Curriculum Vitae
Nicolas Wein, PhD

Center for Gene Therapy
Nationwide Children's Hospital
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Education

Years	Degree	Institution
2011 – 2016	Postdoctoral Scientist	Center for Gene Therapy, Nationwide Childrens Hospital, Columbus, OH, USA (AP Gene Therapy; Neuromuscular Diseases)
2006 – 2010	Ph.D.	Medical Genetics and Functional Genomics, La Timone Medical School, Marseille, France (AP Human genomics and Genetics; Neuromuscular Diseases)
2004 – 2006	M.S.	Medical Genetics and Functional Genomics, La Timone Medical School, Marseille, France (AP Human Genetics)
2001 – 2004	B.S.	Luminy Faculty of Science, Marseille, France (AP cell biology)
2000 – 2001	High School Diploma	Felix Esclangon High School, Manosque, France (AP Biology)

Academic Experience

Full Time Positions

2016-Present Assistant Professor, Departments of Pediatrics and Neurology, The Ohio State University, Columbus, OH, USA.

2016-Present Principal Investigator, Center for Gene Therapy, Nationwide Children's Hospital, Columbus, Ohio.

2011-2016 Postdoctoral Scientist, Center For Gene Therapy, Nationwide Childrens Hospital. Columbus OH, USA.
Principal Investigator: **Pr. Kevin Flanigan**
My main project was focused on the identification of a novel *Internal Ribosome Entry Site* in the *DMD* gene that explained certain asymptomatic or mild patient phenotypes. I developed an *innovative approach* allowing expression of an N-terminal truncated dystrophin that rescues the phenotype of our new Dup2 mouse model of DMD. In addition, I investigated the use of *exon skipping* in order to to restore a wild type DMD transcript for DMD patient derived cell lines carrying exonic duplications. Finally, I'm currently generating pilot data for a *calpain-3 gene transfer* for LGMD2A patients, gene transfer of the *nemo-binding peptide* for DMD and ALS patients, and evaluating a *crispr/cas approach* to correct several muscular dystrophies.

2006 - 2010 PhD Student in the Medical Genetics and Functional Genomics. Marseille, France.
Principal Investigator: **Pr. Nicolas Levy**
My main project focused on the discovery of a *mini-dysferlin* isoform in a mildly affected patient. I was involved in the development of a mini-dysferlin vector for *gene transfer* and studied the use of *exon-skipping* for dysferlinopathies. In addition, I developed *new diagnostic tools* for neuromuscular diseases such as *CGH array* for dysferlin and calpain-3 deficiency and *FACS screening* of dysferlin patient.

2005 - 2006 Research Assistant at INSERM_UMRS491, Marseille France.
Principal Investigator: **Pr. Nicolas Levy**
My main project was to assess the expression and localization of dysferlin in healthy monocytes .

2004 - 2005 Research Assistant at INSERM_UMRS491, Marseille France.
Principal Investigator: **Pr. Nicolas Levy**
I was involved in a study looking at the implication of the farnesylation motif CaaX of *LMNA* in cases of Hutchinson-Gilford Progeria Syndrome.

2003 Summer Intern in the Institute for Developmental Biology, Marseille France.
Principal Investigator: **Dr. Thomas Lecuit**
I was involved in a project focusing on how the germ band of the Drosophilia Melanogaster elongate during development.

Scholastic Honors

2017 Outstanding RINCH Team Award recipients for 2016. Nationwide Childrens Hospital, Columbus, OH, USA

2016 Outstanding poster presentation award at New Direction in Biology and Disease of Skeletal Muscle, Orlando, FL, USA.

2016 Outstanding poster presentation award at the 19th American Society of Gene and Cell Therapy, Washington, DC, USA.

2016 Trainee Travel Award at Nationwide Childrens Hospital, Columbus, OH, USA

2014 *Postdoctoral Scientist of the Year*, Nationwide Childrens Hospital, Columbus, OH, USA.

2014 *Young Investigator Prize* at New Direction in Biology and Disease of Skeletal Muscle, Chicago, IL, USA.

