



Anais Amaya

Postdoctoral Scholar, Stem Cell Transplantation

Bio

INSTITUTE AFFILIATIONS

- Member, Maternal & Child Health Research Institute (MCHRI)

HONORS AND AWARDS

- Elizabeth Nash Memorial Fellowship, Cystic Fibrosis Research Institute (2023)
- Postdoctoral Award, Stanford Maternal and Child Health Research Institute (2023)
- Travel Award, European Society of Gene and Cell Therapy (ESGCT) (2019)
- Travel Award, European Society of Gene and Cell Therapy (ESGCT) (2018)
- Travel Award, American Society of Gene and Cell Therapy (ASGCT) (2018)
- Panos Ioannou Young Investigator Award, Australasian Gene and Cell Therapy Society (AGCTS) (2017)
- CMRI PhD Scholarship, Children's Medical Research Institute (2015)
- USyd International Scholarship, The University of Sydney (2015)
- SI Scholarship for Future Global Leaders, Swedish Institute (2014)
- Summa Cum Laude, Universidad Simon Bolivar (2014)

PROFESSIONAL EDUCATION

- Licenciatura, Universidad Simon Bolivar (2014)
- Doctor of Philosophy, University Of Sydney (2020)
- PhD, University of Sydney , Medicine (2020)

STANFORD ADVISORS

- Matthew Porteus, Postdoctoral Faculty Sponsor

Publications

PUBLICATIONS

- **Highly efficient in vivo hematopoietic stem cell transduction using an optimized self-complementary adeno-associated virus** *MOLECULAR THERAPY METHODS & CLINICAL DEVELOPMENT*
Charlesworth, C. T., Homma, S., Amaya, A. K., Dib, C., Vaidyanathan, S., Tan, T., Miyauchi, M., Nakauchi, Y., Suchy, F. P., Wang, S., Igarashi, K. J., Cromer, M., Dudek, et al
2025; 33 (1)
- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *Molecular biology of the cell*
Riepe, C., Wąchalska, M., Deol, K. K., Amaya, A. K., Porteus, M. H., Olzmann, J. A., Kopito, R. R.

