SCOTT Q. HARPER, Ph.D. **CURRICULUM VITAE**

CURRENT POSITIONS

Associate Professor, Department of Pediatrics
The Ohio State University College of Medicine
Principal Investigator, Center for Gene Therapy
The Research Institute at Nationwide Children's Hospital

Faculty Member,
Integrated Biomedical Sciences Graduate Program and
Molecular, Cellular, and Developmental Biology Graduate Program,
The Ohio State University College of Medicine
Columbus, OH, USA

CONTACT INFORMATION

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EDUCATION

Post-doctoral, University of Iowa College of Medicine, Iowa City, IA, 2002-2007
Mentor: Dr. Beverly Davidson
Ph.D., Cellular and Molecular Biology, University of Michigan,
Ann Arbor, MI, 1996-2002
Mentor: Dr. Jeffrey Chamberlain
B.S., Biology, Saginaw Valley State University, Saginaw, MI, 1996
Summa cum laude

MILITARY SERVICE

Hospital Corpsman (HM2), United States Naval Reserve, 1992-2000
Sailor of the Year, Naval Reserve Center, Saginaw, Michigan, 1998
Outstanding Recruit, Naval Training Center, San Diego, CA, 1992

PERSONAL STATEMENT I am a molecular biologist, and have 20 years of experience in the gene therapy field.

PhD: AAV.Micro-Dystrophin. As a graduate student in Dr. Jeff Chamberlain's lab at the University of Michigan, I investigated dystrophin gene structure and function in transgenic mice, and subsequently used this work to develop the first generation of adeno-associated viral vectors (AAV) to deliver ΔR4-R23 micro-dystrophin, as a gene replacement therapy for Duchenne Muscular Dystrophy (DMD). This invention is now being tested in boys with DMD.

Post-doc: RNAi-based gene therapy for Huntington's Disease. As a post-doctoral fellow in Dr. Beverly Davidson's lab at the University of Iowa, I continued working in the gene therapy field using AAV vectors, but switched focus to develop the first RNAi-based gene therapies for neurodegenerative diseases, including Huntington's Disease and spinocerebellar ataxia type 1. With this work, we were on the vanguard of developing RNAi-based gene therapies and generating novel strategies to design and express artificial inhibitory RNAs for therapeutic purposes.

PI: I began my independent academic position in 2007, and have worked to couple my expertise in AAV-based gene therapy and RNAi to develop the first RNAi-based interventions for dominant neuromuscular diseases, for which therapies are an unmet need. My lab is particularly focused on developing therapies for Facioscapulohumeral muscular dystrophy (FSHD), Limb Girdle Muscular Dystrophy Type 1A (LGMD1A), and Charcot-Marie-Tooth Diseases (CMT2D and CMT1A). We are now working to translate these programs toward clinical trials.

In 2014, I was recipient of the Outstanding New Investigator Award from the American Society of Gene and Cell Therapy (ASGCT).

RESEARCH SUPPORT

NIH NINDS R21/R33 (1 R21 NS105116-01A1 Innovation Grant to Nurture Initial Translational Efforts (IGNITE) (Harper and Burgess, co-PI) 7/1/2018 – 6/30/2021
Gene therapy knockdown and replacement of GARS to treat dominant inherited peripheral neuropathy ($250,000 direct costs per year)

NIH NIAMS P50 Center of Research Translation (CORT) 9/1/2016 – 8/30/2021
(Overall PI: Flanigan; Harper, PI Project 2).
DUX4 inhibition with non-coding RNAs as a therapeutic strategy for FSHD.
Priority Score of 10.

NIH NINDS R21/R33 (Innovation Grant to Nurture Initial Translational Efforts (IGNITE) (Harper, PI; K. Wagner, Co-I) 9/1/2017 – 8/30/2020
In vivo efficacy studies of DUX4-targeted RNAi therapy for FSHD ($250,000 direct costs per year)

Muscular Dystrophy Association (MDA) #418933 (Harper, PI)
"Therapeutic development for FSHD using a new DUX4-expressing knockin model" 8/1/2016 – 7/31/2019

NIH 1U54OD020351-01 (PI, Frankel; Harper, Co-I)
"Jackson Laboratory Center for Precision Genetics: 8/15/2015 – 6/30/2020
From New Models to Novel Therapeutics"

NIH U54 UMMS Wellstone Center (Harper, Co-I; Emerson, PI)
"Biomarkers for therapy of FSHD" 6/1/2015 – 5/31/2023

Muscular Dystrophy Association (MDA) (Bolduc, PI; Bonnemann, Mentor; Harper, Collaborator) 8/1/2017 – 7/31/2020
"Exon skipping for Ullrich congenital muscular dystrophy"

Post-Doctoral Trainee, Dr. Jocelyn Eidahl (Harper, Mentor) 6/1/2017 – 5/31/2019
Friends of FSH/Chris Carrino Foundation Fellowship
"Defining the roles of PTMs on FSHD protein DUX4"

Chris Carrino Foundation Research Grant. (Harper PI).
"Developing a muscle-specific DUX4 inhibition system for FSHD therapy" 8/1/2015 – 7/31/2019

Chris Carrino Foundation Research Grant. (Harper PI). 9/1/2018 – 8/31/2019
"Developing a novel FSHD therapy using N.A.P.A.L.M., a novel nucleic acid-peptide conjugate designed to destroy the DUX4 protein"

Nizar Saad FSH Society Post-doctoral fellowship 3/1/2019 – 2/28/2020
"Identification of natural human DUX4-targeted miRNAs and development of a novel DUX4-targeted miRNA-based gene therapy for FSHD"
(PI: Saad; Mentor: Harper)

FSHD Global Research Foundation (Harper, PI) 1/1/2019 – 12/31/2020
"Developing AAV-follistatin gene therapy alone or in combination with RNAi in a novel mouse model of FSHD"
The goal of this project is to test AAV.Follistatin gene therapy in a mouse model of FSHD

FSH Friends of FSH Research Post-doctoral fellowship to trainee 3/1/2019 – 2/28/2021
"DUX4 mRNA silencing using CRISPR-Cas13"
(PI: Rashnonejad; Mentor: Harper)
The goal of this project is to develop a new gene therapy for FSHD using CRISPR/Cas.

CMT Association 2/1/2019 – 1/31/2021
"Development of a Gene Silencing Approach to Treat CMT1A"
(PI: Kleopas; Co-Investigator: Harper)
The goal of this project is to develop a new gene therapy for Charcot-Marie-Tooth Type 1A

CMT Association 9/1/2018 – 8/31/2021
"Toxicology study of AAV-mediated RNAi therapy in non-human primates"
(PI: Harper)
The goal of this project is to develop a new gene therapy for Charcot-Marie-Tooth Type 2D

Completed Research Support

Nizar Saad FSH Society Post-doctoral fellowship 9/1/2016 – 12/31/2018
miRNAs as potential modifiers of Facioscapulohumeral Muscular Dystrophy (FSHD)
(PI: Saad; Mentor: Harper)

NIH (NIAMS) R01 (1R01AR062123-01) 8/1/2012 –7/31/2018
"DUX4 and the p53 pathway in FSHD pathogenesis" (10 priority score; 1%)

Muscular Dystrophy Association Research Grant (82019115; Co-PI with Dr. Rob Burgess)
"Gene Therapy Approaches to CMT2D" 8/1/2015 – 7/31/2018

"DUX4 exon skipping strategies for FSHD"
NIH TL1 fellowship (TL1TR001069; NCATS)
to PhD Trainee Carlee Giesege (Harper, Mentor) 9/1/2015 – 8/30/2017

Research contract, Debiopharm Inc. (PI: Harper)
Study of Debiopharm compound's ability to suppress DUX4-induced cell death 1/1/2017 – 12/31/2017

FSH Society Research Grant (PI: Harper) 4/1/2016 – 3/31/2017
"Characterization of a Tamoxifen-inducible DUX4 mouse model for FSHD"

Friends of FSH Foundation (PI: Harper) 6/1/2015 – 5/31/2016
"PPMO Therapy for FSHD"

FSH Society Post-doctoral fellowship to trainee 6/1/2015 – 5/31/2017
"Protein chemistry and protein-protein interactions of DUX4"
(PI: Eidahl; Mentor: Harper)

FSH Society Post-doctoral fellowship to Dr. Jocelyn Eidahl 6/1/2015 – 5/31/2016
"Protein chemistry and protein-protein interactions of DUX4"
Role: Mentor; PI: Dr. Jocelyn Eidahl

NIH (NINDS) Translational R21. (1R21NS072260-01, Harper PI). 4/1/2011 – 3/31/2013
"RNAi Therapy for Dominant LGMD1A"

NIH (NINDS) Translational R21. (1R21NS078327-01; Harper PI). "DUX4 inhibition as a therapeutic strategy for FSHD"	4/1/2012 – 3/31/2014
Muscular Dystrophy Association Research Grant (Harper PI) "Development of an Inducible FSHD mouse model"	2/1/2012 – 1/31/2015
FSHD Global Research Foundation (Harper, PI) "DUX4 inhibition as a therapeutic strategy for FSHD"	6/1/2012 – 5/31/2015
NIH Wellstone Graduate Fellowship to PhD Trainee Carlee Giesiege (Harper, Mentor; Grant # U54 HD066409; Mendell, PI)	1/1/2014 – 12/31/2014
NINDS T32 Training Grant Fellowship to Post-Doc Dr. Jocelyn Eidahl (Harper, Mentor; Grant 1T32NS077984-02 "Training in Neuromuscular Diseases" "Protein chemistry and protein-protein interactions of DUX4"	6/1/2014 – 5/31/2015
Chris Carrino Foundation Research Grant. (Harper PI). "Developing a controlled DUX4 inhibition system for FSHD therapy"	7/15/2013 – 7/14/2014
Muscle Disease and Biology NIH T32 Training Grant, Post-Doctoral Fellow, Lindsay Wallace (Harper, Mentor)	2/1/2013 – 1/31/2014
Systems and Integrative Biology Training Grant, 2013Graduate Student Trainee Jackie Domire (Harper, Mentor)	10/1/2011 – 9/30/2013
OSU Muscle Group Fellowship, Post-doc Trainee Lindsay Wallace (Harper, Mentor)	9/1/2011 – 8/31/2012
Facioscapulohumeral Society, Travel Fellowship Grant for Trainee Travel to World Muscle Society Meeting, Perth Australia (FSHS-32012-01) October 2012	October 2012
Facioscapulohumeral Society Research Fellowship Grant (FSHS-82010-02; Harper, PI). "Defining the tissue and cell specificity of the human DUX4 promoter in mice".	2/8/2011 – 2/7/2012
NIH KL2 Clinical and Translational Scholar Award (KL2 RR025754; Harper, PI) "A translational approach toward RNAi therapy for Facioscapulohumeral muscular dystrophy"	6/1/2008 – 5/31/2011
Muscular Dystrophy Association Development Grant (Harper, PI) "RNAi therapy for facioscapulohumeral muscular dystrophy"	7/1/2007 – 6/30/2010
Facioscapulohumeral Society Jeff Jacobs Family Research Fellowship Grant (FSHS-JJFR-001; Harper, PI) "Investigating DUX4 structure and function using rational Mutagenesis"	2/1/2009 – 1/31/2011
Facioscapulohumeral Society Landsman Charitable Trust Research Fellowship Grant (FSHS-LCT-002; Harper, PI) "*In vivo* investigation of DUX4 as a candidate FSHD gene"	2/1/2008 – 1/31/2009

NIH NS047048-01 (Harper, PI; Davidson, Mentor) 7/17/03 – 7/16/06
"Functional effects of Hes6 proteolysis on differentiation"

Hereditary Disease Foundation (Harper, PI) 7/1/2003 – 6/30/2006
"Silencing Huntingtin with siRNA"

NIH Gene Therapy Pilot Grant (Harper, PI; Davidson, Mentor) 10/1/2006 – 5/31/2007
"Autoregulated AAV vectors for inducible RNAi"