2013 *Duchenne Research Award* for the best presentation on Therapy of Duchenne Muscular Dystrophy. 18th World Muscle Society. Asilomar, CA, USA.

2013 Outstanding poster prize at the Muscular Dystrophy Association Scientific meeting, Washington, DC, USA.

2012 Poster award at the 17th World Muscle Society. Perth, Australia

2010 *Young Investigator Prize* at the 15th World Muscle Society, Kumamoto, Japan.

2009 Poster award at the Federative Research Institute 125, Marseille, France.

Scientific skills

Virus	AAV and lentivirus production.
Therapeutic	RNA surgery (exon inclusion/exclusion), gene transfer, premature stop codon read-through assays.
Genomic	Crispr/cas gene editing, DNA extraction and purification, Q-PCR, PCR, site directed mutagenesis, cloning, sequencing, FISH.
Transcription	RNA extraction and purification, RT-PCR, qRT-PCR, siRNA, ribosome profiling, RNA seq.
Protein	Protein extraction and purification, western blot, immuno-staining, mass spectrometry, immunoprecipitation, dual luciferase assay.
Cell Culture	Cells, yeast and bacterial culture, transformation, transfection, AAV and lentiviral transduction.
Diagnostics	MLPA, CGH, FACS.
Other skills	NIH grant writing experience (K99/R00) NIH/NINDS (1K99NS093427-01).
	Managing students, projects, and budgets.
	Preparation of regulatory documents (e.g. PreIND, Provisional Patent Application, Material Transfer Agreement, Confidential Disclosure Agreement, Invention Disclosure, IACUC, IBCSC,).

Administrative Experience

Professional Organization and Scientific Committees

2018-Present Board member of Institutional Biosafety Committee, Nationwide Childrens Hospital, Columbus OH, USA.

2017-Present Organizer of PI grant discussions. Nationwide Childrens Hospital, Columbus OH, USA.

Dec. 2017 Organization of Pr. Charles Thornton visit for the Molecular Life Sciences Seminar Series. The Ohio State University, Columbus. OH. USA.

2014-2016 Board member of the Research in Training Association. Nationwide Childrens Hospital, Columbus OH, USA.

Oct. 2008 Board Member of the conference "Biotechno2008". Marseille, France.

2008 Member of the Scientific Committee for School of Biology, Ecole Doctorale des Sciences de la Vie et de la Santé, Marseille, France.

2006 - 2010 Board Member of the *Hippo these,* a trainee association, Marseille, France.

Symposium/Meeting Chair/Coordinator

May 2018 Mentor at the mentoring event American Society of Gene and Cell Therapy, Chicago, IL, USA.

May. 2018 Poster Judge at the American Society of Gene and Cell Therapy, Chicago, IL, USA

Apr. 2018 Poster Judge at the OSUWMC 2017 Trainee Research Day, Columbus. OH. USA.

Nov. 2017 Poster Judge at the Annual Research Retreat of The Research Institute at Nationwide Children's Hospital, Columbus. OH. USA.

Nov 2017 Poster Judge at the trainee poster day at the Ohio State University, Columbus. OH. USA.

May 2017 Mentor at the mentoring event American Society of Gene and Cell Therapy, Washington DC, USA.

May. 2017 Poster Judge at the American Society of Gene and Cell Therapy, Washington, DC, USA

Apr. 2017 Poster Judge at the OSUWMC 2017 Trainee Research Day, Columbus. OH. USA.

Nov. 2016 Poster Judge at the Annual Research Retreat of The Research Institute at Nationwide Children's Hospital, Columbus. OH. USA.

May 2016 Mentor at the mentoring event American Society of Gene and Cell Therapy, Washington DC, USA.

Nov. 2015 Poster Judge at the Annual Research Retreat of The Research Institute at Nationwide Children's Hospital, Columbus. OH. USA.

October 2015 Session chairman at ActionDuchenne conference, London, UK.

June 2014 Organizer and session chairman of the *Muscle Microenvironment* conference, Columbus. OH. USA.

