CURRICULUM VITAE

PERSONAL DATA

Name:	Scott Q. Harper, PhD
D.O.B.	11/27/71
Address/affiliations:	Assistant Professor, Center for Gene Therapy The Research Institute at Nationwide Children's Hospital and Department of Pediatrics,The Ohio State University 700 Children's Drive, Columbus, OH 43205 Office: Rm WA3015 Lab: Rm W334 Tel: (614) 355-2893 / Fax: (614) 722-5892 *Scott.Harper@Nationwidechildrens.org or Harper.104@osu.edu* Faculty Member, Integrated Biomedical Sciences Graduate Program and Molecular, Cellular, and Developmental Biology Graduate Program, The Ohio State University Columbus, OH 43210

EDUCATION

Year	Degree/Institution
2007	Post-doctoral, University of Iowa College of Medicine, Iowa City, IA.
2002	PhD (Cellular and Molecular Biology), University of Michigan Medical School, Ann Arbor, MI.
1996	BS (Biology), Saginaw Valley State University, Saginaw, MI. *Summa cum laude*

ACADEMIC AWARDS AND HONORS

Year	Award
2006	Recipient, Travel Award, American Society of Gene Therapy, 9th Annual Meeting, Baltimore, MD, May-June 2006.
2005	Recipient, Travel Award, American Society of Gene Therapy, 8th Annual Meeting, St. Louis, MO, June 2005.
2005	Best Basic Science Poster, University of Iowa, Department of Internal Medicine, Research Day Symposium, March 2005.
2003	First Place, Poster Award for Post-Doctoral and Medical Fellows, Center for Aging, University of Iowa, Department of Internal Medicine, Research Day Symposium
2002	Recipient, Travel Award, American Society of Gene Therapy, 5th Annual Meeting, Boston, MA, June 2002.
2001	Invited participant, Press Briefing, American Society of Gene Therapy, 4th Annual Meeting, Seattle, WA, May-June, 2001.

2000	First Place, Poster Award, Cell and Molecular Biology Research Symposium, University of Michigan, Ann Arbor, MI, September 2000.
2000	Pre-doctoral Student Award finalist, American Society of Human Genetics, 50th Annual Meeting, Philadelphia, PA, October 2000.
1996	Graduated *Summa cum laude*, Saginaw Valley State University
1996	Recipient, State of Michigan Competitive Scholarship
1991	Phi Theta Kappa, National Junior College Honor Society, Delta College

TEACHING EXPERIENCE

Year	Course
2009	Faculty moderator, MCDB 890/800 student seminar series
2008	Faculty, Integrated Biomedical Sciences Graduate Program, IBGP 701 Lectures on nucleic acid biochemistry, RNA interference, gene therapy
2003-2005	Lecturer: Human Physiology for Pre-Pharmacy and Pre-Engineering students, University of Iowa College of Medicine, Department of Physiology. Prepared lectures and examination questions, taught lectures, proctored examinations.
1998	Graduate Student Teaching Assistant, *Human Molecular Genetics* (HG541), University of Michigan Medical School, Department of Human Genetics. Course Director: Dr. Diane Robins. Organized and ran a weekly discussion section with 25-35 graduate and undergraduate students. Prepared course materials, graded examinations, tutored. Fall 1999.

COMMITTEE AND ADMINISTRATIVE SERVICE

Year	Committee
2008	Co-Chair, "Oligonucleotide Therapies for Diseases of Muscle and Nerve", Oral Abstract Session, American Society of Gene Therapy, 11th Annual Meeting, Boston, MA 2008
2008	Co-Chair, "Muscle Gene Therapy: Systemic Delivery", Oral Abstract Session, American Society of Gene Therapy, 11th Annual Meeting, Boston, MA 2008
2008	Abstract Review Committee, Musculoskeletal Section, American Society of Gene Therapy 11th Annual Meeting, Boston, MA, 2008
2007-2010	Radiation Safety Committee, The Research Institute at Nationwide Children's Hospital
1998-2000	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	Co-Organizer, Fall CMB/Genetics Short Course (HG 630), "Diseases of Trinucleotide Repeat Expansion", University of Michigan Medical School

MILITARY

Year	Service Details
1992-1999	Hospital Corpsman (HM2), 1st Battalion, 24th Marine Infantry, United States Naval Reserve
1998	Sailor of the Year, Naval Reserve Center, Saginaw, Michigan
1991	Navy League Outstanding Recruit Award, Naval Training Center, San Diego, CA

PROFESSIONAL SOCIETY MEMBERSHIPS

American Society of Gene Therapy
American Society of Human Genetics

PUBLICATIONS

1. **S.Q. Harper.** Progress and challenges in RNAi therapy for Huntington's Disease. (2009) *Archives of Neurology*, accepted for publication.

