Mira (née Patel) Pochron, PhD

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Senior Director of Biology and Translational Research

Collaborative Team Leader | Assay Development | Pharmacodynamic Researcher

Director of Translational Biology and team leader with a solid background in translational research and leader of multidisciplinary, cross functional drug discovery teams. Comprehensive experience directing pre-clinical assets from high throughput screening to IND preparation. Extensive knowledge in designing efficacy and biomarker studies in model animals supporting all stages of preclinical pipeline programs. Experience directing biomarker evaluation, collection and summarization for clinical programs in rare diseases. Noteworthy contributions led to **Oxbryta®**, which received the **Prix Galien award for Best Biotechnology Product in 2021**.

Areas of Expertise

Clinical and Animal Biomarker Specialist | Builder of High Performing Teams | Animal Model Evaluation | Reverse Translational | Structural Biologist | Author of IND and NDA Reports | CRO Management | Clinical Biomarker Operations | Collaborative | Pharmacodynamic Assay Developer | Project Leadership |Sickle Cell Disease | Non-malignant Hematology | Assay Development | Biomarker Sample Management | Clinical Protocol Support and Execution | Author of Clinical Study Report

Education

Doctor of Philosophy (Ph.D.), Chemistry and Biochemistry ***March 2010***
University of California, Santa Cruz

Bachelor of Science (B.S.), Chemistry ***June 2003***
University of California, Santa Cruz

Professional Experience

POCHRON SOLUTIONS, LLC

Translational Medicine and Biomarker Consultant **September 2024 – Present**

- Supported, presented and facilitated discussion at client's advisory board meeting
- Reviewed and contextualized clinical biomarker data
- Strategic planning to increase client's customer base
- Advising VC firms regarding target choice for seed companies

PFIZER (legacy GLOBAL BLOOD THERAPEUTICS)

Director of Translational Biology, Sickle Cell Disease **October 2022-August 2024**

Managed a team of ten researchers that supported four pre-clinical programs by:

- Conducted Proof of Concept studies in *in vitro* and *in vivo* models in support of four pipeline programs for sickle cell disease and other non-malignant hematologic disorders.
- Supported two mid- to late-stage pre-clinical programs with biomarker implementation and pharmacodynamic measurements.
- Assessed two new technologies related to implementation of novel biomarkers.
- Provided direction and oversight for eight academic sponsored research studies to further investigate the mechanism of action of our hemoglobin modifiers voxelotor and osivelotor.
- Designed, and supervised data collection and analysis for animal studies measuring the impact of long-term drug dosing in over 500 sickle cell disease model mice.

In addition, my team supported multiple sickle cell clinical trials by:

- Training four clinical sites and four vendor laboratories on biomarker collection.
- Ensuring appropriate data transfers from vendor labs for analysis.
- Analysis of pharmacodynamic and biomarker data from phase 1 and phase 2 clinical trials of osivelotor.
- Onboarding and overseeing vendor laboratory deliverables and compliance.

GLOBAL BLOOD THERAPEUTICS, South San Francisco, CA ***August 2011 – October 2022***

Head of Translational Biology, Associate Director **August 2021 – September 2022**

Head of Translational Biology, Staff Scientist **September 2019 – August 2021**

- Project leader for a late-stage pre-clinical program, key contributions included designing IND enabling studies to predict efficacious clinical doses and directing studies to understand on-target toxicity.
- Oversight of six pre-clinical collaborations

Directed a team of seven responsible for:

- Overseeing and advising on pharmacodynamic measurements for mid- and late-stage pre-clinical programs.
- Onboarding and supervising vendor laboratories
- Analyzing pharmacodynamic data collected in clinical trials of voxelotor and osivelotor.
- Research liaison for the cross functional voxelotor Project Development Team
- Authoring various reports to support regulatory filings for IND, NDA and EMA.

