

**PACE Initiative Sponsor Advisory Council**  
Inaugural Meeting

Friday, September 25, 2009

**Meeting Highlights, Notes, and Take-Aways:**

1. The meeting was attended by 14 Council members representing 9 Sponsors, 5 PACE/UBC Staff, and one consulting economist (see attached list of attendees and organizations)
2. CER and CER Trial Survey and Results (see attached doc). The survey indicated high concurrence across participants that CER is important to the innovative pharma industry; CER trials will be important; and innovating methods to improve efficiency and usefulness of trials is important.
3. The thrust, mission, activities to date of the PACE Initiative were endorsed.
4. The three working papers presented (“Prior” by Vanness; “Investment” by Basu; “Simulation” by Caro) were well received.
5. The Council provided very useful advice on activities going forward, specifically:
  - a. Major new thrust recommended: Initiate a “Proof of Concept” effort to determine the degree to which a Bayesian adaptive trial design could be more efficient, saving money and time. Specifically, the group endorsed considering ALLHAT (or some other completed prominent CER trial). The idea being to go back in time to the planning of that trial, develop a Bayesian prior that would have applied at the time, via systematic review of evidence available then including a multiple treatment comparisons (MTCs) analysis.
  - b. Use that prior as the basis for a Bayesian adaptive trial design as an alternative to the actual frequentist design that was implemented.
  - c. Develop a simulation of the adaptive design. Obtain the actual data from the ALLHAT trial and use them to carry out the simulation as if the trial were running under an adaptive design instead.
  - d. Determine to what extent the adaptive design would have improved trial execution, timing, cost and possibly outcomes. A nationally-prominent, independent, clinical/statistical-analytical/policy advisory board would be appointed to oversee the integrity of the project.
  - e. Timeline: completion in 12 months (Note: this timeline is highly ambitious; we will revisit once a topic has been selected, protocol developed).
  - f. Cost unclear; special funding will be required.



7. Other issues that came up:

- a. Pragmatic trial can be done not only in Phase 4 but also in Phase 3B. Need to think about the differentiation between the two; especially with FDA, there will be more issues
- b. The new proposed CER National Institute: Will it tackle novel methods in designing comparative trials? Answer: Probably not, and a potential useful role for the PACE Initiative
- c. PACE being catalyst for a major multi-sponsor CER Trial Institute. Industry could band together, fund a CER institute to develop methods/ set up standards for the purpose of counterbalancing whatever may come out of health reform. PACE could be the seed for this endeavor. (Note: this idea was suggested by one Council member and discussed by all, but there was no firm recommendation in going forward. Such an effort is not presently being contemplated by PACE organizers.)
- d. Importance of substantiating that Bayesian adaptive methods for CER trials
  - i. Are credible
  - ii. Result in efficiency in costs and time
  - iii. Can potentially adapt treatment and focus on heterogeneity
- e. Importance of getting clinicians more involved as we generate papers.