2. 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.

3. 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.

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

5. 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.

6. **S.Q. Harper**, P.D. Staber, C.R. Beck, S.K. Fineberg, C.S. Stein, D. Ochoa, and B.L. Davidson. Optimization of feline immunodeficiency viral vectors for RNA interference. (2006), *Journal of Virology* **80**(19), 9371-9380.

7. **S.Q. Harper**, P.D. Staber, X. He, S.L. Eliason, I.H. Martins, L. Yang, H.L. Paulson, R.M. Kotin, and B.L. Davidson. RNA interference improves behavioral and neuropathological abnormalities in a mouse model for Huntington's disease. (2005) *Proceedings of the National Academy of Sciences, USA*. **102**(16), 5820-5825.

8. **S.Q. Harper** and B.L. Davidson. Plasmid-based RNA interference. (2005) *Methods in Molecular Biology* **309**, 219-235.

9. V.M. Miller, R.F. Nelson, C.M. Gouvion, A. Williams, E. Rodriguez-Lebron, **S.Q. Harper**, B.L. Davidson, M.R. Rebagliati, and H.L. Paulson. CHIP suppresses polyglutamine aggregation and toxicity *in vitro* and *in vivo*. (2005) *Journal of Neuroscience* **25**(40), 9152-9161.

10. M. Liu, Y. Yue, **S.Q. Harper**, R.W. Grange, J.S. Chamberlain, and D. Duan. Adeno-associated virus-mediated microdystrophin expression protects young *mdx* muscle from contraction-induced injury. (2005) *Molecular Therapy* **11**(2), 245-256.

11. B.L. Davidson and **S.Q. Harper**. Viral delivery of short hairpin RNAs. (2005) *Methods in Enzymology* **392**, 145-173.

12. H. Xia, Q. Mao, S.L. Eliason, **S.Q. Harper**, I.H. Martins, H.T. Orr, H.L. Paulson, L. Yang, R.M. Kotin, and B.L. Davidson. RNAi suppresses polyglutamine-induced neurodegeneration in a model of spinocerebellar ataxia. (2004) *Nature Medicine* **10**(8), 816-820.

13. M.J. Blankinship, P. Gregorovic, J.M. Allen, **S.Q. Harper**, H.A. Harper, C. Halbert, A.D. Miller, and J.S. Chamberlain. Efficient transduction of skeletal muscle using vectors based on adeno-associated virus serotype-6. (2004) *Molecular Therapy* **10**(4), 671-678.

14. Y. Yue, Z. Li, **S.Q. Harper,** R.L. Davisson, J.S. Chamberlain, and D. Duan. Microdystrophin gene therapy of cardiomyopathy restores dystrophin-glycoprotein complex and improves sarcolemma integrity in the *mdx* mouse heart. (2003) *Circulation* **108**(13), 1626-1632**.**

15. **S.Q. Harper**, M. Hauser, C. DelloRusso, D. Duan, R.W. Crawford, S. Phelps, H.A. Harper, A.S. Robinson, J.F. Engelhardt, S.V. Brooks, and J.S. Chamberlain. (2002) Modular flexibility of dystrophin: Implications for gene therapy of DMD. *Nature Medicine*, **8**(3), 253-261.

16. **S.Q. Harper**, R.W. Crawford, C. DelloRusso, and J.S. Chamberlain. Spectrin-like repeats from dystrophin and α-actinin are not functionally interchangeable. (2002) *Human Molecular Genetics*, **11**(16), 1807-1815.

17. J.M. Scott, S. Li, **S.Q. Harper**, R. Welikson, D. Bourque, C. DelloRusso, S.D. Hauschka, and J.S. Chamberlain. Viral vectors for gene delivery of micro-, mini-, or full-length dystrophin. (2002) *Neuromuscular Disorders,* **12**(S1), S23-29.