HONORS AND AWARDS

- **2014 Outstanding New Investigator,** The American Society of Gene and Cell Therapy. Awarded at the American Society of Gene and Cell Therapy Annual Meeting, Washington, DC, June 2014.
- "New Investigator of the Month", American Society of Gene and Cell Therapy, 2013
- "Featured Alumnus", Cell and Molecular Biology Graduate Program, University of Michigan, Spring 2013
- "Service from the Heart Teamwork Award", Nationwide Children's Hospital, 2011
- Recipient, Travel Award, American Society of Gene Therapy, 9th Annual Meeting, Baltimore, MD, May-June 2006.
- Recipient, Travel Award, American Society of Gene Therapy, 8th Annual Meeting, St. Louis, MO, June 2005.
- Best Basic Science Poster, University of Iowa, Department of Internal Medicine, Research Day Symposium, March 2005.
- First Place, Poster Award for Post-Doctoral and Medical Fellows, Center for Aging, University of Iowa, Department of Internal Medicine, Research Day Symposium
- Recipient, Travel Award, American Society of Gene Therapy, 5th Annual Meeting, Boston, MA, June 2002.
- Invited participant, Press Briefing, American Society of Gene Therapy, 4th Annual Meeting, Seattle, WA, May-June, 2001.
- First Place, Poster Award, Cell and Molecular Biology Research Symposium, University of Michigan, Ann Arbor, MI, September 2000.
- Pre-doctoral Student Award finalist, American Society of Human Genetics, 50th Annual Meeting, Philadelphia, PA, October 2000.
- Graduated *Summa cum laude*, Saginaw Valley State University, 1996
- Recipient, State of Michigan Competitive Scholarship, 1996
- Phi Theta Kappa, National Junior College Honor Society, Delta College, 1991

Awards to Dr. Harper's Trainees

- PhD Student, Carlee Giesige, Travel Award, World Muscle Society Annual Meeting, Mendoza, Argentina, 2018
- Post-doctoral fellow, Dr. Nizar Saad, Poster Award Winner, World Muscle Society Annual Meeting, Mendoza, Argentina, 2018
- PhD Student, Carlee Giesige, Poster Award Winner, World Muscle Society Annual Meeting, Mendoza, Argentina, 2018
- Post-Doctoral Fellow, Dr. Lindsay Wallace, Travel Award, World Muscle Society Annual Meeting, Saint-Malo, France, 2017
- PhD Student, Carlee Giesige, Burrough's Wellcome Fund Travel Award, 2017 travel to Association for Clinical and Translational Science Meeting, Washington, DC, April 2017
- PhD Student, Carlee Giesige, Travel Award, World Muscle Society Annual Meeting, Saint-Malo, France, 2017
- Post-doctoral fellow, Dr. Nizar Saad, Travel Award Winner, NCH Research Trainee Award Association, 2017
- Post-doctoral fellow, Dr. Jocelyn Eidahl, Travel Award, World Muscle Society Annual Meeting, Saint-Malo, France, 2017

- Post-doctoral fellow, Dr. Jocelyn Eidahl, Poster Award Winner, MDA Scientific Conference Poster Session, Arlington, VA, Spring, 2017
- Post-doctoral fellow, Dr. Jocelyn Eidahl, Travel Award Winner, NCH Research Trainee Award Association, 2016
- Outstanding poster award winner, American Society of Gene and Cell Therapy, 19th Annual Meeting, Washington DC, May 2016
- Post-doctoral fellow Dr. Jocelyn Eidahl, Selected as top abstract awardee, NCH Research Retreat, 2015
- Ph.D. student Carlee Giesige, Poster Award Winner, OSU Research Day, 2015
- Ph.D. student Carlee Giesige, Winner, One of top 5 poster presentations, Muscular Dystrophy Association 2015 Scientific Conference, Washington, D.C. March 2015.
- Post-doctoral fellow Lindsay Wallace, Elsevier Top Trainee Poster Awardee, World Muscle Society Meeting, Berlin, Germany, Oct 2014
- Post-doctoral fellow Lindsay Wallace, Travel Award, World Muscle Society Meeting, Berlin, Germany, Oct 2014.
- Post-Doctoral Fellow Dr. Jocelyn Eidahl, recipient of NINDS T32 Training Grant Fellowship, 2014-5
- Post Doctoral Fellow Dr. Jocelyn Eidahl, recipient of FSH Society Post-Doctoral Fellowship, 2015-6
- Graduate Student Carlee Schaefer, Wellstone Graduate Training Fellowship awardee, 2014
- Post-doctoral fellow Lindsay Wallace, Elsevier Top Trainee Poster Awardee, World Muscle Society Meeting, Asilomar, California, October 2013.
- Post-doctoral fellow Lindsay Wallace, Travel Award, World Muscle Society Meeting, Asilomar, California, October 2013.
- Post-doctoral fellow Lindsay Wallace, Elsevier Top Trainee Poster Awardee, World Muscle Society Meeting, Perth, Western Australia, October 2012.
- Ph.D. student Jacqueline Domire, Winner, OSU Research Day poster session, April 2013
- Post-doctoral fellow Lindsay Wallace, Recipient of NIH/NINDS T32 training grant fellowship, "Training in Neuromuscular Diseases", Jan 1, 2013 – December 31, 2013.
- Post-doctoral fellow Lindsay Wallace, Travel Award, World Muscle Society Meeting, Perth, Western Australia, October 2012.
- Ph.D. student Jacqueline Domire, Travel Award, World Muscle Society Meeting, Perth, Western Australia, October 2012.
- Ph.D. student Lindsay Wallace. Winner, One of top 4 Abstracts, Muscular Dystrophy Association 2012 Clinical Conference, Las Vegas, NV, March 4-7, 2012
- Ph.D. student Jacqueline Domire. Recipient, Systems and Integrative Biology Training Grant fellowship, The Ohio State University, 2011-2013
- Ph.D. student Jacqueline Domire. Recipient, Dean's Distinguished Fellowship, The Ohio State University, deferred until 2013-2014
- Ph.D. student Lindsay Wallace. Recipient, OSU Muscle Group Fellowship, The Ohio State University, 2011-2012
- Ph.D. student Lindsay Wallace (abstract presented by Dr. Eric Meadows). Winner, One of Top 5 Abstracts, Muscular Dystrophy Association Meeting, Las Vegas, NV, March 2011
- Ph.D. student Lindsay Wallace, Travel Award, American Society of Cell and Gene Therapy, 14th Annual Meeting, Seattle, WA, May 2011
- Ph.D. student Lindsay Wallace. Winner, Outstanding Pre-Doctoral Basic Award at the 60th Annual American Society of Human Genetics meeting, Washington D.C. (6008 scientific attendees; 1734 trainees; 3149 total presentations), 2010
- Ph.D. student Lindsay Wallace, Travel Award, American Society of Human Genetics, 60th Annual Meeting, Washington, DC, 2010
- Ph.D. student Lindsay Wallace, Recipient, Nationwide Children's Hospital Outstanding Graduate Student Award for 2010. Accompanied by stipend fellowship for 2010.

- Travel Award to Trainee, Ph.D. Student Lindsay Wallace, American Society of Gene Therapy, 12th Annual Meeting, San Diego, CA, 2009

TEACHING EXPERIENCE

- FSH Society Webinar, "Emerging Molecular Therapies for FSHD", Presented Oct 12, 2017, published online by the FSH Society; https://www.fshsociety.org/
- Organizer and Presenter, FSHD Family Day Conference, Sponsored by NCH, The FSH Society, and the P50 CORT Center for Research Translation, Held at Nationwide Children's Hospital, June 11, 2017.
- Co-Course Director and Instructor, BSGP7000, Foundational Concepts in Biomedical Research, IBGP Graduate Program, Autumn 2012-2017. Instructor only from 2018-present.
- Instructor, NCH and Paul D. Wellstone Myology Course, 2012-present
- Instructor, IBGP851, Matching Treatment with Disease, Winter 2012
- Instructor, IBGP797.02, Systems and Integrative Biology (SIB) Training Program Journal Club, Spring 2012
- Instructor, IBGP792, Translational Biomedical Research, Spring, 2012
- Course Director, Integrated Biomedical Sciences Graduate Program, IBGP701 module, 2011 - present
- Instructor, IBGP704. Research Problem Solving, 2010, 2012
- Biomedical Science 582 (BMS582) Special Topics 2. Genetics and Neurological Diseases, 2010 - present
- OSU Faculty, ANAT911.03, Human Embryology. Taught a two hour lecture on limb and somite development, 2009
- OSU Faculty moderator, MCDB 890/800 student seminar series, 2009
- OSU Faculty, Integrated Biomedical Sciences Graduate Program, IBGP 701, Lectures on nucleic acid biochemistry, RNA interference, gene therapy, 2008 - 2011
- University of Iowa College of Medicine, Department of Physiology. Lecturer: Human Physiology for Pre-Pharmacy and Pre-Engineering students, Prepared lectures and examination questions, taught lectures, proctored examinations, 2003 - 2005
- Graduate Student Teaching Assistant, Human Molecular Genetics (HG541), University of Michigan Medical School, Department of Human Genetics. Organized and ran a weekly discussion section with 25-35 graduate and undergraduate students. Prepared course materials, graded examinations, tutored. Fall 1999.
- Co-Organizer, Fall CMB/Genetics Short Course (HG 630), "Diseases of Trinucleotide Repeat Expansion", University of Michigan Medical School, 1998

PUBLICATIONS

In preparation/Under Review

1. J.O. Eidahl, M.E. Hoover, O.E. Branson, L. Zhang, M.A. Freitas, and **S.Q. Harper**. (2019) FSHD candidate protein, DUX4, is post-translationally modified. In preparation for submission.

2. Amini-Chermahini, G., Rashnonejad, A, and S.Q. Harper. (2019). In situ hybridization method for detecting endogenous DUX4 mRNA in vitro and in vivo. In revision at *RNA.*

3. **S.Q. Harper**. (2019) "RNAi therapy for dominant muscular dystrophies and other myopathies." Muscle Gene Therapy. Ed. D. Duan. Springer Press. *In press.*

4. A. Rashnonejad, **S.Q. Harper** (2019). U7-snRNA-mediated exon skipping gene therapy for FSHD. In preparation for submission.

5. A. Rashnonejad, **S.Q. Harper** (2019). CRISPR/Cas13 gene therapy for FSHD. In preparation for submission.

Published

1. C.R. Giesige, L.M. Wallace, K.N. Heller, J.O. Eidahl, N.Y. Saad, A.M. Fowler, N.K. Pyne, M. Al-Kharsan, A. Rashnonejad, G.A. Chermahini, J.S. Domire, D. Mukweyi, S.E. Garwick-Coppens, S.M. Guckes, K.J. McLaughlin, K. Meyer, L.R. Rodino-Klapac, and S**.Q. Harper**. (2018) AAV-mediated follistatin gene therapy improves functional outcomes in the TIC-DUX4 mouse model of FSHD. *JCI Insight* Nov 15;3(22).

2. E. Ansseau, C. Vanderplanck, A. Wauters, **S.Q. Harper**, F. Coppee, A. Belayew. (2017) Antisense Oligonucleotides Used to Target the DUX4 mRNA as Therapeutic Approaches in FaciosScapuloHumeral Muscular Dystrophy (FSHD). *Genes*. 2017, 8, 93, March 3, 2017.

3. L.M. Wallace, N.Y. Saad, N.K. Pyne, A. Fowler, J.O. Eidahl, J.S. Domire, D.A. Griffin, A.C. Herman, Z. Sahenk, L Rodino-Klapac, and **S.Q. Harper**. (2017) Pre-clinical safety studies to support translation of AAV-mediated RNAi therapy for FSHD. *Mol Ther Methods Clin Dev*. Dec 24, 8:121-130.

4. Eidahl, J.O., Giesige, C.R., Domire, J.D., Wallace, L.M., Fowler, A.M., Guckes, S., Garwick-Coppens, S., Labhart, P. and **S.Q. Harper**. (2016) Mouse Dux is myotoxic and shares partial functional homology with its human paralog DUX4..*Human Molecular Genetics.* 2016 Oct 15;25(20):4577-4589.

