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WORK

Research Assistant Professor, College of Medicine, UF, Florida *07/2021-Present*
Center for NeuroGenetics, Department of Molecular Genetics and Microbiology,
Advisor: Prof. Laura P. W. Ranum

Postdoctoral Associate/Fellow, College of Medicine, UF, Florida *2016-2021*
Center for NeuroGenetics, Department of Molecular Genetics and Microbiology,
Advisor: Prof. Laura P. W. Ranum

EDUCATION

Ph.D. in Chemistry, University of Illinois, Urbana-Champaign, Illinois *2011- 2016*
Advisor: Prof. Steven C. Zimmerman
Thesis title: *Design, synthesis, and biological activities of small molecules that target myotonic dystrophy*

B.S. in Chemistry, Hanoi College of Science, VNU, Vietnam *2006- 2011*
Advisor: Prof. Huy Quang Do
Thesis title: *Analysis of compounds in Aloe vera using gas chromatography-mass spectrometry*

RESEARCH EXPERIENCE

Research Assistant Professor, College of Medicine, UF, Florida *07/2021-Present*
Center for NeuroGenetics, Department of Molecular Genetics and Microbiology
Advisor: Prof. Laura P. W. Ranum

- Develop wet-lab and computational methods for identifying novel repeat expansion mutations
- Study roles of repeat expansion mutations in neurodegenerative diseases
- Study repeat expansion pathology in Alzheimer's disease, amyotrophic lateral sclerosis and other neurodegenerative diseases with unknown etiology
- Generate and study patient derived models of ALS and Alzheimer's disease (iPSC and organoid)

Postdoctoral Associate/Fellow, College of Medicine, UF, Florida *2016-2021*
Center for NeuroGenetics, Department of Molecular Genetics and Microbiology
Advisor: Prof. Laura P. W. Ranum

- Studied roles of *C9orf72* repeat-associated non-AUG (RAN) proteins in disease pathogenesis and developed passive immunotherapy strategy for *C9* ALS/FTD using a BAC mouse model and human anti-RAN protein antibodies
- Studied disease pathology using patient autopsy brain tissue and mouse models
- iPSC reprogramming, neural differentiation, and characterization
- Developed and characterized patient-derived organoid models
- Whole genome sequencing and bioinformatics
- Cloned vectors for antibody characterization, recombinant protein production in *E. Coli*, insect cells
- Developed assays to study protein turnover, protein interaction in cells
- Developed assays to study proteasome and autophagy activity in cells
- Generated knockout cell models using CRISPR technique
- Characterized repeat expansion mutations using Southern blot and repeat-crossing PCR
- Antibody development and characterization (immunofluorescence, Western blot, and immunohistochemistry)

- Designed and performed preclinical mouse studies
- Characterized a BAC mouse model of C9 ALS-FTD.
- Designed and developed highly sensitive high-throughput immunoassays
- Discovered biomarkers for neurodegenerative diseases and pharmacological markers for clinical study
- Developed tools to identify novel repeat expansion mutations

Pre-Postdoctoral Associate, College of Medicine, UF, Florida

02-05/2016

Center for NeuroGenetics, Department of Molecular Genetics and Microbiology

Advisor: Prof. Laura P. W. Ranum

- Developed therapeutic strategies for C9orf72 ALS/FTD using a BAC transgenic mouse model and human antibodies
- Characterized antibodies using immunofluorescence, western blot, and immunohistochemistry
- Cloned vectors for antibody characterization using immunofluorescence and western blot
- Mouse handling and characterization

