

Points to Consider

Morning session:

Safety monitoring of children with cancer enrolled in clinical studies is an ethical, scientific, and legal imperative. Multiple documents address the protection of people enrolled in investigations and some specifically address the enrollment of children. None, however, are specific for the context of children with cancer and in general society is willing to tolerate risks for cancer therapy and cancer clinical investigations that are not tolerated in other clinical settings.

We would like to get specific advice on:

- Which sections of which publicly available documents provide the principles upon which safety monitoring for pediatric oncology patients participating in studies should be based. If there is one document, for example ICH E 11, that contains all the principles, then that document should be noted. If the principles are found among multiple documents, then the relevant sections should be identified. If there are no documents that state an important principle, then the need for such a statement or document should be noted.
- We would further solicit input into what specific parameters should- in general- be monitored, how often monitoring should occur, and how the monitoring should be done. This would include monitoring for both acute and delayed toxicities. The concept could be considered a core safety monitoring schedule that should be implemented in any study, independent of phase or agent. Study specific safety monitoring would then be additions to the core monitoring schedule.
- We would also solicit input on when external review or an independent monitoring committee would be recommended and the role of that external review; that is, should it be advisory or should it have the authority to stop a study.

Afternoon Session

The limited number of pediatric oncology patients with a given diagnosis make it unlikely that more than one definitive study can be performed in a timely manner. To enhance the interpretation of clinical results, we would like to solicit advice on:

- What types of non-clinical data is considered informative to complement or supplement clinical results
- What the characteristics or properties of non-clinical models and data should be to effectively add to clinical results.
- If no satisfactory models exist, what characteristics should a non-clinical model have to confirm, extend, or substitute for clinical results.
- If there are a set of postulates that can be identified or should be developed