75th Meeting of the National Cancer Institute (NCI)
NCI Council of Research Advocates (NCRA)
National Institutes of Health (NIH)

Building 40, Room 1201/1203
NIH Campus
Bethesda, Maryland

Monday, September 25, 2017

Members Present

Mr. David Arons, Chair
Dr. Gregory Aune
Mr. Rick Bangs
Ms. Mary Ann Battles
Dr. Senaida Fernandez Poole
Ms. Julie Fleshman
Dr. Sue Friedman
Ms. Shelley Fuld Nasso
Ms. Danielle Leach
Dr. June McKoy (by telephone)
Dr. Roberto Vargas
Dr. Regina M. Vidaver (by phone)

Speakers

Dr. Terri Armstrong, Senior Investigator, Neuro-Oncology Branch, NCI
Mr. David Arons, Chair, NCRA; Chief Executive Officer, National Brain Tumor Society
Ms. Michelle Canady, Deputy Budget Officer, Office of Budget and Finance, NCI
Ms. Holly Gibbons, Deputy Director, Office of Government and Congressional Relations, NCI
Dr. Mark Gilbert, Chief, Neuro-Oncology Branch, NCI
Ms. M. K. Holohan, Director, Office of Government and Congressional Relations, NCI
Dr. Douglas R. Lowy, Acting Director, NCI; Chief, Laboratory of Cellular Oncology, NCI
Ms. Anne Lubenow, Special Assistant for External Relations, NCI
Ms. Laurie Mignone, Budget Officer, Office of Budget and Finance, NCI
Dr. Brigitte Widemann, Chief, Pediatric Oncology Branch, NCI
Ms. Amy Williams, Acting Director, Office of Advocacy Relations; Executive Secretary, NCRA, NCI

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Welcome and Meeting Goals
Mr. Arons, Ms. Williams

Ms. Williams opened the meeting by welcoming members and guests at 9:15 a.m.

Mr. Arons acknowledged the recent passing of NCRA member Ms. Kimberly Newman-McCown and dedicated the meeting to her. He welcomed two new council members, Mr. Rick Bang and Ms. Danielle Leach.

Mr. Arons reviewed the meeting agenda and read an opening statement regarding conflict of interest guidelines for the meeting.

Budget and Legislative Update
M.K. Holohan, Ms. Mignone

Ms. Holohan and Ms. Mignone provided an overview of the budget process and the status of fiscal years 2017 – 2019.

- FY 2017 ends on September 30 and year-end close is an NCI-wide effort. NCI's enacted level is $5.389 billion. The Cancer Moonshot budget is $300 million and carries over from year to year.
- FY 2018 starts on October 1. The continuing resolution (CR) is in place from October 1 through December 8, 2017; NCI will receive a prorated appropriation of 18.9% for 69 days in the CR.
- FY 2019 budget formulation is awaiting funding and policy decisions from the Office of Management and Budget (OMB). NCI also does a Professional Judgment Budget ($6.38 billion proposed), which provides an opportunity to be prospective.
- The chairs of the Appropriation subcommittees (Senator Roy Blunt [R-MO] and Congressman Tom Cole [R-OK) appear to be in lock-step about NIH issues and have been positive about endorsing basic research.
- Typically, the president's budget does not result a full-scale adoption, and there are often dramatic differences in the resolution.
- There has been strong bipartisan and bicameral support for NIH and NCI. More than 20 members of Congress and more than 50 staffers visited NCI in 2017.
- A 12-bill omnibus, which included opportunities for new programs, passed in the House, but the Senate requires an unlikely 60 votes.

<u>Discussion</u>

- Mr. Bangs said that discussing the value of research with the public is important, and that a role for advocates might involve articulating the kind of work being done, the number of lives saved, etc.,—"something to hang our hats on through social media that articulates what the public is getting." Ms. Holohan said that the NIH website shows economic benefits and NCI always aims to integrate that information into every congressional message and briefing. She agreed that there has not been enough public focus on the economic impact of basic research and that promoting messages about research would be "a wonderful thing for advocates to do."