May 2014 Poster Judge at the Ohio State University annual research conference, Columbus. OH. USA.

Oct. 2008 Organizer of the *Biotechno2008* workshop, Marseille, France.

June 2008 Organizer and session chairman of 15th meeting of School of Biology, Ecole Doctorale des Sciences de la Vie et de la Santé, Marseille, France.

Mentoring Committee

2017- Present Candicacy with Emily Nordquist (March 16,2018)

2017- Present Candidacy with Samantha Powers (July 24,2017)

2017- Present Candidacy with Jessica Adair (June 12,2017)

2016- Present Mentoring meeting with Drs Rafael-fortney, Amacher, Lincoln, King (dec 20,2016)

Professional Membership

2017 – Present	Member of Neuroscience Graduate Program
2016 – Present	Member of Biomedical Sciences Graduate Program
2016 – Present	Member of Molecular, Cellular and Developmental Biology
2014 - Present	American Society of Gene and Cell Therapy member
2009 - Present	World Muscle Society member

Patents

Patent issued	#14/785,769	Recombinant Adeno-Associated Virus Delivery of Exon 2-Targeted U7snRNA Polynucleotide Constructs (co-Inventor)
Application Filed	#62/035,395	Methods and Materials for activating an IRES in exon 5 of the DMD gene (co-Inventor)

Teaching Responsabilites/Assignments

Teaching Training

2001 - 2004 Teacher training in being a life sciences and earth professor, Luminy Faculty of Science, Marseille, France.
Duration: 1 semester/year

2001 - 2002 Teacher training in being a sport instructor, Marseille, France.
Duration: 1day/week for 10 months

Teaching Experiences

2017-Present Lecturer for MCDBIO 7890/91 class, The Ohio State University, Columbus, OH, USA.

2016-Present Lecturer for the RNA biology classes, BSGP7000 class, The Ohio State University, Columbus, OH, USA.

2016 Lecturer (Making sense of nonsense: Moving toward an unusual therapeutic strategy for DMD) for the Summer Scientist Program, Nationwide Children's Hospital, Columbus, OH, USA.

2006-Present Supervision and Management of high school, undergraduate, Ph.D. students and technicians in grad and postdoctoral labs.

2008 - 2010 Instructor (classroom and lab) for the Molecular Genetics course at the Neurobiology Institute of the Mediterranean, Marseille, France.

2003 - 2005 Volleyball Instructor, Luminy Faculty of Science of Marseille, France.

Students Mentor

Nationwide Childrens Hospital, Columbus, OH, USA

Postdoctoral scientist.

2018 - Present	Yacidzohara Rodriguez	“neurological disorders and gene therapy”.
2017 - Present	Florence Robriquet	“AAV gene therapy for neuromuscular disorders ”.

Ph.D.

2017 - Present	Jessica Adair	“AAV.CRISPR preclinical evaluation for DMD exon 2 duplications”. Molecular, Cellular, and Developmental Biology Program
2017 - Present	Samantha Powers	“Rett syndrome and gene therapy”.

Students Supervised

Ph.D.

2015 - 2016	Jessica Adair	“AAV.U7 preclinical evaluation for DMD exon 2 duplications”. Molecular, Cellular, and Developmental Biology Program
2013 – 2016	Tabatha Simmons	“Therapeutic exon skipping of DMD exon 2 duplications”. Molecular, Cellular, and Developmental Biology Program

M.D.

2012 – 2013	Andrew Findlay	“Investigation of single exon-skipping for patients carrying DMD exonic duplication”. Medical student, Pre-doctoral research year.

