

CURRICULUM VITAE

Name: Lisa M. Ellerby, Ph.D.

Present Position: Professor
Buck Institute for Research on Aging
Program on Aging

Address: 8001 Redwood Blvd
Novato, California 94945

Telephone: 415-209-2088

Fax: 415-209-2230

E-mail: lellerby@buckinstitute.org

Education: University of California, Santa Cruz
Bachelor of Art, Chemistry, 1985
University of California, Santa Cruz
Doctor of Philosophy, Chemistry, 1989
Postdoctoral Fellow
Department of Biochemistry and Chemistry
University of California, Los Angeles, 1990-1994

Professional Memberships: American Chemical Society, 1996-Present
Society of Neuroscience, 1999-Present
American Association for the Advancement of Science, 1996-Present
Oxygen Society, 1994-1998

NIH Service: NINDS NSD-B Study Section Member 2003-2007
NIH NINDS NSD-B Review Committee Member 2004-2008
NIH NINDS ZRG1 NDBG (02) Review Committee, Ad hoc, 2005
NIH NINDS CDIN-2 Review Committee, Ad hoc, 2004-present
NIH NINDS ZNS1 SRB-M (S1), Ad hoc, 2007
NIH NINDS NSD-B Review Committee Member, Ad hoc 2008-Present
NIH NINDS CDIN Review Committee Member, 2009-2013
NIH ETTN-M Review Committee Member, Ad hoc 2011-Present
NIH MDCN Review Committee Member, Ad hoc 2009-Present

Honors: University of California, Santa Cruz,
Outstanding Teaching Assistant Award, 1987
American Cancer Society Postdoctoral Fellowship, 1992-1994

Editorial Board: Biochemistry Journal, 1999-Present
Plos Huntington's Disease, 2011-Present
Journal of Biological Chemistry, 2003-2015
Journal of Huntington's Disease, Associate Editor, 2012-Present
Journal of Alzheimer's Disease, 2012-Present
American Journal of Stem Cell Research, 2013-Present

Ad Hoc Reviewer: Aging
Biochemistry
Brain Research
Brain Sciences
Cell Death and Differentiation
Cell Metabolism
Cell Reports
Cell Stem Cell
eLIFE
EMBO Journal
Free Radicals in Biology and Medicine
Human Molecular Genetics
International Society of Stem Cell Research
Journal of Neurochemistry
Journal of Biological Science
Journal of Huntington's Disease
Journal of Neuroscience
Molecular Cell
Molecular Psychiatry
Molecular Neurodegeneration
Nature Communications
Nature Medicine
Nature Methods
Neurobiology of Aging
Neurobiology of Disease
Neuron
Pharmaceuticals
Plos Biology
Plos Genetics
PNAS
Stem Cell Reports
Science
Trends in Neuroscience

Research and Teaching Experience:

01/90-06/90 **Postdoctoral Associate in Protein Chemistry and Enzymology,**
University of California, Santa Cruz. Research focus: Protein

folding and mechanism of β -lactamase. Ph.D. advisor Dr. Anthony Fink.

- 06/90-09/90 **Instructor-General Chemistry**, University of California, Santa Cruz.
- 11/90-12/91 **Postdoctoral Associate in Bioinorganic Chemistry and Molecular Biology**, University of California, Los Angeles, in laboratory of Dr. Joan S. Valentine, Member National Academy of Sciences. Research focus: Biosensors, biological and mechanistic studies on the function of copper-zinc superoxide dismutase and ALS.
- 01/92-12/94 **American Cancer Postdoctoral Fellow**, University of California, Los Angeles. Biological and mechanistic studies on the function of copper-zinc superoxide dismutase.
- 01/95-06/99 **Senior Research Associate in Neurodegenerative Disease and Apoptosis**, Co-Investigator, Program on Aging, The Burnham Institute, La Jolla, CA. Research focus: Elucidating cell death pathways in neurodegenerative diseases such as ALS, and polyglutamine expansion disease.
- 07/01-07/08 **Assistant Professor**, Buck Institute for Research on Aging, Novato, CA. Research focus: Elucidating cell death pathways in neurodegenerative diseases such as Huntington's disease, and other polyglutamine expansion diseases.
- 07/08-07/17 **Associate Professor**, Buck Institute for Research on Aging, Novato, CA. Research focus: Elucidating cell death pathways in neurodegenerative diseases such as Huntington's disease, and other polyglutamine expansion diseases.
- 03/17-Present **Professor**, Buck Institute for Research on Aging, Novato, CA. Research focus: Elucidating cell death pathways in neurodegenerative diseases such as Huntington's disease, and other polyglutamine expansion diseases.
- 07/08-Present **Adjunct Professor**, Dominican University, San Rafael, California
- 07/14-Present **Adjunct Professor**, Davis School of Gerontology, University of Southern California

Administrative Responsibilities and Committees:

- 07/00-07/10 **Chemical Safety Officer**, Buck Institute for Research on Aging

07/00-07/10	Chair, Chemical Safety Committee , Buck Institute for Research on Aging
07/00-07/10	Chair's Committee Member , Buck Institute for Research on Aging
03/02-6/06	IACUC Committee Member , Buck Institute for Research on Aging
07/02-Present	Director, MPI, T32 Training Grant in Aging and Disease
07/01-Present	NIH Reviewer
05/04-10/15	Editorial Board, Journal of Biological Chemistry
08/06-08/08	HDSA Scientific Advisory Board
05/07	Reviewer, French National Research Agency
05/07-present	Reviewer, Ataxia UK, Ataxia Foundation USA
05/07-present	Reviewer, Kennedy's Disease Association
05/07-present	Reviewer, Hereditary Disease Foundation
07/07-07/17	Chair or Member, Faculty Recruit Committee , Buck Institute for Research on Aging
02/12	Organizer, Stem Cell Meeting , Buck Institute
01/12	Barrow Foundation Reviews
03/13	Scientific Research in Biomedicine , Europe
07/13-07/14	Chair, Formal Research Series , Buck Institute
07/16	Associate Editor, Journal of Huntington's Disease

BIBLIOGRAPHY

A) Research Overview Ellerby Laboratory

The Ellerby group studies the basic mechanisms of disease progression and pathogenesis in Huntington's disease with an emphasis on the discovery of new therapeutic targets for this disease. Huntington's disease (HD), one of the most extensively studied of these CAG/polyglutamine diseases, is an inherited neurodegenerative disease characterized by involuntary movements, personality changes, dementia, and early death. Huntington's disease (HD) is an autosomal dominant progressive neurodegenerative disorder resulting in specific

neuronal loss and dysfunction in the striatum and cortex. The central theme of our research is to utilize novel approaches to explore the mechanisms, identify therapeutic targets and treatments for the disease. A very exciting research direction for the laboratory has been the use of stem cells models to understand Huntington's disease. Recently, my laboratory has developed an isogenic, allelic induced pluripotent stem cell model of HD using HD patient fibroblasts. We demonstrated that the damage imposed by mutant huntingtin (HTT) can be restored after the genetic mutation is corrected by homologous recombination in HD induced pluripotent stem cells (iPSCs). We have also reported the first use of TALENs and CRISPR for genetic engineering of new lines of iPSC for disease modeling in polyQ disease or HD transplantation. In addition, we have recently reported the use of RNAseq to demonstrate the disruption of striatal neuronal development and the identification of therapeutic targets for HD with this model. We have been able to leverage our expertise in disease modeling work with iPSCs to obtain funding for projects in PD and AD.

Our initial studies in the field focused on post-translational modifications and proteolysis of Huntingtin (HTT). We identified several mechanisms by which expanded polyQ proteins confer toxicity such as cleavage by caspases and calpain. My laboratory in collaboration with Dr. Hayden described the findings that the majority of polyQ disease proteins are cleaved by caspases. I translated seven of the eight proteins and treated each of them with different caspase family members and was excited to find that there was selective and specific cleavage of these substrates. Given the number of caspase substrates in the cell, this was statistically higher than expected and suggests a correlation between caspase substrates and polyQ disease. To understand the effect of this in the pathogenesis of polyQ diseases, we constructed forms of the polyQ expansion disease proteins that were resistant to cleavage and found this significantly attenuated toxicity in cell culture models of these diseases. These studies were done *in vitro*. By providing evidence for proteolysis and cleavage of polyQ mutant proteins by caspases we contributed to the fields understanding of neurodegeneration and cell death mechanisms for this class of diseases as well as AD.

