SEEKING ADVICE FROM FDA, EMA, AND EU HEALTH AUTHORITIES
Process, Strategy, Timing, Questions to Ask

Seeking advice from regulatory Health Authorities (HA) at significant milestones is critical to every Sponsor's development program. Because of the long lead-time, high costs, and great resources required to prepare for and attend these meetings, it is important to make sure that the Sponsor makes the best use of each consultation. This presentation will discuss the different types of FDA and European Scientific Advice meetings and the logistical and strategic planning needed for each meeting. Case studies will be presented as examples of different consultations.

On the logistical side, the planning includes:

- Timeline from start of process to when advice will be received. The consultation must be arranged so the advice is received in time to be useful.
- How to decide which HA's to consult?
- Should the EMA or individual European HA's be consulted?
- If more than one HA is being consulted, how to coordinate the meetings and what to do if there is conflicting advice?
- Are the regulations/guidelines for the content of the meeting request and briefing book?
- What other regulations/guidelines are relevant to the meeting discussion?
- Is a local representative required to arrange the meeting?

On the technical side: Which questions should be asked and when? How to phrase the questions? How to ask forward-thinking questions? What data are needed to justify the Sponsor's position? Examples of different types of meetings such as those listed below will be discussed:

- First-in-human study (such as Pre-IND meeting):
    - Traditional questions ask adequacy of nonclinical, CMC, and clinical trial design.
    - Additional questions that address the Phase 2 program.
- End-of-Phase 2 meetings
    - Justification for why the Sponsor believes they are ready for Phase 3 and the design of the Phase 3 study.
    - Additional questions regarding the entire dataset planned for the future NDA/BLA/MAA
- Pre-Submission meetings (pre-NDA, pre-BLA, pre-MAA)
    - Discusses the overall safety and efficacy results
    - All of the data that will support the marketing application and how the data will be organized.
- Special meetings
    - Special Protocol Assessment
    - Breakthrough Designation
    - CMC/Quality questions
    - Clinical findings

Case studies from consultation for traditional drugs and well-characterized biologics as well as advanced therapies such gene and cellular therapies will be presented as examples. The different focus at meetings to discuss different clinical phases will also be described.