Pediatric Cancer Advocacy and the Childhood Cancer STAR Act

Gregory J. Aune, MD, PhD
Department of Pediatrics Grand Rounds
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Disclosure

Gregory J. Aune, MD, PhD discloses the following relationships with commercial companies:

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Learning Objectives

- Review the major pediatric cancer advocacy organizations and the overall research and clinical environment that necessitates activities in pediatric cancer
- Review a specific example where advocacy efforts directly impacted patient care and outcome
- Identify the major pieces of prior legislation that impact pediatric and pediatric cancer drug development
- Review the components of the Childhood Cancer STAR Act

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!!
My Treatment

- Splenectomy
- 45 Gy radiation to neck, chest, abdomen - “mantle field” and “inverted-Y”
- 8 cycles of chemotherapy with 8 different drugs - 4 cycles MOPP, 4 cycles ABVD
- Supportive care - ineffective antiemetics, severe ileus, no G-CSF

My Late Effects

- Hypothyroidism
- Pulmonary fibrosis
- Infertility
- Aortic stenosis
- 3 vessel coronary artery disease - all blocked >95%
- Secondary skin cancers
- TIA/Stroke
- Diabetes

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
- Organize Texas contingent for 2016 Pediatric Cancer Action Days

Organizations

- There are over 1,000 foundations or organizations in the United States whose mission is to promote greater awareness of childhood cancer and fundraise
- The community is often fractured and it is extremely challenging to achieve consensus
- At times individuals can direct communications toward government research funding agencies that is adversarial
- Huffington Post Blog Post: The Empty Chair of Harold Varmus
- Quote: “It is simply a shame that in my opinion Dr. Varmus does not believe that childhood cancer is an important enough issue to address.”
Alliance for Childhood Cancer

- Founded September 12, 2001
- Represents more than thirty national patient advocacy groups and professional medical and scientific organizations
- Members include AAP, ASPHO, COG, ACS, St. Baldrick’s Foundation, ASCO, ACCR
- Organization emerged out of the 1999 and 2000 childhood cancer summits — goal was to coalesce the community around and focus on shared goals:
  "Increase awareness of childhood cancer, advance research and policies to pr

Coalition Against Childhood Cancer

- Founded in 2011
- Includes over 100 members from small and medium sized organizations
- Focused more on outreach and fundraising and less on policy issues
- Holding their first scientific meeting at Cold Spring Harbor in November 2016

St. Baldrick’s Foundation

- 2nd largest funder of childhood cancer research in the United States (The National Institutes of Health is #1)
- Founded in 1999 when two New Yorkers decide to shave their heads to raise money for childhood cancer
- Annual fundraising now surpasses $30 million annually
- Intently focused on policy issues and spearheaded the creation of the Childhood Cancer STAR Act

(S) Survivorship

- 60% of long-term survivors of childhood cancer will either be dead or suffering from a life-threatening late effect by age 50
- Across the United States less than 30% of survivors receive care in a comprehensive survivorship clinic
  - Our program: approximately 300 out of >3,000 in Bexar county
  - There will be 500,000 long-term survivors of pediatric cancer by 2020

(T) Treatment

- The development of new pediatric anticancer agents is lagging far behind adult cancers. In the last 30 years:
  - 62 new drugs have been FDA-approved for adult cancers
  - 3 for pediatric cancers

(A) Access

- Children are often excluded from compassionate used programs developed by the pharmaceutical industry
- The Josh Hardy case
The National Cancer Institute spends approximately 4% of its total budget on childhood cancers. The private sector funds 2/3 of the research in adult cancers. In children, the private sector investment is essentially zero. Pediatric representation on national cancer policy and advisory board is often overlooked.

The Childhood Cancer Community has three large goals:
1. Increase pediatric cancer research
2. Improve pediatric cancer drug development
3. Provide necessary support to childhood cancer survivors

Review a specific example where advocacy efforts directly impacted patient care and outcome.

The Case of Josh Hardy
- Case centered on the concept of compassionate use
  “Use outside of a clinical trial of an investigational medical product not yet approved by the FDA”

Josh developed an adenovirus infection when he was recovering from a BMT for myelodysplastic disorder.

His physicians asked Chimerix for compassionate access to brincidofovir, a drug in phase 3 trials.
Identify the major pieces of prior legislation that impact pediatric and pediatric cancer drug development

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 incent...
**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)

**Gabriela 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

**21st Century Cures**
- Passed by House 344-77 on July 10, 2015
-Awaiting a vote in the Senate
- Wide Bipartisan Support
- Relevant Components
  - Compassionate use language – Josh Hardy Case
  - NIH Funding – Increase NIH Budget by $10 Billion over 5 years
  - Re-authorization of the Creating Hope Act
- Review the components of the Childhood Cancer STAR Act
Childhood Cancer
STAR Act

The process
• Policy Roundtable
• Consensus Building
• Champions on the Hill Introduce the Bill

In the House, it is H.R. 3381. It was introduced by Congressman Michael McCaul (R-TX), Congressman Chris Van Hollen (D-MD) and Congresswoman Jackie Speier (D-CA) in the summer of 2015

In the Senate, it is S. 1883. It was introduced by Senator Jack Reed (D-Ri) and Senator Shelley Moore Capito (R-WV)

Key Components of the STAR Act

Expanding Opportunities for Childhood Cancer Research:
• Authorize the National Cancer Institute (NCI) to expand existing efforts to collect biospecimens for childhood cancer patients enrolled in NCI-sponsored clinical trials to collect and maintain relevant clinical, biological, and demographic information on all children, adolescents, and young adults with cancer.
Improving Childhood Cancer Surveillance:
- Authorize grants to state cancer registries to identify and track incidences of child, adolescent, and young adult cancer.
- This funding would be used to identify and train reporters of childhood cancer cases, secure infrastructure to ensure early reporting and capture of child cancer incidences, and support the collection of cases into a national childhood cancer registry.

Improving Quality of Life for Childhood Cancer Survivors:
- Enhance research on the late effects of childhood cancers, including a study on insurance coverage and payment of care for childhood cancer survivors;
- Improve collaboration among providers so that doctors are better able to care for this population as they age; and
- Establish a new pilot program to begin to explore innovative models of care for childhood cancer survivors.

Ensuring Patients Access to Publicly Available Compassionate Use Policies:
- Ensure that pharmaceutical companies have publicly accessible compassionate use policies for drugs treating serious or life-threatening conditions, and
- Would require FDA to finalize its guidance for industry on the issue.

Ensuring Pediatric Expertise at the National Institutes of Health (NIH):
- Require the inclusion of at least one pediatric oncologist on the National Cancer Advisory Board
- Improve pediatric research reporting requirements to include childhood cancer.

2016 Pediatric Cancer Action Days

The Results

- 233 Co-sponsors in the House of Representatives
- 22 Co-sponsors in the Senate
- Broad bipartisan support in both chambers
- Likely hearing before the House Energy and Commerce committee as early as late next week
Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has.
-Margaret Mead

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