5. E. Ansseau, J.O. Eidahl, C. Lancelot, A. Tassin, C. Matteotti, C. Yip,, J. Liu, B. Leroy, C. Hubeau, C. Gerbaux. S. Cloet, A Wauters, S. Zorbo, P. Meyer, I. Pirson, D. Laoudj-Chenivesse, R. Wattiez, **S.Q. Harper**, A. Belayew, and F. Coppee (2016). Homologous transcription factors DUX4 and DUX4c associate with cytoplasmic proteins during muscle differentiation. *PLoS One*. 2016 Jan 27;11(1):e0146893. eCollection 2016.

6. Ansseau, E., Domire, J.S., Wallace, L.M., Eidahl, J.O., Guckes, S.M., Giesiege, C.R., Pyne, N.K., Belayew, A., and **S.Q. Harper**. (2015) Aberrant splicing in transgenes containing introns, exons, and V5 epitopes: lessons from developing an FSHD mouse model expressing a D4Z4 repeat with flanking genomic sequences. *PLoS One*. 2015 Mar 5;10(3):e0118813. doi: 10.1371/journal.pone.0118813. eCollection 2015.

7. O'Reilly, M., Federoff, H.J., Fong, Y., Kohn, D.B., Patterson, A.P., Ahmed, N., Aravind, A., Boye, S.E., Crystal, R., DeOliveira, S., Gargiulo, L., **Harper S.Q.**, Ikeda, Y., Jambou, R., Montgomery, M., Prograis, L., Rosenthal, E., Sterman, D.H., Vandenberghe, L.H., Zoloth, L., Abedi, M., Adair, J., Adusumilli, P.S., Goins, W.F., Gray, J., Monahan, P., Popplewell, L., Sena-Esteves, M., Tannous, B., Weber, T., Wierda, W., Gopal-Srivastava, R., McDonald, C.L., Rosenblum, D., and Corrigan, Curay, J. Gene Therapy: Charting a Future Course. Summary of a National Institutes of Health Workshop, April 12, 2013. (2014) *Human Gene Therapy*. Jun;25(6):488-97. doi: 10.1089/hum.2014.045

8. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, D. Sloboda, C. Cavis, C.H. Hakim, M.E. Hauser, J.R. Mendell, S.V. Brooks, and **S.Q. Harper**. (2014) RNAi-mediated gene silencing of mutant myotilin improves myopathy in LGMD1A mice. *Molecular Therapy Nucleic Acids* Apr 29:3e160.

9. Wallace, L.M. Moreo, A., Clark, K.R. and **S.Q. Harper**. Dose-dependent toxicity of humanized *Renilla Reniformis* GFP (hrGFP) limits its utility as a reporter gene in mouse muscle. (2013) *Molecular Therapy Nucleic Acids*. Apr 16;2:e86.

10. **S.Q. Harper**. Molecular dissection of dystrophin identifies the docking site for nNOS. (2013) *PNAS* Jan 8;110(2):387-8.

11. Flanigan, K.M. and **S.Q. Harper**. (2013) "Chapter 32: Facioscapulohumeral Muscular Dystrophy." Muscle Disease: Pathology and Genetics. Ed. H. Goebel, C. Sewry, and R. Weller. ISN Press. DOI: 10.1002/9781118635469.ch32.

12. Wallace, L.M., Liu J., Domire J.S., Garwick-Coppens, S.E., Guckes, S.M., Mendell, J.R., Flanigan K.M., and **S.Q. Harper**. (2012) RNA interference inhibits DUX4-induced muscle toxicity in vivo: Implications for a targeted FSHD therapy. *Molecular Therapy* July;20(7):1417-23.

13. S.N. Pandey, J. Cabotage, R. Shi, M. Dixit, M. Sutherland, J. Liu, S. Muger, **S.Q. Harper**, K. Nagaraju, and Y.W. Chen. (2012) Conditional over-expression of PITX1 causes skeletal muscle atrophy in mice, *Biology Open* 1:629-639.

14. Liu, J. and **S.Q. Harper**. (2012) RNAi-based gene therapy strategies for dominant limb girdle muscular dystrophies. *Current Gene Therapy* 12(4):307-314.

15. S.E. Garwick-Coppens, Adam Herman, and **S.Q. Harper**. (2011) Construction of permanently inducible miRNA expression vectors using site-specific recombinases. *BMC Biotechnology* Nov 16;11:107.

16. L.M. Wallace, S.E. Garwick-Coppens, R. Tupler, and **S.Q. Harper**. (2011) RNA interference improves myopathic phenotypes in mice over-expressing FSHD Region Gene 1 (FRG1). *Molecular Therapy* Nov;19(11):2048-54.

17. S.Q. Harper. (2011) "Neuromethods Preface". RNA Interference Methods. Ed. **S.Q. Harper**. Humana Springer Press, 2011, page vii.

18. R.L. Boudreau, S.E. Garwick-Coppens, L.M. Wallace, J. Liu, and **S.Q. Harper**. (2011) "Rapid Cloning and Validation of MicroRNA Shuttle Vectors: A Practical Guide." RNA Interference Methods. Ed. **S.Q. Harper**. Humana Springer Press, 2011, pages 19-37.

19. L.M. Wallace, S.E. Garwick, W. Mei, A. Belayew, F. Coppee, K.J. Ladner, D. Guttridge, J. Yang, and **S.Q. Harper**. DUX4, a candidate gene for Facioscapulohumeral muscular dystrophy, causes p53-dependent myopathy in vivo. (2010) *Annals of Neurology*, Epub Oct 28; March;69(3):540-52, 2011.

20. Z. Jin, L.M. Wallace, **S.Q. Harper**, and J. Yang. PP2A:B56, a substrate of caspase-3, regulates p53-dependent and p53-independent apoptosis during development. (2010) *Journal of Biological Chemistry*, 285(45):34493-502.

21. L.M. Wallace, S.E. Garwick, and **S.Q. Harper**. (2010) "RNAi therapy for dominant muscular dystrophies and other myopathies." Muscle Gene Therapy. Ed. D. Duan. Springer Press. Pages 99-116.

22. **S.Q. Harper**. Progress and challenges in RNAi therapy for Huntington's Disease. (2009) *Archives of Neurology*, 66(8):933-938.

23. A. Packer, Y. Xing, **S.Q. Harper**, L. Jones, and B.L. Davidson. The bi-functional microRNA mir9/mir9* regulates REST and coREST and is down-regulated in Huntington's disease. (2008) *Journal of Neuroscience*, 28(53):14341-6.

24. J.L. McBride*, R.L. Boudreau*, **S.Q. Harper*** (shared first authorship), A. Mas Monteys, P.D. Staber, I. Martins, B. Gilmore, H. Burstein, R.W. Peluso, B. Polisky, B.J. Carter, and B.L. Davidson. MicroRNA shuttles mitigate short-hairpin RNA mediated toxicity in the brain: Implications for therapeutic development of RNA interference. (2008) *Proceedings of the National Academy of Sciences, USA* 105(15):5868-73.

25. **S.Q. Harper** and Gonzalez-Alegre, P. Lentivirus-mediated RNA interference in mammalian neurons. (2008) *Methods in Molecular Biology* 442, 95-112.

26. P. Allaire, B. Ritter, S. Thomas, J. Burman, A. Denisov, V. Legendre-Guillmen, **S.Q. Harper**, B.L. Davidson, K. Gehring, and P. McPherson. Connecdenn, A DENN domain-containing protein functioning in synaptic vesicle endocytosis. (2006) *Journal of Neuroscience* 26(51), 13202-13212.

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ABSTRACTS

1. G. Amini Chermahini, A. Rashnonejad, and **S.Q. Harper**. An in situ hybridization-based method for detecting *DUX4* RNA expression in vitro. To be presented at the MDA Clinical Meeting, Orlando, FL, April 13-17, 2019.

2. A. Rashnonejad, G. Amini Chermahini, and **S.Q. Harper**. *DUX4* mRNA silencing with CRISPR-Cas13 gene therapy as a prospective treatment for Facioscapulohumeral Muscular Dystrophy, To be presented at the MDA Clinical Meeting, Orlando, FL, April 13-17, 2019. *Oral presentation.*

3. A. Rashnonejad, G. Amini Chermahini, and **S.Q. Harper**. *DUX4* mRNA silencing with CRISPR-Cas13 gene therapy as a prospective treatment for Facioscapulohumeral Muscular Dystrophy, To be presented at the 21st Annual ASGCT Meeting, Washington, DC, April 29-May 2, 2019. *Oral presentation.*

4. K.H. Morelli, N.K. Pyne, L.M. Wallace, A.M. Fowler, **S.Q. Harper**, and R.W. Burgess. Preclinical Gene Therapy Studies Using Allele-Specific RNAi Knockdown of *GARS* to Treat Charcot-Marie-Tooth Disease Type 2D. To be presented at the MDA Clinical Meeting, Orlando, FL, April 13-17, 2019.

5. K.H. Morelli, M. Presa, L. Bogdanik, R.M. Bailey, S.J. Gray, N. K. Pyne, L.M. Wallace, A.M. Fowler, **S.Q. Harper**, C. M. LutZ, and R.W. Burgess. Preclinical Gene Therapy studies for FIG4/CMT4J and GARS/CMT2D. To be presented at the Peripheral Nerve Society meeting, June 22-26, 2019, Genoa, Italy.

6. **S.Q. Harper,** Molecular Therapies for FSHD, Kansas City FSHD Family Day Conference, Sponsored by the KU Medical Center and the FSH Society, Nov 10, 2018, Overland Park, KS.

7. **S.Q. Harper**, FSHD Therapies 101, 2018 FSHD Connect Meeting, Las Vegas, NV, June 9, 2018

8. **S.Q. Harper,** The Pipeline for FSHD Therapies, Targeting DUX4 mRNA, 2018 FSHD Connect Meeting, Las Vegas, NV, June 9, 2018.

9. J.O. Eidahl, L. Zhang, M. Hoover, O. Branson, M. Freitas, and **S.Q. Harper**, Regulation of FSHD candidate protein DUX4, Presented at the 2018 FSH Society FSHD International Research Conference, Las Vegas, NV, June 8, 2018. *Oral Presentation.*

10. N.Y. Saad, M. Al-Kharsan, S. Garwick-Coppens, N. Pyne, and **S.Q. Harper,** The natural microRNA mir-675 reduces DUX4 expression and toxicity in vitro, Presented at the 2018 FSH Society FSHD International Research Conference, Las Vegas, NV, June 8, 2018. *Oral Presentation.*

11. Carlee R. Giesige[1,2], Lindsay M. Wallace[2], Kristin N. Heller[2], Jocelyn O. Eidahl[2], Allison M. Fowler[2], Nettie K. Pyne[2], Nizar Y. Saad[2], Mustafa Alkharsan[2], Afrooz Rashnonejad[2], Gholamhossein Amini Chermahini[2], Jacqueline S. Domire[2], Diana Mukweyi[2], Sara E. Garwick-Coppens[2], Susan M. Guckes[2], K. John McLaughlin[4], Louise R. Rodino-Klapac[2,3] and **Scott Q. Harper**. AAV.RNAi and follistatin gene therapy development in the TIC-DUX4 mouse model of FSHD. Presented at the 2018 FSH Society FSHD International Research Conference, Las Vegas, NV, June 8, 2018. *Oral Presentation.*

12. A. Rashnonejad, and **S.Q. Harper**. AAV.U7-snRNA-mediated exon skipping of the toxic DUX4 gene as a promising therapeutic approach for facioscapulohumeral muscular dystrophy. Presented at the 2018 FSH Society FSHD International Research Conference, Las Vegas, NV, June 8, 2018. *Poster Presentation.*

13. **S.Q. Harper.** Emerging therapeutic approaches for FSHD. Invited keynote talk, Presented at the 23rd World Muscle Society Congress, October 5, 2018, Mendoza, Argentina. Oral presentation.

14. J.O. Eidahl, L. Zhang, M. Hoover, O. Branson, M. Freitas, and **S.Q. Harper**, Regulation of FSHD candidate protein DUX4, 23rd World Muscle Society Congress, October 5, 2018, Mendoza, Argentina. Poster presentation.