Research Assistant in Chemical Biology, UIUC, Illinois

2011-2016

Department of Chemistry

Advisor: Prof. Steven C. Zimmerman

- Designed, modeled, synthesized, purified, and characterized (using NMR, MS, HPLC, LC-MS) novel inhibitors of MBNL1-CUG^{exp} and MBNL1-CCUG^{exp} complexes, and novel CUG^{exp} cleaving agents as RNase mimics for developing therapeutics for myotonic dystrophy
- Developed *in vitro* assays and in-cell assays for investigating protein-RNA interactions and biological activities of small molecules, e.g., in vitro transcription, electrophoretic and anisotropy assays, immunofluorescence, splicing assays, RT-qPCR
- Expressed, purified, and characterized recombinant proteins
- Synthesized RNA from designed sequences or plasmids
- Differentiated fibroblasts to myoblasts using retroviruses
- Developed assays for testing toxicity and stability of small molecules *in vitro* and *in vivo*
- Set-up and managed mammalian cell culture and radioactive laboratories

Research Assistant, Institute of forensic medicine and the EDC, Hanoi, Vietnam

2007-2011

Advisor: Prof. Huy Quang Do

- Extracted natural products from Aloe vera
- Analyzed natural products in Aloe vera using gas chromatography liquid chromatography

Research Assistant in Chemistry, University of Illinois, Urbana-Champaign, Illinois

06-08/2010

Department of Chemistry

Advisor: Prof. Anne M. Baranger

- Optimized electrophoresis mobility shift assays for screening inhibitors of MBNL1-CUG^{exp} complexes

HONORS & AWARDS

Grant awards

R01, NIH (NINDS/NIA)

Nguyen (multi-PI)

07/2022-06/2027

Novel repeat associated non-AUG (RAN) proteins in sALS, sFTD and SBMA: shared pathological features and unifying therapeutic opportunities. Approved by Council, Award pending.

ALSRP Therapeutic Idea Award

Nguyen (co-I)

07/2022-06/2024

Department of Defense

Identifying and targeting novel repeat-associated non-AUG (RAN) proteins in sporadic ALS

K99/R00: Pathway to independence award, NIH/NIA Nguyen (PI) 09/2020-08/2025
Identifying and understanding the role of repeat RNAs and RAN proteins in Alzheimer's disease

McJunkin Family Foundation award, UF Nguyen (PI) 09/2020-08/2021
Studying double-stranded RNA pathology in Alzheimer's disease

MBI Postdoctoral Fellowship, McKnight Brain Institute, UF Nguyen (PI) 09/2018-08/2020
Screening for expanded repeats in samples from Alzheimer's disease and unknown CNS diseases

Recognition

Best short talk, the 4th International Brainstorm Symposium, UF 2020
Best poster award, the 3rd International Brainstorm Symposium, UF 2018
Eastman Travel Award, University of Illinois, Urbana-Champaign, Illinois 2015
C. S. Marvel Fellowship, University of Illinois, Urbana-Champaign, Illinois 2014-2015
Ullyot Fellowship, University of Illinois, Urbana-Champaign, Illinois 2013-2014
Nomination for HHMI fellowship, University of Illinois, Urbana-Champaign 2014
Introducing our authors, ACS Chemical Biology 2013
Outstanding Student Award, Hanoi University of Science 2010
Summer Internship Award, University of Illinois at Urbana-Champaign 2010
Excellent Student Scholarship, Hanoi University of Science 2006-2011

