Community Outreach and Advocacy

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What is the definition of “advocacy”?

The act or process of supporting a cause or proposal

Levels of Advocacy

- Local: lobby our institution and hospital partners to support our mission
- Community: Outreach events
- State: CPRIT (Cancer Prevention and Research Institute of Texas)- Advisory Committee on Childhood Cancers
- National: Congress, White House

Why is Childhood Cancer Advocacy Important?

Isn’t childhood cancer now close to 90% curable?
- Cancer remains the #1 cause of death by disease for children in the United States
- There will be over 500,000 survivors by 2020 and at least 1/3 will suffer from a life-threatening health problem caused by their prior treatments
- Kids don’t vote!!

Pediatric Cancer Advocacy in San Antonio

- LLS Light the night walk
- LLS Little Heroes Prom
- CureSearch Walk
- Kick it for CureSearch
- ACS Relay for Life
- Camp Discovery
- For the Kids Dance Marathon at UTSA
- St. Baldrick’s San Antonio

Collectively these two efforts have raised over $900,000 in five years of events

National Advocacy

- Named to NCI Council of Research Advocates in September 2014 at a White House briefing on childhood cancer
- Attend President Obama’s announcement of the Precision Medicine Initiative in the White House East Room
- Invited to speak at 68th World Health Assembly in Geneva
- Invited to give the keynote address at the kickoff for 2015 Pediatric Cancer Action Days — Childhood Cancer STAR Act announced
- Invite Texas contingent for 2016 Pediatric Cancer Action days
- Invited speaker for the 7th Annual Childhood Cancer Summit in Washington D.C. – September 2016
- Assist NCI Director’s office in highlighting childhood cancer survivorship for the 2016 bypass budge to be released in fall 2016
Examples of Legislation Resulting from National Advocacy Efforts

Best Pharmaceuticals for Children Act (BPCA)
- Originally established in 1997
- Provides pharmaceutical companies an incentive of six additional months of marketing exclusivity if it voluntarily conducts FDA-requested pediatric studies
- As of March 2015, the FDA had issued 464 written requests for BPCA studies and 59 were for oncology products (pediatric studies often completed when market exclusivity is about to expire)

Impact
Increased studies of oncology drugs in children, but has not incentivized development of new drugs

Pediatric Research Equity Act (PREA)
- Enacted by Congress in 2003
- Pre-market requirement that drug sponsors conduct pediatric assessments on drugs developed for adults
- Requirement can be waived if "claimed indication" is not relevant in children
- Orphan indications are exempt

Impact
- Virtually all childhood cancers are orphan diseases
- PREA has had virtually no impact on pediatric studies of adult cancer drugs

Creating Hope Act
- Signed into law by President Obama in 2011
- Creates a market incentive for the development of drugs for rare pediatric diseases
- Sponsor requests that drug or biologic under development be designated a "rare pediatric disease product application"
- If ultimately approved, then the sponsor receives a priority review voucher from the FDA
  - Valuable to the sponsor – get other drugs to market sooner
  - Unlimited transferability
- To date, three vouchers have been sold:
  - BioMarin to Sanofi/Regeneron for $67.5 million
  - Knight Therapeutics to Gilead for $125 million
  - United Therapeutics to AbbVie for $350 million (first drug developed for childhood cancer indication under this system)

Gabriella Miller Kids First Act
- Gabriella Miller

- Earmarked $126 million over 10 years to research in genetics of childhood cancers and birth defects
- Administered via the NIH Common Fund

Childhood Cancer STAR Act: Current Status
- 233 Co-sponsors in the House of Representatives
- 23 Co-sponsors in the Senate
- Broad bipartisan support in both chambers
- Likely hearing before the House Energy and Commerce committee in September 2016