- Mr. Arons asked how the current health care reform proposals will affect cancer research, and how replacement or modification of the Affordable Care Act (ACA) could affect insurance coverage and clinical research. Ms. Holohan said that it is too early to tell how that will play out, but it is important for NCRA to be part of the discussions.
- Ms. Fleshman asked whether money appropriated for the Cancer Moonshot in 2017 would carry over to the next year if not spent. Ms. Mignone said that it would and that annual appropriations need to be committed or obligated by September 30.
- In answer to Ms. Williams question about preventing lapses in funding, Ms. Mignone said that investments are now being made to position for 2018 and beyond and that the majority of funds for grants is "mortgage money" for a period of 5 to 7 years. The Office of Budget and Finance (OBF) does a large amount of tracking to prevent lapses.
- Ms. Battles asked how a government shut-down would impact ongoing clinical trials from a direct patient perspective. Ms. Mignone said that NCI always prepares for this scenario, which would be the "worst possible thing for the intramural program." Only a small cohort of about 600 NCI employees involved in patient care would continue working. Ms. Lubenow said that patients already in the system would receive care, but funding could not be committed for anything that is not already in a protocol. Every shut-down differs in terms of what is permitted, and there is much negotiation to ensure that investments made over years of research are not lost. Ms. Holohan said that while exceptions are made for patient research during research, there are few or none for animal research. She added that Congress has very little interest in a shut-down because of the political risk.

Rare Tumor Patient Engagement Network, Part 2
Dr. Armstrong and Dr. Widemann

Dr. Armstrong briefly reviewed the mission, goals, operation and status of the Rare Tumor Patient Engagement Network.

- Funding has been secured, with planned start dates of October 1, 2017 (intramural) and Spring 2018 (extramural).
- Hiring has begun, with the goal of having key personnel in place in October.
- Protocols and rare tumor clinics are in development. Several therapeutic trials are already open, including one for immunotherapy for central nervous system (CNS) rare tumors.
- Suggestions are needed for partnering with advocacy to overcome challenges such as engaging underrepresented populations, providing virtual support for patients, encouraging patient participation in clinical trials, and maintaining patient engagement over time.

<u>Discussion</u>

- Mr. Bangs asked for a definition of a rare tumor. Dr. Gilbert said that rare tumors are those with an annual U.S. incidence of <50,000. For CNS cancers, the rare tumor incidence is <1000 per year. He added that the strategies being employed for

addressing rare tumors may eventually be extended to more classically defined cancers and molecule subtypes.

- Ms. Leach asked how the network's efforts complement the work already being done in the Children's Oncology Group (COG) and other groups. Dr. Widemann said that the network has reached out to collaborate with COG to study tumors of interest, including rare types of sarcomas. Dr. Armstrong added that the data generated will be housed on the cloud for other investigators to access. The network hopes to partner with other tumor registries to share samples. Dr. Gilbert said that for many adult rare cancers, there is no activity outside of this network, other than anecdotal reports from small institutions, which the network has been able to pull together. Dr. Widemann said that the network also holds rare tumor clinics that bring patients and extramural experts to NCI for in-depth clinical studies. The network is also involved with patient-related outcome studies, which can lead to a substantial understanding of a tumor.
- Dr. Friedman asked how the network is engaging patients and whether patients who act as advisors are compensated for their efforts in promotion and recruitment. Dr. Armstrong said that there is no payment for patients from the Collaborative Ependymoma Research Network (CERN) and that they are working with the Center for Cancer Research (CCR) to understand what they are able to do.
- Dr. Aune said that in San Antonio, there are about 200 to 300 [rare tumor pediatric] patients throughout the city and most are not getting enrolled in rare tumor databanks. He said that he and Ms. Leach would be willing to help identify these patients to improve their situations through research. Dr. Widemann said that this is an important issue where advocates can help and she encouraged Dr. Aune and Ms. Leach to follow up. Dr. Armstrong said that providers are difficult to reach; the network has tried sending mailings using lists from associations. In CERN, patients were asked for names of providers who were then contacted. This direct engagement with patients and follow-up with providers seems to work well. Dr. Gilbert said that this could be done on a more global scale with patients coming to NIH and the 26 centers across the U.S. to establish collaborations with local providers.

Dr. Armstrong asked for the members' thoughts on engaging patients, noting problems in the past with reaching underrepresented populations.

- Dr. Vargas said that the University of California San Francisco (UCSF) has been partnering with organizations that are trusted advocates for underrepresented populations, such as African American congregations. Although the rare tumor population may be small within these groups, he felt there was value in building relationships and trust, and that these organizations can help with designing outreach materials. Dr. Vargas also said that any online materials should translate well into mobile applications noting that low-income people, especially young people, use smart phones as the primary method to access the Internet. He offered to share more information offline.
- Dr. Friedman asked if there will be a conference or event for educating patients about contacting their providers. She said that leadership training for patient advocates is important, and that patients will spread the word through their communication groups (e.g., Facebook pages).