Senior Scientist **March 2017 – August 2019**

Scientist II **March 2014 – February 2017**

- Lead biologist on the voxelotor program
- Project leader for two pipeline programs
- Developed a pharmacodynamics model to be used in phase 1/2 clinical trial for voxelotor.
- Coordinated and troubleshot the analysis of the human samples with the vendor laboratory
- Led the team responsible for preparing and executing the Lab Manual used in the vendor laboratory for the pharmacodynamic measurements.
- Collaborated with informatics to develop a tool for remotely analyzing data collected in London from our phase 1/2a study.
- Oversaw, processed, applied quality review, and analyzed pharmacodynamic data from the Phase 1/2a clinical trials.
- Interacted and aligned with other functions and communicated complex data to internal and external stakeholders.
- Prepared manuscripts and abstracts for data dissemination
- Evaluated new indications by investigating the medical, business and scientific landscapes and proposing new targets
- Supervised two Research Associates

Scientist I **June 2012 – February 2014**

- Established multiple assays for structure-activity relationship (SAR) screening
- Coordinated data collection and compilation for distribution to the project team
- Developed assays for further characterizing lead compounds.

Major Achievements include:

- Conducted primary and secondary screens of compounds and lead profiling using purified Hb, red blood cells and whole blood that led to the discovery of Oxbryta®.
- Developed a novel plate-based assay to measure changes in oxygen affinity that was used to delineate compound SAR
- Developed a viscosity assay to determine the effects of lead compounds on sickle cell blood rheology
- Liased with informatics to develop tools for automated data analysis for non-standard assays

Consulting Scientist **August 2011 – May 2012**

- Established preliminary assay leading to data acquisition for Series A funding.
- Developed a screening assay purified Hemoglobin, RBCs and whole blood, which allowed for the identification of novel and improved compounds
- Developed an activity-based measure of on-rate and off-rate for series characterization that contributed to the design of voxelotor
- Established preliminary rat pharmacodynamics assay for the HbS program

UNIVERSITY OF CALIFORNIA, SAN FRANCISCO

Post-Doctoral Researcher; Levy Lab **March 2010 – April 2011**

- Studied function and cell surface markers on plasmacytoid dendritic cells of healthy and HIV infected individuals
- Used HEK 293 cell lines to study TLR7 and 9 to understand immune activation during HIV infection
- Developed spectroscopic methods for analyzing metabolites of primary immune cells
- Conducted ELISAs to study cytokines produced by immune cells
- Taught medical students a course on the pharmacodynamics of drugs

UNIVERSITY OF CALIFORNIA, SANTA CRUZ

PhD Student; Millhauser Lab **June 2004 – February 2010**

- Characterized the binding pockets of two GPCRs, MC1R and MC4R, providing specificity and agonist/inverse agonist characteristics for drug development
- Determined the structure of two small proteins using 2D NMR
- Designed, synthetically produced, purified and characterized the activity and binding properties of nine Agouti Signaling Protein and Agouti Related Protein chimeric proteins

UNIVERSITY OF CALIFORNIA, SANTA CRUZ

Undergraduate Researcher; Yildiz Lab **June 2002 – August 2003**

- Analyzed the properties of over 8000 knock out mutants of *V. cholerae* to determine the genes necessary for biofilm formation and selected 50+ mutants for further analysis
- Conducted PCR on flanking regions of knockouts to determine the 'knocked out' gene

PUBLICATIONS

1. Zhe Li, Carsten Alt, Kobina Dufu, Athiwat Hutchaleelaha, Qing Xu, Xinchun Zhang, Chien-Ming Li, Peter Rademacher, Caroline Bosmajian, **Mira Patel Pochron**, James R. Partridge, Donna Oksenberg, Brian E. Cathers. "Discovery of Osivelotor (GBT021601): A Potent, Next-Generation Sickle Hemoglobin Polymerization Inhibitor" *ACS Medicinal Chemistry Letters,* (2025)
2. Susanna A Curtis, Elana Friedman, Caterina Minniti, Annie Nguyen Dang, **Mira Pochron**, Merin Thomas, Jaime Betancourt, Leena Vattappally, Andrew Crouch, Julissa Morales, Sean T Campbell. "Concentration of voxelotor in sickle cell disease can be estimated using electrophoresis and high-performance liquid chromatography" *American Journal of Clinical Pathology*, (2024);
3. Qiang, Yuhao, Mengjia Xu, **Mira Patel Pochron**, Madhulika Jupelli, and Ming Dao. "A framework of computer vision-enhanced microfluidic approach for automated assessment of the transient sickling kinetics in sickle red blood cells." *Frontiers in physics* 12 (2024): 1331047.
4. Chonat, Satheesh, Earl Fields, Hannah Baratz, Amanda Watt, **Mira Pochron**, Sandy Dixon, Margaret Tonda, Clark Brown, and David Archer. "Voxelotor improves red blood cell functionality in children with sickle cell anaemia: An ancillary study of the HOPE-KIDS 1 trial." *Ejhaem* 5, no. 1 (2024): 125-130.
5. Dufu, Kobina, Carsten Alt, Steven Strutt, James Partridge, Tzechiang Tang, Vincent Siu, Hilary Liao-Zou, Peter Rademacher, Alexander T. Williams, Cynthia R. Muller, Xin Geng, **Mira Patel Pochron**, Annie Nguyen Dang, Pedro Cabrales, Zhe Li, Donna Oksenberg, Brian E. Cathers. "GBT021601 improves red blood cell health and the pathophysiology of sickle cell disease in a murine model." *British Journal of Haematology* 202, no. 1 (2023): 173-183.
6. Kanne CK, Nebor D, **Pochron M**, Oksenberg D, Sheehan VA. "Rheological Impact of GBT1118 Cessation in a Sickle Mouse Model." *Front Physiol*. 2021 Sep 24;12:742784.
7. Li, Z., **Patel, M**., Oksenberg, D., & Metcalf, B. (2019). "Small-Molecule Approaches for the Treatment of Sickle Cell Disease." In J. J. Bronson (Ed.), *2019 Medicinal Chemistry Reviews* (pp. 29–52).