Undergraduates

2018	Rachel Rafferty	“Alternative delivery method of AAV to target muscle”. Biochemistry undergraduate major at The Ohio State University.
2017	Manu Sanjeev	“Alternative delivery method of AAV to target muscle”. Molecular, Cellular, and Developmental Biology Program.
2017-Present	Daniel Lesman	“Deletion of DMD exons and their therapeutic implications”. Biomedical Science undergraduate major at The Ohio State University.
2017	Avanti Patel	“Deletion of DMD exons and their therapeutic implications”. Summer internship.
2017	Charlene Lopez	“Deletion of part of ABD1 and its therapeutic implication”. Master internship.
Summer 2016	Chloe Gaillet	“Evaluation of exon-skipping following intravascular delivery of PPMO‘’. Summer Education and Research in Clinical Healthcare Internship
2014 – 2016	Jacqueline Yurkosi	‘’Investigating the relationship of dystrophin and syntrophin ‘’.
Summer 2013	Margaret Graham	‘’Evaluation of exon-skipping following intravascular delivery‘’.

High School Students

Summer 2017	Daniel Lesman	“Deletion of DMD exons and their therapeutic implications”. Summer internship.
Summer 2016	Daniel Lesman	“Deletion of part of ABD1 and its therapeutic implication”. Summer internship.
Summer 2015	Nimo Suleyman	‘’Is there another IRES in the dystrophin gene‘’. The Summer Scientist Program
Summer 2015	Naomi Laporte	‘’Evaluation of exon-skipping following intramuscular delivery of PPMO‘’. Summer Education and Research in Clinical Healthcare Internship
Summer 2015	Sravya Gedela	‘’Molecular Cloning of U7‘’. Summer Education and Research in Clinical Healthcare Internship
Summer 2014	Sarah Rogers	‘’Evaluation of exon-skipping following intravascular delivery ‘’. Summer Education and Research in Clinical Healthcare Internship
Summer 2014	Patrick Liu	‘’Characterization of the Dup2 mouse model‘’. Summer Education and Research in Clinical Healthcare Internship
Summer 2012	Margaret King	‘’Construction of the pRDEF plasmid‘’. The Summer Scientist Program.

La Timone Medical school, Marseille, France

Ph.D.

2008 - 2011	Florian Barthelemy	“Therapeutic for dysferlinopathies”. Ecole Doctorale des Sciences de la Vie et de la Santé, Marseille, France.

Undergraduates

2006 - 2010	Virginie Kergourlay	‘’Exploration of dysferlin splicing‘’. University of Saint Charles Science program

University Community Activities

Department Level

2018-Present Board member of Institutional Biosafety Committee, Nationwide Childrens Hospital, Columbus OH, USA.

2017-Present Member of the Neuroscience Graduate Program, The Ohio State University, Columbus, OH, USA.

2016-Present Co-Director of the cell line core, Nationwide Childrens Hospital, Columbus OH, USA.

2016-Present Member of the Biomedical Sciences Graduate Program, The Ohio State University, Columbus, OH, USA.

2016-Present Member of the Molecular, Cellular and Developmental Biology program, The Ohio State University, Columbus, OH, USA.

2016-Present Participating Principal Investigator at Center For Muscle Health and Neuromuscular journal club. Nationwide Childrens Hospital/Ohio State University, Columbus. OH. USA.

2011-2016 Participating Postdoctoral Scientist in the Muscle Biology Disease Journal Club. Nationwide Childrens Hospital/Ohio State University, Columbus. OH. USA.

2006 - 2010 Participating Ph.D student to the La Timone Medical School Journal Club. Marseille. France

Program Level

2016 Lecture on "*how to find a job*" at the Research in Training Association. Nationwide Childrens Hospital. Columbus. OH. USA.

2015 *"Making sense of nonsense mutation: Moving toward an unusual therapeutic strategy for DMD"*. Nationwide Childrens Hospital. Columbus. OH. USA. (07/21/16)

2014-2016 Member of the Research in Training Association. Nationwide Childrens Hospital. Columbus. OH. USA.

2013-Present Mentor, Summer Student Program. Nationwide Childrens Hospital. Columbus. OH. USA.

2011-Present Member, Center for Muscle Health and Neuromuscular Disorders. Nationwide Childrens Hospital/Ohio State University,Columbus. OH. USA.

2008 - 2010 Member, Interdepartmental Research Association "Tous Chercheurs", Neurobiology Institute of the Mediterranean, Marseille. France.

