Trainee Tutorial: Application of Machine Learning in Drug Development with Regulatory Considerations

Training Need

Machine learning (ML) is a computational approach that utilizes data to make predictions. ML approaches have gained significant traction in the past decade; however, their potential in drug development remains to be realized.

General guidance to facilitate appropriate ML applications in drug development have yet to be established. Applying ML methods in drug development should be guided by three questions:

- "What is the drug development need?"
- "What ML methods are most appropriate for the context of the drug development need?"
- "What data can be used to support the development of the methods to address the drug development need?"

These questions lay the foundation for applying ML in drug development and will guide continued development of the field. Additionally, early regulatory collaboration and engagement are needed to develop ML approaches within regulatory standards which can then be disseminated to sponsors.

Tutorial Overview

Format: The tutorial will be divided into two sessions: lecture and hands-on

Goal: Expose trainees to various drug development case studies that apply ML methods while taking regulatory considerations into account.

Learning Objectives

- Understand origin and utility of ML in drug development based on regulatory considerations.
- Learn the ordered best practices for generating and applying ML tools in drug development:
    1. Identification of the drug development need
    2. Selection of appropriate ML methods
    3. Utilization of supporting data
- Implement a Deep Learning (DL) model from scratch for a drug development problem and interpret the results.

Hands-on Session

Problem: Trainees will be given a drug development problem and a synthetic, patient-level dataset to build a predictive DL model for Alzheimer's Disease state classification.

Engagement: After registration, trainees will be grouped into one of 10 teams and matched with a coach to help guide the team through the hands-on session.

During the Event: Speakers will describe the DL model development processes step-by-step allowing trainee teams to meet in break-out rooms where they will build their DL model in R. Coaches will join each team in the break-out rooms to guide DL model development and troubleshoot code. Each team will have the opportunity to share the results of their model at the end of the session.