PUBLICATIONS

Published in peer-reviewed journals

1. Guo, S.; **Nguyen, L.***; Ranum, L. P. W*. RAN proteins in neurodegenerative disease: Repeating themes and unifying therapeutic strategies, *Curr. Opin. Neurobiol.* **2022**, *72*, 160-170. PMID: 34953315 (*co-corresponding author)
2. Tusi, S. K.; **Nguyen, L.**; Thangaraju, K.; Cleary, J. D.; Zu, T.; Ranum, L. P. W. The alternative initiation factor eIF2A plays key role in RAN translation of myotonic dystrophy type 2 CCUG•CAGG repeats. *Hum. Mol. Genet.* **2021**, *30*, 1020-1029. PMID: 33856033
3. Pattamatta, A.; **Nguyen, L.**; Olafson, H.; Scotti, M.; Liu, Y.; Richardson, J.; Berglund, A. J.; Zu, T.; Wang E. T.; Ranum, L. P. W. Repeat length increases disease penetrance and severity in C9orf72 ALS/FTD BAC transgenic mice. *Human Molecular Genetics*, **2021**, *29*, 3900-3918. PMID: 33378537
4. **Nguyen, L.***; Laboissonniere, L. A.*; Guo, S.*; Pilotto, F.; Scheidegger, O.; Oestmann, A.; Hammond J. W.; Li, H.; Hyysalo, A.; Peltola, R.; Pattamatta, A.; Zu, T.; Voutilainen, M. H.; Gelbard, H. A.; Saxena, S.; Ranum, L. P. W. Survival and motor phenotypes in FVB C9-500 ALS/FTD BAC transgenic mice reproduced by multiple labs, *Neuron*, **2020**, *108*, 1-13. (*co-first author). PMID: 33022226
5. Zu, T.; Bardhi, O.; Guo, S.; Ryskamp, D.; Tusi S. K; Liu, Y.; Klippel, K.; Chakrabarty, P.; **Nguyen, L.**; Golde T. E., Sonenberg, N.; Ranum L. P. W. Metformin inhibits RAN translation through PKR pathway and corrects ALS/FTD phenotypes in C9orf72 mouse model. *Proc. Natl. Acad. Sci. U.S.A.* **2020**, *117*(31), 18591-18599. PMID: 32690681
6. **Nguyen, L.**; Montrasio, M.; Pattamatta, A.; Tusi, S. K.; Bardhi, O.; Meyer, K. D.; Hayes, L.; Nakamura, K.; Banez-Coronel, M.; Coyne, A.; Guo, S.; Laboissonniere, L. A.; Gu, Y.; Narayanan, S.; Smith, B.; Nitsch, R. M.; Kankel, M. W.; Rushe, M.; Rothstein, J.; Zu, T.; Grimm, J.; Ranum, L. P. W. Antibody therapy targeting RAN proteins rescues C9 ALS/FTD in C9orf72 mouse model, *Neuron*, **2020**, *105*(4), 645-662. PMID: 31831332

7. **Nguyen, L.**; Cleary, J. D.; Ranum, L. P. W. Repeat associated non-ATG translation: molecular mechanisms and contribution to neurologic disease, *Annu. Rev. Neurosci.* **2019**, *42*, 227-24. PMID: 30909783
8. Chu D. T.; Minh Nguyet N. T.; Nga V. T.; Thai Lien N. V.; Vo D. D.; **Nguyen L.**; Nhu Ngoc V. T.; Son L. H.; Le D. H.; Nga V. B.; Van Tu P.; Van To T.; Ha L. S.; Tao Y.; Pham V. H.; An update on obesity: Mental consequences and psychological interventions, *Diabetes Metab Syndr.* **2019**; *13*(1), 155-160. PMID: 30641689
9. Bai, Y.*; **Nguyen, L.***; Song, Z.*; Peng, S.; Zheng, N.; Lee, J.; Kapoor, I.; Cheng, J.; Chan, W.-Y.; Zimmerman, S. C. Integrating Display and Delivery Functionality with a Cell Penetrating Peptide Mimic as a Scaffold for Intracellular Multivalent Multi-targeting, *J. Am. Chem. Soc.* **2016**, *138*, 9498–9507. (*co-first author). PMID: 27355522
10. Luu, M. L.*; **Nguyen, L.***; Chan, W.-Y.; Zimmerman, S. C. A Potent Inhibitor of Protein Sequestration by Expanded Triplet (CUG) Repeats that Shows Phenotypic Improvements in a *Drosophila* Model of Myotonic Dystrophy, *ChemMedChem* **2016**, *11*, 1428-1435. (*co-first author). PMID: 27245480
11. **Nguyen, L.**; Luu, M. L.; Peng, S.; Serrano, F. J.; Chan, W.-Y.; Zimmerman, S. C. Rationally designed small molecules that target both the DNA and RNA causing myotonic dystrophy type 1, *J. Am. Chem. Soc.* **2015**, 14180–14189. (UIUC news). PMID: 26473464
12. **Nguyen, L.**; Lee, J.; Wong, C.-H.; Zimmerman, S. C. Small Molecules that Target the Toxic RNA in Myotonic Dystrophy Type 2, *ChemMedChem* **2014**, *9*, 2455–2462. (designated VIP, highlighted on the journal cover). PMID: 24938413
13. Wong, C.-H.; **Nguyen, L.**; Peh, J.; Luu, L. M.; Sanchez, J. S.; Richardson, S. L.; Tuccinardi, T.; Ho, T.; Chan, E. H. Y.; Chan, W.-Y.; Baranger, A. M.; Hergenrother, P. J.; Zimmerman, S. C. Targeting toxic RNAs that cause myotonic dystrophy type 1 (DM1) with a bisamidinium inhibitor, *J. Am. Chem. Soc.* **2014**, *136*, 6355–6361. PMID: 24702247
14. Jahromi, A. H.; Fu, Y.*; Miller, K. A.*; **Nguyen, L.***; Luu M. L.; Baranger, A. M.; Zimmerman, S. C. Developing bivalent ligands to target CUG triplet repeats, the causative agent of myotonic dystrophy type 1, *J. Med. Chem.* **2013**, *56*, 9471–9481. (*co-second author). PMID: 24188018
15. Jahromi, A. H.; **Nguyen, L.**; Fu, Y.; Miller, K. A.; Baranger, A. M.; Zimmerman, S. C. A novel CUG^{exp}-MBNL1 inhibitor with therapeutic potential for myotonic dystrophy type 1, *ACS Chem. Biol.* **2013**, *8*, 1037–1043. (Highlighted on the journal cover and UIUC news). PMID: 23480597
16. Do, Q. H.; Pham V. T.; **Nguyen, L.**; Do, Q. C. Analysis of compounds in *Aloe vera* by gas chromatography-mass spectrometry, *Science and Technology office, Vietnam* **2010**, *2*.

