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

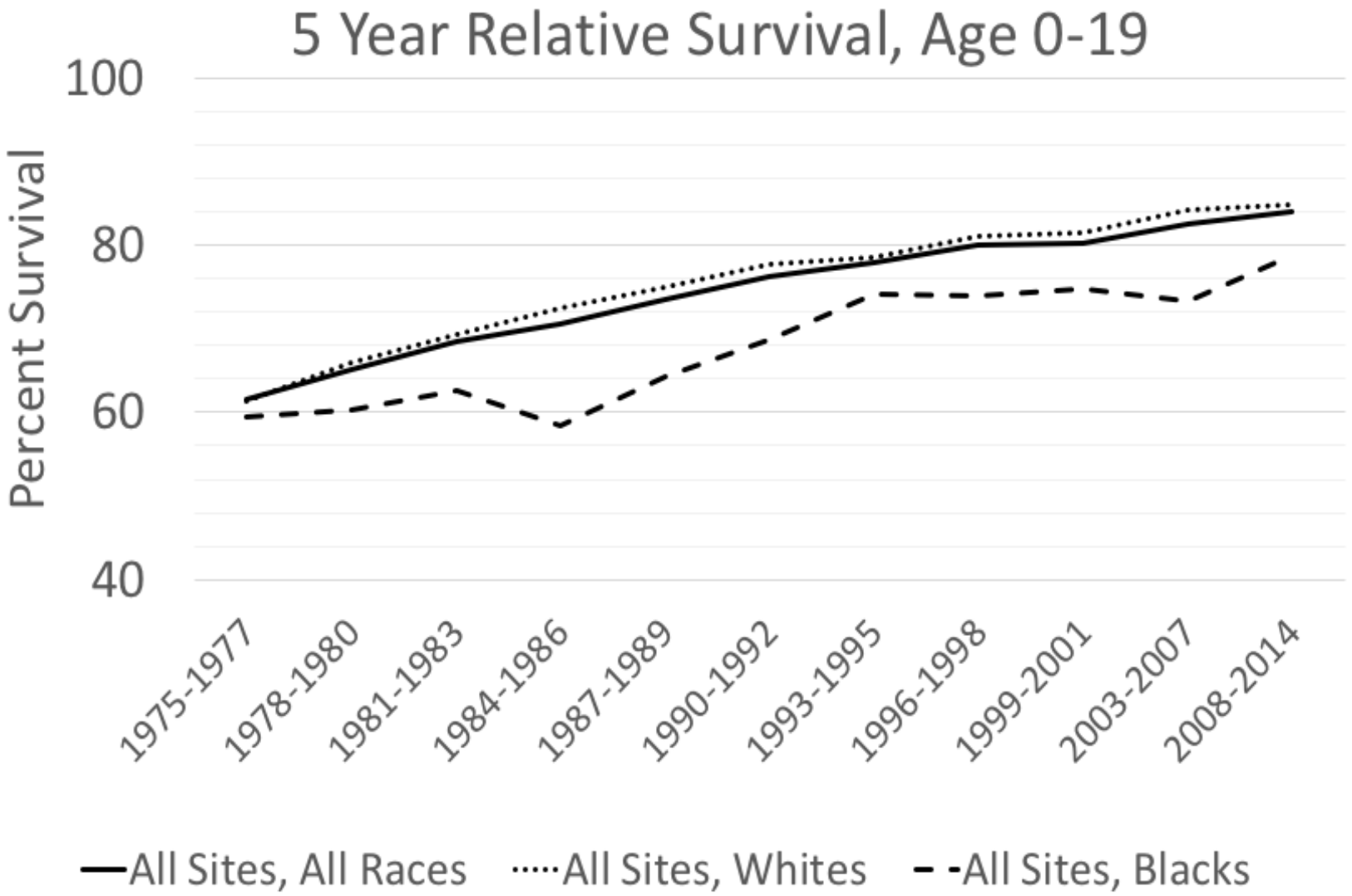
- **Congress strongly encourages efforts to advance pediatric, adolescent, and young adult (AYA) cancer survivor research**
- Authorizes improvements to:
  1. Biospecimen collections and infrastructure
  2. Cancer registry infrastructure
  3. **Research to improve the care of and quality of life for 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

