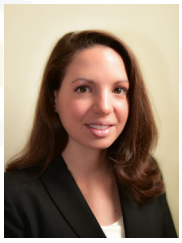
AIMBE FDA Scholars Program

Advancing innovation and discovery from the lab to the marketplace.

Spotlight on the 2016-2017 AIMBE Scholars



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During graduate school, I became interested in better understanding how health care devices transitioned from benchtop research to marketable products. Specifically, the clinical trial process, and the challenges and barriers as new medical devices are developed, was particularly intriguing to me. The AIMBE Scholars Program interested me because it immersed participants into the FDA. I feel the best way to learn is by doing and that is exactly what I have been privileged to do during my time as a scholar. My time as a scholar has been exciting and engaging! Each day, I looked forward to being challenged and growing as I learned about regulatory science first-hand.

As a scholar, I worked in the FDA's Center for Devices and Radiological Health (CDRH) Office of the Center Director (OCD) and the Office of Surveillance and Biometrics (OSB). I was fortunate to work on multiple projects which focused on helping the Center meet one of the 2016-2017 strategic priorities to Partner with Patients. The goals of this priority are to promote a culture of meaningful patient engagement by facilitating interactions with patients and to increase the transparency of patient input as evidence in the Center's decision making. By working on Partnering with Patient projects, I have developed a strong knowledge of pre- and post-market clinical studies for medical devices and have had the great satisfaction of seeing how patient involvement can help shape and improve the regulatory process. The majority of my work has focused in two areas: patient reported outcomes and patient preferences.

Patient-reported outcome (PRO) measures are scientifically developed questionnaires that are used to gather patients' assessments of their own health and well-being. These instruments are valuable tools to measure pain, quality of life, symptom(s), functioning, and the overall wellbeing of patients. PROs are specifically interesting to me as they allow the patient to voice these clinically important factors that would otherwise be immeasurable in a clinical study. Pain, for example, is an important clinical outcome measure that is helpful in assessing a patient's benefit from a treatment, but is difficult to assess in another individual. Due to their utility, PROs are often used as supplemental information to support other clinical outcomes, demonstrate quality of

life improvement, establish patient satisfaction with a treatment, and demonstrate a device's value to payers, providers, and health care systems. My work with PROs has been to evaluate the current field of PROs submitted in pre- and post-market submissions and to assist in encouraging the voluntary submission of PROs as study endpoints. My analysis of current PRO submissions will allow CDRH to better understand current PRO usage and is helpful in identifying target areas where a PRO could be beneficial in reviewing a product. As the use of PROs used in clinical studies increases, therefore also increasing the patient perspective in clinical studies, I am excited to see the elevated patient care that will in turn be developed.

In addition to patient-reported outcomes, my work also involved patient preference studies, which measure the benefit versus risks tradeoffs patients are willing to make in deciding on a treatment choice. Patient preference studies, for example, determine what benefits and risks patients are willing to trade off when comparing treatment options. New devices are developed to treat or to assist in the treatment of patients, therefore, involving the partialities of patients in the regulatory process is beneficial in making impactful regulatory decisions. The patient preference study my work focused on is a first-of-its-kind proof-of-principle study. It is a collaboration between CDRH, the Medical Device Innovation Consortium (MDIC), The Michael J. Fox Foundation (MJFF), Massachusetts Institute of Technology (MIT), and RTI Health Solutions. This project has been stimulating for me as Parkinson's disease has multiple symptoms, yet each patient does not necessarily present each symptom. Developing treatments for diseases like Parkinson's is thus very difficult as each patient is different. Furthermore, this study pushes the field of patient preference in that it not only focuses on the trade-offs patients are willing to make to gain treatment benefits, but also involves taking the benefit-risk data and incorporating it into economic models to determine optimal type I and type II error allowances when designing clinical trials. Historically, a p-value of .05 has been determined as statistically significant in clinical studies. This work is innovative as it seeks to calculate an optimal p-value threshold based on the benefit-risk values of type I and type II errors to patients. My work has involved collaborating with patient scientists and medical device reviewers to determine what benefits and risks are important to patients in determining if they would use a potential new treatment. This data is now being incorporated into the development of a patient preference study for MJFF's technology platform. This project has taught me how scientific processes can be used to gather qualitative information, which can then be analyzed quantitatively to incorporate into a patient preference study. I am excited to see how the field of patient preference studies develop and changes the clinical trial field.

My time as an AIMBE Scholar has been invaluable. My mentors have provided me numerous opportunities and chances to learn and grow as an AIMBE scholar as they provided me essential roles in regulatory science projects. Due to these opportunities, I have developed a working knowledge of the regulatory process and the science behind the practices. I am grateful for being selected as a scholar and for the many opportunities it has provided.