Funding

Active Grant

1/1/18-12/31/18 investigation of the impact of SCN2A mutation

Charlotte and Gwenyth Gray Foundation

Direct Costs: $106,960/yr.

Role: Co-Investigator

6/1/17-5/30/18 Development of a new Duchenne Muscular Dystrophy mouse model

NCH

Direct Costs: $25,000/yr.

Role: Principal Investigator

9/1/16-8/30/21 P50 CORT Center of Research Translation in Muscular Dystrophy Therapeutic Development.

NIH

Direct Costs: $60,000/yr. for Salary Support

Role: Core co-director

Principal Investigator: Kevin Flanigan

Past Grants

9/1/14-9/1/16 Alternate Translational Initiation and Amelioration of Phenotype in the DMD Gene.

Association Francaise contre les Myopathies

Direct costs: €61,336/yr

Role: *Grant Co-writing* and generation of the preliminary data

Principal Investigator: Kevin Flanigan

7/1/13-7/1/15 Successful use of out-of-frame exon 2 skipping induces IRES-driven expression of the N-truncated dystrophin isoform: promising approach for treating other 5' dystrophin mutations.

Ohio State University/Nationwide Children's Hospital Muscle Health and Neuromuscular Disorders Program. This award is a *T32 like fellowship* and was selected among 26 laboratories, with a *competitive renewal*.

Direct Costs: $60,000/yr. for Salary Support

Role: Principal Investigator

3/1/12-3/1/13 Characterization of a novel IRES element in the DMD gene: functional and therapeutic implications.

Philippe Foundation

Direct costs: €5,000/yr. Merit award

Role: Principal Investigator

7/1/07-1/1/11 Functional explorations towards understanding the precise function of normal and mutated dysferlin and testing in vitro/in vivo differential and targeted therapeutic approaches to correct dysferlin defiencies.

Association Francaise contre les Myopathies

Direct costs: €15,600/yr. for Salary Support

Role: Principal Investigator

7/1/06-7/1/09 Functional explorations towards understanding the precise function of normal and mutated dysferlin and testing in vitro/in vivo differential and targeted therapeutic approaches to correct dysferlin defienciesAFM Fellowship.

Association Francaise contre les Myopathies

Direct costs: €100,000/yr

Role: *Grant Co-writing* and generation of the preliminary data

Principal Investigator: Nicolas Levy

7/1/06-7/1/07 Immunolabelling and flow cytometry as new tools to explore dysferlinopathies.

Jain Foundation inc.

Direct costs: $15,000/yr. for Salary Support

Role: Principal Investigator

9/1/05-6/1/06 Student Merit Award Funding (€1,500)

Opockzinsky Foundation

9/1/05-6/1/06 Master Degree Competitive Stipend (€4,500)

Marseille Medical School

Travel Grants

2015	EMBO workshop: Modern DNA concepts and tools for safe gene transfer and modification
2016-2012, 2010, 2009	World Muscle Society travel award
2015, 2014	American Society of Gene and Cell Therapy travel award

Peer-Reviewed Journal Articles

1. **Wein N**, Vulin A, Findlay AR, Gumienny F, Huang N, Wilton SD, Flanigan KM. *Efficient Skipping of Single Exon Duplications in DMD Patient-Derived Cell Lines Using an Antisense Oligonucleotide Approach.* J Neuromuscul Dis. 2017;4(3):199-207

2. Massouridès E, Polentes J, Mangeot P.E, Nectoux J, Deburgrave N, Nusbaum P., Leturcq F., **Wein N**, Flanigan K.M, Peschanski P, Chelly J, Pinset C. *Dp412e: a novel human embryonic dystrophin isoform induced by BMP4 in early differentiated cells.* Skelet Muscle. 2015 Nov 14;5:40.

3. **N. Wein***, A. Vulin*, T.R. Simmons, A.M. Rutherford, A.R. Findlay, J.A. Yurkoski,Y. Kaminoh, K.M. Flanigan. *A new mouse model of DMD: a tool for therapeutic development directed at exon duplications.* Neuromuscul Disord. 2015 Nov;25(11):827-34.