- Ms. Leach asked if social media comments are moderated to ensure accuracy. Dr. Armstrong said that they do not answer direct care questions and focus more on general terms. They have worked on responses with providers in CERN, and wrote a series of vetted tweets.
- In answer to a question from Mr. Arons, Dr. Armstrong said that material is in development to help providers identify patients in their system. They have tried several methods, such as magnets.
- Dr. Friedman asked about the response from providers in the community setting. Dr. Widemann said that reaching out to community and academic centers and creating a good working relationship for the care of patients has led to a series of referrals. When patients learn of efforts to reach out to their doctors, they talk about the positive experience, which breeds good will.
- Ms. Battles asked about gathering data on certain epidemiological issues, such as where patients enter the health care system. Dr. Armstrong said that an outcome survey has questions to address these issues, including risk and environmental exposures. The learnings can inform the primary care practitioners that see the patients. For example, most patients were symptomatic for a year before having diagnostic imaging.
- Dr. Bangs mentioned his institution's rare cancer committee and the DART (Dual Anti-CTLA-4 & Anti-PD-1 blockade in Rare Tumors) trial, and said that some advocates are well-connected and may be very helpful with the process of patient advocacy.

Dr. Armstrong asked the members to discuss what advocacy groups might need or want from a partnership.

- Dr. Friedman said it would be useful to help the groups find board members who are top scientists to ensure that any materials developed are evidence-based. If a group member should speak without evidence, a board member can address the issue while respecting the group's culture.
- Ms. Leach said that it's important to be inclusive of the various groups that address a specific disease or group of diseases; working with coalitions would be helpful. Dr. Widemann said that an open process allows any advocacy group to apply. It is not possible for the network to engage 1000 groups, and they need criteria to determine which ones are appropriate to the network's mission, and whether the network is helpful to theirs. Dr. Friedman suggested allowing an interested group to be engaged in a study design. This will give a sense of these who are most committed and scientifically oriented.
- Ms. Williams said that the Office of Advocacy Relations is the "front door" for the advocacy community and can help determine appropriate organizations for partnerships and can facilitate partnerships.

Dr. Armstrong asked for suggestions on the best ways to help foster relationships with patients, provide virtual support, and maintain communication.

- Mr. Bangs said that each advocacy group should be evaluated on a case-by-case basis to determine what is specific and complementary for each group.

- Ms. Fleshman says that "stars will rise to the top" and should be the ones to engage and invite to meetings. Dr. Widemann expressed some concern about inclusiveness and rivalries among groups. Ms. Williams said that her office can help facilitate any such issues that arise.
- Dr. Friedman suggested sharing as much data as possible with patients. For example, let them know how many of them responded to a request and were contacted. Any response to information that they share is really engaging.
- Dr. Vargas said that most of his work involves facilitating partnerships between scientists and the community and could share resources offline.

Advocate Engagement in Precision Medicine Clinical Trials
Mr. Arons and Ms. Williams

Mr. Arons asked the members to consider some of the issues raised in a recent article ("A Cancer Conundrum: Too Many Drug Trials, Too Few Patients," by Gina Kolata, *The New York Times*, August 12, 2017, https://nyti.ms/2vsMRXf) that he shared with them before the meeting.

Discussion

- Dr. Friedman said that post-research care is a concern if patients cannot get access to a drug at the end of a clinical trial.
- Ms. Fleshman said that for pancreatic cancer, once issue is trying to determine where molecular profiling is being done as a standard of care. Pancreatic cancer is still being treated as one disease.
- Mr. Bangs cited issues with finding and accessing major clinical trials, and in providing data to clinicaltrials.gov. Dr. Friedman said that there is a problem with lack of uniformity across different search criteria, such as those for HER2-negative cancers. Ms. Battles said that patients may not find an existing trial because it is not accurately represented; patients still rely on their doctors to learn about clinical trials. Dr. Friedman said that with personalized medicine, it is rare that any one patient fits into only one category.
- Dr. Aune said that investigators are always faced with too few patients for clinical trials, but this is being addressed by collaborative research across the country and the world. He noted that participation is better for pediatric trials than adult trials and that recruitment may become more difficult as further subsets are defined. Ms. Leach noted that institutions will refer to their own trials first if they have a strong program, and perhaps offer outside choices, but there is much competition among institutions.
- Mr. Arons asked if the advocacy community is doing enough to prepare for precision medicine and understanding how to educate patients about the molecular world and testing. Ms. Battles said that the challenge to advocacy groups is to educate the public about precision medicine. Creating a vision for 2025 would be good. Patients also want to be able to own their data. Dr. Friedman noted that some of this has already entered the commercial space, as with 23andMe. However, many tests have not been validated and clinicians as well as patients do know how to use the data.

