Molecular Characterization Initiative and the Potential for Future Cohort Studies

2023 Childhood Cancer Data Initiative Annual Symposium
Breakout Session #1

NIH NATIONAL CANCER INSTITUTE

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MCI & Future Cohorts: Discussion Overview

- Develop a cohort from the Molecular Characterization Initiative (MCI) to follow patients throughout their lives
  - Incorporate an updated NIH diagnosis list which includes diagnosis, location, stage, metastatic status, treatment, and toxicity encountered during treatment
  - Collect accurate, cumulative dose treatment information and other medication information
  - Conduct disease specific assessments of data quality within the first year of MCI data collection
  - Open MCI to more potential participants, including subsequent cancers

MCI & Future Cohorts: Discussion Overview

- Possible new cohorts
  - Children and AYAs with genetic predisposition syndromes
  - Children and AYAs receiving cellular therapy
  - A landscape analysis may support development of a standardized data dictionary

MCI & Future Cohorts: Discussion Overview

- Other considerations
  - Consider establishing central repository of registry data, to be connected through CCDI participant index with MCI
  - Consider using objective non-contact approaches (e.g., available detailed residential history) for broad exposure/etiology studies
  - Consider using self-report surveys to establish remote data on patients
  - Consider starting surveys at 2 years after treatment begins

MCI & Future Cohorts: Discussion Questions

- What hypothesis(es) would drive the development of a cohort study?
- How best can disease-specific experts and advocates be engaged in study development and planning?
- How important is it to collect any/all retrospective data related to possible pre-natal exposures?
- Should the successful design and conduct of cohort studies be considered as a metric of the success/ utility of CCDI's MCI?

MCI & Future Cohorts: Discussion Questions

- How could one envision cohort studies in specific pediatric brain tumor patients whose tumors have been genomically characterized?
  - As a single, brain tumor cohort or multiple histology-specific cohorts?
  - If, as a single cohort, what critical data elements should be considered?
- How important are natural history studies in discrete brain tumor populations or are sufficient natural history data lacking in any specific histologies?

MCI & Future Cohorts: Discussion Questions

- What additional data are needed to be captured longitudinally, over and above that provided currently by Project: Every Child to satisfy a cohort study design? What processes/procedures need to be developed to capture data that would make a cohort study feasible and meaningful?
- What control data (population) are required for optimal cohort study design and conduct?
- How many and in what specific indications should cohort studies be considered?

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