18. S.B. Chun, C.D. Han, S.F. Hahn, **S.Q. Harper**, P.J. Savickas, D.M. Meunier, L. Li, T. Yalcin. Phase behavior of polystyrene/polybutadiene and polystyrene/hydrogenated polybutadiene mixtures: Effect of the microstructure of polybutadiene. (1998) *Macromolecules*, **31**, 394-402.

INVITED SYMPOSIA AND WORKSHOPS

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

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

3. **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.

4. **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.

5. **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.

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

ABSTRACTS

1. 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.***

2. 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.

3. 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.

4. 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.

5. 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.

6. 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.

7. 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.

8. 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.

9. **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.

10. 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.

11. 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.

12. 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.

13. **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****.*

14. 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****.*

15. 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.

16. 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.

17. **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.

18. **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***.

19. **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.

20. **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.

21. 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.

22. 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.

23. **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.

24. 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.

25. **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.***

26. 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.

27. **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.***

28. 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.

29. 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.

30. **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.

31. **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***.

32. **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.***

33. 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.

EDITORIAL BOARDS

Associate Editor
Life Sciences (2007-2010)

American Society for Gene Therapy, 11th Annual Meeting, Abstract Review Committee, Musculoskeletal Section, (2008 -)

PEER REVIEWS

Scientific Journals

Human Gene Therapy (2008)
Journal of Neuroscience Methods (2008)
Neoplasia (2008)
Expert Opinion on Therapeutic Patents (2008)
Annals of Neurology (2008)
Archives of Neurology (2008)
American Journal of Pathology (2007-8)
Life Sciences (2007)
Biotechniques (2003-2005)
Nucleic Acids Research (2005)
FEBS Letters (2005-2006)

Funding Agencies and Other

Medical Research Charities Group
Research Fellowships in Rare Diseases, Ireland (2005).

North Carolina Biotechnology Center, Research Grant Program (2007).

Auburn University BioGrant Program (2007).

Association Française contre les Myopathies (2008).

National Research Foundation, South Africa (2008).

CURRENT GRANT SUPPORT

Facioscapulohumeral Society Jeff Jacobs Family Research Fellowship Grant (FSHS-JJFR-001; Harper, PI) 2/1/2009 – 1/31/2010
"Investigating DUX4 structure and function using rational Mutagenesis"

Muscular Dystrophy Association Development Grant (Harper, PI) 7/1/2007 – 6/30/2010
"RNAi therapy for facioscapulohumeral muscular dystrophy"

NIH KL2 Clinical and Translational Scholar Award 2/1/2009 – 1/31/2011
(KL2 RR025754; Harper, PI)
"A translational approach toward RNAi therapy for Facioscapulohumeral muscular dystrophy"

Startup Funds, The Research Institute at Nationwide Children's Hospital (Harper, PI). 6/1/2007 – 5/31/2012

PREVIOUS GRANT SUPPORT

Facioscapulohumeral Society Landsman Charitable Trust Research Fellowship Grant (FSHS-LCT-002; Harper, PI) 1/18/08 – 1/17/09
"*In vivo* investigation of DUX4 as a candidate FSHD gene"

Post-Doctoral National Research Service Award (NRSA) 7/17/03 – 7/16/06
NIH NS047048-01 (Harper, PI; Davidson, Mentor)
"Functional effects of Hes6 proteolysis on differentiation"

Hereditary Disease Foundation (Harper, PI) 7/1/03 – 6/30/06
"Silencing Huntingtin with siRNA"

NIH Gene Therapy Pilot Grant (Harper, PI) 10/1/06 – 5/31/07
"Autoregulated AAV vectors for inducible RNA interference"

PATENTS

U.S. Patent No. 6,869,777. Chamberlain J.S. and Harper S.Q. "Mini-dystrophin nucleic acid sequences".

Singapore Patent No. 109,361. Davidson, B.L., Gonzalez-Alegre, P., Miller, V.M., Paulson, H.L., and Harper, S.Q. "Allele-specific siRNA-mediated gene silencing".

Pending:
European Patent No. WO2006083800. Davidson, B.L. and Harper, S.Q. "Nucleic Acid Silencing of Huntington's Disease Gene".

U.S. Patent US2005255086. Davidson, B.L. and Harper, S.Q. "Nucleic Acid Silencing of Huntington's Disease Gene".