Manuscripts in preparation

Nguyen, L.; Thangaraju, K.; Ajredini, R.; Klippel, K.; Ranum, P.; Zu, T.; Pletnikova, O.; Yachnis, T.; Prokop, S.; Tronscoso, Y.; Golde, T. E.; Wang, E. T.; Ranum, L. P. W. Novel repeat expansion mutations increase risk and produce polymeric proteins in Alzheimer's disease patient autopsy brains.

PATENTS

Ranum, L. P. W.; **Nguyen, L.** Methods for treating RAN protein-associated neurological diseases. *Pending*

Zimmerman, S. C.; Luu, L. M.; **Nguyen, L.** Bisamidinium-based inhibitors for the treatment of myotonic dystrophy, US Patent Application 15/502,474

Ranum, L. P. W.; **Nguyen L.** MSD assay to detect dipeptide RAN proteins in *C9orf72* ALS/FTD. *Pending*

Zimmerman, S. C.; Luu, L. M.; **Nguyen, L.** Compounds and Methods for Myotonic Dystrophy Therapy, US Patent Application 14/822,796

PRESENTATIONS

Oral presentations

Department of Molecular Genetics and Microbiology and Scripps Research, University of Florida, 04/2022, RNA club invited talk: *RAN proteins and RAN translation in neurodegenerative disease: Therapeutic targets and tools for genome exploration.*

Department of Neurosciences, UCSD, 04/2022, invited talk: *Decoding the repeatome to explore biological functions and roles in disease.*

Neuroscience symposium, David Geffen School of Medicine, UCLA, 01/2022, invited talk: *Studying roles of repeat expansions in neurodegenerative disease.*

Department of Neuroscience, University of Florida, 09/2021, invited talk: *RAN proteins in neurodegenerative disease: therapeutic targets and tools for genome exploration.*

The RNA Institute, University at Albany, SUNY, 09/2021, invited talk: *RAN proteins as therapeutic targets for C9orf72 ALS/FTD.*

