



## Fabrizia Urbinati

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### Bio

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#### ACADEMIC APPOINTMENTS

- Senior Research Scientist, Innovative Medicines Accelerator (IMA)

### Publications

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#### PUBLICATIONS

- **Development of a Novel AAV Gene Therapy for the Treatment of Dilated Cardiomyopathy**  
Chiola, S., Sen, D., Kay, M., Xu, J., Champe, M., Urbinati, F., Karakikes, I.  
CELL PRESS.2025
- **Identification and validation of an optimal stuffer sequence for AAV gene therapy applications using AI/ML**  
Sen, D., Xu, J., Champe, M., Urbinati, F., Maslova, A., Ketz, N., Sriraman, A., Nipko, J., Elliott, A.  
CELL PRESS.2025
- **High-throughput transcriptome analyses from ASPIRO, a phase 1/2/3 study of gene replacement therapy for X-linked myotubular myopathy.** *American journal of human genetics*  
Andreoletti, G., Romano, O., Chou, H., Sefid-Dashti, M. J., Grilli, A., Chen, C., Lakshman, N., Purushothaman, P., Varfaj, F., Mavilio, F., Biciato, S., Urbinati, F.  
2023
- **Development of Hematopoietic Stem Cell-Engineered Invariant Natural Killer T Cell Therapy for Cancer** *CELL STEM CELL*  
Zhu, Y., Smith, D. J., Zhou, Y., Li, Y., Yu, J., Lee, D., Wang, Y., Di Biase, S., Wang, X., Hardoy, C., Ku, J., Tsao, T., Lin, et al  
2019; 25 (4): 542+
- **Pre-clinical Development of a Lentiviral Vector Expressing the Anti-sickling beta AS3 Globin for Gene Therapy for Sickle Cell Disease** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*  
Poletti, V., Urbinati, F., Charrier, S., Corre, G., Hollis, R. P., Fernandez, B., Martin, S., Rothe, M., Schambach, A., Kohn, D. B., Mavilio, F.  
2018; 11: 167-179
- **Gene Therapy for Sickle Cell Disease: A Lentiviral Vector Comparison Study** *HUMAN GENE THERAPY*  
Urbinati, F., Fernandez, B., Masiuk, K. E., Poletti, V., Hollis, R. P., Koziol, C., Kaufman, M. L., Brown, D., Mavilio, F., Kohn, D. B.  
2018; 29 (10): 1153-1166
- **Improving Gene Therapy Efficiency through the Enrichment of Human Hematopoietic Stem Cells** *MOLECULAR THERAPY*  
Masiuk, K. E., Brown, D., Laborada, J., Hollis, R. P., Urbinati, F., Kohn, D. B.  
2017; 25 (9): 2163-2175
- **Preclinical studies for a phase 1 clinical trial of autologous hematopoietic stem cell gene therapy for sickle cell disease** *CYTOTHERAPY*  
Urbinati, F., Wherley, J., Geiger, S., Fernandez, B., Kaufman, M. L., Cooper, A., Romero, Z., Marchioni, F., Reeves, L., Read, E., Nowicki, B., Grassman, E., Viswanathan, et al  
2017; 19 (9): 1096-1112

