



Maximizing Data Generation for Rare Diseases in Biopharma

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PTC is a science-driven, global biopharmaceutical company focused on the discovery, development, and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Biological assessment of clinical samples from rare or pediatric diseases however is often constrained due to limited sample number and size. This limitation typically denies investigational deep dives into underlying biology as priority is given to traditional clinical chemistry and pharmacokinetic analyses. Insufficient sample number and low biofluid volumes also persist in preclinical studies where genetically modified animals can be quite small, expensive, or hard to breed. These drawbacks are particularly problematic in complex biological systems, devoid of a singular cause-effect relationship, and in need of a greater basic understanding of disease pathology. Therefore, my group at PTC Therapeutics develops analytical strategies compatible with diverse technologies that enable multi-omic analyses from limited sample requirements. Here I present some of our initial attempts to add imaging mass spectrometry to our bioanalytical suite.

Biography:

Dr. Joey C. Latham is currently a Senior Director (Bioanalytical) and an Equality, Diversity, & Inclusion (ED&I) global ambassador at PTC Therapeutics. Dr. Latham began his scientific pursuits as an undergraduate at Texas Tech University. After completing bachelor's degrees in both Chemistry and Mathematics, he attended Vanderbilt University for his doctoral studies. Under the tutelage of Dr. Hassane Mchaourab (Molecular Physiology and Biophysics), Joey received a National Eye Institute training grant to investigate the chaperone function of the lens protein α -crystallin. While working on his dissertation at RISØ National Laboratory in Denmark during an NSF exchange program, Joey helped develop an optical biosensor using MEMs fabrication techniques in ISO 3 cleanrooms. This work, published in *Science*, led to a start-up company for the detection of molecular interactions in picoliter volumes (Molecular Sensing Inc.). After completing his Ph.D., Joey spent the next several years as a

postdoctoral researcher and then research faculty focused on imaging mass spectrometry in the lab of Dr. Richard Caprioli at Vanderbilt University. He then transitioned to the pharmaceutical industry, joining Allergan in Irvine, CA as a scientist in the drug safety evaluation unit. Following stops at Elan Pharmaceuticals and Genentech in South San Francisco, Joey has spent the past ten years working in the rare pediatric disease space.