Childhood Cancer Policy Roundtable, part III
February 27, 2015
Meeting Summary
& Collaborative Action Items

The meeting began with a brief welcome from Dan Smith, President of AdvocacySmiths, and a quick recap of the process to date, including the recent work group meetings. Dan stated the purpose of this meeting was to finish a collaborative strategy process that began six months ago, to coalesce around a cogent set of priorities and identify a path forward. Dan reviewed the agenda for the day, noting that the group, including participants both in the room and on the phone, would first hear report backs from each work group leader and the full group would then have an opportunity to further refine, and thereafter, agree upon the consensus goals. Then the group would discuss tactics, or "how" each goal will be accomplished. Finally, the group would work to agree upon a set of concrete tasks/follow up items that will be executed, under the direction of a Steering Committee, to achieve the goals. This meeting summary is the third and final report of agreed upon action items.

A full list of participants is available at the end of this summary.

WORK GROUP REPORT BACKS, DISCUSSION AND ALIGNMENT ON GOALS

MAXIMIZING DISCOVERY THROUGH RESEARCH

David Arons presented the Maximizing Discovery Through Research work group draft goals and after a brief discussion and some relatively minor amendments and clarifying comments, the full group agreed upon the following goals:

(1) Legislative Goal: Increase childhood cancer research funding

- NIH/HCI Appropriations for FY 2016
- Gabriella Miller Kids First Research Act Appropriations
- Department of Defense Pediatrics Research Funding
- Review Structure and Effectiveness of the Pediatric Subcommittee of Oncologic Drug Advisory Committee (PedsODAC) (harmonize with Accelerating Development work group)

(2) Agency Goal: Increase effectiveness of agencies as funders and as partners

- Improve and increase transparency of NCI/NIH childhood cancer research related funding
- Enable Children's Oncology Group (COG) to have both sufficient funding and flexibility to maintain and start new trials as needed
    - It was recommended (by LeAnn Jackson) that the group also consider working with the Pediatric Brain Tumor Consortium on this topic

- Ensure that an increase in participation on peer review groups by pediatric oncology exerts and on advisory committees by patient advocates
    - It was pointed out (by Ruth Hoffman) that NCI does not make recommendations on NCAB representatives, those decisions come from the White House
    - It was recommended (by Beth Anne Baber) the group continue pressure on White House through emails and calls
- Maintain close working relationship with Pediatrics Match trial and advocate for its success

(3) Agency Goal: Pediatric cancer use of FY15 Gabriella Miller Kids First Research Act funding

- Advocate for use of Kids First funding to be used for pediatric cancer in FY15

ACCELERATING DEVELOPMENT & AVAILABILITY OF PROMISING TREATMENTS

David Pugach (filling in for James Baumberger) presented the Accelerating Development & Availability of Promising Treatments work group draft goals and after a brief discussion and some minor amendments and clarifying comments, the full group agreed upon the following goals:

(1) Legislative Goal: Explore modifications to Pediatric Research Equity Act (PREA) / Best Pharmaceuticals for Children Act (BPCA) to optimize effectiveness for pediatric cancer: Ensure that BPCA and PREA are working optimally for childhood cancer

- Expand PREA to include mechanism of action
- Work to eliminate the exclusion of rare disease trials under orphan drugs under PREA
- Explore BPCA modifications to improve effectiveness and transparency
- Monitor and maintain oversight of the structure and effectiveness of the Pediatric Subcommittee of Oncologic Drugs Advisory Committee (PedsODAC) (harmonize with Maximizing Discovery work group)

(2) Legislative Goal: Engage in ongoing House and Senate FDA reform initiatives (21st Century Cures) to ensure proposals advance cures for children with cancer

- Expanded access issues
    - No current position being taken, the group agreed to continue monitoring the situation as it develops through congressional action
- Orphan Product Extensions Now (OPEN) Act
    - No current position or action is being taken but it remains a priority for monitoring and may be raised as potential action item later
- Caroline Pryce Walker Conquer Childhood Cancer Act to be reviewed and considered at a later time for action
- Respond effectively to these congressional initiatives and move toward more proactivity

