sugiyama H., Inoue K., Ogawa H., Tatekawa T., Hirata M., Kudoh T., Akiyama T., Murakami A., Maekawa T. (1996) Growth inhibition of human leukemic cells by WT1 (Wilms' tumor gene) antisense oligonucleotides: implications for the involvement of WT1 in leukemogenesis. *Blood*, 87: 2878-84.

Zannini M., Francis-Lang H., Plachov D., Di Lauro R. (1992) Pax8, a paired domain containing protein, binds to a sequence overlapping the recognition site of a homeodomain and activates transcription from two thyroid specific promoters. *Mol. Cell. Biol.* 12:4230-4241.

Zapata-Benavides P., Tuna M., Lopez-Berestein G., Tari A.M. (2002) Downregulation of Wilms' tumor 1 protein inhibits breast cancer proliferation. *Bioch. Biophy. Res. Communication*, 295: 784-790.

Zon L.I., Yamaguchi Y., Yee K., Albee E.A., Kimura A., Bennett J.C., Orkin S.H., Ackerman S.J. (1993) Expression of mRNA for the GATA binding proteins in human eosinophils and basophils: potential role in gene transcription. *Blood*, 81:3234-3241.

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