To understand if the proteolysis of *in vivo*, my group evaluated if caspases were activated in polyQ expansion diseases, whether the cleavage products were observed in mouse models and human HD postmortem tissue. We then evaluated the impact of proteolysis of ataxin-7 in SCA7. This study describes a critical mechanism for neurodegeneration in polyglutamine expansion diseases, and shows that cleavage by caspase-7 is required for SCA7 neurodegeneration, behavioral phenotypes, and shortened lifespan in transgenic mice. Proteolytic cleavage products in SCA7 patients and mouse models are an early pathological change, yet the relationship between ataxin-7 proteolysis and disease progression in SCA7 had not been addressed. To determine if caspase cleavage is a critical event in SCA7 neuronal degeneration, we generated transgenic mice expressing mutant ataxin-7 (D266N) resistant to caspase-7 proteolysis. In collaboration with Dr. Al La Spada, we compared ataxin-7-92Q-D266N transgenic mice to SCA7 mice lacking the D266N mutation. We documented inhibition of proteolysis, improved motor performance, decreased neurodegeneration, and an extended lifespan by greater than 3-fold in the SCA7 D266N mice. These are the first genetic studies to demonstrate in a transgenic model of polyglutamine expansion that lifespan can be extended 3-fold by a single point mutation, and identify inhibition of caspase-7 cleavage of ataxin-7 as an important therapeutic strategy for this disease. We also evaluated if specific caspase family

members were involved in disease progression by performing genetic cross with knockout mice lacking the caspases to HD mouse models. My work along with others in the field established the importance of these enzymes in disease progression and pathogenesis and suggested the caspases maybe a significant therapeutic target in the diseases.

Along with studies on proteases, my laboratory has carried out some of the first studies to fully map the PTMs of the HTT protein using mass spectrometry. For example, we used mass spectrometry to define the phosphorylation sites on the HTT protein and found that modulation of phosphorylation at the calpain site of cleavage controlled susceptibility to proteolysis. Further, we demonstrated how posttranslational modifications of the polyQ proteins influence proteolysis or toxicity for ataxin-7 and androgen receptor. In addition, we mapped the acetylation sites in the HTT protein and linked this modification to pharmacological treatment with HDAC inhibitors. We also mapped several key acetylation sites in Tau which have impact on AD disease pathology and progression. This body of work contributes to understanding the influence of PTMs on neurological diseases. We now are finishing up a project on a novel PTM in HTT that provides physiological evidence for a nuclear smaller form of HTT. This work will tie a number of key publications and observations in the field together.

Another major focus of the laboratory is to identify small molecules or novel therapeutic targets to treat HD. We have used a multitude of new approaches to carry out this goal. We have used unbiased siRNA screens to the druggable genome to identify new therapeutic targets for HD. We identified MMPs and RRAS as new targets for HD. In addition, we have developed small molecules or screened for new ones in cellular striatal models of HD. One particularly attractive target we are exploring is the enzyme DGK ϵ which is involved in lipid metabolism and when inhibited normalizing lipid metabolism in HD models.

Our most recent work focuses on the use of induced pluripotent stem cells to model and identify therapeutic targets in HD and the use of CRISPR/Cas9 to genetically correct HD *in vivo*. The abstracts for two recently awarded RO1s on these two topics are shown below:

Genetic Correction of Mutant Huntingtin *in Vivo*

The proposed research is intended to determine if a monogenetic disease such as Huntington's disease (HD) can be treated by using homologous recombination (HR) to genetically correct the disease containing allele *in vivo*. The approach will utilize a genome engineering system, CRISPR (Clustered Regulatory Interspaced Short Palindromic Repeats) delivered *in vivo*. In recent work in HD-iPSCs (induced pluripotent stem cells), we utilized homologous recombination to genetically correct the disease containing cells and found this completely reverses HD disease phenotypes. We have adapted the CRISPR technology to carry out genetic correction/expansion and have found very high levels of homologous recombination in human cells (Preliminary Results). The overall goal is to use this technology *in vivo* and carry out genetic correction of disease-containing mutations such as the CAG expansion in huntingtin (HTT). This work will serve as a proof of concept to demonstrate utility not only for monogenic neurodegenerative diseases but also for other types of genetic diseases. To achieve our goals we will carry out the following aims: Specific Aim 1. To demonstrate robust CRISPR-mediated recombination of the mutant HTT in human HD patient-derived neural cells using lentivirus; Specific Aim 2. To demonstrate robust CRISPR-mediated recombination in mouse models of HD

expressing the human HTT protein using viral delivery; Specific Aim 3. To develop a protein-mediated delivery system using the nickase Cas9 D10A protein, gRNA and DNA to mediate homologous recombination in human-patient HD neural stem cells and HD mouse models. These studies will advance our understanding of how to perform genetic correction in cells derived from the brain. The successful demonstration of *in vivo* genetic correction of the disease allele in the brain would be a major leap forward in neuroscience.

Identifying Factors Regulating Medium Spiny Neuron Differentiation or Maintenance as Therapeutic Targets for Huntington's Disease using Induced Pluripotent Stem Cells

Huntington's disease (HD) is a fatal, dominantly inherited neurodegenerative disorder that primarily affects neurons in the striatum and cortex, and for which there is currently no effective treatment. HD is caused by a CAG expansion in the huntingtin gene leading to a polyglutamine (polyQ) expansion in the encoded protein (HTT), and patients with a CAG expansion greater than 38 repeats exhibit chorea, psychological problems, and cognitive decline. Expression of mutant HTT leads to selective neuronal dysfunction and degeneration despite its ubiquitous expression pattern. Recent advances in stem cell research suggest that patient induced pluripotent stem cells (iPSCs) may provide novel models of disease and new treatments for diseases. These studies will utilize iPSCs derived from HD patients (HD-iPSCs) as a human model of HD. Using genetic engineering, we generated an isogenic allelic HD-iPSC series for HD modeling (CAG repeat of 21, 45, 72, 100). To understand the molecular basis for the CAG repeat expansion dependent disease phenotypes in NSCs, we performed transcriptomic analysis of HD iPSCs and HD neural stem cells (NSCs) compared to isogenic controls. Differential gene expression and pathway analysis pointed to TGF- β and netrin-1 as the top dysregulated pathways, and dysregulated genes were enriched for those involved in neuronal development and the formation of the dorsal striatum. The disrupted striatal and neuronal networks could be modulated to correct HD phenotypes and provide therapeutic targets. Therefore, the isogenic HD-iPSCs with corrected alleles provides mechanistic insights into the disease process and allows the identification of novel therapeutic targets for HD. Indeed our studies suggest that factors that lead to the maturation or maintenance of medium spiny neurons (MSNs) are likely to ameliorate Huntington's disease phenotypes. We have found that netrin leads to enhanced rate of maturation of MSNs with increased spontaneous electrical activity and increased levels of DARPP-32. We will investigate the following aims in this application: **Specific Aim 1.** We will characterize the cellular and functional deficits in normal iPSCs, HD-iPSCs, and genetically corrected HDiPSCs differentiated into medium spiny neurons using "omics" approaches; **Specific Aim 2.** Using DARPP-32 genomic elements that direct gene expression specifically in mature MSNs, we will develop a marker of mature MSNs and identify factors that mediate differentiation and maintenance of MSNs for this cellular HD model; **Specific Aim 3.** We will determine if factors that promote MSN differentiation or maintenance ameliorate HD phenotypes in mouse models of the disease. Therapeutic targets will be identified and new treatments for HD will be explored.

B) Original Publications (Citations 17,155; h-index 50; i10-index 71)

1. Fink AL, Ellerby LM, Bassett PM. Conformational Changes in the Inactivation of β -lactamase by penicillin sulfones. *J. Am. Chem. Soc.* 111:6871-6873, 1989.

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C) Manuscripts in Preparation/Submitted

1. O'Brien, R, DeGiacomo F, Zhang, N, Schilling B, Gibson BW, Ellerby LM. Mapping the Acetylation sites of full-length Htt. In preparation.
2. Gafni J, Zafar K, Holcomb, J, Crippen D, Chen S, Hermel E, Melov S Ellerby LM. Calpain Activation in Huntington's Disease R6/2 Mouse Model. *Cell Death and Disease*, in revision.
4. Zafar K, Ellerby LM. Neurogenesis is Required for Functional Recovery in Huntington's Disease, in preparation.
5. Holcomb J, Zafar K, Ellerby LM. FGF Family Members are Neuroprotective in Huntington's Disease, in preparation.
6. Papanikolaou T, Sampath V, Ellerby LM. FOXO is a Regulator of Ataxin-7 Function, in preparation.
7. Cong X, Cheng JM, Gafni J, Gibson BW, Ellerby LM. Caspase-7 Phosphorylation Site at the Active Site Regulates Activity, in preparation.

D) Book Chapters and Reviews

1. Studies of CuZnSOD in *Sacharomyces cerevisiae*. Gralla, E.B., Ellerby, L.M. and Valentine, J.S., in "Biological Oxidants and Antioxidants" Cardenas, E., ed., 1994.
2. Copper-zinc Superoxide Dismutase: Mechanistic and biological studies. Valentine, J.S., Ellerby, L.M., Graden, J.A., Nishida, C.R., Gralla, E.B., in "Metals in Biology" Nato, 1994.
3. Dentatorubral-pallidoluysian Atrophy (DRPLA): Model for Huntington's Disease and Other Polyglutamine Diseases. Ross, C.A., Ellerby, L.M., Wood, J.D., and Nucifora, F.C. in "Neurodegenerative Diseases – Neurobiology Pathogenesis and Therapeutics." Beal MF et al, eds., December 2004.
4. Spinal Ataxia Type 7: Clinical Features to Cellular Pathogenesis, Garden, G.A., Truant, R., Ellerby, L.M, LaSpada, A. in "Genetic Instabilities and Neurological Diseases." Eds Wells, R, Ashizawa, T., July, 2006.
5. Stem Cell Therapy and Huntington's disease: an update, Papanikolaou, T., An, M., Ningzhe, Z., Zafar, K. in "Neurogenesis and CNS Disease" Eds Jin, K, March, 2009.

