Improving Outcomes for Pediatric, Adolescent and Young Adult Cancer Survivors

Request for BSA Concept Approval, RFA in Response to the STAR Act

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The Childhood Cancer Survivorship, Treatment, Access and Research (STAR) Act

- Congress strongly encourages efforts to advance pediatric, adolescent, and young adult (AYA) cancer survivor research
- Authorizes improvements to:
  1. Biospecimen collections and infrastructure
  2. Cancer registry infrastructure
  3. Research to improve the care of and quality of life for survivors
  4. Additional survivorship care provisions
The STAR Act – Six Key Research Areas

1. Survivor outcomes
2. Barriers to follow-up care
3. Familial, socioeconomic, and environmental factors
4. Indicators used for long-term follow-up
5. Risk factors, predictors and molecular basis identification
6. Targeted interventions to reduce the burden of morbidity

Consideration of health disparities, minorities or other medically underserved populations
Background – Growing Population of Survivors

Growing population of survivors
Persistent disparities
Estimated 630,000 cancer survivors age 0 – 39 in US

1SEER 9 areas. Based on follow-up of patients into 2015. Expected survival rates are derived from the U.S. Annual Life Tables.
Background – Survivors have significantly more chronic health conditions per person than community controls¹

### Background - data from Observational Studies

#### Healthcare Delivery
- Unmet needs for long-term follow-up
- Care often delivered by provider not familiar with late effects
- Continuity of care in information across multiple providers and settings

#### Adverse Effects

**Physical**
- Symptoms (fatigue, sleep disturbances, peripheral neuropathy)
- Impaired physical function
- Neurocognitive impairments
- Late treatment effects (endocrine, cardiopulmonary, 2º malignancies)
- Accelerated aging and comorbidity
- Fertility concerns
- Adverse body composition

**Psychosocial**
- Psychological Distress
- Disrupted social development
- Financial hardship, insurance coverage, school/employment difficulties

**Behavioral**
- Reduced physical activity
- Potential for risky behaviors (alcohol, tobacco, non-adherence)
- Obesity
NIH Portfolio: 102 Pediatric and/or AYA Cancer Survivor Projects (active 2017, survivor at day 1 of diagnosis)
RFA Purpose

- To support the scientific development of interventions to address adverse physical and psychosocial effects in survivors of pediatric and/or AYA cancers.
RFA: Improving Outcomes for Pediatric, Adolescent and Young Adult Cancer Survivors

- Focus: Development, testing and/or scaling of innovative, feasible, and effective interventions to address physical and psychosocial adverse effects in survivors of pediatric and/or AYA cancers.

- Responsive proposals may include:
  - Development and preliminary testing of a novel intervention
  - Testing efficacy in a phase II or III trial
  - Effectiveness testing in real-world settings
  - Dissemination/implementation studies

- Responsive proposals should include:
  - Meaningful proximal endpoints
RFA: Improving Outcomes for Pediatric, Adolescent and Young Adult Cancer Survivors

- Clinical trial required

- 6-8 U01s

- Anticipate that scope of projects will be diverse; anticipate that some projects can be supported through modular grant budgets

- $4.8M Total Annual Set Aside for the RFA, ($24M 5-year total)

- 2 receipt dates (April 2019, February 2020)
Summary

- Proposed RFA will:
  - Address priorities encouraged by the STAR Act
  - Leverage insights from previous NCI investments in observational studies that confirm the burden of morbidity
  - Yield research-tested interventions that improve outcomes for the growing number of survivors of pediatric and AYA cancers

Future concept ideas will more broadly address the 6 STAR Act areas (PAR in development)
Response to BSA Feedback

Clarifications

 What is the broader NCI strategy to address all components of the STAR Act?
 What are plans for future concept ideas?
 Where does health promotion fit in, specifically, is it within the models of care or development and testing of interventions?
 Is development and testing of interventions across the spectrum from feasibility/acceptability/preliminary efficacy (phase I-II) to effectiveness (phase III) and implementation trials is being solicited by this RFA?
 How will the U01 mechanism will be used and what size of awards will be allowed?

Recommendations

 Two receipt dates were suggested to improve the quality of applications.
 The subcommittee emphasized the importance of biospecimen collection more generally for future exploration of hypotheses about etiology, mechanisms and outcomes.
 It was noted that in some instances follow-up of participants may be warranted.