15. Carlee R. Giesige[1,2], Lindsay M. Wallace[2], Kristin N. Heller[2], Jocelyn O. Eidahl[2], Allison M. Fowler[2], Nettie K. Pyne[2], Nizar Y. Saad[2], Mustafa Alkharsan[2], Afrooz Rashnonejad[2], Gholamhossein Amini Chermahini[2], Jacqueline S. Domire[2], Diana Mukweyi[2], Sara E. Garwick-Coppens[2], Susan M. Guckes[2], K. John McLaughlin[4], Louise R. Rodino-Klapac[2,3] and **Scott Q. Harper**. Progressive myopathy in a new mouse model of Facioscapulohumeral muscular dystrophy (FSHD) facilitates development of targeted molecular therapies. 23rd World Muscle Society Congress, October 5, 2018, Mendoza, Argentina. Poster presentation. **Poster award winner.**

16. N.Y. Saad, M. Al-Kharsan, S. Garwick-Coppens, N. Pyne, and **S.Q. Harper,** The natural microRNA mir-675 reduces DUX4 expression and toxicity in vitro, Presented at the 23rd World Muscle Society Congress, October 5, 2018, Mendoza, Argentina. Poster presentation. **Poster award winner.**
17. A. Rashnonejad, and **S.Q. Harper**. AAV.U7-snRNA-mediated exon skipping of the toxic DUX4 gene as a promising therapeutic approach for facioscapulohumeral muscular dystrophy. Presented at the ASGCT Annual Meeting, Chicago, IL 2018. *Oral Presentation.*

18. C.R. Giesige[1,2], L.M. Wallace[2], K.N. Heller[2], J.O. Eidahl[2], A.M. Fowler[2], N.K. Pyne[2], N.Y. Saad[2], M. Alkharsan[2], A. Rashnonejad[2], G. Amini Chermahini[2], J.S. Domire[2], D. Mukweyi[2], S.E. Garwick-Coppens[2], S.M. Guckes[2], K. John McLaughlin[4], L.R. Rodino-Klapac[2,3] and **S.Q. Harper**. Progressive myopathy in a new mouse model of Facioscapulohumeral muscular dystrophy (FSHD) facilitates development of targeted molecular therapies. *Oral presentation, Presented at the ASGCT annual meeting, Chicago, Illinois 2018.*

19. L.M. Wallace, D.A. Griffin, N.K. Pyne, J.S. Domire, L.R. Rodino-Klapac, and **S.Q. Harper**. Translating DUX4-targeted RNAi therapy for FSHD. Poster Presentation at World Muscle Society, St. Malo, France, October 2017.

20. J.O. Eidahl, and **S.Q. Harper**. Regulation of FSHD protein DUX4. Poster Presentation at World Muscle Society, St. Malo, France, October 2017.

21. L.M. Wallace, D.A. Griffin, N.K. Pyne, J.S. Domire, L.R. Rodino-Klapac, and **S.Q. Harper**. Translating DUX4-targeted RNAi therapy for FSHD. Presented at the 2017 Muscular Dystrophy Association Scientific Conference, Arlington, VA, March 2017. ***Oral presentation.***

22. Carlee Giesige, Kristin N. Heller, Lindsay M. Wallace, Jacqueline S. Domire, Diana Mukweyi, Jocelyn O. Eidahl, Sara E.Garwick-Coppens, Susan M. Guckes, Louise R. Rodino-Klapac, and **Scott Q. Harper**. "Developing the first mouse model of FSHD that recapitulates myopathy-related phenotypes." Oral presentation at Association for Clinical and Translational Science, Washington DC, April 2017.

23. Carlee Giesige, Kristin N. Heller, Lindsay M. Wallace, Jacqueline S. Jocelyn O. Eidahl, Diana Mukweyi, S Sara E.Garwick-Coppens, Susan M. Guckes, Louise R. Rodino-Klapac, and **Scott Q. Harper**. "Generation of a new, inducible model of FSHD that develops overt myopathic phenotypes." Poster Presentation at World Muscle Society, St. Malo, France, October 2017

24. J.O. Eidahl, M.E. Hoover, O.E. Branson, L. Zhang, M. Freitas, **S.Q. Harper**. Identification and Characterization of DUX4 Post-Translational Modifications". Presented at the Muscular Dystrophy Association Scientific Conference, Arlington, VA, March 19-22, 2017. (*Poster award winner*).

25. N.Y. Saad, S. Garwick-Coppens, and **S.Q. Harper**. Mir-675 reduces DUX4 expression and confers resistance to DUX4 toxicity in FSHD myoblasts: A framework to define the DUX4-targeted miRNome, Presented at the 2017 Muscular Dystrophy Association Scientific Conference, Arlington, VA, March 19-22, 2017.

26. K.H. Morelli, N. Pyne, A. Fowler, J.S. Domire, **S.Q. Harper**, and R.W. Burgess. A personalized gene therapy approach for CMT2D. Presented at the MDA Scientific Conference, Arlington, VA, 2017. (*Poster award winner*).

27. L.M. Wallace, D. Griffin, N. Pyne, J.S. Domire, L. Rodino-Klapac, and **S.Q. Harper**. Translating DUX4-targeted RNAi therapy for FSHD. Presented at FSH Society International Research Meeting, Boston, MA, November 2016. (oral presentation).

28. L.M. Wallace, D. Griffin, N. Pyne, J.S. Domire, L. Rodino-Klapac, and **S.Q. Harper**. Translating DUX4-targeted RNAi therapy for FSHD. Presented at FSHD Wellstone Meeting, Worcester, MA, November 2016. (poster).

29. L.M. Wallace, D. Griffin, N. Pyne, J.S. Domire, L. Rodino-Klapac, and **S.Q. Harper.** Toxicology for DUX4-targeted microRNAs. Presented at the American Society of Gene and Cell Therapy Annual Meeting, Washington, D.C. June, 2016.

30. L.M. Wallace, C.R. Giesiger, D.A. Griffin, L.R. Rodino-Klapac, and **S.Q. Harper**. RNAI therapy for dominant LGMD1A. Presented at the American Society of Gene and Cell Therapy Annual Meeting, Washington, D.C. June, 2016. *(Outstanding Poster Presentation Award)*

31. N. Saad, S. Garwick-Coppens, and **Scott Q. Harper**. miR-675 reduces DUX4 expression and confers resistance to DUX4 toxicity in FSHD myoblasts: a framework to define the DUX4-targeted miRNome. Presented at FSH Society International Research Meeting, Boston, MA, November 2016. (oral presentation)

32. J.O. Eidahl, M.E. Hoover, O.E. Branson, L. Zhang, M. Freitas, **S.Q. Harper**. Protein Chemistry of DUX4. Presented at FSH Society International Research Meeting, Boston, MA, November 2016. (oral presentation)

33. J.O. Eidahl, C.R. Giesige1,J.S. Domire, L.M. Wallace, A. M. Fowler, S.M. Guckes, S.E. Garwick-Coppens, P. Labhart, **S.Q. Harper**. Mouse Dux is myotoxic and shares partial functional homology with its human paralog DUX4. Presented at the FSHD Wellstone Meeting, Worcester, MA September 2016. (*oral presentation*)

34. J.O. Eidahl, M.E. Hoover, O.E. Branson, L. Zhang, M.Freitas, **S.Q. Harper**. Protein Chemistry and Protein-Protein Interactions of DUX4. Presented at the NCH Research Retreat 2016.

35. **S.Q. Harper**. DUX4 inhibition as a therapeutic strategy for FSHD. New Directions in Skeletal Muscle Biology Meeting, Orlando, FL, June 29 – July 2, 2016. (invited talk)

36. Carlee R. Giesige, Jacqueline Domire, Lindsay M. Wallace, Diana Mukweyi, Kristin Heller, Susan Guckes, Sara Coppens, Louise Rodino-Klapac and **S.Q. Harper**. Characterization of an Inducible DUX4 Mouse Model for FSHD. Presented at FSH Society International Research Meeting, Boston, MA, November 2016.

37. **S.Q. Harper**, "DUX4 inhibition as a therapeutic strategy for FSHD." Presented at IGBMC, Strasbourg, France, September 2015. (Invited talk)

38. L.M. Wallace, J.S. Domire, C.R. Giesige, and **S.Q. Harper**. Toxicology for DUX4-targeted microRNAs. Presented at the FSH Society International Research Consortium, Boston, MA, October 2015.

39. E. Ansseau, J.O. Eidahl, C. Lancelot, A. Tassin, C. Matteotti, C. Yip,, J. Liu, B. Leroy, C. Hubeau, C. Gerbaux. S. Cloet, A Wauters, S. Zorbo, P. Meyer, I. Pirson, D. Laoudj-Chenivesse, R. Wattiez, **S.Q. Harper**, A. Belayew, and F. Coppee. The Translocation of DUX4 and DUX4c during myoblast differentiation allows their association with

nucleo-cytoplasmic proteins associated with mRNP granules. Presented at the FSH Society International Research Consortium, Boston, MA, October 2015

40. J.O. Eidahl, C.R. Giesige, J.S. Domire, L.M. Wallace, A. Fowler, S. Guckes, and **S.Q. Harper**. Defining the functional overlap between mouse Dux and human DUX4. Presented at the FSH Society International Research Consortium, Boston, MA October 2015.

41. J.O. Eidahl and **S.Q. Harper.** Protein chemistry and protein-protein interactions of DUX4. Presented at the FSH Society International Research Consortium, Boston, MA October 2015.

42. **S.Q. Harper**, "Understanding pathogenesis and developing therapies for FSHD." Presented at The University of Missouri, Department of Molecular Microbiology and Immunology, Columbia, MO, Feb 23, 2016. (Invited talk)

43. **S.Q. Harper**, "DUX4-targeted RNAi therapy for FSHD." Presented at the Friends of FSH Research Summit, Portland, OR, February 29-March 1, 2016. (Invited talk)

44. **S.Q. Harper,** "Translating FSHD." Presented at the Children's Hospital of Pennsylvania/University of Pennsylvania (CHOP/UPENN) Gene Therapy and Vaccines section, April 4, 2016.

45. American Society of Gene and Cell Therapy, Washington D.C. May 4-7, 2016. (**SQ Harper**, ASGCT faculty; 2 posters).

46. New Directions in Biology and Disease of Skeletal Muscle Conference, Orlando, FL, June 29-July 2. (**SQ Harper**, invited speaker)

47. K.H. Morelli, J.S. Domire, N. Pyne, **S.Q. Harper**, and R.W. Burgess. The use of humanized mouse models to validate disease association of a de novo GARS variant and to test a novel gene therapy strategy for CMT2D. To be presented at the Human Genome meeting, Feb 28 – March 2, 2016, Houston, TX.

48. L.M. Wallace, J.S. Domire, C.R. Giesige, and **S.Q. Harper**. Toxicology for DUX4-targeted microRNAs. Presented at the FSH Society International Research Consortium, Boston, MA, October 2015. ***Oral presentation***

49. E. Ansseau, J.O. Eidahl, C. Lancelot, A. Tassin, C. Matteotti, C. Yip,, J. Liu, B. Leroy, C. Hubeau, C. Gerbaux. S. Cloet, A Wauters, S. Zorbo, P. Meyer, I. Pirson, D. Laoudj-Chenivesse, R. Wattiez, **S.Q. Harper**, A. Belayew, and F. Coppee. The Translocation of DUX4 and DUX4c during myoblast differentiation allows their association with nucleo-cytoplasmic proteins associated with mRNP granules. Presented at the FSH Society International Research Consortium, Boston, MA, October 2015. ***Oral presentation***

50. J.O. Eidahl, C.R. Giesige, J.S. Domire, L.M. Wallace, A. Fowler, S. Guckes, and **S.Q. Harper**. Defining the functional overlap between mouse Dux and human DUX4. Presented at the FSH Society International Research Consortium, Boston, MA October 2015. ***Oral presentation***

51. J.O. Eidahl and S.Q. Harper. Protein chemistry and protein-protein interactions of DUX4. Presented at the FSH Society International Research Consortium, Boston, MA October 2015. ***Oral presentation***

52. K.H. Morelli, L. Griffin, J.S. Domire, N. Pyne, A. Fowler, **S.Q. Harper**, A. Antonellis, R.W. Burgess. Validation of disease association of a de novo GARS variant and development of a "humanized" mouse model for preclinical studies. (2015). Presented at the Peripheral Nerve Society Meeting, Quebec City, Canada, June 28 – July 2, 2015.