8. Hutchaleelaha, A., **Patel, M. P.**, Washington, C., Siu, V., Allen, E., Oksenberg, O., Gretler, D. D., Mant, T., Lehrer-Graiwer, J. (2019) "Pharmacokinetics and Pharmacodynamics of Voxelotor (GBT440) in Healthy Adults and Patients With Sickle Cell Disease." *Br. J. Clin. Pharmacol.*

9. Howard, J., Hemmaway, C. J., Telfer, P., Layton, D. M., Porter, J., Awogbade, M., Mant, T., Gretler, D. D., Dufu, K., Hutchaleelaha, A., **Patel, M.**, Siu, V., Dixon, S., Landsman, N., Tonda, M., Lehrer-Graiwer, J. (2019) A "Phase 1/2 Ascending Dose Study and Open-Label Extension Study of Voxelotor in Patients with Sickle Cell Disease." *Blood*, 133, 1865-1875

10. Dufu, K., **Patel, M. P.**, Oksenberg, D., Cabrales, P. (2018) GBT440 Improves Red Blood Cell Deformability and Reduces Viscosity of Sickle Cell Blood under Deoxygenated Conditions. *Clin. Hemorheol. Microcirc.*, 70, 95-105

11. **Patel, M. P.**, Siu, V., Silva-Garcia, A., Xu, Q., Li, Z., Oksenberg, D. (2018) Development and Validation of an Oxygen Dissociation Assay, a Screening Platform for Discovering, and Characterizing Hemoglobin – Oxygen Affinity Modifiers. *Drug Des. Devel. Ther.*, *12*, 1599–1607.

12. Metcalf, B., Chuang, C., Dufu, K., **Patel, M.P**., Silva-Garcia, A., Johnson, C., Lu Q., Partridge, J.R., Patskovska, L., Patskovsky, Y., Almo, S. C., Jacobson, M. P., Hua, L., Xu, Q., Gwaltney, S.L., Yee, C., Harris, J.R., Morgan, B. P., James, J., Xu, D., Hutchaleelaha, A., Oksenberg, D., Li, Z., Paulvannan, K. (2017) Discovery of GBT440, an Orally Bioavailable R State Stabilizer of Sickle Cell Hemoglobin. *ACS Med. Chem. Lett.*, *8*, 321–326.

13. Geng, X., Dufu, K., Hutchaleelaha, A., Xu, Q., Li, Z., Li, C., **Patel, M.P.**, Vlahakis, N., Lehrer-Graiwer, J. & Oksenberg, D. (2016) Increased hemoglobin–oxygen affinity ameliorates bleomycin-induced hypoxemia and pulmonary fibrosis. *Physiological Reports*, 4.

14. Oksenberg, D., Dufu, K., **Patel, M.P.**, Chuang, C., Li, Z., Xu, Q., Silva-Garcia, A., Zhou, C., Hutchaleelaha, A., Patskovska, L., Patskovsky, Y., Almo, S.C., Sinha, U., Metcalf, B.W. & Archer, D.R. (2016) GBT440 increases haemoglobin oxygen affinity, reduces sickling and prolongs RBC half-life in a murine model of sickle cell disease. *British Journal of Haematology*, 175, 141–153.