6. iPSC-based Drug Screening for Huntington's Disease, Brain Research, Zhang N, Bailus BJ, Ring KL, Ellerby LM, 1638(Pt A):42-56.
7. Diseases of Protein Folding: Huntington's Disease and Amyotrophic Lateral Sclerosis, Bailus, B and Ellerby, LM, in "Encyclopedia of Cell Biology", Elsevier, 2016.
8. Huntington's Disease and Stem Cells, Ring, K, O'Brien, R, Ellerby, LM, in "Stem Cells" Taylor and Francis, 2016.
9. Using Genome Engineering to Understand Huntington's Disease, Bailus, B Zhang, N and Ellerby, LM in R. Jaenisch et al. (eds.), Genome Editing in Neurosciences, Research and Perspectives in Neurosciences, DOI 10.1007/978-3-319-60192-2_9.

E) Patent Applications

1. Sol-Gel Encapsulated Enzyme. Zink, J.I., Dunn, B., Valentine, J.S., Ellerby, L.M., Nishida, F., Nishida, C., Yamanaka, S. A. Serial No. 07/744,524, 1993.
2. A Cell-Free System of Mitochondria-Dependent Apoptosis, and Methods of Use, Thereof. Ellerby, H.M, Bredesen, D.E., Ellerby, L.M. Serial No. 08/919,801, 1997.
3. Hunter-Killer Peptides and Methods of Use. H.M. Ellerby, Ellerby, L.M., Bredesen, D.E. Rabizadeh, S. U.S. Patent Application No. 60/558,448, 2004.
4. Fibroblast Growth Factor-2 Promotes Neurogenesis and Neuroprotection and Prolongs Survival in Huntington's Disease. Ellerby, L.M., Greenberg, D.A., Jin, K.L., Andersen, J. U.S. Patent Application No. 60/701,752, 2006; European Patent Application No. 06788314.0 – 1521 / 1904092, 2006.
5. Caspase Inhibitors and Uses Thereof. Ellerby, L.M., Ellman, J. Levy, M. U.S. Patent Application P018US, 2009.
6. The Treatment of Huntington's Disease with Serotonin Receptor Antagonists. Ellerby, L.M., Hughes, R.E. 61/384,151, 2010
7. Provisional Patent: BUCK Ref. BI-449; WAVS Ref. BUCKP063PUS "ZIKA as a Cell Penetrating Peptide for Delivery to the Brain, 32606537, 2018.

F) Abstracts, Meetings and Presentations

1. Symposium on Neurobiology & Neuroendocrinology of Aging "Using Stem Cells to Model Huntington's Disease" Austria, July 15-19, 2018
2. XDP Meeting, "Elucidating factors that promote patch and matrix MSN differentiation for XDP disease modeling and drug discovery" MGH, June 8-10, 2018
3. Buck Institute Journal Club, "The "Big Bang" For Modern Glial Biology", June 26, 2018
4. NIH Study Group, Special Emphasis Panel, RFA, "Interdisciplinary Research to Understand the Complex Biology of Resilience to Alzheimer's Disease Risk", June 22, 2018

5. NIH Study Group, Special Emphasis Panel, "Expand the Human Genome Editing Repertoire", May 24, 2018
6. AFAR Big Awards, Review Panel, June 7, 2018
7. 7th Ataxia Investigators Meeting, "Disease Modeling and Therapeutic Targets for Huntington's Disease", National Ataxia Foundation, Philadelphia, Pennsylvania, April 2-5, 2018
8. NIH NINDS/ZNS1 SRB-A(16) Study Group, March 12, 2018
9. UCSF BUCK Meeting, "New Technology Applied to Neurodegenerative Disease and Aging", Buck Institute for Research on Aging, October 13, 2017
10. NIH ZNS1 T32 Study Group, Washington DC, October 30-31, 2017
11. BAC Meeting, "New Technology Applied to Neurodegenerative Disease and Aging", Buck Institute for Research on Aging, October 16-17, 2017
12. Neuropharmacology and Human Stem Cell Models, "Modeling Huntington's Disease with Stem Cells", Banbury, September 10-13, 2017
13. Society of Biomolecular Imaging and Informatics (SBI2) 4th Annual "High Content" Conference, "Therapeutic Targets in Huntington's Disease" September 13-15, San Diego, 2017
14. NIH Common Fund, Francis Collins, NIH Director, Making Somatic Cell Genome Editing Therapies a Reality Workshop, July 24, 2017
15. NIH CMND Study Group, San Francisco, CA, June 1-2, 2017
16. Collaborative Center for XDP, Workshop, Boston, MA, May 8-9, 2017
17. NIH ZNS1 SBR-A(10) Integrated Review Study Group, Teleconference, May 3, 2017
18. Southern Methodist University Biology Seminar Series, "Using Stem Cells to Model HD and Identify Therapeutic Targets", Dallas, April 21, 2017
19. 13th Annual Huntington Disease Research Symposium, HDSA, "Huntington's Disease: Therapeutic Targets" UC San Francisco, Mission Bay Campus-Genetech Hall, November 19, 2016
20. Buck Institute for Research on Aging Geoscience Course, "Huntington's Disease and Aging", Buck-USC Graduate Program (GERO 601), Buck Institute, November 10, 2016
21. NIH NOMD Study Group, Crystal City, VA, October 24-25, 2016
22. NIH MDCN Integrated Review Study Group, Teleconference, October 21, 2016
23. Institute of Biology Paris-Seine, University Pierre and Marie Curie, "Approaches to Identifying Therapeutic Targets in Huntington's Disease", Paris, France, September 30, 2016
24. Institute of Biology Paris-Seine, University Pierre and Marie Curie, Thesis committee member, Jessica Voisin, "FOXO in HD stress resilience", Paris, France, September 29, 2016

25. Columbia University, Thesis committee member, Leora Fox, "Autophagy-linked FYVE Protein Mediates the Turnover of Mutant Huntingtin and Modifies Pathogenesis in Mouse Models of Huntington's disease", New York, New York, September 15, 2016
26. 10th Neurodegenerative Conditions Research and Development, "Using Huntington's Disease with Induced Pluripotent Stem Cells to Identify Therapeutic Targets", Boston, MA, September 11-12, 2016
27. NIH ZRG1 BDCN-Q, NIH Special Emphasis Study Group, July 15, 2016
28. NIH NST-1 BDCN-W, NIH Special Emphasis Study Group, June 16, 2015
29. ISSCR 2016 Annual Meeting 22-25, Faculty mentor luncheon and poster judge, San Francisco June, 2016
30. Fondation IPSEN, Genome Editing in Neurosciences, "Using Genome Engineering to Understand Huntington's Disease", Paris, France, April 22, 2016
31. Buck Institute for Research on Aging Geoscience Course, "Huntington's Disease and Aging", Buck-USC Graduate Program (GERO 601), Buck Institute, April 14, 2016
32. XDP Consortium, "Using iPSC to understand XDP, Harvard University, March 2, 2016
33. CHDI meeting, Huntington's Disease Therapeutics, February 22-25, 2016
34. HDSA local chapter, Huntington's Disease Therapeutics, January 23, 2016
35. Calico Presentation, "DGKepsilon as a Therapeutic Target in HD an Epilepsy", Buck Institute, January 6, 2016
36. Faculty Chalk Talk, "Using Genome Engineering Tools to Understand Aging and Disease" Buck Institute, October 5, 2015
37. ZRG1 BDCN-W, NIH Special Emphasis Study Group, September 24, 2015
38. 9th Neurodegenerative Conditions Research and Development, "System Biology Approaches to Understand Huntington's Disease with Induced Pluripotent Stem Cells", Philadelphia, PA, September 9-10, 2015
39. Calico Presentation, "Using CRISPR/Cas9 for HD treatments", Buck Institute, July 15, 2015
40. NIH NST-2, NIH Study Group, Washington, DC, June 29-30, 2015
41. Gordon Conference, CAG Triplet Repeat Disorders, "Modeling Huntington's Disease with Induced Pluripotent Stem Cells" Lucca (Barga), Italy, May 31-June 5, 2015
42. The American Society for Neural Therapy and Repair, "Systems Biology Approaches to Modeling HD with Induced Pluripotent Stem Cells" Sheraton Sand Key Resort, Clearwater Beach, Florida, USA
43. NIH ZRG1 MDCN-Q, Special Emphasis Study Group, March 27th, 2015
44. Touro University, "Huntington's Disease Stem Cells and Therapeutic Targets", Vallejo, California, March 10, 2015
45. CHDI Conference, "The role of TGF- β in Huntington's Disease" Palm Springs, California, February 23-26, 2015

