CIRM Disease Team Grant DR2A -05415

Mesenchymal Stem Cells Engineered to Express BDNF

for the Treatment of Huntington's Disease

Publication List 2012 - 11/19/2015

1. Olson SD, Pollock K, Kambal A, Cary W, Mitchel GM, Tempkin J, Stewart S, McGee J, Bauer G, Kim HS, Tempkin T, Wheelock V, Annett G, Dunbar G and Nolta J. Genetically Engineered Mesenchymal Stem Cells as a Proposed Therapeutic for Huntington's Disease. *Mol Neurobiol* 2012 Feb;45(1):87-98. Epub 2011 Dec 9.

2. Pollock K, Stewart, H Cary W, Nelson H Nacey C, Pepper K, Fink KD, Gruenloh W, Annett G, Tempkin T, Wheelock V, and Nolta JA. Mesenchymal stem cells engineered to produce BDNF for the treatment of Huntington's disease. American Society for Neural Therapy and Repair Annual Meeting, 2014.

3. Wheelock V, Fink KA, and Yarborough M. "Ethical considerations regarding a first-in-human stem cell gene therapy trial for Huntington's disease." Platform session, American Society of Bioethics and Humanities 2014 Annual Meeting, San Diego CA, October 2014.

4. Pollock K, Stewart H, Cary W, Nelson H, Hendrix K, Nacey C, Pepper K, Fink K, Gruenloh W, Annett G, Tempkin T, Wheelock V and Nolta J. Human Mesenchymal Stem/Stromal cells engineered to produce BDNF for the future treatment of Huntington's disease. Eight Annual Huntington's disease Clinical Research Symposium, Minneapolis MN, November 2014.

5. Tempkin T, DeCarli C, Scher L, Farias S, Duffy A., Fink K, Annett G, Brunberg J, Yarborough M, Hersch S, Stout J, Alyward E, Martin A, Kjer L, Swadell D, Nolta J, Wheelock V. PRE-CELL: A Pre-Cellular Observational Study in Early Huntington's Disease. Eight Annual Huntington's disease Clinical Research Symposium, Minneapolis MN, November 2014; *Neurotherapeutic*s 2015;12(1): 263-284.

6. Duffy A, Sifry-Platt M, Tempkin T and Wheelock V. "Protecting privacy in Huntington's disease predictive testing: A survey of Attitudes, Beliefs and Concerns." Eighth Annual Huntington's disease Clinical Research Symposium, Minneapolis MN, November 2014; *Neurotherapeutic*s 2015;12(1): 263-284.

7. Stewart H, Pollock K, Cary W, Nelson K, Nacey C, Pepper K, Fink KD, Gruenloh W, Annett G, Tempkin T, Wheelock V, Nolta JA. "Engineered mesenchymal stem cells to overexpress BDNF for the treatment of Huntington's disease." 44th Annual Society for Neuroscience Meeting 2014, Washington, D.C.

8. Fink KD, Deng P, Jandela J, Cary W, Torrest A, Kalomoiris, S, Nacey C, Stewart H, Pollock K, Pepper K, Gruenloh W, Annett G, Tempkin T, Wheelock V, Segal DJ, Nolta JA. "Allele-specific modification of the mutant huntingtin gene with transcription activator-like effectors." 44th Annual Society for Neuroscience Meeting, Washington 2014, D.C.

9. Pollock K, Stewart H, Nelson H, Fink K, Cary W, Hendrix K, Deng P, Torrest A, Gutierrez J, Nacey C, Peppe K, Gruenloh W, Bauer G, Annett G, Tempkin T, Wheelock V and Nolta JA. Genetically Engineered Mesenchymal Stem Cells as a Proposed Therapeutic for Huntington's disease. Presidential Symposium, American Society for Neural Therapy and Repair, 2015. *Cell Transplantation*, Vol. 24(4), pp. 751–777, 2015 0963-6897/15. DOI: http://dx.doi.org/10.3727/096368915X687804

10. Fink KD, Deng P, Torrest A, Stewart H, Pollock K, Gruenloh W, Annett G, Tempkin T, Wheelock V, Nolta JA. Developing stem cell therapies for juvenile and adult-onset Huntington's disease. *Regen Med*. 2015 Aug;10(5):623-46.

11. Torrest A, Deng P, Kalomoiris S, Cary W, Gutierrez JR, Pepper K, Gruenloh W, Annett G, Tempkin T, Wheelock V, Segal DJ, Nolta JA, and Fink KD. "Allele-specific silencing of the mutant huntingtin gene in primary fibroblast and neural cultures with transcription activator-like effectors." Society for Neuroscience Annual Meeting (October 17-21, 2015) Chicago IL.