53. L.M. Wallace, J. Liu, S.E. Garwick-Coppens, S.M. Guckes, C. Smith, J. McBride and **S.Q. Harper**. The DUX4 promoter mouse: the next generation. Presented at the 19th International Congress of the World Muscle Society, Berlin, Germany, October 2014.

54. J.O. Eidahl, J. Liu, and S.Q. Harper. "Protein Chemistry and Protein-Protein Interactions of DUX4." Presented at the 2014 FSH Society International Research Consortium, San Diego, CA, Oct 17-18, 2014. ***Oral presentation.***

55. L.M. Wallace, J.S. Domire, and S.Q. Harper. "Developing a DUX4-targeted RNAi-based gene therapy for FSHD". Presented at the 2014 FSH Society International Research Consortium, San Diego, CA, Oct 17-18, 2014. ***Oral presentation.***

56. L.M. Wallace, J. Liu, S.E. Garwick-Coppens, S.M. Guckes, C. Smith, Y. Krom, S. van der Maarel, J. McBride and **S.Q. Harper**. "The DUX4 promoter mouse: an updated characterization." Presented at the 2013 Facioscapulohumeral Society International Research Consortium Meeting, Cambridge, MA, Oct 21, 2013. ***Oral presentation.***

57. J.S. Domire, L.M. Wallace, S.M. Guckes, and **S.Q. Harper**. "DUX4 regulates expression of the pro-apoptotic gene, p63." Presented at the 2013 Facioscapulohumeral Society International Research Consortium Meeting, Cambridge, MA, Oct 21, 2013.

58. L.M. Wallace, J. Liu, S.E. Garwick-Coppens, S.M. Guckes, and **S.Q. Harper**. The DUX4 promoter is expressed in FSHD-affected tissues. Presented at the 18th International Congress of the World Muscle Society, Asilomar Conference Grounds, California, USA, October 2013.

59. S-R.A. Hussain, L.M. Wallace, D. Griffin, C. Montgomery, **S.Q. Harper**, Z. Sahenk, and K.R. Clark. "Viral mediated acute mouse model for DM1." Presented at the 16th Annual Meeting of the American Society of Gene and Cell Therapy, Salt Lake City, UT, May 2013.

60. L.M. Wallace, A. Moreo, K.R. Clark, and **S.Q. Harper**. "Dose dependent toxicity of hrGFP limits its utility as a reporter gene in mouse muscle." Presented at the 16th Annual Meeting of the American Society of Gene and Cell Therapy, Salt Lake City, UT, May 2013.

61. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, D. Sloboda, C. Cavis, C.H. Hakim, M.E. Hauser, J.R. Mendell, S.V. Brooks, and **S.Q. Harper**. "RNAi therapy for LGMD1A." Presented at the 16th Annual Meeting of the American Society of Gene and Cell Therapy, Salt Lake City, UT, May 2013. ***Oral presentation.***

62. J.S. Domire, L.M. Wallace, S.M. Guckes, J. Liu, and **S.Q. Harper**. "DUX4 regulates expression of the pro-apoptotic gene P63." Presented at the 2013 Muscular Dystrophy Association Scientific Conference, Washington, D.C. April 21-24, 2013.

63. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, D. Sloboda, C. Cavis, C.H. Hakim, M.E. Hauser, J.R. Mendell, S.V. Brooks, and **S.Q. Harper**. "RNAi-mediated gene silencing of MYOT improves histopathology and whole muscle function in a mouse model of LGMD1A." Presented at the 2013 Muscular Dystrophy Association Scientific Conference, Washington, D.C. April 21-24, 2013.

64. L.M. Wallace, J. Liu, S.E. Garwick-Coppens, S.M. Guckes, and **S.Q. Harper**. "The DUX4 promoter is expressed in FSHD-affected tissues." Presented at the 2013 Muscular Dystrophy Association Scientific Conference, Washington, D.C. April 21-24, 2013.

65. E. Ansseau, C. Vanderplanck, L.M. Wallace, A. Tassin, J.S. Domire, S.M. Guckes, C. Yip, D. Laoudj-Chenivesse, F. Coppee, S. Wilton, **S.Q. Harper** and A. Belayew. "Evaluation of new antisense oligomers targeting the DUX4

mRNA as a therapeutic strategy for FSHD." Presented at the 2013 Muscular Dystrophy Association Scientific Conference, Washington, D.C. April 21-24, 2013. ***Oral presentation.***

66. L.M. Wallace, J. Liu, S.E. Garwick-Coppens, and **S.Q. Harper**. "The DUX4 promoter is expressed in FSHD-affected tissues". Presented at the 2012 Facioscapulohumeral Society International Research Consortium Meeting, San Francisco, CA, Nov 6, 2012.

67. J. Domire, L.M. Wallace, S.M. Guckes, J. Liu, and **S.Q. Harper**. "DUX4 regulates expression of the pro-apoptotic gene, p63". Presented at the 2012 Facioscapulohumeral Society International Research Consortium Meeting, San Francisco, CA, Nov 6, 2012.

68. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, C. Davis, S.V. Brooks, M.A. Hauser, J.R. Mendell, and **S.Q. Harper**. "RNAi therapy for dominant LGMD1A". Presented at the 5th Biennial New Directions in Biology and Disease of Skeletal Muscle Conference, New Orleans, LA, June 17-21, 2012.

69. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, M.A. Hauser, J.R. Mendell, and **S.Q. Harper**. "RNAi therapy for dominant LGMD1A". Presented at the 15th Annual Meeting of the American Society and Gene and Cell Therapy, Philadelphia, PA, May 2012.

70. L.M. Wallace, J. Liu, J.S. Domire, S.E. Garwick-Coppens, and **S.Q. Harper**. "RNAi inhibits DUX4-induced muscle toxicity in vivo: implications for a targeted FSHD therapy". Presented at the 15th Annual Meeting of the American Society and Gene and Cell Therapy, Philadelphia, PA, May 2012. **Trainee travel award winner.**

71. L.M. Wallace, J. Liu, and **S.Q. Harper**. The DUX4 promoter is preferentially expressed in FSHD-affected tissues. Presented at the 2012 Muscular Dystrophy Association Clinical Conference, Las Vegas, NV, March 4-7, 2012. **Award winner – top abstract (1 of 4 awardees).**

72. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, D. Nelson, C. Davis, S.V. Brooks, M.E. Hauser, J.R. Mendell, and **S.Q. Harper**. RNAi therapy for dominant LGMD1A. Presented at the 16th International Congress of the World Muscle Society, Almancil, Algarve, Portugal, Oct 18-22, 2011. ***Oral presentation.***

73. L.M. Wallace, S.E. Garwick-Coppens, J. Liu, J.S. Domire, and **S.Q. Harper**. RNA interference inhibits DUX4-induced muscle toxicity in vivo: Implications for a targeted FSHD therapy. Presented at the 16th International Congress of the World Muscle Society, Almancil, Algarve, Portugal, Oct 18-22, 2011. ***Oral presentation.***

74. **S.Q. Harper.** RNAi therapy for dominant muscular dystrophies. Presented at the 2nd RNAi Research and Development Conference, San Francisco, CA, July7-8, 2011. ***Oral presentation.***

75. J. Liu, L.M. Wallace, S.E. Garwick-Coppens, M.A. Hauser, J.R. Mendell, and **S.Q. Harper**. RNAi therapy for dominant LGMD1A. Poster presentation at the 14th Annual American Society of Gene and Cell Therapy Meeting, Seattle, WA, May 18-21, 2011.

76. L.M. Wallace, S.E. Garwick-Coppens, and **S.Q. Harper**. RNA Interference improves myopathic phenotypes in mice over-expressing FRG1. Presented at the 14 Annual American Society of Gene and Cell Therapy Meeting, Seattle, WA, May 18-21, 2011. ***Oral presenation and Travel Award Winner.***

77. E. Meadows, L.M. Wallace, K. Flanigan, J.R. Mendell, and **S.Q. Harper**. FSHD pathogenesis and RNAi-based therapy development for dominant muscular dystrophies. Presented at the Muscular Dystrophy Association National Scientific Conference, Las Vegas NV, March 13-16, 2011. **Award winner – top abstract (1 of 5 awardees).**

78. S. E. Garwick, L. M. Wallace, and **S.Q. Harper**. Developing RNAi therapy for FSHD candidate genes. Presented at the 60th Annual American Society of Human Genetics meeting, Washington, D.C. November, 2010.

79. L.M. Wallace, S.E. Garwick, W. Mei, A. Belayew, F. Coppee, K. Ladner, D. Guttridge, J. Yang, and **S.Q. Harper**. DUX4 over-expression recapitulates FSHD-associated phenotypes in vivo. Presented at the 60th Annual American Society of Human Genetics meeting, Washington, D.C. November, 2010. ***Oral presentation. Trainee Award Winner.***

80. **S.Q. Harper.** A vector-based approach to understand FSHD pathogenesis and develop potential RNAi therapies. Presented at the XII International Congress on Neuromuscular Diseases (ICNMD), Naples, Italy, July, 2010. ***Oral presentation.***

81. L.M. Wallace, S.E. Garwick, W. Mei, A. Belayew, J. Yang, and **S.Q. Harper**. DUX4 promotes FSHD-associated pathology *in vivo*. Presented at the Facioscapulohumeral Muscular Dystrophy 2009 International Research Consortium Meeting, Watertown, MA, November 2009. ***Oral presentation.***

82. S.E. Garwick, L.M. Wallace, and **S.Q. Harper.** RNAi therapy for FSHD. Presented at the 59th Annual Meeting of the American Society of Human Genetics, Honolulu, HI, October 2009. ***Oral presentation.***

83. L.M. Wallace, S.E. Garwick, W. Mei, A. Belayew, J. Yang, and **S.Q. Harper**. DUX4 promotes FSHD-associated pathology in vivo. Presented at the 59th Annual Meeting of the American Society of Human Genetics, Honolulu, HI, October 2009.

84. L.M. Wallace, S.E. Garwick, W. Mei, J. Yang, and **S.Q. Harper**. Developing RNAi therapy for FSHD. Presented at the American Society for Gene Therapy Annual Meeting, San Diego, CA, May-June 2009. ***Oral presentation.***

85. L.M. Wallace, S.E. Garwick, and **S.Q. Harper**. DUX4 causes muscle toxicity *in vivo*. Presented at the FSH Society Annual Meeting, Philadelphia, PA, October 2008. ***Oral presentation.***

86. S.E. Garwick, J.L. Allen, L.M. Wallace, J.A. Torres, R. Tupler, and **S.Q. Harper**. RNAi Targeting of FRG1: A Potential Therapy for Facioscapulohumeral Muscular Dystrophy. Presented at the American Society of Gene Therapy, 11th Annual Meeting, Boston, MA, May 31-June 4, 2008.

87. J.L. McBride, R.L. Boudreau, **S.Q. Harper**, P.D. Staber, A. Mas-Monteys, I.H. Martins, B. Polisky, B.J. Carter, and B.L. Davidson. RNA interference as a potential therapy for Huntington's disease. Keystone Symposia on RNAi, MicroRNA, and Non-Coding RNA. Whistler, British Columbia, Canada, March 25-30, 2008.

88. S.K. Fineberg, B.J. He, **S.Q. Harper**, and B.L. Davidson. Mir-34a may promote neural fate commitment in adult murine neural progenitor cells. Society for Neuroscience, 37th Annual Meeting, San Diego, CA, November 7, 2007.

89. A. Mas-Monteys, **S.Q. Harper**, B.L. Gilmore, P. Staber, C. Schaffer, B. Polisky, C. Vargesse, and B.L. Davidson. Allele-specific silencing of mutant huntingtin for Huntington's Disease therapy. American Society of Gene Therapy 10th Annual Meeting, Seattle, WA, May-June 2007.

90. R.L. Boudreau, A. Mas-Monteys, **S.Q. Harper**, and B.L. Davidson. In vitro and in vivo evaluation of shRNAs and miRNA shuttles for therapeutic RNAi. American Society of Gene Therapy 10th Annual Meeting, Seattle, WA, May-June 2007.