MAXIMIZING DELIVERY: CARE, QUALITY OF LIFE, SURVIVORSHIP, AND CAREGIVER SUPPORT

Vickie Buenger presented the Maximizing Delivery work group draft goals and after a brief discussion and clarifying comments, the full group agreed upon the following goals:

(1) Legislative Goal: Align with other coalitions working within the Care, Quality of Life, Survivorship, and Caregiver space on legislation to ensure appropriate coverage and payment for high-quality care furnished across multiple settings and by a range of providers for pediatric treatment, palliative care, and survivorship

- Monitor and incorporate legislative recommendations as appropriate and agreed to coming our of the Institute of Medicine Quality of Life meeting on March 9 & 10
- Patient Quality of Life Coalition (PQLC) legislative recommendations
    - Monitor PQLC legislative initiatives and incorporate as appropriate and agreed to
    - Encourage Roundtable members to join PQLC to provide pediatric voice
- Align with global palliative care community
    - Seek to hold the US accountable for signing the 2014 World Health Organization (WHO) resolution on palliative care through working with the International Pediatric Palliative Care Group (Ruth Hoffman agreed to send info – International Pediatric Palliative Care Network? http://www.icpcn.org/)

(2) Legislative Goal: Explore access and payment gap issues and policy options across the entire range of pediatric cancer experience

- Identify access issues related to coding and payer relations and work to offer solutions
- Work to cover psychosocial services

(3) Agency Goal: Hold federal agencies accountable for improving access and coverage across the entire range of the pediatric cancer experience, especially after end of treatment

- Work to ensure that the NCI Childhood Cancer Survivorship Study continues to receive high priority for funding and support within NCI for medical and psychosocial research, particularly with the recruiting and monitoring of the second cohort
    - Get NCI, Office of Cancer Survivorship, to list pediatric cancer as a priority agenda item, on their website: The group agreed to approach the Office of Cancer Survivorship (Julie Rowland, Director), as part of the approach to NCI

(4) Agency Goal: Explore access and payment gap issues and administrative options across the entire range of the pediatric cancer experience

- Work to ensure consistent definitions of standard of care to ease coding and gain more access to hard-to-obtain services
- Work to ensure that standard language for patients includes "psychosocial" needs
- Work to ensure that care is delivered and coordinated across the gaps that currently exist, including:
    - Transition from pediatrics to adult
    - Complications and treatments related to late effects
    - Access across the continuum

GROUP STRATEGY SESSION & AGREED UPON ACTION ITEMS

Working within the framework of the agreed upon goals (above), the group endeavored to identify the "how" or the tactics the group will employ to achieve the goals. Dan reminded the group that the "how" requires real resources (time, talent, treasure) and each participating organization is asked to contribute both knowledge and action to achieve collaborative success for the childhood cancer community. Below is a list of the agreed upon tactics and each has been assigned a lead. It should be noted that the group acknowledged that a tactic without assigned resources/leadership/sweat equity would be ultimately fall off the list.

➢ ACTION ITEM 1:

A common thread and thus a foremost priority for the group will be to join other advocacy efforts to press for an overall increase in the NIH and NCI budgets, because without that overall increase, it will be extremely difficult to increase funding for pediatric research. The group agreed that the childhood cancer community could be an extremely powerful voice in those efforts, but that it also would expect to not be exploited in the process and also that once the overall increases are achieved, that it will fight to obtain its fair share for the childhood cancer community's priorities.

This will be accomplished through several avenues, but the first and most tangible is through the ongoing appropriations process in Congress. The group will send a letter to Congress by March 26 asking for funding increases for NIH/NCI in line with the OVAC request, as well as specific requests for report language relevant to childhood cancer (TBD), including a possible request for NCI to do one or two formal reports for transparency sake. The group also discussed the need to create a compelling message for this effort and to that end, the group will attempt to capture moving patient and family stories and share those with legislators and the media to advance these efforts. It was also agreed that the priorities addressed in the letter would be used during respective Action Days on the Hill in 2015, and beyond as appropriate.