- **Selection-free genome editing of the sickle mutation in human adult hematopoietic stem/progenitor cells** *SCIENCE TRANSLATIONAL MEDICINE*  
DeWitt, M. A., Magis, W., Bray, N. L., Wang, T., Berman, J. R., Urbinati, F., Heo, S., Mitros, T., Munoz, D. P., Boffelli, D., Kohn, D. B., Walters, M. C., Carroll, et al  
2016; 8 (360): 360ra134
- **CRISPR/Cas9-Mediated Correction of the Sickle Mutation in Human CD34(+) cells** *MOLECULAR THERAPY*  
Hoban, M. D., Lumaquin, D., Kuo, C. Y., Romero, Z., Long, J., Ho, M., Young, C. S., Mojadidi, M., Fitz-Gibbon, S., Cooper, A. R., Lill, G. R., Urbinati, F., Campo-Fernandez, et al  
2016; 24 (9): 1561-1569
- **Reactivating Fetal Hemoglobin Expression in Human Adult Erythroblasts Through BCL11A Knockdown Using Targeted Endonucleases** *MOLECULAR THERAPY-NUCLEIC ACIDS*  
Bjurstrom, C. F., Mojadidi, M., Phillips, J., Kuo, C., Lai, S., Lill, G. R., Cooper, A., Kaufman, M., Urbinati, F., Wang, X., Hollis, R. P., Kohn, D. B.  
2016; 5: e351
- **Potentially therapeutic levels of anti-sickling globin gene expression following lentivirus-mediated gene transfer in sickle cell disease bone marrow CD34(+) cells** *EXPERIMENTAL HEMATOLOGY*  
Urbinati, F., Hargrove, P. W., Geiger, S., Romero, Z., Wherley, J., Kaufman, M. L., Hollis, R. P., Chambers, C. B., Persons, D. A., Kohn, D. B., Wilber, A.  
2015; 43 (5): 346-351
- **Enrichment of Human Hematopoietic Stem/Progenitor Cells Facilitates Transduction for Stem Cell Gene Therapy** *STEM CELLS*  
Baldwin, K., Urbinati, F., Romero, Z., Campo-Fernandez, B., Kaufman, M. L., Cooper, A. R., Masiuk, K., Hollis, R. P., Kohn, D. B.  
2015; 33 (5): 1532-1542
- **Correction of the sickle cell disease mutation in human hematopoietic stem/progenitor cells** *BLOOD*  
Hoban, M. D., Cost, G. J., Mendel, M. C., Romero, Z., Kaufman, M. L., Joglekar, A. V., Ho, M., Lumaquin, D., Gray, D., Lill, G. R., Cooper, A. R., Urbinati, F., Senadheera, et al  
2015; 125 (17): 2597-2604
- **The human ankyrin 1 promoter insulator sustains gene expression in a beta-globin lentiviral vector in hematopoietic stem cells** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*  
Romero, Z., Campo-Fernandez, B., Wherley, J., Kaufman, M. L., Urbinati, F., Cooper, A. R., Hoban, M. D., Baldwin, K. M., Lumaquin, D., Wang, X., Senadheera, S., Hollis, R. P., Kohn, et al  
2015; 2: 15012
- **beta-globin gene transfer to human bone marrow for sickle cell disease** *JOURNAL OF CLINICAL INVESTIGATION*  
Romero, Z., Urbinati, F., Geiger, S., Cooper, A. R., Wherley, J., Kaufman, M. L., Hollis, R. P., de Assin, R., Senadheera, S., Sahagian, A., Jin, X., Gellis, A., Wang, et al  
2013; 123 (8): 3317-3330
- **Genotoxic Potential of Lineage-specific Lentivirus Vectors Carrying the beta-Globin Locus Control Region** *MOLECULAR THERAPY*  
Arumugam, P. I., Higashimoto, T., Urbinati, F., Modlich, U., Nestheide, S., Xia, P., Fox, C., Corsinotti, A., Baum, C., Malik, P.  
2009; 17 (11): 1929-1937
- **The 3' Region of the Chicken Hypersensitive Site-4 Insulator Has Properties Similar to Its Core and Is Required for Full Insulator Activity** *PLOS ONE*  
Arumugam, P. I., Urbinati, F., Velu, C. S., Higashimoto, T., Grimes, H., Malik, P.  
2009; 4 (9): e6995
- **Mechanism of Reduction in Titers From Lentivirus Vectors Carrying Large Inserts in the 3' LTR** *MOLECULAR THERAPY*  
Urbinati, F., Arumugam, P., Higashimoto, T., Perumbeti, A., Mitts, K., Xia, P., Malik, P.  
2009; 17 (9): 1527-1536
- **A novel human gamma-globin gene vector for genetic correction of sickle cell anemia in a humanized sickle mouse model: critical determinants for successful correction** *BLOOD*  
Perumbeti, A., Higashimoto, T., Urbinati, F., Franco, R., Meiselman, H. J., Witte, D., Malik, P.  
2009; 114 (6): 1174-1185
- **The woodchuck hepatitis virus post-transcriptional regulatory element reduces readthrough transcription from retroviral vectors** *GENE THERAPY*

Higashimoto, T., Urbinati, F., Perumbeti, A., Jiang, G., Zarzuela, A., Chang, L., Kohn, D. B., Malik, P.  
2007; 14 (17): 1298-1304

- **Multilineage hematopoietic reconstitution without clonal selection in ADA-SCID patients treated with stem cell gene therapy** *JOURNAL OF CLINICAL INVESTIGATION*  
Aiuti, A., Cassani, B., Andolfi, G., Mirolo, M., Biasco, L., Recchia, A., Urbinati, F., Valacca, C., Scaramuzza, S., Aker, M., Slavin, S., Cazzola, M., Sartori, et al  
2007; 117 (8): 2233-2240
- **Pathophysiology and therapy for haemoglobinopathies. Part II: thalassaemias.** *Expert reviews in molecular medicine*  
Urbinati, F., Madigan, C., Malik, P.  
2006; 8 (10): 1-26
- **Retroviral vector integration deregulates gene expression but has no consequence on the biology and function of transplanted T cells** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*  
Recchia, A., Bonini, C., Magnani, Z., Urbinati, F., Sartori, D., Muraro, S., Tagliafico, E., Bondanza, A., Stanghellini, M. T., Bernardi, M., Pescarollo, A., Ciceri, F., Bordignon, et al  
2006; 103 (5): 1457-1462
- **Competitive engraftment of hematopoietic stem cells genetically modified with a truncated erythropoietin receptor** *HUMAN GENE THERAPY*  
Urbinati, F., Lotti, F., Facchini, G., Montanari, M., Ferrari, G., Mavilio, F., Grande, A.  
2005; 16 (5): 594-608
- **WBSCR14, a gene mapping to the Williams-Beuren syndrome deleted region, is a new member of the Mix transcription factor network** *HUMAN MOLECULAR GENETICS*  
Cairo, S., Merla, G., Urbinati, F., Ballabio, A., Reymond, A.  
2001; 10 (6): 617-627