

Professor Adrian Thrasher

Selected Publications

1. Ali, R.R., G. Sarra, C. Stephens, M. de Alwis, J. W.B. Bainbridge, P. M. Munro, S. Fauser, M. B. Reichel, C. Kinnon, D. M. Hunt, S. S. Bhattacharya, Thrasher, A.J. Restoration of photoreceptor ultrastructure and function in retinal degeneration slow mice by gene therapy. *Nature Genet.* 25: 306-310, 2000.
2. Thrasher, A.J. WASp in immune-system organization and function. *Nat Rev Immunol.* 2(9):635-46, 2002.
3. Cavazzana-Calvo, M., Thrasher, A.J., Mavilio, F. The future of gene therapy. *Nature*, 427: 779-781, 2004.
4. Gaspar, H.B., Parsley, K., Howe, S., King, D., Gilmour, K., Sinclair, J., Brouns, G., Schmidt, M., Von Kalle, C., Barington, T., Jacobsen, M., Christensen, H.O., AlGhonaim, A., White, H., Smith, J., Levinsky, R., Ali, R.R., Kinnon, C., Thrasher, A.J. Gene therapy of X-linked severe combined immunodeficiency using a pseudotyped gammaretroviral vector. *Lancet* 364: 2181-2187, 2004.
5. Yanez, R., Balaggan, K., MacNeil, A., Smith, A.J., Buch, P., Howe, S., Duran, Y., Maclaren, R.E., Anderson, P., Kinnon, C., Ali, R.R., Thrasher, A.J. Effective gene therapy with non-integrating lentiviral vectors *Nature Medicine* 12: 348-353, 2006
6. Ott, M., Schmidt M, Schwarzwaelder K, Stein S, Siler U, Koehl U, Glimm H, Kuhicke K, Schilz A, Kunkel H, Naundorf S, Brinkmann A, Deichmann A, Fischer M, Ball C, Pilz I, Dunbar C, Du Y, Jenkins NA, Copeland NG, Luthi U, Hassan M, Thrasher AJ, Hoelzer D, von Kalle C, Seger R, & Grez M. Correction of X-linked chronic granulomatous disease by gene therapy, augmented by insertional activation of MDS1-EV11, PRDM16 or SETBP1. *Nature Medicine* 12(4), 401-409, 2006.
7. Gaspar, H.B., Bjorkegren E., Parsley, K., Gilmour, K., King, D., Sinclair J., Adams S., Fairbanks L.D., Gaspar J., Henderson L., Davies, E.G., Veys, P., Kinnon C., Thrasher, A.J. Successful reconstitution of immune function in ADA-SCID by stem cell gene therapy following cessation of PEG-ADA and use of mild pre-conditioning. *Mol. Ther* 14(4): 505-513, 2006.
8. Thrasher A; Gaspar H; Baum C; Modlich U; Schambach A; Candotti F; Otsu M; Sorrentino B; Scobie L; Cameron E; Blyth K; Neil J; Hacein-Bey Abina S; Cavazzana-Calvo M; Fischer A. Gene Therapy: SCID-X1 transgene leukaemogenicity. *Nature*, 443, E5-E6, 2006
9. Schwarzwaelder, K., Howe, S.J., Schmidt, M., Brugman, M., Deichmann, A., Glimm, H., Schmidt, S., Prinz, C., Wissler, M., King, D.J.S., Zhang, F., Parsley, K.L., Gilmour, K.C., Sinclair, J., Bayford, J., Peraj, R., de Ridder, D., Pike-Ovezet, K., Wagemaker, G., Kinnon, C., Abel, U., Staal, F., Gaspar, H.B., Thrasher, A.J. (joint senior author), von Kalle, C. Gammaretrovirus-mediated correction of SCID-X1 is associated with skewed vector integration site distribution in vivo. *J.Clin.Inv*, 117 (8), 2241-2249, 2007
10. Moulding, D.A., Blundell, M.P., Spiller, D.G., White, M.R.H., Cory, G.O., Calle, Y., Kempski, H., Sinclair, J., Ancliff, P.J., Kinnon, C., Jones, G.E., Thrasher, A.J. Unregulated actin polymerization by WASp causes defects of mitosis and cytokinesis in X-linked neutropenia. *J.Exp.Med*, 204(9): 2213-2224, 2007.
11. Bainbridge, J., Smith, A., Barker, S., Scott, R., Henderson, R., Balaggan, K., Fitzke, F., Holder, G., Stockman, A., Tyler, N., Petersen-Jones, S., Bhattacharya, S., Thrasher, A.J., Carter, B., Rubin, G., Moore, A., Ali, R. Improvement of Visual Function following RPE65 Gene Therapy for Leber Congenital Amaurosis. *N Eng. J Med.* In press, 2008.