PSI Scientific Committee Webinar

Longitudinal modelling: Time to take the next step?

November 18, 2019

Motivation

- Escalating costs of drug development creates sustainability challenge for pharmaceutical industry: increasing efficiency is critical
- Different approaches have been proposed: e.g., adaptive designs, biomarkers, platform trials, risk-based monitoring, etc.
- Some involve significant changes to drug development practice and processes
- Focus today on analysis approach to improve drug development efficiency which can be used with data currently collected in trials

Opportunities and Challenges

- Longitudinal data routinely collected in most clinical trials, but rarely fully utilized: focus on change from baseline and response rates at fixed visit
- Analyses based on parametric longitudinal models, such as mixed-effects models, offer potential information efficiency gains and better understanding of drug effect (e.g., speed of onset)
- Such methods require additional assumptions about response longitudinal profiles, variability between and within subjects, correlation structures, etc.
- Better understanding of pros and cons of and greater familiarity with such approaches are critical for broadening their use and acceptance in drug dev.