4. F. Barthélémy, **N. Wein*,** C. Blouin*, V. Mouly, L. Garcia, G. Butler-Browne, C. Lamaze, N. Lévy, M. Krahn, M. Bartoli. *Recovery of Dysferlin Function after Exon-Skipping.* JND. 2015 Sept; 2(3):281-290.

5. Findlay A.; **Wein N.**, Kaminoh K; Taylor L.; Dunn D., Mendell J.; King W.; Pestronk A.; Florence J.; Mathews K.; Finkel R.; Swoboda K.; Howard M.; Day J.; McDonald C.; Weiss R.,Flanigan K. for the United Dystrophinopathy Project. *Clinical phenotypes as predictors of the outcome of DMD exon 45 skipping.* Ann Neurol. 2015 Apr;77(4):668-74.

6. **Wein N,** Vulin A, Falzarano MS, Szigyarto A, Maiti B, Findlay A, Uhlen M, Bakthavachalu B, Messina S, Kaminoh Y, Vattemi G, Perrone D, Taylor L, Gualandi F, Weiss RB, Schoenberg D, Howard M, Ferlini A, Flanigan KM. *Using out of frame exon skipping to induce IRES-driven expression of an N-truncated dystrophin isoform: A novel therapeutic approach to mutations in the 5' region of DMD.* Nat Med. 2014 Sep;20(9):992-1000.

7. A.Vulin, **N.Wein**; D. Strandjord; A.R. Findlay; B. Maiti; M.T. Howard; Y.J. Kaminoh; L.E. Taylor; W.C. Ray; J.M. Ervasti; K.M. Flanigan. *DMD and the role of the ZZ domain of dystrophin: making sense of missense mutations.* Hum Mutat. 2014 Feb;35(2):257-64.

8. Meregalli M, Navarro C, Sitzia C, Farini A, Montani E, **Wein N**, Razini P, Beley C, Cassinelli L, Parolini D, Belicchi M, Parazzoli D, Garcia L, Torrente Y. *Full-length dysferlin expression driven by engineered human dystrophic blood derived CD133+ stem cells.* FEBS J. 2013 Dec;280(23):6045-60.

9. Flanigan KM, **Wein N**, Howard MT, Weiss RB. *Becker muscular dystrophy with widespread muscle hypertrophy and a non-sense mutation of exon 2.* Neuromuscul Disord. 2013 Feb;23(2):192.

10. Blandin G, Beroud C, Labelle V, Nguyen K, **Wein N**, Hamroun D, Williams B, Monnier N, Rufibach LE, Urtizberea JA, Cau P, Bartoli M, Lévy N, Krahn M. *UMD-DYSF, a novel locus specific database for the compilation and interactive analysis of mutations in the dysferlin gene.* Hum Mutat. 2012 Mar;33(3):E2317-31.

11. Bartoli M, Nègre P, **Wein N**, Bourgeois P, Pécheux P, Lévy N, Krahn M. *Validation of Comparative Genomic Hybridization Arrays for the detection of genomic rearrangements of the calpain-3 and dysferlin genes.* Clin Genet. 2012 Jan;81(1):99-101

12. **Wein N*,** Krahn M*, Bartoli M*, Lostal W, Courrier S, Bourg-Alibert N, Nguyen K, Vial C, Streichenberger N, Labelle V, DePetris D, Pécheux C, Leturcq F, Cau P, Richard I and Lévy N. *A Naturally Occurring Human Minidysferlin Protein Repairs Sarcolemmal Lesions in a Mouse Model of Dysferlinopathy.* Sci Transl Med. 2010 Sep 22;2(50):50ra69.

13. Lévy N, **Wein N**, Barthelemy F, Mouly V, Garcia L, Krahn M, Bartoli M. *Therapeutic exon 'switching' for dysferlinopathies?* Eur J Hum Genet. 2010 Sep;18(9):969-70.

14. **Wein N**, Avril A, Bartoli M, Beley C, Chaouch S, Laforêt P, Behin A, Butler-Browne G, Mouly V, Krahn M, Garcia L, Lévy N. *Efficient bypass of mutations in dysferlin deficient patient cells by antisense-induced exon skipping.* Hum Mutat. 2010 Feb;31(2):136-42.