91. J.L. McBride, **S.Q. Harper**, P.D. Staber, I. Martins, H. Burstein, R.W. Peluso, B. Polisky, B. Carter, and B.L. Davidson. Viral delivery of shRNAs as a potential therapy for Huntington's Disease. American Society of Gene Therapy 10th Annual Meeting, Seattle, WA, May-June 2007.

92. S.K. Fineberg, **S.Q. Harper**, and B.L. Davidson. Brain-expressed microRNAs modulate neural progenitor cell proliferation and differentiation in culture. Society for Neuroscience 36th Annual Meeting, Atlanta, GA, 2006.

93. **S.Q. Harper**, P.D. Staber, A. Mas Monteys, B.L. Gilmore, and B.L. Davidson. Optimization of RNAi for HD Therapy. Hereditary Disease Foundation HD2006 Meeting: Changes, Advances, and Good News, Cambridge, MA, August 11-13, 2006.

94. P.D. Staber, C. Vargeese, I. Martins, B. Polisky, A. Mas Monteys, **S.Q. Harper**, and B.L. Davidson. Synthetic siRNAs for Huntington's disease therapy. Hereditary Disease Foundation HD2006 Meeting: Changes, Advances, and Good News, Cambridge, MA, August 11-13, 2006.

95. J.L. McBride, **S.Q. Harper**, P.D. Staber, I.H. Martins, H. Burstein, R.W. Peluso, B. Polisky, B.J. Carter, and B.L. Davidson. Non-allele specific RNA interference in the CAG140 knock-in mouse model of Huntington's disease. Hereditary Disease Foundation HD2006 Meeting: Changes, Advances, and Good News, Cambridge, MA, August 11-13, 2006.

96. A. Mas Monteys, **S.Q. Harper**, B.L. Gilmore, P.D. Staber, C. Schaffer, B. Polisky, C. Vargeese, and B.L. Davidson. Allele-specific silencing of mutant huntingtin for Huntington's disease therapy. Hereditary Disease Foundation HD2006 Meeting: Changes, Advances, and Good News, Cambridge, MA, August 11-13, 2006.

97. **S.Q. Harper**, P.D. Staber, C. Rowley, S. Fineberg, D. Ochoa, C. Stein, and B.L. Davidson. Optimization of feline immunodeficiency viral vectors for RNA interference. American Society of Gene Therapy 9th Annual Meeting, Baltimore, MD, May 31-June 4, 2006. *Molecular Therapy* 2006; 13(S): S141. ***Oral Presentation***.

98. J.L. McBride, **S.Q. Harper (presenting author)**, P.D. Staber, I.H. Martins, H. Burstein, R.W. Peluso, B. Polisky, B.J. Carter, and B.L. Davidson. Non-allele specific RNA interference in the CAG140 knock-in mouse model of Huntington's disease. American Society of Gene Therapy 9th Annual Meeting, Baltimore, MD, May 31-June 4, 2006. *Molecular Therapy* 2006; 13(S): S159. ***Oral Presentation***.

99. P.D. Staber, C. Vargeese, I.H. Martins, B. Polisky, A. Mas-Monteys, **S.Q. Harper**, B.L. Davidson. Synthetic siRNAs for Huntington's Disease Therapy. American Society of Gene Therapy 9th Annual Meeting, Baltimore, MD, May 31-June 4, 2006. *Molecular Therapy* 2006; 13(S): S36.

100. A. Mas-Monteys, **S.Q. Harper**, B.L. Gilmore, P.D. Staber, C. Schaffer, B. Polisky, C. Vargeese, and B.L. Davidson. Allele-specific silencing of mutant huntingtin for Huntington's disease therapy. American Society of Gene Therapy 9th Annual Meeting, Baltimore, MD, May 31-June 4, 2006. *Molecular Therapy* 2006; 13(S): S274.

101. **S.Q. Harper**, P.D. Staber, C.R. Rowley and B.L. Davidson. Huntingtin-specific RNA-interference using feline immunodeficiency viral vectors. The Society for Neuroscience 35th Annual Meeting, Washington, D.C., November 12-16, 2005.

102. **S.Q. Harper**, P.D. Staber, X. He, S.L.Eliason, I.H. Martins, Q. Mao, L. Yang, H.L. Paulson, R.M. Kotin, and B.L. Davidson. AAV-delivered RNAi improves cellular and motor phenotypes in a mouse model for Huntington's disease. American Society of Gene Therapy 8th Annual Meeting, St. Louis, MO, June 2-6, 2005. ***Oral presentation***.

103. **S.Q. Harper**, P.D. Staber, X. He, S.L. Eliason and B.L. Davidson. Creating a gold-standard model for RNAi-based HD gene therapy. Hereditary Disease Foundation HD2004 Meeting: Changes, Advances, and Good News, Cambridge, MA, August 12-15, 2004.

104. **S.Q. Harper,** P.D. Staber, S.L. Eliason, X. He, I.H. Martins, Q. Mao, H.L. Paulson, R.M. Kotin, and B.L. Davidson. AAV-delivered RNAi causes cellular and motor improvements in a mouse model for HD. The Society for Neuroscience 34th Annual Meeting, San Diego, CA, October 23-27, 2004.

105. P.D. Staber, **S.Q. Harper**, S.L. Eliason, C.R. Rowley, X. He, I.H. Martins, Q. Mao, H. Paulson, P. Gonzalez-Alegre, and B.L. Davidson. Huntingtin gene silencing by lentivirus-delivered shRNA leads to phenotypic improvement in an HD mouse model. The Society for Neuroscience 34th Annual Meeting, San Diego, CA, October 23-27, 2004.

106. R.L. Boudreau, H. Xia, **S.Q. Harper**, W.T. Talman, and B.L. Davidson. Inducible RNAi-therapy for polyglutamine disease. The Society for Neuroscience 34th Annual Meeting, San Diego, CA, October 23-27, 2004.

107. **S.Q. Harper**, P.D. Staber, C.R. Rowley, X. He, I.H. Martins, Q. Mao, H.L. Paulson, and B.L. Davidson. Gene silencing of human huntingtin using lentivirus-delivered shRNA. The American Society of Gene Therapy 7th Annual Meeting, Minneapolis, MN, June 2-6, 2004. *Molecular Therapy* 2004 9(S): S80.

108. H. Xia, Q. Mao, S.L. Eliason, N. Kiewiet, J. Critchfield, I.H. Martins, **S.Q. Harper**, X. He, R.M. Kotin, H.Y. Zoghbi, H.T. Orr, H.L. Paulson, and B.L. Davidson. RNAi therapy for dominant neurodegenerative diseases. The American Society of Gene Therapy 7th Annual Meeting, Minneapolis, MN, June 2-6, 2004. *Molecular Therapy* 2004 9(S): S273.

109. **S.Q. Harper**, P.D. Staber, S.K. Fineberg, H.L. Paulson, and B.L. Davidson. Silencing huntingtin with siRNA. The American Society of Gene Therapy 6th Annual Meeting, Washington. DC, June 4-8, 2003. *Molecular Therapy* 2003 7(S): S317. ***Oral presentation***.

110. Y. Yue, Z. Li, **S.Q. Harper,** R.L. Davisson, J.S. Chamberlain, and D. Duan. Microdystrophin gene therapy of cardiomyopathy in *mdx* mice. The American Society of Gene Therapy 6th Annual meeting, Washington, DC. June 4-8, 2003. *Molecular Therapy* 2003, 7(S): S319.

111. **S.Q. Harper**, M.J. Blankinship, D. Duan, H.A. Harper, R.W. Crawford, C.L. Halbert, J.F. Engelhardt, A.D. Miller, and J.S. Chamberlain. Progress toward gene therapy of Duchenne muscular dystrophy using truncated four-repeat dystrophin and AAV6. The American Society of Gene Therapy 5th Annual Meeting, Boston, MA. ***Oral presentation.***

112. Z. Li, Y. Yue, **S.Q. Harper**, J.F. Engelhardt, R.L. Davisson, J.S. Chamberlain, and D. Duan. AAV-mediated micro-dystrophin expression restores dystrophin glycoprotein complex (DGC) in both the heart and diaphragm of the *mdx* mouse. The American Society of Gene Therapy 5th Annual Meeting, Boston, MA.

113. S.D. Hauschka, S. Abmayr, **S.Q. Harper**, H.A. Harper, S. Li, R.W. Crawford, J.M. Scott, M.T. Little, and J.S. Chamberlain. Dystrophin expression in cultured canine DMD skeletal muscles using mouse mck gene regulatory cassettes. The Xth Annual Congress on Neuromuscular Diseases, Vancouver, British Columbia, Canada, July 7-12, 2002.

114. **S.Q. Harper**, C. DelloRusso, R.W. Crawford, H.A. Harper, J.F. Engelhardt, D. Duan, and J.S. Chamberlain. Delivery of functional four-repeat micro-dystrophin to *mdx* muscle via different adeno-associated virus serotypes. The American Society of Human Genetics 51st Annual Meeting, San Diego, CA, Oct 12-16; *American Journal of Human Genetics* 2001.

115. **S.Q. Harper**, C. DelloRusso, R.W. Crawford, H.A. Harper, and J.S. Chamberlain. Functional correction of muscular dystrophy by four-repeat micro-dystrophins. Society of Gene Therapy 4th Annual Meeting, Seattle, WA, May 30-June 3; *Molecular Therapy* 2001; 3(S): S140. ***Oral presentation***.

116. **S.Q. Harper**, R.W. Crawford, C. DelloRusso, and J.S. Chamberlain. Prevention of muscular dystrophy in *mdx* mice by four-repeat micro-dystrophins. American Society of Human Genetics 50th Annual Meeting, Philadelphia, PA, Oct 6, 2000; *American Journal of Human Genetics* 2000; 67(S); 429. ***Pre-Doctoral Student Award Finalist.***

117. G. Crawford, C.N. Lumeng, **S.Q. Harper**, T. Hutchinson, S. Phelps, and J.S. Chamberlain. Co-localization of PDZ-domain proteins with utrophin and dystrophin at the neuromuscular junction. American Society of Human Genetics 48th Annual Meeting, Denver, CO, Oct. 27-31; *American Journal of Human Genetics* 1998; 63(S):A178.

INVITED SYMPOSIA, WORKSHOPS, and TALKS

1. Invited participant, Industry Collaborative for Therapy Development in FSHD, Organized by the FSH Society, Silver Spring, MD, March 12, 2019.

2. **S.Q. Harper.** Emerging therapeutic approaches for FSHD. Invited talk, to be presented at the inaugural international meeting of FSHD patient advocacy groups, hosted by the FSH Society, June 18, 2019, Marseille, France

3. **S.Q. Harper.** Emerging therapeutic approaches for FSHD. Invited keynote talk, Presented at the 23rd World Muscle Society Congress, October 5, 2018, Mendoza, Argentina. Oral presentation.

4. **S.Q. Harper,** Molecular Therapies for FSHD, Kansas City FSHD Family Day Conference, Sponsored by the KU Medical Center and the FSH Society, Nov 10, 2018, Overland Park, KS.

5. **S.Q. Harper**, FSHD Therapies 101, 2018 FSHD Connect meeting, Las Vegas, NV, June 9, 2018.

6. **S.Q. Harper,** The Pipeline for FSHD Therapies, Targeting DUX4 mRNA, 2018 FSHD Connect Meeting, Las Vegas, NV, June 9, 2018.

7. **S.Q. Harper**, Science Week Participant (Patient Outreach), FSHD Global Research Foundation, Touring Auckland, NZ; Brisbane, Sydney, Perth and Melbourne, Australia, September, 2017.

8. **S.Q. Harper,** Developing Models and Molecular Therapies for FSHD, Grand Rounds, University of Kansas Medical Center, August 17-18, 2017.

9. **S.Q. Harper**, "DUX4 Inhibition as a therapeutic strategy for FSHD", Presented at the New Directions in Skeletal Muscle Biology Meeting, Orlando, FL, June 29-July 2, 2016.

10. **S.Q. Harper**, "Translating FSHD". Presented at the Center for Gene Therapy, Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, April 4, 2016.

11. **S.Q. Harper, "**Progress toward RNAi therapy for FSHD", FSH Research Summit, Portland, OR, Feb 29-Mar 1, 2016.

12. **S.Q. Harper,** Panel Member, Discussion of Therapeutic Strategies for FSHD, FSH Society Patient Connect Meeting, November 11, 2016.