15. Kaushik, S., Teque, F., **Patel, M.**, Fujimura, S.H., Schmidt, B. & Levy, J.A. (2013) Plasmacytoid Dendritic Cell Number and Responses to Toll-Like Receptor 7 and 9 Agonists Vary in HIV Type 1-Infected Individuals in Relation to Clinical State. *AIDS Research and Human Retroviruses*, 29, 501–510.

16. Huang, Z., Chinen, M., Chang, P.J., Xie, T., Zhong, L., Demetriou, S., **Patel, M.P.**, Scherzer, R., Sviderskaya, E. V, Bennett, D.C., Millhauser, G.L., Oh, D.H., Cleaver, J.E. & Wei, M.L. (2012) Targeting protein-trafficking pathways alters melanoma treatment sensitivity. *Proceedings of the National Academy of Sciences of the United States of America*, 109, 553–8.

17. Beaumont, K.A., Smit, D.J., Liu, Y.Y., Chai, E., **Patel, M.P.**, Millhauser, G.L., Smith, J.J., Alewood, P.F. & Sturm, R.A. (2012) Melanocortin-1 receptor-mediated signalling pathways activated by NDP-MSH and HBD3 ligands. *Pigment Cell & Melanoma Research*, 25, 370–374.

18. Swope, V.B., Jameson, J.A., McFarland, K.L., Supp, D.M., Miller, W.E., McGraw, D.W., **Patel, M.**, Nix, M.A., Millhauser, G.L., Babcock, G.F. & Abdel-Malek, Z.A. (2012) Defining MC1R Regulation in Human Melanocytes by Its Agonist α-Melanocortin and Antagonists Agouti Signaling Protein and β-Defensin 3. *Journal of Investigative Dermatology*, 132, 2255–2262.

19. Beaumont, K.A., Wong, S.S., Ainger, S.A., Liu, Y.Y., **Patel, M.P.**, Millhauser, G.L., Smith, J.J., Alewood, P.F., Leonard, J.H. & Sturm, R.A. (2011) Melanocortin MC(1) receptor in human genetics and model systems. *European journal of pharmacology*, 660, 103-110.

20. **Patel, M.P.**, Cribb Fabersunne, C.S., Yang, Y.-K., Kaelin, C.B., Barsh, G.S. & Millhauser, G.L. (2010) Loop-swapped chimeras of the agouti-related protein and the agouti signaling protein identify contacts required for melanocortin 1 receptor selectivity and antagonism. *Journal of molecular biology*, **404**, 45–55.

21. Seballos, L., Richards, N.G.J., Stevens, D.J., **Patel, M.P.**, Kapitzky, L., Lokey, R.S., Millhauser, G.L. & Zhang, J.Z. (2007) Competitive binding effects on surface-enhanced Raman scattering of peptide molecules. *Chemical Physics Letters*. 447, 335-339.

PRESENTATIONS

Dec 2023 *American Society of Hematology, San Diego*

"Predictive Biomarker Analysis from the GBT021601 Survival Study in Townes Sickle Mice."

Host: 66th Annual Meeting & Exposition for American Society of Hematology

Feb 2021 *Pharmaceutical & BioScience Society, San Francisco Bay*

"Oxbryta ® (Voxelotor) Tablets for Sickle Cell Disease: The R&D Journey and Lessons Learned"

Host: Jointly organized by PBSS (Pharmaceutical & BioScience Society) and CLSA (California Life Science Association) symposium series "Success Stories of Recently Approved Drugs and Lessons Learned"

May 2009 *University of California, San Francisco*

"Determining the necessary components of Agouti Signaling Protein and Agouti-Related Protein for Melanocortin Receptor Binding"

Host: 5th Annual Chemical Biology in the Bay Area Research Day

August 2007 *San Francisco*

"Becoming a Scientist Educator: Reflections on Professional Development Programs for Scientist"

Host: Bay Area Institute 2007

PUBLISHED PATENTS

Compounds for treating acute respiratory distress syndrome or a negative effect thereof
Dufu, K.N., **Patel, M.P.**, and Lehrer-Graiwer, J.E.
WO Application 2016043849A2, March 24, 2016

Composition and methods for the modulation of hemoglobin(s)
Sinha, U., Metcalf, B.W., Oksenberg, D., Dufu, K.D., and **Patel, M.P.**
United States Patent Application 20160038474 A1, February 11, 2016