BIOGRAPHICAL SKETCH

NAME: **Gabsang Lee, Ph.D., D.V.M.**

eRA COMMONS USER NAME (credential, e.g., agency login): **GABSANGLEE**

POSITION TITLE: **Professor (at Johns Hopkins University School of Medicine)**

EDUCATION/TRAINING

INSTITUTION AND LOCATION	DEGREE	Completion Date	FIELD OF STUDY
Seoul National University, Seoul, Korea	B.S.&D.V.M.	02/2000	Veterinary Medicine
Seoul National University, Seoul, Korea	Ph.D.	02/2004	Veterinary Medicine
Sloan Kettering Institute, New York, NY	Postdoc	08/2011	Developmental Biology/ Stem cell biology

A. Personal statement

During my postdoctoral training, I have worked on the derivation of various cell types from human induced pluripotent stem cells (hiPSCs) for disease modeling and drug discovery (*Lee et al., Nature Biotech., 2007; Lee et al., Nature, 2009; Lee et al., Nature Protocols, 2010; Lee et al., Nature Biotech., 2012*). As an independent investigator at the Johns Hopkins University School of Medicine (since 2011), my group has continuously worked using stem cell technologies to shape new paradigms. Recently, our group has published research papers, including development of a new congruent human Schwann cell model system to study a genetic neural disorder, Charcot-Marie-Tooth 1A (**1**. *Mukherjee-Clavin et al., Nature Biomedical Engineering)*, comparing ocular and spinal motor neurons of amyotrophic lateral sclerosis to identify a new potential drug (**2**. *Lee et al., Nature Neuroscience, 2021*), derivation of functional and engraftable satellite cells from hPSCs for muscular dystrophies (**3.** *Sun et al., Cell Stem Cell, 2022*) and establishment of a new optogenetic alpha-synuclein aggregation system to find a drug candidate for Parkinson's disease (**4.** *Kim et al., Cell Stem Cell, 2023*). In summary, my group has the energy, determination, and innovation necessary to plan and execute the effective strategies for accomplishing the innovative goals.

1. Mukherjee-Clavin B, Mi R, Kern B, Choi IY, Lim HT, Oh YH, Lannon B, Kim KJ, Bell S, Hur JK, Hwang WC, Habib O, Baloh RH, Eggan K, Brandacher G, Hoke A, Studer L, Kim YJ, **Lee G**. Comparison of three congruent patient-specific cell models for the modelling of a human genetic neurological disorder. **Nature Biomedical Engineering** 2019 3:571-582. PMCID: PMC6612317

2. Lee H, Lee JJ, Park NY, Dubey SK, Kim T, Ruan K, Lim SB, Park S-H, Ha S, Kovlyagina I, Kim K-T, Kim S, Oh Y, Kim H, Kang S-U, Song M-R, Lloyd TE, Maragakis NJ, Hong YB, Eoh H, **Lee G**. Multi-omic analysis of selectively vulnerable motor neuron subtypes implicates altered lipid metabolism in ALS. **Nature Neuroscience** 2021 Dec 21. PMID: 34782793

3. Sun C, Kannan S, Choi IY, Lim HT, Zhang H, Chen GS, Zhang N, Park S-H, Serra C, I SR, Lloyd TE, Kwon C, Lovering RM, Lim SB, Andersen P, Wagner KR, **Lee G**. Human pluripotent stem cell-derived myogenic progenitor cells engraft to become quiescent functional satellite cells *in vivo*. **Cell Stem Cell** 2022 Apr 7;29(4):610-619.PMID: 35395188

4. Kim MS, Ra AE, Kweon SH, Seo BA, Ho AS, Oh Y, **Lee G**. Advanced human iPSC-based preclinical model for Parkinson's disease with optogenetic alpha-synuclein aggregation **Cell Stem Cell** 2023 Jul 6;30(7):973-986. PMID: 37339636

B. Positions, Scientific Appointments, and Honors

Positions and Scientific Appointments

2202-current Professor, Institute of Cell Engineering, Department of Neurology, at Johns Hopkins University School of Medicine (Baltimore, USA)

2016-2021 Associate Professor, Institute of Cell Engineering, Department of Neurology, at Johns Hopkins University School of Medicine (Baltimore, USA)

2011-2016 Assistant Professor, Institute of Cell Engineering, Department of Neurology, at Johns Hopkins University School of Medicine (Baltimore, USA)
2008-2011 Postdoctoral Research Associate at Sloan Kettering Institute (New York, USA)
2007-2008 Research Professor at Korean University Medical Center (Seoul, Korea)
2007-2007 BK21 Research Professor at Seoul National University (Seoul, Korea)
2004-2007 Postdoctoral Researcher at Sloan Kettering Institute (New York, USA)