13. **S.Q. Harper**, "DUX4 inhibition as a therapeutic strategy for FSHD." Presented at IGBMC, Strasbourg, France, September 2015.

14. **S.Q. Harper**, "Developing mouse models to understand the mechanisms of DUX4-associated muscle damage in FSHD", Presented at the 2015 Muscular Dystrophy Association Scientific Conference, Washington, D.C., March 13, 2015.

15. **S.Q. Harper**, "AAV DUX4 models of FSHD pathophysiology", Presented at the UMMS Wellstone Center for FSH Muscular Dystrophy Research, 2015 Retreat, Worcester, MA March 4, 2015.

16. **S.Q. Harper**, "Understanding Disease Mechanisms and Developing Therapies for FSHD", Presented at Indiana University, Department of Medical and Molecular Genetics, School of Medicine, February 25, 2015.

17. **S.Q. Harper**, "Mouse models for FSHD and preclinical testing", Presented at the New Directions in Biology and Disease of Skeletal Muscle Conference, Chicago, IL, July 1, 2014.

18. **S.Q. Harper**, "Translating Facioscapulohumeral muscular dystrophy". Outstanding New Investigator Talk at the American Society of Gene and Cell Therapy 17th Annual meeting, Washington, DC, May 23, 2014.

19. **S.Q. Harper**, "Translating FSHD". Presented at Mayo Clinic, Rochester, MN, April 2014.

20. **S.Q. Harper**, "Translating FSHD". Presented at Northwestern University, Department of Pathology, September 16, 2013, Chicago, IL.

21. **S.Q. Harper**, "Startup Funds Don't Last Forever: A Junior PI's Path Toward Funding and Growing a New Lab." Presented at The American Society of Gene and Cell Therapy 16th Annual Meeting, Education Session 123 entitled Navigating a Difficult Funding Climate, May 15, 2013, Salt Lake City, UT.

22. **S.Q. Harper,** Invited Expert Panelist and Presenter, NIH Workshop entitled "Gene Therapy: Charting a Future Course." April 12, 2013, Bethesda, MD.

23. **S.Q. Harper**, "DUX4 inhibition as a therapeutic strategy for FSHD." Presented at the meeting of the FSHD Global Society, Perth, Australia, October 2012.

24. **S.Q. Harper**, "Translating Facioscapulohumeral muscular dystrophy." Presented at the Center for Gene Therapy Seminar Series, University of Iowa Carver College of Medicine, Iowa City, IA, June 25, 2012.

25. **S.Q. Harper**, "Translating Facioscapulohumeral muscular dystrophy." Presented at Department of Neurology Grand Rounds, University of Iowa Carver College of Medicine, Iowa City, IA, June 26, 2012.

26. **S.Q. Harper**, "Therapeutic applications of regulatory RNAs." Education Session #113, Presented at the 15th Annual Meeting of the American Society of Gene and Cell Therapy, Philadelphia, PA, May 16, 2012.

27. **S.Q. Harper**, "Potential strategies for FSHD." Muscular Dystrophy Association Clinical Conference, Las Vegas, NV, March 4-7, 2012.

28. **S.Q. Harper**, "Translating Facioscapulohumeral Muscular Dystrophy." Department of Biological Sciences, Wright State University, Dayton, OH, February 13, 2012.

29. **S.Q. Harper**, "DUX4 inhibition as a therapeutic strategy for FSHD." Wellstone Cooperative Muscular Dystrophy Research Center Meeting. October 13-14, 2011. Columbus, OH.

30. **S.Q. Harper**, "DUX4 as an FSHD candidate gene and target of RNAi therapy." Department of Neurology, University of Michigan Medical School, Ann Arbor, MI, March 11, 2011.

31. **S.Q. Harper,** Invited participant, "This Month in Muscular Dystrophy Podcast", February 2011. https://itunes.apple.com/us/podcast/dr.-scott-harper-discusses/id502589471?i=126963973&mt=2

32. **S.Q. Harper**, "DUX4 as Candidate Gene for FSHD." Wellstone Cooperative Muscular Dystrophy Research Center Meeting. October 7, 2010. Philadelphia, PA.

33. **S.Q. Harper**, "FSHD mechanistic models and DUX4 pathogenesis." 2010 FSH Society International Patient and Researcher Network Meeting. July 30 – August 1, 2010. Las Vegas, NV.

34. **S.Q. Harper,** Invited Expert Panelist, National Institutes of Health meeting on "Proposed Revisions to the NIH Guidelines for Research Involving Recombinant DNA Molecules". Panel II: Human Gene Transfer Involving Synthetic Nucleic Acids, Arlington, Virginia, June 23, 2009.

35. **S.Q. Harper**, Keynote Address: "Designer Nanomedicine: RNAi Therapeutics". 93rd Annual Meeting of the South Dakota Academy of Science, Chamberlain, SD, April 4, 2008.

36. **S.Q. Harper**, "Choosing the right inhibitory RNA". Presented at the Society for Neuroscience, 37th Annual Meeting, San Diego, CA, November 1, 2007.

37. **S.Q. Harper**, "RNAi-mediated gene therapy for polyglutamine repeat disorders". Presented at the American Society for Neurochemistry 37th Annual Meeting, Portland, OR, March 11-15, 2006. *Colloquium: "RNA interference in neurodegenerative disorders: from discovery to therapeutics".* Journal of Neurochemistry 2006; 96(S):14.

38. **S.Q. Harper**, "Targeting Huntington's disease: Clinical opportunities". Presented at the Lexington Conference on RNA Therapy for Neurodegenerative Disease, Lexington, KY, April 13, 2006.

39. **S.Q. Harper**, "RNAi Therapy: Potential applications for treating disease." Education Session #105; Presented at the American Society of Gene Therapy 9th Annual Meeting, Baltimore, MD, May 31, 2006.

40. **S.Q. Harper**, Workshop: "Gene Silencing." Presented at the 6th International Congress of Neuroendocrinology, Pittsburgh, PA, June 20, 2006.

GENERAL PROFESSIONAL ACTIVITIES

PROFESSIONAL SOCIETY MEMBERSHIPS

- American Society of Gene and Cell Therapy
- American Society of Human Genetics
- World Muscle Society

COMMITTEE, ADMINISTRATIVE AND OTHER SERVICE

- Admissions Committee, Discovery Prep Post-Baccalaureate Program, The Ohio State University, 2019
- Chair, ASGCT Oligonucleotide and RNAi Committee 2018-9
- Member, ASGCT Oligonucleotide and RNAi Committee 2016-2019
- Abstract Reviewer, "Musculoskeletal Gene Therapy", 2019 American Society of Gene and Cell Therapy Meeting, Washington, DC
- OSU Faculty Senator, 2014-2017
- Member, Scientific Advisory Board, Charcot-Marie-Tooth Association
- Member, Neuromuscular Scientific Advisory Panel, Roche Switzerland
- Grant Reviewer, Muscular Dystrophy UK
- Institutional Biosafety Committee member, 2014-present
- NCH Junior Faculty Awards Committee, 2015-2017.
- OSU CCTS L-Pilot Program Advisory Board, 2015-present

- OSU Faculty Hearing Committee, 2015-present
- OSU Faculty Senate (Senator, elected); 2014 – 2016
- Co-Chair, Employee Research Awards Committee, 2009-2012
- NCH Capital Equipment Budget Requests Committee (2012-2013)
- OSU Department of Pediatrics Promotion and Tenure Committee (2014-15)
- NCH Capital Equipment Budget Requests Committee (2012-2013)
- Abstract Reviewer, "Musculoskeletal Gene Therapy", 2016 American Society of Gene and Cell Therapy Meeting, Washington, DC
- Abstract Reviewer, "Clinical Translation of Vector Production and Protocol Preparation", 2015 American Society of Gene and Cell Therapy Meeting, New Orleans, LA, 2015.
- 2014 – 2016: Appointed Member, Promotion and Tenure Committee, Department of Pediatrics, The Ohio State University College of Medicine
- Poster Session judge, 2015 Muscular Dystrophy Association Scientific Conference, Washington, D.C., March 12, 2015.
- Organizer and Co-Moderator, Muscle Diseases – Mechanisms of Disease platform session, 2015 Muscular Dystrophy Association Scientific Conference, Washington, D.C., March 13, 2015.
- Organizer and Co-Moderator, Science Policy and Economics Education Session, The American Society of Gene and Cell Therapy, 17th Annual Meeting, May 21, 2014
- Poster Session judge, The American Society of Gene and Cell Therapy, 17th Annual Meeting, May 2014
- Co-Moderator, Platform Session 3 (FSHD Models), Facioscapulohumeral Society 2013 International Research Consortium, Cambridge, MA, October 21, 2013
- Invited ad hoc reviewer, NIH Recombinant DNA Advisory Committee (RAC), 134th Meeting, June 11-12, 2013, NIH campus, Bethesda, MD.
- Member, Oligonucleotide and RNAi Therapeutics Committee, American Society of Gene and Cell Therapy, 2013-2016.
- Co-Moderator, Education Session entitled "Navigating a Difficult Funding Climate", 16th Annual Meeting of the American Society of Gene and Cell Therapy, Salt Lake City, UT, May 2013.
- Co-Moderator, Oral Abstract Session on "Oligonucleotide and RNAi Therapeutics", 16th Annual Meeting of the American Society of Gene and Cell Therapy, Salt Lake City, UT, May 2013.
- Co-Moderator, Platform Session 4 (Therapy), Facioscapulohumeral Society 2012 International Research Consortium, San Francisco, CA, November 2012
- Abstract Review Committee, "Oligonucleotide therapy", American Society of Gene and Cell Therapy 16 Annual Meeting, Salt Lake City, UT
- Ad hoc Committee Member, Medical Advisory Committee, Muscular Dystrophy Association, 2012 – 2013
- Committee Member, Scientific Equipment Capital Budget Requests Review Committee, TRINCH, 2012 - 2013
- Co-Chair, Education Session #113, "Regulatory RNAs", 15th Annual Meeting of the American Society of Gene and Cell Therapy, Philadelphia, PA, May 16, 2012
- Scientific Advisor, 3rd RNAi Research and Development Conference, Boston, MA, May 30-31, 2012
- Co-Moderator, Platform Presentations, Facioscapulohumeral Society 2011 International Research Consortium and Research Planning Meeting, Watertown, MA, 2011
- Co-Moderator, Platform Presentations, Facioscapulohumeral Society 2011 International Research Consortium and Research Planning Meeting, Watertown, MA, November, 2010
- Co-Moderator, Platform Presentations and Breakout Session, Facioscapulohumeral Society 2010 International Research Consortium and Research Planning Meeting, Watertown, MA, October 21-22, 2010
- Member, Education Committee, American Society of Gene and Cell Therapy, 2010-2013
- Member, Junior Investigator Taskforce, American Society of Gene and Cell Therapy, 2010-11
- Abstract Review Committee, Musculoskeletal Gene and Cell Therapy Section, American Society of Gene and Cell Therapy (ASGCT) 13th Annual Meeting, Washington, D.C, 2010
- OSUMC Research Day, Poster Session Judge, 2009-2011

- Invited Expert Panelist, National Institutes of Health meeting on "Proposed Revisions to the NIH Guidelines for Research Involving Recombinant DNA Molecules". Panel II: Human Gene Transfer Involving Synthetic Nucleic Acids, Arlington, Virginia, June 23, 2009.
- Co-Chair, Employee Awards Committee, The Research Institute at Nationwide Children's Hospital, 2009-2012
- Co-Chair, "Oligonucleotide Therapies for Diseases of Muscle and Nerve", Oral Abstract Session, American Society of Gene Therapy, 11th Annual Meeting, Boston, MA 2008
- Co-Chair, "Muscle Gene Therapy: Systemic Delivery", Oral Abstract Session, American Society of Gene Therapy, 11th Annual Meeting, Boston, MA 2008
- Abstract Review Committee, Musculoskeletal Section, American Society of Gene Therapy 11th Annual Meeting, Boston, MA, 2008
- Radiation Safety Committee, The Research Institute at Nationwide Children's Hospital, 2007-2010
- Admissions Committee, University of Michigan Medical School Program in Biological Sciences (PIBS), 1998-2000
- Student Representative, Cell and Molecular Biology Program Committee, University of Michigan Medical School, 1998-2000
- Student Recruitment Planning Committee, Cell and Molecular Biology Program, University of Michigan Medical School, 1998-2000
- Co-Organizer, Fall CMB/Genetics Short Course (HG 630), "Diseases of Trinucleotide Repeat Expansion", University of Michigan Medical School, 1998