2023: mbcE23080336

- **Assessment of Pre-Clinical Liver Models Based on Their Ability to Predict the Liver-Tropism of Adeno-Associated Virus Vectors.** *Human gene therapy*
Westhaus, A., Cabanes-Creus, M., Dilworth, K. L., Zhu, E., Salas Gomez, D., Navarro, R. G., Amaya, A. K., Scott, S., Kwiatek, M., McCorkindale, A. L., Hayman, T. E., Frahm, S., Perocheau, et al
2023; 34 (7-8): 273-288
- **Novel human liver-tropic AAV variants define transferable domains that markedly enhance the human tropism of AAV7 and AAV8.** *Molecular therapy. Methods & clinical development*
Cabanes-Creus, M., Navarro, R. G., Zhu, E., Baltazar, G., Liao, S. H., Drouyer, M., Amaya, A. K., Scott, S., Nguyen, L. H., Westhaus, A., Hebben, M., Wilson, L. O., Thrasher, et al
2022; 24: 88-101
- **Attenuation of Heparan Sulfate Proteoglycan Binding Enhances In Vivo Transduction of Human Primary Hepatocytes with AAV2** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
Cabanes-Creus, M., Westhaus, A., Navarro, R., Baltazar, G., Zhu, E., Amaya, A. K., Liao, S. H. Y., Scott, S., Sallard, E., Dilworth, K. L., Rybicki, A., Drouyer, M., Hallwirth, et al
2020; 17: 1139-1154
- **Efficient in vivo editing of OTC-deficient patient-derived primary human hepatocytes** *JHEP REPORTS*
Ginn, S. L., Amaya, A. K., Liao, S. H. Y., Zhu, E., Cunningham, S. C., Lee, M., Hallwirth, C., Logan, G. J., Tay, S. S., Cesare, A. J., Pickett, H. A., Grompe, M., Dilworth, et al
2020; 2 (1): 100065
- **Inhibition of proliferation in primary human hepatocytes following in vivo AAV-mediated genome editing**
Amaya, A. K., Ginn, S. L., Liao, S. Y., Zhu, C., Smyth, C. M., Logan, G. J., Lisowski, L., Alexander, I. E.
MARY ANN LIEBERT, INC.2019: A18
- **Efficient in vivo editing of patient-derived primary human hepatocytes**
Ginn, S. L., Amaya, A. K., Liao, S. Y., Zhu, E., Cunningham, S. C., Hallwirth, C. V., Tay, S. S., Lee, M., Pickett, H. A., Grompe, M., Dilworth, K., Lisowski, L., Alexander, et al
MARY ANN LIEBERT, INC.2019: A2-A3
- **Codon-Optimization of Wild-Type Adeno-Associated Virus Capsid Sequences Enhances DNA Family Shuffling while Conserving Functionality** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
Cabanes-Creus, M., Ginn, S. L., Amaya, A. K., Liao, S. H. Y., Westhaus, A., Hallwirth, C. V., Wilmott, P., Ward, J., Dilworth, K. L., Santilli, G., Rybicki, A., Nakai, H., Thrasher, et al
2019; 12: 71-84
- **Successful in vivo editing of patient-derived primary human hepatocytes**
Amaya, A. K., Ginn, S. L., Liao, S. Y., Zhu, C., Lee, M., Pickett, H. A., Hallwirth, C. V., Cunningham, S. C., Lisowski, L., Alexander, I. E.
MARY ANN LIEBERT, INC.2018: A26-A27
- **Gene therapy clinical trials worldwide to 2017: An update** *JOURNAL OF GENE MEDICINE*
Ginn, S. L., Amaya, A. K., Alexander, I. E., Edelstein, M., Abedi, M. R.
2018; 20 (5): e3015
- **Evaluation of Recombinant Adeno-Associated Virus-Based Genome Editing Reagents for Homology-Directed Repair to Target a Human Liver Locus In Vivo**
Ginn, S. L., Amaya, A. K., Liao, S. H. Y., Zhu, C., Hallwirth, C. V., Tay, S. S., Cunningham, S. C., Logan, G. J., Dilworth, K., Lisowski, L., Alexander, I.
CELL PRESS.2018: 113
- **Successful In Vivo Editing of the OTC Locus in Primary Human Hepatocytes Xenografted into the FRG Mouse Liver**
Amaya, A. K., Ginn, S. L., Liao, S. H. Y., Zhu, C., Lee, M., Pickett, H. A., Hallwirth, C. V., Cunningham, S. C., Logan, G. J., Dilworth, K., Lisowski, L., Alexander, I. E.
CELL PRESS.2018: 448-449
- **TREATMENT OF GENETIC LIVER DISEASE BY AAV-MEDIATED GENOME EDITING AND SELECTIVE EXPANSION OF REPAIRED HEPATOCYTES**
Amaya, A. K., Zhu, C., Ginn, S. L., Hallwirth, C. V., Tay, S. S., Cunningham, S. C., Logan, G. J., Alexander, I. E.
WILEY.2018

- **DISCOVERY OF A LIVER-SPECIFIC ENHANCER-PROMOTER ELEMENT IN THE 3 ' UTR OF THE WILD-TYPE AAV2 GENOME PROVIDES NOVEL INSIGHTS INTO AAV VECTOR SAFETY IN THE HUMAN LIVER**

Logan, G. J., Dane, A. P., Hallwirth, C. V., Smyth, C. M., Wilkie, E. E., Amaya, A. K., Zhu, E., Khandekar, N., Ginn, S. L., Liao, S., Cunningham, S. C., Sasaki, N., Tam, et al
WILEY.2018

- **Identification of liver-specific enhancer-promoter activity in the 3 ' untranslated region of the wild-type AAV2 genome** *NATURE GENETICS*

Logan, G. J., Dane, A. P., Hallwirth, C. V., Smyth, C. M., Wilkie, E. E., Amaya, A. K., Zhu, E., Khandekar, N., Ginn, S. L., Liao, S. H. Y., Cunningham, S. C., Sasaki, N., Cabanes-Creus, et al
2017; 49 (8): 1267-+