Honors and Award

Excellent Thesis Presentation from Seoul National University, Korea (2004), Travel Award from International Society of Stem Cell Research (2007), New York Stem Cell Foundation Druckenmiller Fellowship (2009) Travel Scholarship from International Consortium of Stem Cell Networks, Canada (2011), New York Stem Cell Foundation Robertson Investigator (2011), ISSCR Abstract Review Committee (2012–), Maryland Stem Cell Research Award (2012, 2013, 2017, 2018), Blaustein Pain Research Award (2013), Program Committee member, Maryland Stem Cell Conference (2014), FSH Society Max Weintraub Memorial Research Fellowship (2015), Mirowski Award for Cardiovascular Research (2015), Organizer for Institute for Cell Engineering Regenerative Medicine Research Seminar Series (2015-2020), Johns Hopkins Catalyst Award (2016), STAR (Strategy to Accelerate Research) member in the CMT Association (2016-2018), the Johns Hopkins ISCRO Committee (2016-2023), SAB member in the CMT Research Foundation (2018- 2023), In-Depth Review Committee for the Director in the Institute for Basic Science (South Korea) (2023)

Scientific Review Activity

• *Grant/applications Review*

NIH Study Section (USA), Telethon Foundation (Italy), Medical Research Council (UK), Biotechnology Research Grant in North Carolina (USA), Helis Marvin Foundation (USA), CMT Association (USA), ICTR ATIP grant program (USA), Muscular Dystrophy Association (USA and UK), ANR (France), Research Grant Council (Hong Kong, China), Samsung Research Foundation (South Korea), Kavli Foundation (USA), JHU PURA program (USA), JHU FARMS Foundation (USA), Israel Science Foundation (Israel), the Hopkins Heart Initiative (USA),

• *Editorial Board*

2018– Experimental Neurology

• *Journal Review*

Biological Psychiatry, Biomaterials, BMC Pediatrics, Brain Research, Cell, Cell Reports, Cell Reprogramming, Cells, Cell Stem Cell, Cellular and Molecular Life Science, Expert Opinion on Biological Therapy, FASEB Journal, Frontiers Neuroscience, Future Medicine, Gene Therapy, International Journal of High Throughput Screening, International Journal of Molecular Sciences, Journal of Biomolecular Screening, Journal of Biological Chemistry, Journal of Cell Science, Journal of Cellular Physiology, Journal of Clinical Investigation, Journal of Molecular Science, Journal of Visualized Experiments, Nature Communications, Nature Neuroscience, Neurological Research, Nuclei Acid Research, Oncology Reports, PLOS One, Proteomics, Science Advances, Scientific Reports, Stem Cells, Stem Cell and Development, Stem Cell International, Stem Cell Reports, Stem Cell Research, Stem Cells Translational Medicine, Translational Research

Educational Activity

• Mentoring/Trainees: 16 postdoctoral fellows, 2 graduate students, 4 research technicians, 12 visiting/undergraduate research assistants.

• Classroom Teaching: NeuroCog I Course in Neuroscience Graduate Course (2010–present), Advanced Topic in Human Genetics Course (2013–present), and Translational Science Topics in Interdisciplinary Medicine (TIME) course (2016–2020)

• Admission Committees for Neuroscience and Biomedical Engineering (BME) Graduate Students, Johns Hopkins School of Medicine

Lab Honors (selected)

2016 Assistant Prof. (tenure-track), KyungHee University School of Medicine (YongJun Kim)
2017 Senior Researcher, Samsung Medical Center (Minjeong Kim)
2017 Assistant Prof. (tenure-track), Chungbuk National University (Yubyeol Jeon)

2018 Assistant Prof. (tenure-track), Hanyang University, School of Medicine (Yohan Oh)
2021 Scientist, Cedar-Saini Medical Center (Hojae Lee)
2021 Senior Scientist, Vita Therapeutics (Congshan Sunny Sun)
2021 Merkin Postdoctoral Fellowship (Seong-Hyun Park)
2022 Assistant Prof. (tenure-track), Johns Hopkins Univ. School of Medicine (Bipasha Mukherjee-Clavin)
MSCRF Postdoctoral Fellowship; Congshan Sunny Sun, 2106; Minseong Kim, 2017; Suchan Lee, 2017; Seong-Hyun Park, 2020; Wonjin Yun, 2022).