Expanded repeat disorders: from mechanisms to therapies meeting, UK Dementia Research Institute, 06/2021, selected talk, and rapid-fire talk: *Identification of repeat expansion mutations using deactivated-Cas9-based repeat enrichment and detection (dCas9READ).*

Myology institute, University of Florida, 02/2020, invited talk: *Antibody therapy targeting RAN proteins rescues ALS/FTD in C9orf72 mouse model.*

McKnight Brain Institute, University of Florida, 06/2020, Neuromedicine seminar series, invited talk: *Immunotherapy for C9orf72 ALS/FTD.*

The 4th International Brainstorm Symposium, Center for NeuroGenetics, College of Medicine, University of Florida, 01/2020, selected short talk presentation: *Antibody therapy targeting RAN proteins rescues ALS/FTD in C9orf72 mouse model.* Selected best talk.

The 3rd International Brainstorm Symposium, Center for NeuroGenetics, College of Medicine, University of Florida, 01/2018, selected talk and poster presentation: *Immunotherapy development for C9ORF72 ALS/FTD using a BAC transgenic mouse model and human antibodies targeting RAN proteins.* Selected best poster.

Center for NeuroGenetics, College of Medicine, University of Florida, Gainesville, Florida, 09/2015, invited talk: *Small molecules targeting expanded CCUG and CUG repeats as potential therapeutics for myotonic dystrophy.*

International Myotonic Dystrophy Consortium meeting, Paris, France, 06/2015, Therapeutic development section, Therapeutic Strategies: *Small molecules that control the fate of CUG repeat RNA transcripts-the causative agent in myotonic dystrophy type 1.*

National ACS meeting, San Francisco, California, 08/2014, Biologically related molecules and processes section, Division of Organic Chemistry: *Small molecules that control the fate of CUG repeats-the causative agent in myotonic dystrophy type 1.*

Novartis Symposium, Urbana, Illinois, 04/2014, oral presentation: *Targeting a toxic RNA that causes myotonic dystrophy type 1 with a bisamidinium inhibitor.*

Allerton Conference, Monticello, Illinois, 11/2013: *Bis-amidinium based ligands as potential therapeutics for myotonic dystrophy.*

National ACS meeting, Indianapolis, Indiana, 9/2013, Biologically related molecules and processes section, Division of Organic Chemistry: *Biological activity of rationally designed inhibitors for CUG^{exp}-MBNL as potential leads for myotonic dystrophy type 1.*

Poster presentations

Neurodegenerative diseases: Biology & Therapeutics, Cold Spring Harbor Laboratory, 12/2020: *Identification of repeat expansion mutations using novel deactivated-Cas9-based tools.*

Alzheimer's Association International Conference® (AAIC) Neuroscience Next meeting, 11/2020: *Identification of repeat expansion mutations using novel deactivated-Cas9-based tools.*

Antibody Engineering and Therapeutics meeting, San Diego, 12/2019: *Antibody therapy targeting RAN proteins rescues ALS/FTD in C9orf72 mouse model.*

Neurodegenerative diseases: Biology & Therapeutics, Cold Spring Harbor Laboratory, 12/2018: *Antibody therapy targeting RAN proteins rescues ALS/FTD in C9orf72 mouse model.*

The 2018 College of Medicine Celebration of Research, College of Medicine, University of Florida, 02/2018: *Immunotherapy development for C9ORF72 ALS/FTD using a BAC transgenic mouse model and human antibodies targeting RAN proteins.*

The 3rd International Brainstorm Symposium, Center for NeuroGenetics, College of Medicine, University of Florida, 01/2018, selected talk and poster presentation: *Immunotherapy development for C9ORF72 ALS/FTD using a BAC transgenic mouse model and human antibodies targeting RAN proteins.*

The American Society of Human Genetics annual meeting, Orlando, Florida, 09/2017, poster presentation: *Immunotherapy development for C9ORF72 ALS/FTD using a BAC transgenic mouse model and human antibodies targeting RAN proteins.*

