Carola Maturana

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Professional Profile

Highly motivated and results-driven Research Scientist with over 6 years of experience in the field of gene therapy, specializing in AAV vector development and validation. Proven ability to lead and execute complex research projects, driving innovation and contributing to the advancement of preclinical studies. Expertise in biochemical, molecular and cellular biology, data analysis, and interdisciplinary collaboration, as well as a strong understanding of the biopharmaceutical industry and its regulatory landscape.

- AAV vector production, purification, and validation
- AAV viral vector design and Titer measurement
- Design *in-vivo* preclinical studies
- Primary cell culture and cell line management
- SOP Development & Implementation
- Software: SnapGene, GraphPad, QuPath

Experience

DIORASIS Therapeutics Inc — *Durham, NC*
RESEARCH SCIENTIST, R&D — *May 2024 – Present*

- Developed and executed *in-vitro* assays supporting AAV gene therapy programs for glaucoma.
- Independently managed human primary cell and cell lines, DNA/RNA extraction, and cell transfection.
- Implemented PCR, RT-PCR, qPCR, and ELISA techniques to analyze data, contributing to project success.
- Drafted standard operating procedures (SOPs) and testing protocols, ensuring consistent and high-quality data collection.
- Collaborated effectively with cross-functional teams to ensure project timelines were met and deliverables were achieved.

IVIEW Therapeutics Inc. — *Cranbury, NJ*
RESEARCH SCIENTIST, R&D — *January 2023 – May 2024*

- Developed and executed *in-vitro* and *ex-vivo* translational assays for AAV gene therapy programs in glaucoma.
- Managed human primary cell, cell lines, and human eye tissue models, DNA/RNA/protein extraction, PCR, RT-PCR, qPCR, ELISA, titer and endotoxin assays, and IF.
- Optimized and validated biodistribution and AAV transduction efficacy assays using ELISA to accurately measure ROCK activity.
- Contributed to method validation plans and drafted testing protocols and SOPs, ensuring robust data integrity.
  - Poster presentation in ASCGT Annual Meeting (2024)
  - International Patent Application (2024)

Princeton University — *Princeton, NJ*
ASSOCIATE RESEARCH SCHOLAR — *October 2021 – December 2022*

- Developed and validated AAV expression cassette elements to enhance transgene target specificity and expression.
- Cultured SCG neuron primary cells, N2A, and HEK293T cells, performing retro-orbital and intravitreal injections in mice.
- Utilized Histology (cryostat, vibratome), IF, IHC, RNA/DNA FISH to generate comprehensive data.
  - International Patent Application (2022)
  - Manuscript published in Gene Therapy (2023)

Princeton University — *Princeton, NJ*
POSTDOCTORAL RESEARCH ASSOCIATE — *October 2017 – September 2021*

- Identified and validated neuronal promoters from the alphaherpesvirus genome
- Cultured primary neuron cells and cell lines, performing retro-orbital injections in mice/rats, intravitreal, intravenous, intramuscular, stereotactic injection, tereotactic injection, anesthesia, perfusion and mouse/rat dissection.
- Validated transgene expression and vector biodistribution in brain, spinal cord, and peripheral organs by Histology, qPCR, IHC, IF, FISH, AAV Titer measurements, WB, FIJI and QuPath imaging software.

- Developed a single-cell AAV genome quantification assay with a mathematical model
- Characterized alphaherpesvirus reactivation mechanisms in PRV and HSV
  - International Patent Application (2020)
  - Poster presentation at ASCGT Annual Meeting (2022)
  - Manuscripts published in Molecular Therapy-Methods & Clinical Development (2020), Gene Therapy (2021, Editor's Choice for May 2023 cover), Current Protocols (2022), Journal of Virological Methods (2023), Frontiers in Virology (2024)

Education

PhD Physiological Sciences, *Pontificia Universidad Catolica de Chile*

Master Biological Sciences, *Pontificia Universidad Catolica de Chile*

Master Neuroscience, *Universidad de Valparaiso, Chile*

Bachelor Biochemistry, *Universidad Austral de Chile*

Selected Publications

- **Maturana, C. J.** & Engel E.A. (2024). Persistent transgene expression in peripheral tissues one year post intravenous and intramuscular administration of AAV vectors containing the alphaherpesvirus latency-associated promoter 2. Frontiers in Virology
- **Maturana, C. J.**, (2023). Engineered compact pan-neuronal promoter from Alphaherpesvirus LAP2 enhances target gene expression in the mouse brain and reduces tropism in the liver. Gene Therapy
- **Maturana, C. J.**, Chan, A., Verpeut, J. L., & Engel E. A. (2023). Local and systemic administration of AAV vectors with alphaherpesvirus latency-associated promoter 2 drives potent transgene expression in mouse liver, kidney, and skeletal muscle. Journal of Virological Methods
- Chan, A., **Maturana, C. J.**, & Engel, E. A. (2022). Optimized formulation buffer preserves Adeno-associated virus-9 infectivity after 4°C storage and freeze/thawing cycling. Journal of Virology Methods
- **Maturana, C. J.**, Verpeut, J. L., & Engel, E. A. (2022). Single-Cell Quantification of Triple-AAV Vectors Genome Coexpressed in Neurons. Current Protocols
- **Maturana, C. J.**, Verpeut, J. L., Kooshkbaghi, M., & Engel E. A. (2021). Novel tool to quantify with single-cell resolution the number of incoming AAV genomes co-expressing in the mouse nervous system. Gene Therapy
- Laval, K., **Maturana, C. J.**, & Enquist, L. W. (2020). Mouse Footpad Inoculation Model to Study Viral-Induced Neuroinflammatory Responses. JoVE (Journal of Visualized Experiments)
- **Maturana, C. J.**, Verpeut, J. L., Pisano, T. J., Dhanerawala, Z. M., Esteves, A., Enquist, L. W., & Engel, E. A. (2020). Small Alphaherpesvirus Latency-Associated Promoters Drive Efficient and Long-Term Transgene Expression in The Central Nervous System. Molecular Therapy-Methods & Clinical Development

Patents

- Borras, T., Hong, G., Liang, B., Wu, L., & **Maturana, C. J.** (2024). Self-complementary AAV vectors carrying dominant negative RhoA and methods of use to treat ocular diseases. Patent Application No. PCT/US2023/074351
- **Maturana, C.J.** & Engel, E. A. (2022). Compositions and methods for cell-specific expression of target genes. Patent Application No. PCT/US2022/080438
- Engel, E. A., **Maturana, C.J.**, & Enquist, L.W. (2020). Adeno-associated viral vectors containing alphaherpesvirus promoter sequences. International Patent Application No. PCT/US2020/016787
- Sáez, J. C., Lagos, C., & **Maturana, C.J.** (2018). Selective connexin hemichannels blockers for the treatment of epilepsy. U.S. Patent Application No. US15/556,205