46. Duke University, "Modeling Huntington's Disease with Induced Pluripotent Stem Cells" Durham, North Carolina, January 20, 2015
47. CHDI Stem Cell Platform Conference, "Using Isogenic HD Cell Lines to Model Huntington's disease", Princeton, New Jersey, January 7-9, 2015
48. University of California, Davis, "Modeling Huntington's Disease with Induced Pluripotent Stem Cells", Davis, California, December 12, 2014
49. Society for Free Radical Biology and Medicine 21st Annual Meeting, "Discovery New Therapeutic Targets for Huntington's Disease using Stem Cells", Seattle, Washington, November 19-23, 2014
50. NIH NST-2 Study Group, Washington, DC, October 27-28, 2014
51. EMBL conference, "Modeling Huntington's Disease with iPSCs", Stem Cells in Cancer and Regenerative Medicine, Heidelberg, Germany, October 9-12, 2014
52. American Chemical Society, Bay Area Chapter Meeting, "Using Chemical Approaches to Understand Huntington's Disease", Buck Institute, September 25, 2014
53. 11th Annual Huntington Disease Research Symposium, HDSA, "Huntington's Disease: Using iPSCs to Identify New Therapeutic Targets" UC San Francisco, Mission Bay Campus-Genetech Hall, September 13th, 2014
54. HDF Meeting, "Systems Biology Approaches to HD", Boston, MA, August 6-10, 2014
55. NIH ZRG1 MDCN-E Study Group, Teleconference, July 9, 2014
56. Abcam Stem Cell Meeting, "Stem Cells in Huntington's Disease", Singapore, June 26, 2014
57. ISSCR Meeting, "Systems Biology Approaches to HD", Vancouver, Canada, June 18-23, 2014
58. NIH CDIN Study Group, Washington, DC, June 14-15, 2014
59. Baylor School of Medicine, "Stem Cells in Huntington's Disease", May 13, 2014
60. CHDI, Modifiers Workshop, " HD-iPCS: RNAseq analysis", Los Angeles, CA, November 15, 2013
61. SFN, Neurobiology of Disease Workshop: Human Brain Disorders in a Dish: Induced Pluripotent Stem Cell Models of Disease, "Modeling Huntington's disease with Induced Pluripotent Stem Cells" San Diego, November 8, 2013
62. Case Western Reserve, Department of Physiology and Biophysics, "What can Stem Cells Tell us About Huntington's Disease", Cleveland, Ohio, October 28, 2013
63. San Antonio Nathan Shock Aging Center, Stem cells and Aging. "Stem Cells in Huntington's Disease and Aging" Majan Ranch, Bandera, Texas, October 17-20, 2013
64. Treat PolyQ disease Course, "MMPs in Huntington' Disease", "Stem Cells and Huntington's Disease", "Women in Science", Corsica, France, October 7-11, 2013

65. SFN, Neurobiology of Disease Workshop: Human Brain Disorders in a Dish: Induced Pluripotent Stem Cell Models of Disease, Dress rehearsal, Washington, DC, September 6, 2013
66. Northern California Board Meeting, HDSA, Novato, CA, July 27, 2013
67. Gordon Research Conference, Chair, Emerging Methods and Models: Illuminating New Paths to Therapeutics”, Waterville Valley Resort, Waterville Valley, NH, June 22-23, 2013
68. The International Society of Stem Cell Research, “Disease Modeling for HD”, Boston, Massachusetts, June 12-15, 2013
69. The Buck Advisory Council, “Panel Discussion: Lost in Translation: Why Do So Many Experimental Drugs for Neurodegenerative Disorders Fail”, Novato, CA, May 20-21, 2013
70. UCSF Ground Rounds, “What Stem Cells Can Tell Us About Huntington’s Disease”, San Francisco, CA, May 17, 2013
71. CHDI Meeting, “PTMs in Huntington’s Disease Workshop”, New York, NY, May 8-9, 2013
72. Keck Graduate Institute, 3rd Annual Research Symposium, Huntington’s Disease: From Basic Science to Therapy, “Modeling Huntington’s Disease with Induced Pluripotent Stem Cells”, Claremont, CA April 4-5, 2013
73. CHDI Meeting “System Biology Approaches to HD”, New York, February 28, 2012
74. NIH CDIN Study Group, San Francisco, CA, February 14-15, 2013
75. The Buck Advisory Council, “Fixing the Brain: Collaboration to Cure Neurodegenerative Diseases”, Muscat, Oman, November 10-12, 2012
76. NIH ZRG1 ETTN-C Study Group, Small Business: Neuropharmacology, New Orleans, Louisiana, October 11-12, 2012
77. NIH CDIN Study Group, Washington, DC, Oct 4-5, 2012
78. Euro Huntington’s Disease Meeting, “Stem Cell Models of Huntington’s Disease”, Stockholm, Sweden, September 13-16, 2012
79. “HDF Meeting, “Genetic Correction of HD Phenotypes in Induced Pluripotent Stem Cells”, Boston, August 1-4, 2012
80. Biological Modifiers Working Group, “Stem Cell Models of Huntington’s Disease”, Boston, August 1, 2012
81. CHDI Meeting “Target validation establishing whether high priority targets such as syntaxin-1, RNF128, DGK-epsilon and R-RAS *in vivo* modulate HD disease progression and pathophysiology”, Boston, July 30-31, 2012
82. NIH CDIN Study Group, Washington, DC, June 7-8, 2012
83. Buck Advisory Council Meeting, “Therapies for Neurodegenerative Diseases”, The Buck Institute for Research on Aging, May 20-22, 2012

84. HDF, Milton Wexler Interdisciplinary Workshop, "Huntingtin Protein" UCLA, May 9-10, 2012
85. CHDI Meeting "Target validation establishing whether high priority targets such as syntaxin-1, RNF128, DGK-epsilon and R-RAS *in vivo* modulate HD disease progression and pathophysiology", Buck Institute for Research on Aging, April 2, 2012
86. Organizer and Speaker, 2012 Buck Symposium: Stem Cell Research and Aging, "Designing Stem Cells in Huntington's Disease" Buck Institute for Research on Aging, March 1-2, 2012.
87. HD Therapeutics Conference: A Forum for Drug Discovery & Development, CHDI Foundation, "Defining Posttranslational Modifications in Huntingtin" Palm Springs, California, February 27-March 1, 2012
88. 9th Annual Huntington Disease Research Symposium, HDSA, "Therapeutic Targets in Huntington's Disease" UC San Francisco, Mission Bay Campus-Genetech Hall, February 11th, 2012
89. NIH, ZCA1 RPRB-7 Study Group, Spore in Lymphoma, Brain Head/Nick and Lung Cancers, and Sacroma, Washington, DC, February 8-9, 2012
90. CHDI Meeting "Target validation establishing whether high priority targets such as syntaxin-1, RNF128, DGK-epsilon and R-RAS *in vivo* modulate HD disease progression and pathophysiology", Buck Institute for Research on Aging, February 2, 2012
91. CHDI Meeting "Target validation establishing whether high priority targets such as syntaxin-1, RNF128, DGK-epsilon and R-RAS *in vivo* modulate HD disease progression and pathophysiology", New York, NY, November, 2011
92. Thermavance, Inc., "Huntington's Disease: Target Validation" South San Francisco, October, 2011
93. Buck Institute for Research on Aging, Novato, CA, Scientific Advisory Board Meeting, "Huntington's Disease: Proteolysis and Neurogenesis", August, 2011
94. Chair, NIH ZRG1 MDCN-E Study Group, Teleconference, July 15, 2011
95. NIH ZRG1 ETTN-C Study Group, Small Business: Neuropharmacology, Seattle, Washington, June 23-24, 2011
96. CAG Triplet Repeat Disorders, Session Chair "Protein Cell and Genomics Effects" Lucca, Italy, June 5-10, 2011
97. NIH CDIN Study Group, Washington, DC, June 2-3, 2011
98. Buck Institute for Research on Aging, Novato, CA, Internal Geroscience Seminar, "Huntington's Disease: Proteolysis and Neurogenesis", June 15, 2011
99. Neurogenetics Affinity Group & Consortium for Neuropsychiatric Phenomics, UCLA, Los Angeles, CA, "Huntington's Disease: Targets and Therapeutics", April 28, 2011
100. BioMarin, Novato, CA "Target Validation in Huntington's Disease", March 29, 2011