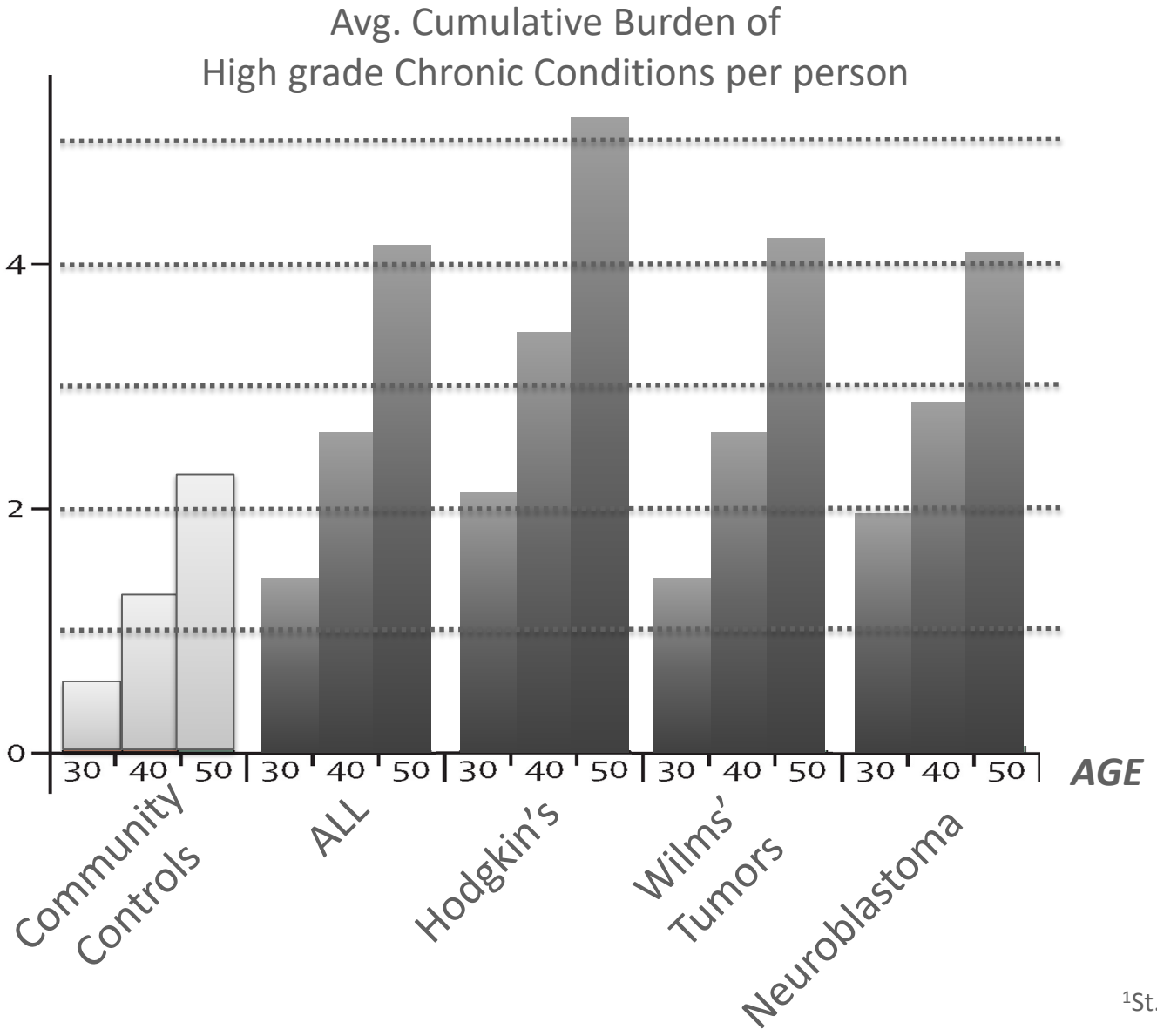
Background – Growing Population of Survivors