Simultaneously, and with a target completion date of March 31, the group will write two agency letters, one to the director of NIH and another to the director of the NCI. The group agreed to have Dr. Adamson review a draft of the letter prior to submission. The letters will comprehensively address a variety of agenda items and areas of concern for the childhood cancer community (e.g. distribution of funding from Gabriella Miller Kids First research Act) and will lay the groundwork for specific follow up with the agency (e.g. meetings with program administrators etc.).

Going forward, the group also agreed that these respective letters will be created annually and serve as the outline for the group's agenda with Congress and the NIH/NCI.

NIH/NCI APPROPS letter to Congress will include three things [DEADLINE MARCH 26]:

1. Ask for a NIH/NCI funding increase of $33B, aligned with One Voice Against Cancer (OVAC)
2. Assess report language
3. Call out one or two specifics asks of NCI to do formal reports

Letter work group volunteers:

- Kevin Mathis
- David Pugach
- David Arons
- Gavin Lindberg
- Jonathan Agin

NCI AGENCY LETTER(S) written to both Acting Director Lowy* and Collins. Both letters to include [Target deadline: March 31]:

1. NIH increased funding request of $33B, aligned with OVAC

2. Language regarding advocacy indicating the group will follow their activity and maintain pressure
3. Reference presumption of precision medicine $ directed to peds
4. Include requests to meet with specific people
5. Gabriella Miller Kids First Research Act
6. Final-ish letter draft to be sent to Dr. Adamson for review

Letter work group volunteers:

- David Arons
- David Pugach
- Susan Weiner
- Ruth Hoffman
- Steve Crowley
- Kevin Mathis

*Since this meeting, Director Varmus announced his intention to step-down by the end of March, assuming the leadership responsibility will be Acting Director Lowy.

➢ ACTION ITEM 2:

On March 25, the Food and Drug Administration will hold a public stakeholder meeting to gather comments in preparation for their report to Congress on BPCA and PREA. The meeting information can be found here: https://www.federalregister.gov/articles/2015/02/26/2015-03974/pediatric-stakeholder-meeting-request-for-comments. In preparation for the meeting, the group, under leadership of Susan Weiner, will: conduct analysis by talking to industry, talk to Dr. Adamson, compile data, talk to FDA, encourage member attendance, and regroup after the meeting to discuss priorities and possible action items.

PREPARATION FOR MARCH 25 FDA STAKEHOLDER MEETING, public comments:

1. The group agreed to:
    a. Prep work (see #2)
    b. Encourage attendance
    c. Listen
    d. Congregate after to talk about priorities
2. Prep work and deeper analysis before March 25, Susan Weiner & James Baumberger to put together a work group (the week of March 16) that will:
    a. Talk to industry
    b. Talk to Dr. Adamson
    c. Find data
    d. Talk to FDA (Dr. Reaman)

➢ ACTION ITEM 3:

A work group has been formed (and has already met) to strategize how to maintain and strengthen the Department of Defense research funding. The groups seeks to ensure the integrity of current funding levels for current programs (at minimum) and hopes to develop a strategy for the creation of a new pediatric-specific peer review program.

DEPARTMENT OF DEFENSE work group to focus building out the DoD initiative better by:

1. Short-term: maintain what does exist in the current program

2. Long-term: establish an entirely new program for childhood cancer

DoD work group volunteers:
- David Arons
- LeAnn Jackson

➢ ACTION ITEM 4:

The group agreed to write a letter to the Institute of Child Health and Human Development regarding the Childhood Cancer Survivorship Study. The letter will emphasize the high-priority nature of funding and support within NCI for medical and psychosocial research, particularly with the recruiting and monitoring of the second cohort.