101. CHDI Meeting "Target validation establishing whether high priority targets such as syntaxin-1, RNF128, DGK-epsilon and R-RAS *in vivo* modulate HD disease progression and pathophysiology", Princeton, NJ, April 26, 2011
102. External Advisory Board, Buck Institute for Research on Aging, Novato, CA, "HDACs in Neurodegeneration and Aging", February 23, 2011
103. 8th Annual UCSF/UCD HD Research Symposium, Genetech Hall, UCSF Mission Bay, "Huntington's Disease: Targets and Therapeutics", February 5, 2011
104. NIH CDIN Study Group, Washington, DC, January 27-28, 2011
105. Buck Institute for Research on Aging, Novato, CA, Internal Geroscience Seminar, "Huntington's Disease Research Directions", December 15, 2010
106. ACARM8, All California Ataxia Research Meeting, "Therapeutic Strategies for Huntington's Disease", December 12, 2010
107. Sonoma State University, "Therapeutic Targets in Huntington's Disease", Rohnert Park, CA, November 19, 2010
108. NIH CDIN Study Group, Washington, DC, October 7-8, 2010
109. CHDI Meeting "Huntington's Disease: Target Validation", New York, NY, October 6, 2010
110. NIH CDIN Study Group, Washington, DC, June 10-11, 2010
111. NIH NCGC CHDI Meeting "Post-translational Modifiers of Huntington's Disease", Rockville, MD, March 11, 2010
112. 7th Annual UCSF/UCD HD Research Symposium, Genetech Hall, UCSF Mission Bay, February 20, 2010
113. NIH ETTM-C-02 Study Group, February 17, 2010
114. NIH BDCN CDIN Study Group, Washington, DC, January 28-29, 2010
115. NIH BDCN-Y-04 Special Emphasis Panel, October 26, 2009
116. NIH NINDS CDIN Study Group, Washington, DC, September 24-25, 2009
117. NIH NIA ARRA P30 Core Grants, July 15, 2009
118. NIH NINDS CDIN Study Group, Bethesda, MD, June 11-12, 2009
119. NIH NINDS NSDB Study Group, San Diego, California, February 26-27, 2009
120. Buck Institute for Research on Aging, Geroscience Meeting, HDACs and Neurodegeneration, February 25, 2009
121. CHDI, "Polyglutamine Disease and Proteolysis" February 15, 2009
122. NIH CMND Study Group, Washington, DC, February 10-11, 2009
123. UCSF, "Polyglutamine Disease and Proteolysis" December 15, 2008
124. Buck Institute for Research on Aging, Therapeutic Targets in Huntington's Disease, November 12, 2008

125. Buck Institute for Research on Aging, Therapeutic Targets in Huntington's Disease, October 28, 2008
126. NIH ZRG1 BDCN-Y Study Group, San Francisco, California, October 14, 15-2008
127. NIH CMND Study Group, San Francisco, California, October 23-24, 2008
128. ACARM7, All California Ataxia Research Meeting, Target Validation in HD, September 14, 2008
129. NIH NINDS NSDB Study Group, Washington, DC, February 28-29, 2008
130. CHDI 3rd Annual Huntington Disease Therapeutics Conference, February 04 - 07, 2008
131. 5th Annual UCSF/UCD HD Research Symposium, Genetech Hall, UCSF Mission Bay, January 26, 2008
132. NIH ZAG1 Z1J-4 (J3) Cell Quiescence, Washington, DC, December 13-14, 2007
133. The Biology of Calpains in Health and Disease. "Calpains and Huntington's Disease" Snow Mass, Colorado, July 14-19, 2007
134. Anthony L Fink Symposium, "Enzymes and Neurodegenerative Diseases" University of California, Santa Cruz, California, May 26, 2007
135. CAG Triplet Repeat Disorders, "HD Target Validation: siRNA Screens in HD Cell Culture Models" Aussois, France, May 13-18, 2007
136. CAG Triplet Repeat Disorders, "Impact of Normal Function/Metabolism of Polyglutamine Proteins on Disease", Structured Discussion Leader, Aussois, France, May 13-18, 2007
137. Emory University, "Huntington's Disease: Proteolysis and Neuroprotection" Whitehead Auditorium, Whitehead Biomedical Research Bldg, Emory University, Atlanta, Georgia, April 16, 2007
138. NIH NINDS NSDB Study Group, Washington, DC, February 22-23, 2006
139. 2nd Annual Huntington Disease Therapeutic Conference: A Forum for Drug Discovery & Development, "Quantification of Huntingtin Cleavage Products Using an Enzyme-Linked Immunosorbent Assay", Palm Springs, California, February 5-8, 2007
140. National Ataxia Foundation All California Ataxia Research Meeting (ACARM), "Huntington's Disease and Neurogenesis" Embassy Suites, San Francisco, California, November 19, 2006
141. NIH NINDS NSDB Study Group, Washington, DC, October 25-27, 2006
142. Buck Institute Scientific Advisory Board Review, "PolyQ disease" Presentation to Scientific Advisory Board at the Buck, Annual Review, September 28-29, 2006
143. 2nd Annual Dependence Receptors Symposium, "Factors Determining the Toxicity of the Androgen Receptor and other Polyglutamine Proteins." Buck Institute, September 14-16, 2006
144. HD 2006: Changes, Advances, and Good News, Session Chair, Royal Sonesta Hotel, Boston, August 11-13, 2006

145. NIH NINDS NSDB Study Group, Savoy Hotel, Washington, DC, June 21-22, 2006
146. Huntington's Disease Society of America 2006 Northern California Chapter Convention, "Update on HD Research" UC Davis MIND Institute, Davis, CA, May 6, 2006
147. NIH NINDS NSDB Study Group, Renaissance Hotel, Washington, DC, February 21-22, 2006
148. NIH ZRG1 NDGB-A Study Section, Washington, DC, February 13-14, 2006
149. NIH ZRG1 NDBG09F Study Section, Washington Circle Hotel, Washington, DC, December 28, 2005
150. CHDI (High Q Foundation) Conference, Howard Hughes Center, Los Angeles, CA, November 3, 2005
151. NIH ZRG1 NDBG Study Group, Washington, DC, October 24-25, 2005
152. NIH/NINDS Study Section, Bethesda, MD, October 20-21, 2005
153. Symposium in Honor of Joan Selverstone Valentine, "Neurogenesis and Huntington's Disease" University of California, Los Angeles, September 2, 2005
154. NSD-B Study Section, Rockville, MD, June 23-24, 2005
155. AACR 99th Annual Meeting, "Abraxane (ABI-007) acts synergistically with targeted antiangiogenic pro-apoptotic peptides (HKP) in MDA-MB-435 human tumor xenografts." Anaheim/Orange County, California, April 16-20, 2005
156. ASBMB Annual Meeting and IUBMB Conference, "Mass spectrometric characterization of immunoprecipitated full-length huntingtin: Post-translational modification, April 1-5, 2005
157. ZRG1 CDIN Study Section, Washington, DC, March 23-25, 2005
158. NIH/NINDS NSDB Study Section, Arlington, Virginia, February 24-25, 2005
159. NIH ZRG1 NDBG02S Study Section, Mitochondria and Neurodegeneration, Washington, DC, February 15-17, 2005
160. Buck Institute Public Seminar. "Movement disorders and New Hope for Therapy" February 10, 2005
161. Winter Conference on Brain Research, Breckenridge, CO, January 22-27, 2005
162. Johns Hopkins University, Mock NIH Review for HD center grant, "Mass spectrometric characterization of immunoprecipitated full-length huntingtin: Post-translational modification" January 6, 2005
163. American Society for Cell Biology 4th Annual Meeting, "Mass spectrometric characterization of immunoprecipitated full-length huntingtin: Post-translational modification" Washington, DC, December 4-8, 2004
164. Huntington's Disease Research Symposium, "FGF-2 promotes neurogenesis and neuroprotection and prolongs survival in HD transgenic mice" University of California, San Francisco, December 4, 2004

165. Society For Neuroscience Annual Meeting. "Fibroblast growth factor-2 promotes neurogenesis and neuroprotection and prolongs survival in a transgenic mouse model of Huntington's disease." San Diego, California, October 23-27, 2004
166. Kennedy's Disease Conference and Symposium. "Kennedy's Disease: Mouse Models and Mechanistic Studies" Presentation and panel Discussion. San Diego, California, October 21-22, 2004
167. NIH/NINDS NSD-B Peer Review Panel Study Group, San Francisco, California, October 13-15, 2004
168. HDSA Eighth Annual Scientific and Coalition for the Cure Meeting, "Inducible Huntington's Disease Models Expressing Full-length Huntingtin: Identification of an N-Terminal Fragment of Mutant Huntingtin" Cleveland, OH, October 1-3, 2004
169. HDSA Meeting: Huntington Disease's Proteolysis Team Progress Update, Vancouver, Canada, August 27-29, 2004
170. HDF Symposium, HD 2004: Changes, Advances and Good News (CAG)_n, Cambridge, MA, August 12-15, 2004
171. FEBS Advanced Course, New Molecular Strategies to Treat Neurodegenerative Diseases, "Kennedy's disease: Mechanistic Studies on Motor Neuron Loss" Ofir, Portugal, July 17-23, 2004
172. NIH/NINDS NSD-B Peer Review Panel Study Group, Washington DC, June 21-24, 2004
173. NIH/NINDS NSD-B Peer Review Panel Study Group, Washington DC, June 17-18, 2004
174. ASBMB Annual Meeting and IUBMB Conference, "Kennedy's Disease: Altered MAP Kinase, CBP and p53 Signaling" Boston, Massachusetts, June 12-16, 2004
175. IPAM Proteomics Workshop II, Medical Applications and Protein Networks, Organizer and Presenter, UCLA, "Mass Spectrometry Applied to Questions in Polyglutamine Expansion Diseases" April 19-23, 2004
176. Encino Tarzana Medical Center Continuing Medical Education Program, "Age-Dependent Neurodegenerative Diseases and Cell death" Tarzana Hospital, Tarzana, California, March 2, 2004
177. NIH/NINDS NSD-B Peer Review Panel Study Group, Washington DC, February 26-27, 2004
178. Buck Institute Staff Seminar Series, "Kennedy's disease: Mouse Models and Therapeutics" February 18, 2004
179. Winter Conference on Brain Research, "Pathogenic Mechanisms and Therapeutic Implications in Models of Alzheimer's and Huntington's Diseases" Copper Mountain, Colorado, January 24-30, 2004
180. Buck Institute Staff Seminar Series, "Therapeutic Strategies for Huntington's Disease" December 17, 2003
181. American Society for Cell Biology 43rd Annual Meeting. "TSAP11: A Novel Regulator of the Androgen Receptor" San Francisco, California, December 13-17, 2003