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

[1]SEER 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 controls[1]



Chronic Conditions across a variety of organ systems	
Neoplasms	Pulmonary
Cardiovascular	Endocrine
Renal	Musculoskeletal
Haematological	Neurological
Ocular	Reproductive
Gastrointestinal	Infections
	Auditory

[1]St. Jude Lifetime Cohort, Bhakta N, Liu Q, Ness KK, et al. Lancet 390:2569-2582, 2017

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, 2^o malignancies)
- Accelerated aging and comorbidity
- Fertility concerns
- Adverse body composition

Psychosocial

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

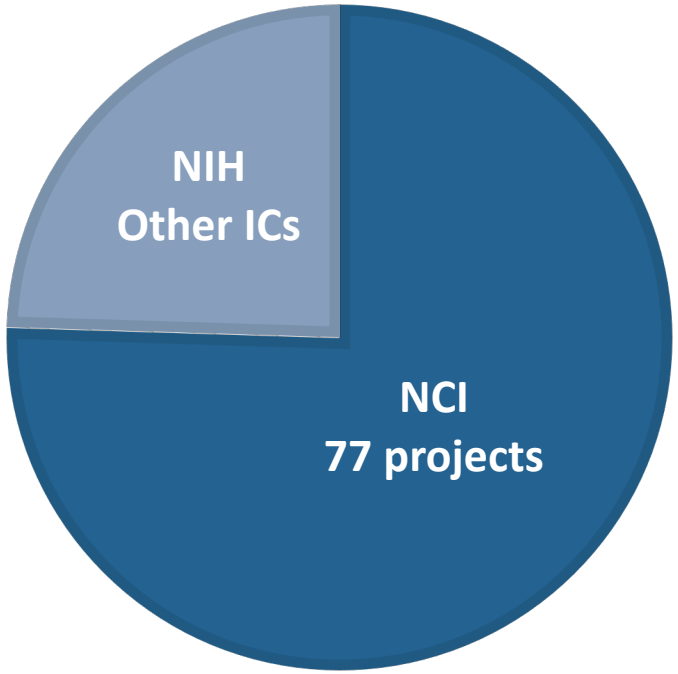
Behavioral

- 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)

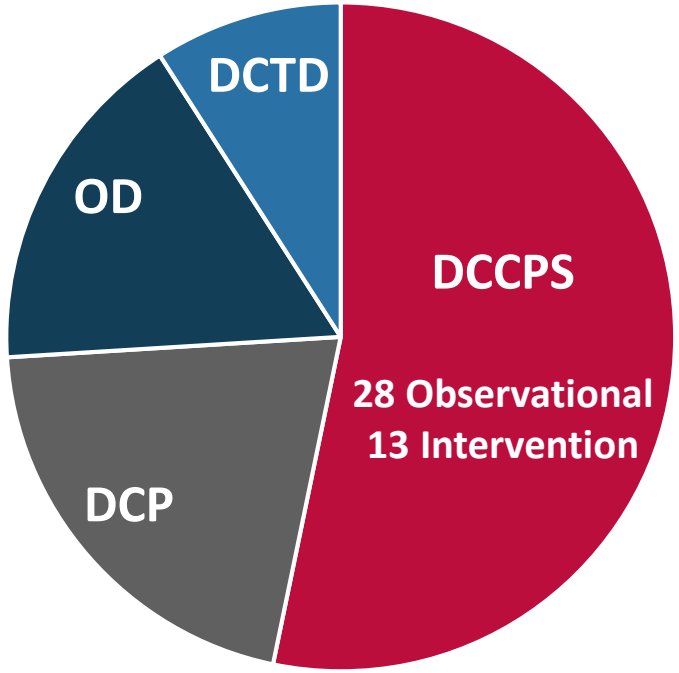
NIH

102 projects



NCI

77 projects
43 Observational, 34 Intervention



RFA Purpose

- To support the scientific development of **interventions** to address adverse physical and psychosocial effects in survivors of pediatric and/or AYA cancers

Improve Healthcare Delivery

Prevent or Mitigate Adverse Effects

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.

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