Patient Preference Studies in Pediatric Oncology Therapeutic Product Development

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Therapeutic Product Development for Pediatric Cancer

• Accomplished through coordinated, national, multi-center clinical trial infrastructure since mid 1950s

• Unique pediatric clinical practice model: Integration of clinical research and care

• Relatively rare condition (15,000 cases/yr. in US); requires collaboration

• Research success: >80% of children with cancer are cured

• Cancer remains the leading cause of death from disease in children
Product Development (cont.)

• Substantial proportion of childhood cancer survivors experience disabling/life-shortening late effects of successful cancer therapy; current focus of new therapy reduction-reduce late effects

• Unmet medical need for new therapies continues: Benefit:risk assessment includes erosion of excellent outcome results with therapy reduction to decrease late toxicities.

• Highly leverages adult cancer therapeutics discovery and development

• Primary goal of therapy: CURE; differs from adult cancer therapy goal of Prolongation of survival
Clinical Research in Life Threatening Diseases in Children

- Prospect for direct clinical benefit to study subject required (minor increase over minimal risk)
- No placebo controls
- Parental consent required
- Assent required for patients 14 years of age and older
- Highly emotionally charged environment for adequate information exchange
Challenges to Patient Preference Approaches in Pediatric Cancer

• Limited concept/content understanding of health-related benefits and risks
• Health related vocabulary not measurable until age 5 with variable trajectory (depending on concept, eg. pain, fatigue) until age 8 when dependable responses can be assumed: survey instruments
• Limited understanding of abstract concepts
• Significant cross-cultural differences re. degree of information sharing between parents and children
• Variability in educational systems: reading comprehension and survey instruments
Patient Preference Approaches

• Age related differences in benefits perspectives: adolescent concerns related to body image, immediate well being, fertility vs. “invasive” procedures, numbers of injections in younger children.

• Understanding of prognostic risk group, stage of disease, and phase of treatment significantly impact patient preferences of both children and parents.

• Frontline treatment decision preferences likely to be same/similar in children* and parents when cure is primary treatment objective.

• To encompass the totality of pediatric population, survey tools for both children( age appropriate) and parents required.
Patient Preference

• Appreciation of short term risks (immediate adverse events) and risks of late or long term toxicities may likely differ between parents and children and requires different survey techniques.

• Patient preferences (child responders and parents alike) must be interpreted in the context of the clinical situation.

• Clinical trial designs based on patient preferences likely limited to issues of treatment scheduling, formulations, and routes of administration, site of care, etc.