182. Huntington's Disease Research Update. "Cell Death Proteases in Huntington's Disease" UC Davis Medical Center, Sacramento, California, December 6, 2003
183. Weekly Department of Biochemistry & Biophysics Seminar Series. "Role of Calpains in Huntington's Disease" Oregon State University, Corvallis, Oregon, November 14, 2003
184. Society For Neuroscience Annual Meeting. "Role of Calpains in Huntington's Disease" New Orleans, Louisiana, November 8-12, 2003
185. Kennedy's Disease Conference and Symposium. "Kennedy's Disease: Proteolytic Cleavage Events" New Orleans, Louisiana, November 4-7, 2003
186. NIH/NINDS NSD-B Peer Review Panel Study Group, Washington DC, October 15-18, 2003
187. Dependence Receptor Conference: Seeing How the Other Half Die. "Androgen Receptor & Dependence" La Fondation des Trielles, France, July 2-7, 2003
188. NIH/NINDS NSD-B Peer Review Panel Study Group, Washington DC, June 26-27, 2003
189. "Polyglutamine Expansion Diseases." Taught one-week Graduate Course. University of Coimbra, Coimbra, Portugal, May 12-16, 2003
190. Gordon Research Conference: CAG Triplet Repeat Disorders. "Kennedy's Disease: MEK1/2 Pathway is Required for Cell-Death Induced by Mutant Androgen Receptor." Il Ciocco, Barga, Italy, May 4-9, 2003
191. Huntington's Disease Society of America, "Toward a Cure: An Update on Research for the Lay Perspective." HDSA Annual California Chapter Convention, UC Davis Medical Center, Davis, California, May 3, 2003
192. ASBMB Board & Experimental Biology Meeting, "Editorial Board Training." San Diego, California, April 10-14, 2003
193. Economics of Aging Seminar "Highlights of Buck Institute Research." Margaret Todd Senior Center, Novato, California, March 26, 2003
194. Winter Conference on Brain Research, "Proteolytic Cleavage Events in Huntington's Disease." Snowbird, Utah, January 27-30, 2003
195. Poster Presentation at the Society for Neuroscience. S.J. Shoesmith-Berke, F.A. Flores Schmied, L.M. Ellerby, E.R.P. Brunt, H.L. Paulson. "Caspase-Mediated Proteolysis of the Polyglutamine Disease Protein Ataxin-3." Society for Neuroscience, Orlando, Florida, Nov 2-7, 2002
196. Poster Presentation at the Society for Neuroscience. J. Gafni, L. Ellerby. "Huntington's Disease: The Role of Calpain Cleavage." Society for Neuroscience, Orlando Florida, November 2-7, 2002
197. Poster Presentation at the Society for Neuroscience. M.A. LaFevre-Bernt, L. M. Ellerby. "Kennedy's Disease: MEK 1/2 Pathway is Required for Cell-Death Induced by Mutant Androgen Receptor." Society for Neuroscience, Orlando, Florida, November 2-7, 2002
198. Poster Presentation at the Society for Neuroscience. J.E. Young, L. Gouw, L.J. Ptacek, Y.H. Fu, L.M. Ellerby "Spinocerebellar Ataxia Type 7: Caspase Cleavage of Ataxin-7 Alters

- Nuclear Location and Cytotoxicity." Society for Neuroscience, Orlando, Florida, November 2-7, 2002
199. The Kennedy's Disease Association invitation KDA Conference and Symposium, "Reaching for the Stars" Baltimore, Maryland, October 13-15, 2002
 200. "A Time for All Ages" the Marin County Commission on Aging. Marin County television show hosted by Jack Hanson. Interview of Dr. L. Ellerby and Dr. D. Greenberg on Aging Research and HD at the Buck Institute. Broadcast throughout August, 2002
 201. Buck Institute Staff Seminar Series, "Huntington's Disease" August 21, 2002
 202. Hereditary Disease Foundation. "Kennedy's Disease: MEK1/2 Pathway is Required for Cell-Death Induced by Mutant Androgen Receptor." Boston, Massachusetts, August 9-12, 2002
 203. "Kennedy's Disease" Buck Institute Informal Seminar Series, July 30, 2002
 204. Scientific Panel member for the Huntington's Disease Society of America, North California Chapter, Annual Chapter Convention. UC Davis Medical Center, Davis, California, May 4, 2002
 205. Huntington's Disease Society of America. "Calpain Activation in Huntington's Disease." Chicago, Illinois, April 26-28, 2002
 206. Huntington's Disease Society of America. "Kennedy's Disease: Toxic Fragments Synergistically Enhance Androgen Receptor Induced Cell Death." Chicago, Illinois, April 26-28, 2002
 207. Poster Presentation at the Society for Neuroscience. "Kennedy's Disease: Toxic Fragments Synergistically Enhance Androgen Receptor Induced Cell Death." Society for Neuroscience, San Diego, California, November 10-15, 2001
 208. Presentation for the Scientific Advisory Board for the Buck Institute Symposium. "Specific Caspase Interaction and Amplication are Involved in Selective Neuronal Vulnerability in Huntington's Disease" September 9, 2001
 209. "Huntington's Disease" Buck Institute Staff Seminar Series, August 12, 2001
 210. Gordon Research Conference. "Specific Caspase Interaction and Amplication are Involved in Selective Neuronal Vulnerability in Huntington's Disease" CAG Triplet Repeat Disorders, Gordon Conference. Mount Holyoke College, South Hadley, Massachusetts, July 15-20, 2001
 211. "Kennedy's Disease" Buck Institute Informal Seminar Series, July 15, 2001
 212. Poster Presentation. Huntington Disease Society of America, Philadelphia, Pennsylvania, May 4-5, 2001
 213. Chemistry Seminar Series. "Huntington's Disease: Role of Cell Death Proteases." University of California, Santa Cruz, March 19, 2001
 214. "Proteases in Polyglutamine Expansion Disease" Joint Buck Institute and Parkinson's Institute Symposium, January, 2001

215. Poster Presentation at the Society for Neuroscience. "Specific Caspase Interaction and Amplication are Involved in Selective Neuronal Vulnerability in Huntington's Disease" New Orleans, Louisiana, November 4-8, 2000
216. Poster Presentation. Huntington Disease Society of America, Miami, Florida October 23-28, 2000
217. Buck Institute Staff Seminar Series, "Polyglutamine Repeat Disease" October 18th, 2000
218. Poster Presentation. Hereditary Disease Symposium, Boston, Massachusetts August 18-20, 2000
219. Oral Presentation. "Huntington's Disease: Role of Cell Death Proteases." Winter Conference on Brain Research, Breckenridge, Colorado, January 24-26, 2000
220. Gordon Conference Poster, The Structural and Kinetic Requirements for pH-Independent Superoxide Dismutase Catalysis, Joan S. Valentine, Lisa M. Ellerby, Janet A. Graden, and Diane E. Cabelli* Department of Chemistry and Biochemistry, University of California, Los Angeles, 1995
*Chemistry Department, Brookhaven National Labs, Upton, New York
221. Oxygen Society, 1995. Glutathione Levels in *Saccharomyces cerevisiae* Lacking Key Antioxidant Enzymes Lisa M. Ellerby, Grace Huang, Edith B. Gralla, Joan S. Valentine Department of Chemistry and Biochemistry, University of California, Los Angeles, 1995

G) Research Support

ACTIVE (direct cost/year)

R01 NS094422 (Ellerby) 09/1/16 – 08/31/21
NIH/NINDS \$250,000

Genetic Correction of Mutant Huntingtin in Vivo

We have recently developed the tools to use CRISPR/Cas9 to genetically correct the mutant Htt gene in human cells and in vivo HD mouse models. This application will extend this technology to genetically correct mouse models of HD.

