Well-Being of Child and Family Participants in Phase 1 Pediatric Oncology Clinical Trials

Stacey Crane, PhD, RN, CPON®, Joan E. Haase, PhD, RN, FAAN, and Susan E. Hickman, PhD

Cancer remains the leading cause of death by disease in children aged 14 years and younger (Siegel, Miller, & Jemal, 2016). Although five-year survival rates for pediatric cancers have improved overall to 83%, for some pediatric cancers, the five-year survival rate is only 67% (Siegel et al., 2016). New therapies are needed to improve outcomes for children with cancer. Phase 1 clinical trials (P1Ts) are the first step in testing new medical therapies in humans and are essential to the development of innovative therapies for children with cancer (Kim et al., 2008; Lee, Skolnik, & Adamson, 2009).

Although the need for P1Ts is generally accepted, P1Ts raise ethical concerns (Agrawal & Emanuel, 2003; Berg, 2007; Crites & Kodish, 2013; de Vries et al., 2011; Ekert, 2013; Estlin, Cotterill, Pratt, Pearson, & Bernstein, 2000; Hazen, Zyzanski, Baker, Drotar, & Kodish, 2015; Kearns & Morland, 2014; Miller & Joffe, 2008; Oberman & Frader, 2003; Weinfurt et al., 2012). Goals of P1Ts are traditionally to determine the maximum-tolerated dose of the therapy, describe the action of the therapy in humans, and reveal side effects (Kim et al., 2008; Lee et al., 2005). The National Cancer Institute updated P1T templates to include preliminary determination of efficacy as a secondary trial objective (Weber et al., 2015). P1Ts are primarily conducted to determine how innovative therapies may safely be given. Although not intended to directly benefit participants, healthcare providers and researchers hope that P1Ts will directly benefit at least some participants.

The Declaration of Helsinki requires that “while the primary purpose of medical research is to generate new knowledge, this goal can never take precedence over the rights and interests of individual research subjects” (World Medical Association, 2013, p. 2). The ethical challenge of P1Ts is to ensure that the well-being of participants is supported throughout the trial.

Ethical concerns regarding P1Ts are more complex in children with cancer, in part because children