Invited Talks (listed since 2019)
Research talks in Stem Cell Symposium at Washington University in St. Louis (virtual 2022), International Society of Stem Cell Research (San Francisco, 2022), Annual Meeting of the Organoid Society (virtual 2022), Gene Therapy Initiative Meeting at the Gilbert Family Foundation (Detroit, 2022), Hanyang University Medical Center (Seoul, Korea, 2022), Association of Korean Neuroscientist (virtual 2022), Institute of Basic Science (Daejeon, Korea, 2022), Korea Advanced Institute of Science & Technology (Daejeon, Korea, 2022), Fusion-Tech Center at Hanyang University (Seoul, Korea, 2022), Pohang University of Science and Technology (Pohang, Korea, 2022), Annual Samsung Gene and Cell Therapy Symposium (Seoul, Korea, 2022), Korean Brain Research Institute (Deagu, Korea, 2022), Korean Society for Brain and Neural Sciences (Songdo, Korea, 2022), Body Myositis Research Center (virtual 2022), RYR-1 Foundation (virtual, 2022), Medical University of South Carolina (Charleston, virtual, 2021), Seminar in University of Georgia (Athens, virtual, 2021), Annual Seminar of Charcot-Marie-Tooth Research Foundation (Washing DC, 2021, virtual), Packard ALS Center, (Baltimore, 2021, virtual), Annual Seminar of International Chemical Biology Society (Atlanta, 2020, virtual), Korean Society for Stem Cell Research (South Korea, 2020, virtual), Korean Association for Laboratory Animal Science, (South Korea, 2020, virtual), NYSCF Fellowship awardees alumni meeting (New York, 2019), Charcot Marie Tooth Research Foundation, (Orlando, 2019), Distinguished Grand Rounds speaker, Department of Pathology's Grand Rounds at the Johns Hopkins Hospital (Baltimore, 2019)

C. Contributions to Science

1) My recent study has been focusing on how we can study genetic diseases with human induced pluripotent stem cells (hiPSCs), in particular neural crest-related disorders. Based on our original protocol for cell specification toward neural crest, peripheral neurons and Schwann cells (cited in >490 papers), I have modeled multiple diseases with patient-specific hiPSCs (cited in >940 papers), followed by large-scale of drug screening with patient-hiPSC-derived cells, and found several promising compounds (cited in >190 papers). Further, my lab just published a novel approach to convert human fibroblasts into induced neural crest (iNC)(cited in >120 papers), which verified our findings of patient-specific hiPSC studies. My efforts set a new paradigm how to utilize new reprogramming technologies for confronting human genetic disorders in peripheral nervous system and skeletal muscle.
   a. **Lee G**, Kim HS, Elkabetz Y, Al Shamy G, Panagiotakos G, Barberi T, Tabar V, Studer L. Isolation and directed differentiation of neural crest stem cells derived from human embryonic stem cells. **Nature Biotechnology** 2007 25:1468-1475. *PMID 18037878*
   b. **Lee G**, Papapetrou EP, Kim HS, Chambers SM, Tomishima MJ, Fasano CA, Viale A, Tabar V, Sadlain M, Studer L. Modeling Pathogenesis and Treatment of Familial Dysautonomia using patient-specific iPSCs. **Nature** 2009 461: 402-406. *PMCID: PMC2784695*
   c. Kim YJ, Lim HT, Li Z, Oh Y, Kovlyagina I, Choi IY, Dong XZ, **Lee G**. Generation of multipotent induced neural crest by direct reprogramming of human postnatal fibroblasts with a single transcription factor. **Cell Stem Cell** 2014 15:497-506. PMID: 25158936

2) There are many detrimental diseases in the nervous system, but there is very limited therapeutic intervention, which is mostly due to the lack of humanized model system. We have focused on developing humanized model systems.
   a. Oh YH, Cho GS, Li Z, Hong I, Zhu R, Kim MJ, Kim YJ, Tampakakis E, Tung L, Huganir R, Dong X, Kwon C, **Lee G**. Functional coupling with cardiac muscle promotes maturation of hPSC-derived sympathetic neurons. **Cell Stem Cell** 2016 PMCID: PMC4996639
   b. Oh Y, Zhang F, Wang Y, Lee EM, Choi IY, Lim H, Mirakhori F, Li R, Huang L, Xu T, Wu H, Li C, Qin C-F, Wen Z, Wu Q-F, Tang H, Xu Z, Jin P, Song H, Ming G-L, **Lee G**. Zika virus directly infects human peripheral neurons and induces cell death. **Nature Neuroscience**. 2017 20:1209-1212. PMCID:

PMC5575960

c. Zeltner N, Fattahi F, Dubois NC, Saurat N, Lafaille F, Shang L, Zimmer B, Tchieu J, Soliman MA, **Lee G,** Casanova JL, Studer L. Capturing the biology of disease severity in a PSC-based model of familial dysautonomia. **Nature Medicine.** 2016 22:1421-7. PMCID: PMC5555047

d. Lee H, Lee JJ, Park NY, Dubey SK, Kim T, Ruan K, Lim SB, Park S-H, Ha S, Kovlyagina I, Kim K-T, Kim S, Oh Y, Kim H, Kang S-U, Song M-R, Lloyd TE, Maragakis NJ, Hong YB, Eoh H, **Lee G**. Multi-omic analysis of selectively vulnerable motor neuron subtypes implicates altered lipid metabolism in ALS. **Nature Neuroscience** 2021 Dec 21. PMID: 34782793