R01 NS100529 (Ellerby) 09/01/16 – 08/30/21
NIH/NINDS \$428,015

Identifying Factors Regulating Medium Spiny Neuron Differentiation or Maintenance as Therapeutic Targets for Huntington's Disease using Induced Pluripotent Stem Cells

These studies will utilize induced pluripotent stem cells (iPSCs) derived from HD patients (HD-iPSCs) as a human model of HD. Using genetic engineering, we generated an isogenic allelic HD-iPSC series for HD modeling (CAG repeat of 21, 45, 72, 100). Recent advances in stem cell research suggest that iPSCs may provide novel models of disease and new treatments for diseases. Our recently generated isogenic HD-iPSCs with corrected alleles will allow novel mechanistic insights into the disease process and establish methods for their eventual use *in vivo*.

1R01 NS074408-01 (Ellerby) 07/01/13 – 06/30/18

NIH \$250,000

Matrix Metalloproteinases: Therapeutic Targets for Huntington's Disease

Huntington's disease (HD) is a fatal autosomal dominant neurodegenerative disease characterized by emotional disturbances, uncontrolled movements and loss of intellectual abilities. We will determine how MMPs affect the processing of huntingin (Htt) in brain samples of Huntington's disease mouse models. Furthermore, we will investigate if pharmacological or genetic reduction of MMP-10 or MMP-14 modifies disease progression or pathogenesis in HD mouse models. By crossing MMP-10 or MMP-14 knockout mice to HD mouse models, we will determine if deficiency of MMPs in the brain can ameliorate HD-like pathologies or behavioral deficits in HD mice. We will also use MMP inhibitors to treat HD mouse models and determine if HD pathogenesis or disease progression is modified by treatment. Together, our studies will determine if MMP are valid targets for HD treatment.

R21 NS095312 (Ellerby)

09/1/15 – 08/31/18

NIH \$150,000

Comprehensive Synaptosome Proteomics Targeting HD Protein Expression, PTMs & Synthesis

An essential goal of quantitative proteomics is to understand how proteins in cells and tissues change in their expression levels and posttranslational modification (PTM) status, ideally with knowledge of their spatial and temporal reorganization, protein interaction networks and functional status. As we have extensive experience in mouse models of Huntington's disease (HD), we propose to use an Htt-polyQ expanded knockin mouse, HdhQ175 compared to littermate controls (B6/J background), as our model system. Synaptosomes isolated from the cortex and striatum will be examined using these proteomic strategies, as these brain regions are known to be effected in HD. In summary, this rich integrated data set of changes in synaptic protein levels, half-lives, and PTM status will provide a greater understanding of the synapse that is integral to the function of the brain and will help elucidate the dysfunctions underling neuronal diseases of the brain.

NIH T32 AG000266-17 (Campisi, Ellerby)

04/29/00–

04/29/23

NIA \$647,654

This postdoctoral training Program will prepare scientists from a range of disciplines for independent careers in research that aims to understand the mechanisms of aging and age-related disease. We will train the next generation of scientists and provide them with the broad knowledge, interdisciplinary skills and scientific interactions they will need to alleviate, through research, the enormous human and financial burdens caused by aging and age-related diseases. (reviewed 10/19/16; score slightly better than 2012; await NIA pay line results).

Private Donor Research in Epilepsy (Ellerby)

04/01/13– 03/31/16

NA \$169,000

Therapeutics for Treatment of Epilepsy

This research will evaluate DGKepsilon inhibitors in HD epilepsy models.

Collaborative Center for X-linked Dystonia PD (Ehrlich, Ellerby) 02/01/16– 07/31/18
Harvard Medical School \$244,003

Understanding XDP using mouse models and human iPSCs
This work is directed at understanding how the XDP mutations cause the disease. The aim of this collaboration is to characterize the XDP iPSC and develop methods to make MSNs characteristic of striosome vs. patch. We will apply this expertise to the novel induced pluripotent stem cell lines to understand the molecular mechanisms of N-TAF1 mutations in XDP.

U19AG023122 (Cummings) 05/01/16-9/30/17
CPMC/NIH/NIA \$70,000

Longevity Consortium Pilot Project (Ellerby)
Resilience Pathways modeling human longevity-promoting ApoE variants in iPSCs
Role: Subaward PI
The aims of this pilot project are; **Specific Aim 1:** To test if ApoE is a neuroprotective factor in neurodegenerative disease and aging we will generate novel of models of aging using human induced pluripotent stem cells (iPSCs) and; **Specific Aim 2** will determine whether longevity-promoting ApoE variants enhance stress resistance and survival and identify the pathways relevant to the neuroprotective effects of the various variants.

PAST

U19AG023122 (Cummings) 05/01/16-9/30/17
CPMC/NIH/NIA \$70,000

Longevity Consortium Pilot Project (Ellerby)
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Delos Pharmaceuticals (Ellerby) 04/01/14 – 03/31/15
NA

Rapalogs in HD
This sponsored research grant will test Rapalogs in HD models and evaluate the mechanism of action for mTOR1 and mTOR2 inhibition in HD.

R01 NS056420 (Ellerby) 12/15/07 – 11/30/14
NIH/NINDS \$208,490

Huntington's Disease and Neurogenesis

Mechanistic studies directed at understanding the role of neurogenesis in Huntington's disease.

RL1 NS062413 (Ellerby: 7 of 11) **09/30/07 – 07/30/14**
NIH/NINDS/NCRR Interdisciplinary Res Consortium (U54) \$225,000

HDACs in Neurodegeneration and Aging

Our hypothesis is that compromised acetylation homeostasis is coupled to neurodegeneration and identification of particular HDAC family members involved in this process will identify therapeutic targets critical to neurodegeneration and aging.

RL1 GM084432 (Hughes: 6 of 11) **09/28/07 – 06/30/12**
NIH/NIGMS/NCRR Interdisciplinary Res Consortium (U54) \$245,250

Protein Interactions and Protein Conformation in Aging and Disease

We propose to define and characterize protein interactions and protein conformational states relevant to a network involving human orthologs of proteins known to increase longevity when mutated in model systems of aging.

A-4066 (Ellerby, Hughes) **02/01/11 – 06/01/13**
CHDI, Inc. \$1,490,660

Joint Steering Committee

Target Validation Establishing Whether High Priority Targets Such as Syntaxin-1, RNF128, DGK-Epsilon and R-RAS *in Vivo* Modulate Huntington's Disease Progression and Pathophysiology.

A-3589 (Ellerby) **03/15/07 – 10/15/11**
CHDI, Inc. \$386,701

Development of Enzyme-Linked Immunosorbent Assay (ELISA)-Based Assays Quantifying Caspase Cleavage Products of Huntingtin (Htt)

This research proposal will continue our first year efforts in which we have developed reagents and assays to quantify caspase cleavage products of huntingtin (*Htt*).

A-3515 (Ellerby) **09/01/10 – 08/31/11**
CHDI, Inc. \$258,994

Characterization of Post-Translational Modifications of Huntingtin (Htt) Protein

Our aim is to map the post-translational modification (PTM) in the huntingtin (*Htt*) protein that have an impact on *Htt* turnover and toxicity.

RL1 NS062413-03S2 (Ellerby) **01/15/10 – 06/30/11**
NIH Research Supplement to Promote Diversity \$39,064

Supplement to: *HDACs in Neurodegeneration and Aging*

A-3507 N/A (Ellerby) **06/15/09 – 12/31/10**
CHDI, Inc. \$266,760

Huntington's Disease target discovery and validation

We have completed three orthogonal large-scale screens for modifiers of huntingtin (*Htt*) pathogenesis, an RNAi-based screen to detect suppressors of mutant *Htt* potentiated apoptosis

and a large-scale screen for Htt interacting proteins. We are in the process of validating hits from these screens and understanding their mechanisms of action.

P30 AG025708 (Campisi) **09/1/09 – 06/30/10**
NIH/NIA Pilot Supplement Award (Ellerby) \$35,000
Basic Mechanisms in Aging and Age Related Disease; Pilot: Effect of Klotho on Huntington's Disease
To develop a Nathan Shock Center of Excellence at the Buck Institute for basic mechanisms in aging and age related disease.

R01 NS040251 (Ellerby) **09/28/99 – 06/30/10**
NIH \$219,267
Triplet Repeat Disease: Requirement for Caspase Cleavage
The purpose of the R01 is to investigate the underlying mechanism(s) that contribute to cell dysfunction and death in polyglutamine expansion diseases. The major goals of this project are: 1. Does the generation of polyglutamine disease transgenic mice with the caspase-cleavage sites abolished slow the progression *in vivo*? 2. What domains or amino acids are required for the caspase interactions with polyglutamine expansion disease proteins? 3. Can we identify and clone the proteins interacting with polyglutamine and caspases in the apoptosome-like complex? 4. Can we define the caspases that are required for cell-death initiation utilizing dominant-negative caspase transgenic mice? 5. Is generation of a toxic fragment required for the mediation of transcriptional dysregulation in polyglutamine diseases?

R01 NS44921 (Greenberg) **12/15/02 – 11/30/07**
NIH \$237,500
VEGF in Neuroprotection and Neurogenesis
The goal of this project is to determine the mechanisms through which vascular endothelial growth factor protects neurons from hypoxic-ischemic insults and promotes neurogenesis in the adult brain.

R01 NS40251 (Ellerby) **02/15/05 – 06/30/07**
NIH Supplement \$21,000
Triplet Repeat Disease: Requirement for Caspase Cleavage
Research supplement for individuals with disabilities.