The 2017 College of Medicine Celebration of Research, College of Medicine, University of Florida, 02/2017, poster presentation: *Antibodies as tools for research on C9orf72 ALS/FTD.*

Community focus symposium, Center for NeuroGenetics, College of Medicine, University of Florida, Gainesville, 07/2016, poster presentation: *Antibodies as tools for research on C9orf72 ALS/FTD.*

CBI Career Development Conference, 6/2013, Urbana, Illinois, poster presentation, *Biological activity of rationally designed inhibitors for CUG^{exp}-MBNL as potential leads for Myotonic Dystrophy type 1.*

The second Symposium on Natural Products Research and Development in Hanoi, Vietnam, 12/2010, poster presentation, *Analysis of compounds in Aloe vera from Vietnam by gas chromatography-mass spectrometry.*

TEACHING EXPERIENCE

Graduate teaching assistant, UIUC Fall 2012
Course: CHEM 242 and CHEM 243
Responsibilities: Developed the websites

Graduate teaching assistant, University of Illinois, Urbana-Champaign Spring 2012
Course: CHEM 437
Responsibilities: Developed synthesis procedures

Graduate teaching assistant, University of Illinois, Urbana-Champaign Fall 2011

Responsibilities: developed course materials including selecting topics and preparing quizzes for *iChemistry* project

Graduate teaching assistant, University of Illinois, Urbana-Champaign
Course: CHEM 445 and 447
Responsibilities: Develop fluorescence experiments

Spring 2011

MENTORING EXPERIENCE

Mentor for Luke Nourie, University of Florida rotation student
Mentor for Rodrigo F. Tomas, University of Florida rotation student,
Mentor for Kelena Klippel, University of Florida master student
Mentor for Savannah J. Weeks, University of Florida rotation student
Mentor for Avery C. Engelbrecht, University of Florida rotation student
Mentor for Mesfin M. Gobena, University of Florida rotation student
Mentor for University of Illinois, Urbana-Champaign junior graduate and undergraduate students (JuYeon Lee, To (Kevin) Wang, Elissia Franklin, Shannon Davies)

Fall 2021
Fall 2020
Fall 2020
Spring 2020
Fall 2018
Fall 2018
2012-2016

SERVICE & OUTREACH

Peer reviewer

European Journal of Clinical Microbiology & Infectious Diseases, Clinical Genetics

2017-present

Affiliations

American Chemical Society
American Society of Human
Genetics

2013-2015
2017-present

Workshop

Antibody Engineering and Therapeutics meeting, San Diego, 12/2019, *Introduction to Antibody Engineering*

External activities

Alachua Habitat for Humanity
Role: Volunteer
Ronald McDonald House Charities (North Central Florida)
Role: Volunteer
The Vietnamese Professionals Network in the U.S.
Role: Member
Antibody Engineering meeting
Role: Attendee
Target ALS annual meeting, Boston, Massachusetts
Role: Postdoc fellow
RNA metabolism in Neurological Disease, Chicago, Illinois
Role: Attendee
MDA Muscle walk, Springfield, Illinois
Role: Attendee and fundraiser
Myotonic Dystrophy Foundation annual conference, Washington DC
Role: Attendee
Vietnamese Student Community at UIUC, Illinois
Role: Vice president
Hanoi AISEC
Role: Attendee

09/2016-Present
11/2017-Present
02/2018-Present
12/2017
2017-2019
09/2015
05/2015
09/2014
2012-2013
03/2011

REFERENCES

Laura P. W. Ranum

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Director of Center for NeuroGenetics

Department of Molecular Genetics and Microbiology

College of Medicine

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Maurice Swanson

Professor

Associate Director of Center for NeuroGenetics

Department of Molecular Genetics and Microbiology

University of Florida

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Roger Adams Professor of Chemistry

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University of Illinois at Urbana-Champaign

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Urbana, IL 61801

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Andrew Berglund

Empire Professor of Innovation

Director, The RNA Institute and SUNY

Department of Biological Sciences

University at Albany

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