3) Recently we have another research focus on studying muscular dystrophies with hiPSCs. Our group has developed a novel myogenic specification for Duchenne muscular dystrophy, and identified disease-related phenotypes, which was pharmacologically rescued. We are continuing this line of study to understand human myogenesis and their relevant diseases.

a. Choi IY, Lim HT, Estrellas K, Mula J, Cohen TV, Zhang Y, Donnelly CJ, Richard JP, Kim YJ, Kim HS, Kazuki Y, Oshimura M, Li HL, Hotta A, Rothstein J, Maragakis N, Wager K, **Lee G**. Concordant but varied phenotypes among patient-specific myoblasts of Duchenne muscular dystrophy revealed by a human iPSC-based model. **Cell Reports** 2016 15:2301-12. PMID: 27239027

b. Choi IY, Lim HT, Cho HJ, Oh Y, Chou B-K, Bai H, Cheng L, Kim YJ, Hyun S-H, Kim H, Shin JH, **Lee G**. Transcriptional landscape of myogenesis from human pluripotent stem cells reveals a key role of TWIST1 in maintenance of skeletal muscle progenitors. pii: e46981. **eLIFE** PMCID: PMC6996923

c. Sun C, Choi IY, Rovira Gonzalez YI, Andersen P, Talbot CC Jr, Iyer SR, Lovering RM, Wagner KR, **Lee G**. Duchenne muscular dystrophy hiPSC-derived myoblast drug screen identifies compounds that ameliorate disease in mdx mice. Jun 4;5(11):e134287. **JCI Insight** PMCID: PMC7308059

d. Sun C, Kannan S, Choi IY, Lim HT, Zhang H, Chen GS, Zhang N, Park S-H, Serra C, I SR, Lloyd TE, Kwon C, Lovering RM, Lim SB, Andersen P, Wagner KR, Lee G. Human pluripotent stem cell-derived myogenic progenitor cells engraft to become quiescent functional satellite cells in vivo. 2022 Apr 7;29(4):610-619. **Cell Stem Cell** PMID: 35395188

4) Some of my contributions are through collaborations with my colleagues, mostly on hiPSC studies, including cell specification, modeling genetic and infection diseases, and high throughput screening approaches, to address multiple biological questions.

a. Choi J, Lee S, Clement K, Mallard W, Tagliazucchi GM, Lim H, Choi IY, Ferrari F, Tsankov A, Pop R, **Lee G,** Rinn J, Meissner A, Park PJ, and Hochedlinger K. A comparison of genetically matched cell lines re veals the equivalence of human iPSCs and ESCs. **Nature Biotechnology** 2015 33:1173-81*. PMCID: PMC4847940*.

b. A Dynamic Unfolded Protein Response Contributes to the Control of Cortical Neurogenesis. Laguesse S, Creppe C, Nedialkova DD, Prévot PP, Borgs L, Huysseune S, Franco B, Duysens G, Krusy N, **Lee G,** Thelen N, Thiry M, Close P, Chariot A, Malgrange B, Leidel SA, Godin JD, Nguyen L. **Developmental Cell.** 2015 35:553-67. *PMID 26651292.*

c. Transneuronal Propagation of Pathologic α-Synuclein from the Gut to the Brain Models Parkinson's Disease. Kim S, Kwon SH, Kam TI, Panicker N, Karuppagounder SS, Lee S, Lee JH, Kim WR, Kook M, Foss CA, Shen C, Lee H, Kulkarni S, Pasricha PJ, **Lee G**, Pomper MG, Dawson VL, Dawson TM, Ko HS. **Neuron.** 2019 ;103(4):627-641 *PMCID: PMC6706297*

5) To share our newly developed protocols and opinions, I have published protocols and reviews papers.

a. Choi IY, Lim HT, **Lee G***. Efficient generation human induced pluripotent stem cells from human somatic cells with sendai-virus. 2014 86:51406. *PMCID: PMC4179475* **JoVE. ** Corresponding Author***

b. Lim HT, Choi IY, **Lee G***. Profiling individual human embryonic stem cells by quantitative RT-PCR. 2014 87:51408. *PMCID: PMC4209783* **JoVE ** Corresponding Author***

c. Kim YJ, **Lee G***. Candidate ALS Therapeutics Motor toward "In Vitro Clinical Trials". **Cell Stem Cell.** 2013 12:633-644. *PMID 23746968* **** Corresponding Author***

Complete List of Published Work in MyBibliography:
https://www.ncbi.nlm.nih.gov/myncbi/1deyyuflJkcAm/bibliography/public/