



# 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.

