Observational studies and novel interventional approaches for rare pediatric cancers

2023 Childhood Cancer Data Initiative Annual Symposium

Breakout Session #6

NIH NATIONAL CANCER INSTITUTE

3/30/2023

Observational studies and novel interventional approaches

- Goal: In this breakout session, participants will discuss the national rare cancer initiative and how to translate these findings to novel interventional approaches, barriers and opportunities in conducting both these types of studies in the rare cancers space within pediatrics and AYA community
- Seeking community input: Initial thoughts and input on the framework of the national strategy for rare cancers

- Facilitators: Mary Frances Wedekind and Ted Laetsch
- Scribe: Christina Vivelo

Summary of breakout

- Everyone enthusiastic with this proposal
- Incorporate advocacy and patient voices early
- Initiate conversations with FDA early to ensure data can be utilized as external controls as we build this infrastructure
- Utilize the successful programs to learn from then engage these programs by determining where we can help them through this infrastructure
- One of the important aspects is the remote enrollment
  - Make a “share-care” model to engage the home team
  - Patients can receive opinions without having to go anywhere
  - May provide care for the underserved population

Summary of breakout

- Engaging the AYA community needs careful thought
  - Many cancers in the AYA population are seen by numerous other adult specialties, not just oncologist
- Molecular tumor boards could be impactful for the treating home physician and patients
  - Need to aggregate those already being utilized or discuss where we can be helpful to not replace/duplicate

Discussion Starters

- How do we best engage disease experts in the project?
- How do we best engage and leverage the advocacy experts in the project?
- Where do we start? How do we navigate determining which cancer types we should have a more dedicated focus on?
- How will we measure success? What are the metrics?
- How can we most efficiently transition from natural history/registry studies to therapeutic studies? What are the steps required to make such a transition?
- What major logistical hurdles do we envision as barriers to opening?
- What infrastructure of CCDI would be needed to ensure the success of this? Do these resources exist? If not how rapidly do we need to develop them

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