

## Citable abstracts

1. García-Layana A, Thumann G, Groll J: **Age macular degeneration: etiology, prevention, individualized therapies, cell therapy, and tissue engineering.** J Ophthalmol 2014; 2014:287893
2. Fernández-Robredo P, Sancho A, Johnen S, Recalde S, Gama N, Thumann G, Groll J, García-Layana A: **Current treatment limitations in age-related macular degeneration and future approaches based on cell therapy and tissue engineering.** J Ophthalmol 2014; 2014:510285
3. Kropp M, Chronopoulos A, Conti A, Harmening N, Pouillot S, Izsvák Z, Marie C, Scherman D, Thumann G: **Safety studies for cell-based gene therapy (TargetAMD project): in vivo exclusion of tumorigenicity and proof of cell product quality.** Human Gene Therapy 2016 ; 27(11): A120-A120.
4. Thumann G, Kropp M, Harmening N on behalf of the entire TargetAMD Consortium: **Transposon-based, targeted ex vivo gene therapy to treat age-related macular degeneration: the TargetAMD project.** Human Gene Therapy 2016 ; 27(11): A123-A123.
5. Harmening N, Sealy G, Johnen S, Kropp M, Ronchetti M, Aranda P, Marie C, Scherman D, Izsvák Z, Thumann G: **Translation of GLP-grade electroporation of primary pigment epithelial cells to GMP-grade GTMP manufacturing for clinical use.** Human Gene Therapy 2016 ; 27(11): A108-A108.
6. Kropp M, et al.: Safety studies for cell-based gene therapy (TargetAMD project): in vivo exclusion of tumorigenicity and proof of cell product quality. Human Gene Therapy 2016 ; 27(11): A108-A108.
7. Diarra S, et al.: pFAR4/SB100X-mediated PEDF gene delivery in iris pigment epithelial cells isolated from patients' iridectomies mimicking a therapeutic approach for the treatment of neovascular AMD. Human Gene Therapy 2016 ; 27(11): A108-A108.
8. Johnen S, et al.: Developments in the pFAR4/SB100X-mediated PEDF gene delivery in primary human pigment epithelial cells for the treatment of neovascular AMD. Human Gene Therapy 2016 ; 27(11): A108-A108.
9. Ivics Z: The Sleeping Beauty Transposon System for Molecular Therapy. ASGCT 2016
10. Harmening N, Sealy G, Kropp M, Marie C, Scherman D, Ronchetti M, Aranda P, Fernandez V, Johnen S, Izsvák Z, Thumann G: **Optimized Non-Viral Transfection of human RPE and IPE cells used for a Gene-Therapeutic Treatment of neovascular AMD.** Investigative Ophthalmology & Visual Science 2016 ; 57(12).
11. Prat-Souteyrand C, Harmening N, Kropp M, Sealy G, Izsvák Z, Scherman D, Marie C, Johnen S, Thumann G: **Human PEDF optimized gene for transposon-based gene therapy to treat age-related macular degeneration.** Investigative Ophthalmology & Visual Science 2016; 57(12).
12. Kropp M, Chronopoulos A, Conti A, Harmening N, Pouillot S, Marie C, Scherman D, Izsvák Z, Thumann G: **Results of a biodistribution study of Venus transfected pigment epithelial cells transplanted subretinally in rabbits.** Investigative Ophthalmology & Visual Science 2016 ; 57(12).
13. Hernandez M, et al.: **Efficacy of Rat Primary Cells transfected with Pigment Epithelium-Derived Factor using the Sleeping Beauty Transposon system in choroidal neovascularization.** Investigative Ophthalmology & Visual Science 2016 ; 57(12).
14. Ivics Z: **Engineering the genome with the Sleeping Beauty transposon system. Conference on transposition and genome engineering 2015.**
15. Dobias A, et al.: **Lack of Tumorigenic Potential of PEDF-Transfected Primary Human Pigment Epithelial Cells in Soft Agar Assays.** DOG 2015
16. Thumann G: **The TargetAMD project – Using Free of antibiotic resistance gene (pFAR4) miniplasmids for a Sleeping Beauty (SB100X) mediated gene therapy to treat neovascular Age-Related Macular Degeneration (nAMD).** Human Gene Therapy 26(10): A81-A81
17. Kropp M, Tian S, Harmening N, Johnen S, Scherman D, Marie C, Izsvák Z, Thumann G. (2015). **Over-expression of PEDF by PEDF-transfected primary pigment epithelial cells does not induce tumorigenicity.** Human Gene Therapy 26(10): A81-A81.