N/A (Ross) **12/01/05 – 01/30/09**
NIH PPG Subcontract (Ellerby) \$120,000
Research Center Without Walls for Huntington's Disease
This proposal evaluates proteolysis of huntingtin (Htt) in PC12 cell lines and post-translational modifications.

N/A (Ellerby) **07/01/01 – 06/30/03**
HDSA \$37,500

Cell Death Proteases in Huntington's Disease

The principle hypothesis of this proposal is that the physical interaction of specific caspases with huntingtin (Htt) as well as the regional and cellular distribution of caspases in the striatum contributes to the selective neuronal loss observed in Huntington's disease. We found that caspase-2, caspase-6, and caspase-7 recruit Htt in an apoptosome-like complex. Caspase-2 and caspase-7 bound the full-length huntingtin protein while caspase-6 bound the N-terminal caspase cleavage product. We propose the following Specific Aims in order to further elucidate the mechanism of mutant Htt-mediated cell death: (1) What domains or amino acids are required for the huntingtin/caspase interaction? (2) Can we identify and clone the proteins interacting with huntingtin and caspase-2 in the apoptosome-like complex?

T32 AG020495 (Bredesen, Ellerby)

05/01/02 – 04/30/07

Ellerby, Co-Investigator, oversee T32 training grant for Institute
NIH

\$250,000

Training in Age-Related Disease and Aging Research

This proposal provides training for five postdoctoral scholars each year in aging and age-related diseases.

N/A (Ellerby)

01/01/01 – 12/31/03

MDA

\$122,388

Characterization of Cell-Death Proteases in Spinal Muscular Atrophy

The long-term objective of these studies is to develop an effective therapy for dominantly inherited, late onset, neurodegenerative diseases caused by expansions in CAG repeats. In recent work, we have found specific caspase family members interact with androgen receptor, and this finding of the interaction between caspases and polyglutamine-containing neurodegeneration associated proteins also offers a new target for therapeutic consideration.

R01 NS38144 (Ross)

07/01/04 – 06/30/05

NIH Subcontract (Ellerby)

\$40,619

Transgenic Models of Huntington's Disease

This proposal evaluates proteolysis of huntingtin in Huntington's disease transgenics.

N/A (Bredesen, Ellerby)

01/01/97 – 12/31/99

Muscular Dystrophy Association

\$62,856

Familial ALS: Mechanism of Initiation

The long term objective of these studies is to develop effective therapy for sporadic and familial amyotrophic lateral sclerosis (ALS): by determining the chemical mechanism by which the mutations in *sod1* lead to familial ALS (FALS), then determining which resultant cellular abnormalities of FALS are shared by sporadic ALS.

RO1NS35155 (Bredesen, Ellerby)

06/01/97 – 03/31/00

NIH

\$155,940

Familial ALS: Mechanism of Initiation

The major goals of this project are: 1) To determine the profile of oxidants for which CuZnSOD mutants catalyze oxidation-reduction reactions at higher rates than wild type CuZnSODs; 2) To determine the potential targets of the redox reactions catalyzed by mutant CuZnSODs; 3) To

determine whether the altered redox chemistry displayed by the mutants is attributed to an underlying abnormality in metal binding or migration.

N/A (Bredesen/M. Ellerby) **06/15/01 – 06/14/05**
American BioSciences Inc. \$650,000
Novel and Killer Peptides
Sponsored agreement for research in the field of novel and killer peptides.

H) Mentored Postdoctoral Scholars

NIH F32 NS043937 (Juliette Gafni, Ph.D.) **01/01/03 – 12/31/05**
NINDS \$46,420
The Role of Calpain in Huntington's Disease
This proposal investigates the role of calpains in proeolytic cleavage of huntingtin and the activation of calpains in Huntington's disease.

NIH F32 NS043823 (Michelle LaFavre-Bernt, Ph.D.) **08/01/03 – 07/31/05**
NINDS \$46,420
Mechanism of Androgen Receptor Cytotoxicity

This proposal describes mechanistic studies directed at understanding the role of caspase activation and proteolysis in polyQ repeat mediated cell death.

NIH T32 AG020495 (Bredesen, Ellerby) **09/01/04 – 08/28/07**
NIA \$196,236
Trainee: Vanitha Sampath, Ph.D.
The Role of Sirtuins in SCA7
This proposal investigates the role of FOXO and Sirtuins in the pathogenesis of SCA7.

HighQ (Ellerby, Hughes, Gibson) **05/01/05 – 04/30/12**
Trainee: Xin Cong, Ph.D. \$219,349
Mass Spectrometry of Huntingtin
This proposal evaluates the impact of posttranslational modifications of huntingtin on Huntington's disease.

NIH T32 AG000266-10 (Campisi, Ellerby) **05/01/07 – 04/30/08**
NIA \$538,649
Trainee: Shona Monkerjee, Ph.D.
Mitochondrial Deletions in Huntington's Disease
This proposal investigates the role of mitochondrial deletions in Huntington's disease.

NIH T32 AG000266-10 (Campisi, Ellerby) **09/16/08 – 09/15/10**
NIA \$498,296

Trainee: Mahru An, Ph.D.

Characterization of iPS cells for Huntington's disease

This proposal seeks to characterize genetically modified induced pluripotent stem cells derived from Huntington's Disease patient somatic cells.

NIH F32 NS070491 (Carlotta Duncan, Ph.D.)

04/30/10 – 04/29/12

NINDS

\$98,932

Chaperone-mediated Autophagy in Spinocerebellar Ataxia Type 7 (SCA7)

The objective of this project is to understand how ataxin-7 is normally broken down in neurons, and how this process is deficient in polyQ-expanded ataxin-7.

NIH F32 NS080551 (Robert O'Brien, Ph.D.)

07/01/12 – 06/30/14

NINDS

Huntington's Disease: Analysis of Proteolysis

\$99,942

The proposal focuses on analysis of ROSA stop mice generated that express proteolytic cleavage products of Htt.

I) Mentored Graduate Students

Dominican University

08/26/08 – 05/31/10

Trainee: Jan Marie Cheng, M.S.

Post-Translational Modifications of Huntingtin and Caspases Attenuate Cellular Toxicity

Masters Thesis

Dominican University

08/26/11 – 05/31/13

Trainee: Bachir Hadid, M.S.

Role of Neurogenesis in Huntington's Disease and Aging

Masters Thesis

San Francisco State University

08/26/16 – 05/31/17

Trainee: Alexander Embusch, M.S.

Altered Expression of Matrix Metalloproteinases in Huntington's Disease Neural Stem Cells

Derived from HD Patient Induced Pluripotent Stem Cells

Masters Thesis

Dominican University

08/26/16 – 05/31/18

Trainee: Lakshika Madushani

Preclinical Evaluation of Matrix Metalloproteinase Inhibitors and Protein Kinase C Activators in Cell and Mouse Models of Huntington's Disease

Masters Thesis

Currently mentoring one Ph.D. student in our joint Aging Ph.D. program with University of Southern California.

J) Mentored Summer Interns

Trainee: Pete Lee	06/16/03 – 07/25/03
Trainee: Cassandra Peirano	06/16/03 – 08/08/03
Trainee: Shana Matthews	06/14/04 – 07/30/04
Trainee: Mike Steinbaugh	06/20/04 – 08/27/04
Trainee: Michelle Lee	06/08/05 – 08/05/05
Trainee: Gabriel Ocker	06/22/05 – 07/29/05
Trainee: Leah Tsang	06/22/05 – 08/05/05
Trainee: Stephanie Lynch	06/26/06 – 08/11/06
Trainee: Sophie Ellerby	07/09/07 – 08/01/08
Trainee: Lina Saeed	06/23/08 – 08/31/08
Trainee: Brian Rus	06/22/09 – 07/31/09
Trainee: Ella Saeed	06/22/09 – 07/31/09
Trainee: Justin Zamoyski	06/01/10 – 07/30/10
Trainee: Katherine Pond	07/01/12 – 09/01/12
Trainee: Raymond Ching	07/01/12 – 09/01/12
Trainee: Jeffrey Gu	06/01/18 – 08/01/18
Trainee: Madhav Mathur	06/01/18 – 09/01/18
Trainee: Stan Moroz	06/01/18 – 09/01/18

K) Research Associates who obtained Ph.D.

Amy Lin, Ph.D., Boston University

Jessica Young, Ph.D., University of Washington, now Assistant Professor at UW

L) Pharmaceutical Consultant

BioMarin Pharmaceuticals, 2014-present

Theravance Pharmaceutical, 2014

Asubo Pharmaceuticals, Inc, 2011

K) References

Professor Juan Botas, Baylor College of Medicine

Professor Harry Orr, University of Mennesota

Professor Marie-Francoise Chesselet, UCLA

Professor Leslie Thompson, UCLA

Professor Judith Campisi, Buck Institute

Professor Simon Melow, Buck Institute

Professor Gordon Lithgow, Buck Institute

Professor Christian Neri, INSERN

Professor William Yang, UCLA