15. **Wein N**, Krahn M, Courrier S, Bartoli M, Salort-Campana E, Nguyen K, Fernandez C, Pouget J, Fossat C, Depetris D, Leturcq F, Cau P, Levy N. *Immunolabelling and flow cytometry as new tools to explore dysferlinopathies.* Neuromuscul Disord. 2010 Jan;20(1):57-60.

16. Krahn M, Borges A, Navarro C, Schuit R, Stojkovic T, Torrente Y, **Wein N**, Pécheux C, Lévy N. *Identification of different genomic deletions and one duplication in the dysferlin gene using multiplex ligation-dependent probe amplification and genomic quantitative PCR.* Genet Test Mol Biomarkers. 2009 Aug;13(4):439-42.

Review Articles

17. **Wein N**, Alfano L, Flanigan KM**.** *Genetic and Treatment Advances in Duchenne and Becker Muscular Dystrophy.* Ped Clinics N America 2015 Jun;62(3):723-42.

18. Barthélémy F, **Wein N**, Krahn M, Lévy N, Bartoli M. *Translational research and therapeutic perspectives in dysferlinopathies.* Mol Med. 2011 Sep-Oct;17(9-10):875-82.

FDA related documents

2018 IND application. Phase I/IIa Systemic Gene Delivery Clinical Trial of scAAV9.U7.ACCA for Exon 2 Duplication-Associated Duchenne muscular dystrophy

2017 PreIND application. Phase I/IIa Systemic Gene Delivery Clinical Trial of scAAV9.U7.ACCA for Exon 2 Duplication-Associated Duchenne muscular dystrophy

Reviewer Experience

2013-Present Reviewer for Annals of Neurology, Molecular Therapy, PLOS One, Molecular Therapy – Nucleic Acids, Archive of Neurology, Journal of Neuromuscular Disorders, EMBO mol med, BMC journal, BioMed Research international, Neuromuscular Disorders, Reproductive BiomedicineOnline, The International Journal of Neuroscience.

Presentations

Meeting Presentations (not including poster)

Oral Communication

2017 "*A single neonatal delivery of an AAV mediated exon skipping results in long-term dystrophin expression that prevents pathologic features in a mouse model of Duchenne muscular dystrophy.*". 20th American Society of Gene and Cell Therapy, Washington, DC, USA. (05/10/17)

2016 "*A single neonatal delivery of an AAV mediated exon skipping results in long-term dystrophin expression that prevents pathologic features in a mouse model of Duchenne muscular dystrophy.*". 2016 Nationwide Children's Annual Research Retreat, Columbus, OH, USA. (11/17/16)

2015 "*Early expression of delCH1 dystrophin isoform reverse or prevents muscular dystrophy in the Dup2 mouse*". 2015 Nationwide Children's Annual Research Retreat, Columbus, OH, USA. (11/16/15)

2015 "*Intramuscular and Systemic Induction of the N-Truncated Dystrophin By Out-Of-Frame Exon 2 Skipping Restores Muscle Function in the Dup2 Mouse, Providing Further Support for a Therapeutic Pathway for 5' DMD Mutations*". 18th American Society of Gene and Cell Therapy, New Orleans, LA, USA. (5/13/15)

2015 "*Induction of the N-truncated dystrophin by out-of-frame exon 2 skipping prevent or restores muscle function in the Dup2 mouse, providing further support for a therapeutic pathway for 5' DMD mutations*". EMBO workshop: Modern DNA concepts and tools for safe gene transfer and modification, Evry, France. (3/31/15)

2014 "*Characterization of a novel Internal Ribosomal Entry Site in the DMD gene: functional and therapeutic implications*". 17th American Society of Gene and Cell Therapy. Washington, DC. USA. (05/24/14)

2013 "*Induction of the N-truncated dystrophin by out-of-frame exon 2 skipping prevent or restores muscle function in the Dup2 mouse, providing further support for a therapeutic pathway for 5' DMD mutations*". 18th World Muscle Society. Asilomar, CA. USA. (10/04/13)