18. Prat-Souteyrand C, Tobalem S, Harmening N, Kropp M, Johnen S, Scherman D, Marie C, Izsvák Z, Thumann G. (2015). **"Stable genomic integration of PEDF in primary pigment epithelial cells transfected with the Sleeping Beauty transposon system to treat age-related macular degeneration (AMD)."** Human Gene Therapy 26(10): A80-A80.
19. Diarra S, et al.: **pFAR4/SB100X-mediated PEDF gene delivery in primary murine pigment epithelial cells.** Human Gene Therapy 26(10): A81-A81.
20. Ivics Z: **The sleeping beauty transposon system and its applications in molecular medicine.** FEBS3+ 2015.
21. Ivics Z: **The Sleeping Beauty transposon system for genetic engineering in stem cells.** GSCN 2015.

22. Scherman D, Pastor M, Marie C, Harmening N, Kropp M, Johnen S, Izsvak Z, Thumann G. (2015). **The use of mini-plasmids free of antibiotic resistance markers for a gene therapeutical approach to treat AMD.** 1st world Congress on Electroporation. Portoroz, Slovenia.
23. Ivics Z: **Target site selection by sleeping beauty.** 6<sup>th</sup> FASEB conferene on mobile DNA in mammalian genomes 2015.
24. Kropp M, Harmening N, Johnen S, Tian S, Scherman D, Marie C, Izsvák Z, Thumann G. (2015). **pFAR4 miniplasmids in combination with the Sleeping Beauty transposon system allow efficient transfection of freshly isolated iris pigment epithelial cells.** *Invest. Ophthalmol. Vis. Sci.*, 56(7):2316.
25. Thumann G, Marie c, Izsvák Z, Scherman D, Walter P, Johnen S: **The Use of Plasmids Free of Antibiotic Resistance Markers for Safe and Efficient *Sleeping Beauty*-mediated PEDF Gene Delivery into Primary Pigment Epithelial Cells.** *Investigative Ophthalmology & Visual Science* 2015; 56(7).
26. Harmening N, Kropp M, Johnen S, Marie C, Scherman D, Izsvák Z, Thumann G: **The use of mini-plasmids free of antibiotic resistance markers for a gene therapeutical approach to treat AMD.** *Investigative Ophthalmology & Visual Science* 2015; 56(7).
27. Hernandez M, et al.: **Biodistribution of Rat Primary Cells transfected with SB100X transposon-mediated Pigment Epithelium-Derived Factor (PEDF) gene in Brown Norway rats after subretinal injection.** *Investigative Ophthalmology & Visual Science* 2015; 56(7).
28. Dobias A, et al.: **Analysis of Potential Tumorigenicity of PEDF-Transfected Primary Human Retinal Pigment Epithelial Cells Using Soft Agar Assay.** *Investigative Ophthalmology & Visual Science* 2015; 56(7).
29. Kropp M, Harmening N, Johnen S, Tian S, Scherman D, Marie C, Izsvák Z, Thumann G: **Transfection of freshly isolated pigment epithelial cells with pFAR4 miniplasmids using the *Sleeping Beauty* (SB100X) transposon system.** *Human Gene Therapy* 2014; 25(11): A106-A106.
30. Garcia Garcia L, et al.: **Use of Plasmids Free of Antibiotic Resistance Markers in Non-Viral Transfection of Pigment Epithelium-Derived Factor in Rat Primary Cells.** EVER 2014.
31. Marie C, et al.: **The merging of the antibiotic-free pFAR4 miniplasmids with the *Sleeping Beauty* transposon system mediates higher transgene delivery in human cells.** ASGCT 2014.