12. Deng P, Fink KD, Torrest A, Kalomoiris S, Cary W, Gutierrez JR, Gruenloh W, Annett G, Tempkin T, Wheelock V, Segal DJ, and Nolta JA. "Patient Centric Gene Targets for silencing the Huntington's disease mutation in Adult and Juvenile HD." Society for Neuroscience Annual Meeting (October 17-21, 2015) Chicago IL.

13. Torrest A, Deng P, Kalomoiris S, Cary W, Gutierrez JR, Pepper K, Gruenloh W, Annett G, Tempkin T, Wheelock V, Segal DJ, Nolta JA, and Fink KD. "Allele-specific silencing of the mutant huntingtin gene in primary fibroblast and neural cultures with transcription activator-like effectors." Faculty for Undergraduate Neurosciences Meeting (October 19, 2015), Chicago, IL

14. Wheelock V, Tempkin T, Duffy A, Martin A, Mooney L, Scher L, Farias S, Swadell D, DeCarli C, Brunberg J, Li C-S, Yarborough M, Dayananthan A, Stout J, Hersch S, Aylward E, Fink KD, Annett G and Nolta J. "PRE-CELL: Preparing for a future planned Phase 1 trial of genetically-modified stem cells over-expressing BDNF in patients with Huntington's disease." Ninth Annual Huntington's Disease Clinical Research Symposium (October 2015), Tampa FL.

15. Pollock K, Stewart H, Nelson H, Fink KD, Cary W, Hendrix K, Torrest A, Deng P, Gutierrez J, Nacey C , Pepper K, Gruenloh W, Bauer G, Annett G, Tempkin T, Wheelock V, and Nolta, JA. "Genetically Engineered Mesenchymal Stem Cells as a Proposed Therapeutic for Huntington's disease." Ninth Annual Huntington's Disease Clinical Research Symposium (October 2015), Tampa FL.

16. Moscovitch-Lopatin M, DiFiglia M, Kegel-Gleason K, Ritch JJ, Rosenthal SJ, Sapp E, Wheelock V, Duffy A, Chopra V, Rosas HD, Hersch SM. "A Novel Translational Bioassay for Conformers of Mutant Huntingtin." Ninth Annual Huntington's Disease Clinical Research Symposium (October 2015), Tampa FL.

17. Dayananthan A, Kuo J, Duffy A, Chang C, Parikh P, Evans, J, Ginwalla C, Wheelock V. "Status Dystonicus Presenting as Status Epilepticus in a Juvenile Huntington's Disease Patient." Ninth Annual Huntington's Disease Clinical Research Symposium (October 2015), Tampa FL.

18. Duffy A, Martin A, Michie M, O'Keafe M, Yarborough M and Wheelock V. First-In-Human stem cell trials in Huntington's Disease: A Bioethics Survey. Ninth Annual Huntington's Disease Clinical Research Symposium (October 2015), Tampa FL.

Submitted:

Manuscripts:

Pollock K, Dahlenburg H, Nelson H, Fink KD, Cary W, Hendrix K, Annett G, Torrest A' Deng P, Gutierrez J, Nacey C, Pepper K, Kalomoiris S, Anderson JD, McGee JL, Gruenloh W, Fury B, Bauer G, Duffy A, Tempkin T, Wheelock V and Nolta, JA. Human Mesenchymal Stem Cells Genetically Engineered to Overexpress Brain-derived Neurotrophic Factor Improve Outcomes in Huntington's disease Mouse Models. *Molecular Therapy.*

Moscovitch-Lopatin M, DiFiglia M, Kegel-Gleason K, Ritch JJ, Rosenthal SJ, Sapp E, Duffy A, Wheelock V, Chopra V, Rosas HD, Hersch SM. A Novel Translational Bioassay for Conformers of Mutant Huntingtin.

2016 AAN Meeting Abstracts:

Pollock K, Stewart H, Nelson H, Fink KD, Cary W, Hendrix K, Torrest A, Deng P, Gutierrez J, Nacey C , Pepper K, Gruenloh W, Bauer G, Annett G, Tempkin T, Wheelock V, and Nolta, JA. Genetically Engineered Mesenchymal Stem Cells as a Proposed Therapeutic for Huntington's disease.

Wheelock V, Tempkin T, Duffy A, Martin A, Mooney L, Scher L, Farias S, Swadell D, DeCarli C, Brunberg J, Yu L, Li C-S, Yarborough M, Dayananthan A, Stout J, Moscovitch-Lopatin M, Hersch S, Fink KD, Annett G and Nolta J. PRE-CELL: Clinical and novel biomarker measures of disease progression in a lead-in-observational study for a planned Phase 1 trial of genetically-modified mesenchymal stem cells over-expressing BDNF in patients with Huntington's disease.

Tempkin T, Duffy A, Martin A, Michie M, O'Keafe M, Yarborough M and Wheelock V. First-In-Human stem cell trials in Huntington's Disease: A Bioethics Survey.