2013 "*Induction of the N-truncated dystrophin by out-of-frame exon 2 skipping prevent or restores muscle function in the Dup2 mouse, providing further support for a therapeutic pathway for 5' DMD mutations*". Research in Training Association, Columbus, OH. USA. (10/18/13)

2010 "*Efficient bypass of mutations in dysferlin deficient patient cells by antisense-induced exon skipping*" N. Wein et al., 15th World Muscle Society. Kumamoto, Japan. (10/15/10)

2010 "*AON induced exon-skipping in dysferlinopathies*" Young scientist conference, Association Francaise contre les Myopathies Paris, France (June 2010)

2010 "*AON induced exon-skipping in dysferlinopathies*". Myogenesis, Cassis, France. (May 2010)

2010 "*Therapeutics exon-skipping in dysferlinopathies*", Institut Fédératif de Recherche, Marseille, France. (April 2015)

2009 "*AON induced exon-skipping in dysferlinopathies*" Societe Francaise de Myologie, Hendaye, France. (October 2009)

2008 "*Partial functionality of a mini-dysferlin molecule identified in a patient affected with a moderately severe primary dysferlinopathy*" Research Day Annual meeting, Ecole Doctorale des Sciences de la Vie et de la Santé, Marseille, France. (May 2008)

2008 "*Partial functionality of a mini-dysferlin molecule identified in a patient affected with a moderately severe primary dysferlinopathy*" Myogenesis, Cassis, France. (May 2008)

Invited/Visiting Presentations

2017 "Making sense of nonsense mutation: Moving toward an unusual therapeutic strategy for DMD". Institut de Myologie, Paris, France. (11/13/17)

2017 "Gene Therapy update for Duchenne Muscular Dystrophy". Braedan's Bridge inc. Sylvania, OH, USA. (10/14/17)

2017 "Development of gene therapy vectors for Duchenne Muscular Dystrophy and Myotonic Dystrophy type 1". Nationwide Children's Hospital. Columbus, OH, USA. (11/03/17)

2017 "Principles of Gene Editing". Myology Course. Nationwide Children's Hospital. Columbus, OH, USA. (08/30/17)

2016 *"Principles of Gene Editing"*. Myology Course. Nationwide Children's Hospital. Columbus, OH, USA. (08/30/16)

2015 *"Making sense of nonsense mutation: Moving toward an unusual therapeutic strategy for DMD"*. ActionDuchenne, Department of Integrative Biology and Physiology. University of Minnesota. Minneapolis, MN, USA (12/20/15)

2015 *"Exonic Duplication and 5' mutations in DMD"*. ActionDuchenne, London, UK. (11/06/15)

2015 "*Intramuscular and Systemic Induction of the N-Truncated Dystrophin By Out-Of-Frame Exon 2 Skipping Restores Muscle Function in the Dup2 Mouse, Providing Further Support for a Therapeutic Pathway for 5' DMD Mutations"*. Department of Physiology, Anatomy and Genetics, University of Oxford, Oxford, UK.

2015 "*Making sense of nonsense mutation: Moving toward an unusual therapeutic strategy for DMD"*. Institute of Biotherapies, Inserm UMR-S 1089, Nantes, FR.

2010 "*Functional explorations towards understanding the precise function of normal and mutated dysferlin and testing in vitro/in vivo differential and targeted therapeutic approaches to correct dysferlin defiencies"*. Neurogenetics Branch, National Institute of Health, Bethesda, MD, USA.

2009 "*Partial functionality of a mini-dysferlin molecule identified in a patient affected with a moderately severe primary dysferlinopathy*" Institut de Myologie, Paris, France.

Specialized skills

- Software : Illustrator, Photoshop, GantProject, ImageJ, Graphpad Prism, VectorNTI

Foreign Languages

- French : Native tongue
- English : spoken and written

Activities and interests

- Volley-ball, Guitar, Sailing, Climbing, Diving
- Reading