CHILDHOOD CANCER SURVIVORSHIP STUDY letter
1. Beth Anne Baber gathering info
2. Danielle Leach/Vickie Buenger to take on the letter writing

➢ ACTION ITEM 5:

The group agreed to participate in identifying childhood cancer priorities for inclusion in a comprehensive cancer omnibus bill. Overall, the bill would seek to increase transparency at NIH and NCI and outline a list of boards necessary to ensure the transparency. The final list of items for inclusion has yet to be determined, but a few complied by Gavin Lindberg during the Roundtable process for consideration are: release of an "advocate friendly" explanation of the 4% level of support for pediatric cancer research, produce an annual report on childhood cancer research from NCI, create a new Deputy Director for Pediatric Oncology at NCI, conduct an NCI sponsored annual meeting with the pediatric cancer advocacy community to discuss the state of research (NIAMS Coalition Outreach and Education Day is an example from another institute), provide accounting from the NIH regarding support for pediatric cancer outside of the NCI, achieve agreement from the NCI to include a pediatric cancer expert on any study section reviewing a pediatric cancer grant application, and convene a state-of-the-science conference on childhood cancer (or individual malignancies) and issue program announcements addressing unmet needs in treatment/research.

COMPREHENSIVE CANCER BILL (Omnibus), will seek to:
1. Increase transparency at NIH/NCI
2. Review list complied by Gavin (see paragraph above)
3. Create/review a list of boards to be compiled and circulated by Kevin Mathis
    a. Include psycho-oncology
    b. List, representation should reference/cross walk all the issues

➢ ACTION ITEM 6:

A few members of the group felt strongly about holding the US accountable for signing the 2014 World Health Organization (WHO) resolution on palliative care, specifically suggesting a pilot program for childhood cancer patients.

HOLD US ACCOUNTABLE FOR SIGNING WHO RESOLUTION by suggesting a pilot program for childhood cancer. Effort to be led by Ruth Hoffman with help from Connie Connor.

➢ ACTION ITEM 7:

The group agreed to press the Children's Oncology Group (COG) to mandate a standard of care for pediatric cancer patients. Through mandating a standard of care, the group seeks to ensure payment solutions for hard-to-obtain services, including but not limited to: neuropsych testing, fertility testing & treatment, dental coverage, implants, hearing aids, mental health services, PET scans post-treatment, bone density and Dexa scans, appropriate oncology referrals, high dose vitamin D, dietician/nutrition counseling, growth hormone, testosterone replacement, hair replacement/implants, improving access to survivorship internists (especially instances with out of network/Medicaid issues), ankle foot orthotics, and improved payment plan (or in some instances increasing billable hours) for survivorship programs. It was suggested that the group review and consider the National Comprehensive Cancer Network (NCCN) guidelines where appropriate.

ASK COG TO MANDATE STANDARD OF CARE, work group volunteers:

- Vicki Brown (volunteered to lead)
- Connie Connor
- Kara Bryant

STEERING COMMITTEE FOR CONTINUED LEADERSHIP & GUIDANCE

As a result of the six-month Roundtable process, participants agreed to a robust agenda and a comprehensive list of action items. To help drive the process and provide leadership in tackling the ambitious outline of action items, it was suggested and subsequently agreed that a Steering Committee be formed. The Steering Committee was carefully crafted to evenly represent both The Alliance for Childhood Cancer and the Coalition Against Childhood Cancer (CAC2) and consists of the following members:

- Kevin Mathis, ex-officio advisor & convener
- David Pugach, (Alliance)
- David Arons (Alliance)
- Danielle Leach (Alliance)
- Vickie Buenger (CAC2)
- Gavin Lindberg (CAC2)
- Steve Crowley (CAC2)
- Susan Weiner (Alliance & CAC2)

Maintaining momentum will require continuous involvement and feedback. Everyone is encouraged to stay involved and push forward. AdvocacySmiths is grateful for the opportunity to work with all members of the Roundtable process. The creation of the strategy outlined in this document could not have been accomplished without the dedication and hard work of each participant.

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