

Breakout session 2A - DISEASE CLASSIFIERS

Session leaders: Drs. Ellis, Taube and Ransohoff

morning

Definition of clinical utility of classifier assays

- Must the assay drive a treatment/management decision? Must clinical utility always relate to improved outcomes?
- Does utility apply only in the setting of a difficult clinical decision regarding treatment/management? Are treatment options a prerequisite for utility of an assay? What if current indicators are adequate? Inadequate?
- If a new test is less invasive, easier to run, cheaper, does that have an impact on its clinical utility? Is affordability relevant? If so, how is affordability assessed?

Evidence needed

- Where do disease classifiers fit in terms of driving treatment/management decisions? Is it necessary to understand the biology behind the clinical association? Is it sufficient to identify different risk groups or identify groups with likely target pathways? (1)
- Is it sufficient to show outcomes improve when the test is used? Does evidence always have to be generated in the same organ or context it will be used? Has the assay been tested in the context in which it will be used?
 - Does it work with the specimens of choice?
 - Does it clearly define the population of interest?
 - Has it been demonstrated that the identified population benefits from one treatment vs another?

Relevant endpoints

- Are the endpoints for classifiers different than for predictive or monitoring assays?
- Does what constitutes a clinically relevant endpoint depend on the disease?

afternoon

Evidence generation

- Randomized controlled trials are the standard. When other types of studies can/should be considered? Are there any cases in which nothing less than RCT can be considered?
- What are other options? When are other study design options appropriate and when are they not?
- How do low-prevalence markers and small sample sizes affect the ability to evaluate clinical utility and are there efficient and/or effective study designs?

Role of NCI

- What are the other interested agencies and organizations? What roles do they play?
- Does the NCI have a unique role? How can the NCI facilitate and accelerate evaluation of clinical utility?
- Might the clinical trials network be utilized differently to generate important evidence?

Please review the following article. It will be used to illustrate the challenges faced in trying to answer the questions above.

1. M. C. Cheang *et al.*, Responsiveness of intrinsic subtypes to adjuvant anthracycline substitution in the NCIC.CTG MA.5 randomized trial. [*Clinical cancer research : an official journal of the American Association for Cancer Research* **18**, 2402 \(Apr 15, 2012\).](#)