32. Thumann G: **Safety strategies in transposon-based plasmids for gene therapy.** Jules Gonin 2014.
33. Thumann G: **The Use of Plasmids Free of Antibiotic Resistance Markers for Safe and Efficient *Sleeping Beauty*-Mediated PEDF Gene Delivery into Primary Pigment Epithelial Cells.** ARVO 2014.
34. Dobias A, et al.: **Non-Viral *Sleeping Beauty*-Mediated Transfection of Retinal Pigment Epithelial Cells.** ARVO 2014
35. Marie C: **pFAR4, a vector devoid of Antibiotic Resistance marker, promotes high and/or sustained expression levels in cultured cells and organs.** Minicircle & DNA Vector Conference 2014.
36. Thumann G: ***Ex vivo* Gene Therapy for the Treatment of Age-Related Macular Degeneration.** Retina 2014.
37. Harmening N, et al.: **Non-viral transfection of primary cells using plasmids free of antibiotic resistance markers.** SFTCG 2014.
38. Ivics Z: **Transposons for molecular medicine.** DGGT 2014
39. Scherman D: **History of plasmid size reduction.** Minicircle & DNA Vector Conference 2014.
40. Scherman D: **New plasmid and transposon for personalized Medicine and Age-related Macular Degeneration.** BIT's World Gene Convention 2013.
41. Marie C: **The merging of pFAR4 miniplasmids with the *Sleeping Beauty* transposon system mediates higher transgene delivery in human cells.** *Human Gene Therapy* 24(12): A59-A59.
42. Thumann G: **Transposon-Based, Targeted *Ex Vivo* Gene Therapy to Treat Age-Related Macular Degeneration (AMD).** *Human Gene Therapy* 24(12): A59-A59.
43. Harmening N, Johnen S, Izsvák Z, Kropp M, Diarra S, Thumann G. (2013). **Use of *Sleeping Beauty* Transposase mRNA for Safe and Efficient Gene Delivery in Pigment Epithelial Cells.** *Human Gene Therapy* 24(12): A59-A59.
44. Johnen S, et al.: **Efficient *Sleeping Beauty*-mediated transposition of primary pigment epithelial cells with PEDF delivered by plasmids free of antibiotic resistance markers (pFARs).** *Human Gene Therapy* 24(12): A59-A59.
45. Ivics Z: **The *Sleeping Beauty* transposon system for molecular medicine.** *Human Gene Therapy* 24(12): A59-A59.
46. Marie C, et al.: **pFARs, plasmids free of antibiotic resistance markers, display high-level transgene expression in vitro and in vivo and high efficiency as transposon/transposase vectors,** CTGE 2013
47. Ivics Z and Izsvák Z: **Transposon-Mediated PEDF Gene Delivery into Primary Pigment Epithelial Cells for the Treatment of Retinal Degenerative Diseases.** CTGE 2013
48. Johnen S, et al.: **Transposon-mediated PEDF gene delivery into primary pigment epithelial cells for the treatment of retinal degenerative diseases.** CTGE 2013

49. Johnen S, et al.: **Transposon-Mediated PEDF Gene Delivery into Primary Pigment Epithelial Cells for the Treatment of Retinal Degenerative Diseases.** CTGE 2013
50. Binder S, et al.: **Human retinal pigment epithelial transplantation – are we too slow?** SOE 2013
51. Thumann G, et al.: **Cell-based therapy for the treatment of retinal degeneration.** SOE 2013
52. Scherman D, et al.: **Improved efficiency biosafe plasmid for sustained expression various tissues in von-viral gene therapy.** ASGCT 2013
53. Binder S, et al.: **Human RPE transplantation – are we too slow?** DOC 2013
54. Thumann G, et al.: **Cell-based therapy for the treatment of retinal degeneration.** DOC 2013
55. Ivics Z, et al.: **Transposons for molecular medicine.** 5<sup>th</sup> FASEB conference on mammalian mobile DNA 2013
56. Johnen S, et al.: **Stable electroporation-mediated gene transfer into primary cells as treatment for age-related macular degeneration.** electrochemotherapy, 2<sup>nd</sup> international users' meeting 2013
57. Scherman D, *et al.*: **New development in non viral gene delivery and therapy.** ESGCT 2012.