EDITORAL BOARDS / ABSTRACT REVIEW

- Associate Editor, Life Sciences (2007-2009)
- American Society for Gene Therapy, 11th Annual Meeting, Abstract Review Committee, Musculoskeletal Section, (2008)
- American Society for Gene and Cell Therapy, 13th Annual Meeting, Abstract Review Committee, Musculoskeletal Section (2010)
- American Society for Gene and Cell Therapy, 16th Annual Meeting, Abstract Review Committee, Oligonucleotide and RNAi therapy (2013)
- American Society for Gene and Cell Therapy, 18th Annual Meeting, Abstract Review Committee, Clinical Translation of Vector Production and Protocol Preparation (2015)
- Abstract Reviewer, Muscular Dystrophy Association, 2015 Scientific Conference, Muscle Diseases – Mechanisms of Disease (2015)
- American Society for Gene and Cell Therapy, 21st Annual Meeting, Abstract Review Committee, Musculoskeletal Section (2019)

AD HOC PEER REVIEWS

Scientific Journals

- Nature Communications (2016-8)
- Cell Reports (2014, 2017)
- PNAS (2012-7)
- Neurology (2011-2012, 2018)
- Journal of Clinical Investigation (2012, 2017)
- PLOS ONE (2011-3, 2017)
- PLOS Genetics (2016, 17)
- American Journal of Human Genetics (2011)

- Human Molecular Genetics (2011-2019)
- Human Genetics (2011)
- Neuromuscular Disorders (2010-2017)
- Journal of Molecular Biology (2010)
- Muscle and Nerve (2010, 2016)
- Molecular Therapy (2009, 2011-9)
- Molecular Therapy Nucleic Acids (2013-9)
- Gene Therapy (2016)
- Expert Opinion on Orphan Drug Dev (2016)
- BMC Medical Genetics (2017)
- Journal of Biotechnology (2009)
- Expert Opinion on Biological Therapies (2009)
- Human Gene Therapy (2008, 2011, 2017-8)
- Journal of Neuroscience Methods (2008)
- Neoplasia (2008)
- Expert Opinion on Therapeutic Patents (2008)
- Annals of Neurology (2008. 2011-2, 2017)
- Archives of Neurology (2008, 2011)
- American Journal of Pathology (2007-8)
- Life Sciences (2007)
- Biotechniques (2003-2005)
- Nucleic Acids Research (2005, 2010, 2013)
- FEBS Letters (2005-2006)

Funding Agencies and Other

- Grant Reviewer, Charcot-Marie-Tooth Association, 2019
- Member, Scientific Advisory Board, Charcot-Marie-Tooth Association
- Invited Reviewer, ASGCT Career Development Grants study section, October 2018.
- Ad hoc Study Section Member, NSD-B, NINDS Innovation Grants to Nurture Initial Translational Efforts (IGNITE) Program, October 2018
- Ad hoc Study Section member, NIH ZNS SRB-B (60) Special Emphasis Panel, Scientific Review Branch, NINDS, 2018
- Ad hoc Study Section member, NIH ZRG1 MOSS K(57) Special Emphasis Panel, Center for Scientific Review, 2017
- Ad hoc Study Section member, Department of Defense, Congressionally Directed Medical Research Programs, Musculoskeletal Disorders, Technology and Therapeutic Development Award, 2017.
- Ad hoc Study Section member, Department of Defense, Congressionally Directed Medical Research Programs, Duchenne Muscular Dystrophy Research Program - Mechanism, Detection, and Prognosis, 2016.
- Muscular Dystrophy UK, grant reviewer, 2016-2018
- Pennsylvania Department of Health, Grant Reviewer, 2016.
- Qatar National Research Fund, Grant Reviewer, 2015-16.
- U.S. Department of Defense, U.S. Army Medical Research and Materiel Command, Congressionally Directed Medical Research Programs, 2015 Duchenne Muscular Dystrophy Research Program (DMDRP) study section, ad hoc member
- NIH KL2 Program Study Section, The Ohio State University College of Medicine, March 6, 2015
- Invited ad hoc reviewer, NIH Recombinant DNA Advisory Committee (RAC), 134th Meeting, June 11-12, 2013, NIH campus, Bethesda, MD.
- Muscular Dystrophy Association, Medical Advisory Committee Ad Hoc Reviewer, 2012 - 3
- Association Française contre les Myopathies (2008-2012).
- MJ Murdock Charitable Trust Research Program for Life Sciences (2011).
- Jesse's Journey, The Foundation for Gene and Cell Therapy (2010).
- Medical Research Council, South Africa (2010).
- External Examiner for Ph.D. Thesis. Mr. Nicholas Franich, The University of Auckland, New Zealand, School of Graduate Studies (2009).
- National Research Foundation, South Africa (2008).
- North Carolina Biotechnology Center, Research Grant Program (2007).
- Auburn University BioGrant Program (2007).
- Medical Research Charities Group, Research Fellowships in Rare Diseases, Ireland (2005).

TRAINEES

- Afrooz Rashnonejad, Ph.D., Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital, Columbus, OH 43205, Advisor, 2017 - present
- Nizar Saad, Ph.D., Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital, Columbus, OH 43205, Advisor, 2015 – present
- Gholamhossein Amini-Chermahini, M.D. Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital, Columbus, OH 43205, Advisor, 2017 – present
- Mustafa Al-Kharsan, M.D. Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital, Columbus, OH 43205, Advisor, 2017 – present
- Jocelyn Eidahl, Ph.D., Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital, Columbus, OH 43205
  Advisor, 2013 – 2018. Current position: Licensing Associate, NCH Technology Transfer Office
- Carlee Giesige, B.S., Integrated Biomedical Sciences Graduate Program (IBGP), The Ohio State University College of Medicine, Thesis Adviser, 2013 – 2018. ***Earned PhD, 2018***. Current position: Post-doc, Beaumont Hospital, Royal Oak, Michigan
- Diana Mukweyi, B.S., Biomedical Sciences Graduate Program, Discovery Prep Post-Baccalaureate Program, The Ohio State University, Advisor, 2016-2017. Current position: Family leave.
- Juwan Copeland, B.S., Biomedical Sciences Graduate Program, Discovery Prep Post-Baccalaureate Program, The Ohio State University, Advisor, 2018-2019. Current position: Beginning a PhD program at Baylor in Fall 2019.
- Matthew Guggenbiller, High School and College Summer Student, 2017-present, Sophomore at University of Notre Dame.
- Alex Cash, Undergraduate Summer Student, 2017-present, Junior at Denison University, Ohio.
- Andrew Palo, High School Summer Student, 2015. Currently a Mechanical Engineering Student at OSU.
- Johanna Lee, High School Summer Student, 2018. Will attend The University of Michigan undergraduate in Fall 2019.
- Eugenie Ansseau, Ph.D., Visiting Post-Doctoral Fellow, University of Mons, Belgium, Research Advisor, 2012
- Cassandre Yip, Visiting Masters Student, Erasmus Program, University of Mons, Belgium, Research Advisor, 2012
- Jackie Domire, B.S., Integrated Biomedical Sciences Graduate Program (IBGP), The Ohio State University College of Medicine, Thesis Adviser, 2011 – 2013. ***Earned M.S. August 2013***. *Newly Appointed Director of the Psoriasis Foundation.*
- Lindsay Wallace, B.A., Molecular, Cellular, and Developmental Biology Graduate Ph.D. Program (MCDB), The Ohio State University College of Medicine (OSU COM), Thesis Advisor, 2008 – 2012. ***Earned Ph.D. May 2, 2012.*** Currently research scientist and program manager in the Harper lab.
- Jian Liu, Ph.D., Post-doctoral fellow, 2008-2012.
  - *Current position: Research Scientist at University of Illinois.*
- Kerry Powell, M.D., Physician Research Fellow, The Research Institute at Nationwide Children's Hospital, Department of Hematology and Oncology, Fellowship Advisor, 2008 – 2010.
  - *Current position: Private practice physician*
- Aaron Bruns, Undergraduate Researcher, The Ohio State University, Research Advisor, 2008 – 2009
  - *Current position: Ph.D. student in Biochemistry, The Ohio State University, Columbus, Ohio*
- Jorge Torres, Undergraduate Researcher, Capital University, Columbus, Ohio, Research Advisor, 2007 – 2008
  - *Current position: M.B.A. student, Capital University, Columbus, Ohio*

Co-mentor – Kathryn Morelli, University of Maine, Jackson Laboratories. Mentor: Dr. Robert Burgess.

PATENTS

- European Patent 12816988.5; EP2736539, **S.Q. H**arper, J. Liu, S. Garwick, L.M. Wallace, "Recombinant virus products and methods for inhibition of expression of DUX4". Issued Aug 23, 2017.
- Canadian Patent #2,596,588, **S.Q. H**arper and Beverly L. Davidson, "Nucleic Acid Silencing of Huntington's Disease Gene", Issue June 27, 2017.
- U.S. Patent #9,388,410. **S.Q. Harper** and Beverly L. Davidson, "Alternative Export Pathways for Vector Expressed RNA Interference", Issue July 12, 2016
- U.S. Patent #9,469,851. **S.Q. Harper**, J. Liu, S. Garwick-Coppens, L.M. Wallace. "Recombinant virus products and methods for inhibition of expression of DUX4." Issued October 18, 2016.
- U.S. Patent #9,133,482. **S.Q. Harper**, J. Liu. "Recombinant virus products and methods for inhibition of expression of Myotilin." Issued September 15, 2016.
- Australian Patent #2014202848**. S.Q. Harper** and B.L. Davidson, "Alternative export pathways for vector expressed RNA Interference", Issued April 28, 2016.
- Canadian Patent #2670967, **S.Q. Harper** and B.L. Davidson. Alternative Export Pathways for Vector Expressed RNA Interference", Issue July 12, 2016, Issued May 20, 2016.
- U.S. Patent #9,260,716, **S.Q. Harper**, Haibin Xia, Q. Mao, R. Boudreau, H. Paulson, B.L. Davidson, "RNA INTERFERENCE SUPPRESSION OF NEURODEGENERATIVE DISEASES AND METHODS OF USE THEREOF", Issued February 16, 2016.
- U.S. Patent No. 8,227,592. Inventors: **Harper, S.Q.** and Davidson, B.L. "Alternative export pathways for vector expressed RNA interference", Filed November 28, 2007; Issued July 24, 2012.
- European Patent No. 1366160. Inventors: Chamberlain J.S. and **Harper S.Q.** "Mini-dystrophin nucleic acid and peptide sequences". Filed Oct 4, 2001, Issued July 9, 2008.
- Singapore Patent No. 109361. Inventors: Davidson, B.L., Gonzalez-Alegre, P., Miller, V.M., Paulson, H.L., and **Harper, S.Q.** "Allele-specific siRNA-mediated gene silencing". Issued Sep 28, 2007.
- U.K. Patent No. 2407091. Inventors: Davidson, B.L., Gonzalez-Alegre, P., Miller, V., Paulson, H, and **Harper S.Q.** "Allele-specific siRNA-mediated gene silencing." Filed May 26, 2003, Issued Feb 14, 2007.
- Australian Patent No. 2005200827. Inventors: Davidson, B.L., Gonzalez-Alegre, P., Miller, V., Paulson, H, Xia, H., and **Harper S.Q.** "Allele-specific siRNA-mediated gene silencing." Filed Feb 24, 2005, Issued July 14, 2005.
- U.S. Patent No. 6,869,777. Inventors: Chamberlain J.S. and **Harper S.Q.** "Mini-dystrophin nucleic acid sequences". Issued March 22, 2005.
- U.S. Patent 9,133,482, Inventors: Liu, J. and **Harper, S.Q**. "Recombinant virus products and methods for inhibition of expression of myotilin." Issued Sept 15, 2015.