- Dr. Aune said that in another 10 to 15 years, molecular profiling is likely to become as commonplace as histology is now, and the role of advocacy groups will be to ensure that patients have access to baseline information.
- Dr. Bangs said that advocacy groups need to encourage patients to seek and consider clinical trials, whether they decide to participate or not. Also, as precision medicine "picks up speed," the issue of second opinions also needs to be encouraged.
- Dr. Vargas said that precision medicine also means making better use of big data and using better analyses. He noted that under-represented populations are being left out of cutting edge technology advances and that there are currently not many Spanish-speaking genetic counselors across the Americas.
- Mr. Arons said that areas for patient advocacy related to molecular profiling include: defining the challenges, keeping up with unvalidated tests (who is doing them and what are their qualifications), addressing insurance reimbursement, assessing safety of the tests and the capability of clinicians using them, and addressing communities least likely to be engaged.
- Dr. Friedman asked about convening a group of all NIH institutes to discuss the clinical trial issues. Ms. Fleishman said that there is a tremendous opportunity for a large-scale campaign for clinical trials, but a huge gap remains. Efforts made in 2000 to educate the public died. She noted that for pancreatic cancer, enrollment in trials increased as patients received more information and resources. Enrollment had been 4%, but increased to 12% for patients who called for information, and 20% for those with molecular profiling.
- Dr. Aune said that MATCH was the fastest accruing trial that NCI has ever done and asked how some of those recruitment efforts can be applied to other trials. Ms. Williams said that MATCH had many highly accruing sites and included all tumor types and a variety of different facets.
- Dr. Friedman suggesting educating patients about trials that are specific vs. nonspecific. Some trials are more nuanced and patients who do not qualify for one trial may qualify for another.
- Mr. Arons suggested that NCI consider doing a landscape analysis of standards and evaluations, which is a difficult task for non-profit advocacy groups. Ms. Leach said that her organization carried out an analysis on drug development research that involved an external firm, about 7 to 8 months, external reviewers, and a real commitment by a collaborative coalition.

Action item: Members will discuss the issues raised at this meeting with their respective coalitions. Members will plan to have a one-hour group call a month later to share their findings.

Action item: Members will suggest a partnership plan for engaging advocacy groups in the Rare Tumor Patient Engagement Network initiative.

Update from the NCI Acting Director
Dr. Lowy

Dr. Lowy welcomed Ms. Leach and Mr. Bangs to the NCRA. He provided updates on NCI activities, including the new collaboration with the Veterans Administration (VA) as well as the Cancer Moonshot.

- Dr. Julie Rowland is retiring from the Office of Cancer Survivorship (OCS), which she has led since 1999. During that period, there has been an enormous increase in the number of cancer survivors in the U.S., and many dimensions of survivorship have been identified as well. Dr. Rowland thanked the NCRA members for their support and acknowledged the important role of advocacy in improving survivorship.
- Dr. Norman "Ned" Sharpless will be the new NCI director pending final vetting. Dr. Lowy accepted his invitation to stay on as principle deputy director.
- Stephen White (Division of Cancer Biology) has been actively monitoring the impact of the recent hurricanes and reaching out to researchers at the University of Houston.
- The Grant Support Index (GSI) is focusing on reducing the number of investigators with many grants.
- The Next Generation Research Initiative is focusing on increasing support for Early Stage Investigators (ESIs) and Early Established Investigators (EEIs). The number of NIH-funded investigators under age 45 years has decreased since 1990, with some stabilization since 2005. For NCI, funding has been trending up for this age group but leveling off. There has been a substantial decline for investigators under age 40, which may be due to an increase in the training period for this group as well as other factors. NCI is increasing the number of investigators deemed "early stage" who never had a major grant from NIH.
- NCI-MATCH has enrolled 6397 patients as of September 17, 2017; 5482 received test results, 983 had a gene abnormality matching an available treatment, and 689 patients enrolled for treatment. About 50% of the 25 treatment arms are fully accrued. Assay success rate has been 94%. The rare variant initiative began in May 2017; a process for qualifying other commercial and academic sequencing labs will be posted to encourage additional accrual to the adult MATCH trial. The pediatric MATCH trial began in July 2017 and differs from the adult trial in that participants need to have a rigorous assessment of germ-line configuration.
- NAVIGATE (NCI and VA Interagency Group to Accelerate Trials Enrollment) is a collaborative effort between NCI and the VA to enable more VA patients to enroll in NCI national clinical trials. The effort provides infrastructure funding support to VA sites as well as an executive oversight committee.
- Cancer Moonshot RFAs (10) have been released and will be funded by the end of this week. Ten partnerships, contracts and supplements will also be funded at this time, including an NCI collaboration with the Department of Energy (DOE) on predictive modeling, as well as a smoking cessation program and two initiatives aimed at pediatric cancers.

Discussion

- Ms. Fuld Nasso asked about plans for the OCS after Dr. Rowland retires. Dr. Robert Croyle said that the office has evolved and diversified beyond just social factors, into

epidemiological, long-term, and outcomes issues. The office has recently focused on the infrastructure needed to scale up its goals. Cancer survivor cohort studies are being funded in a number of locations. A main strategic goal is improving the leverage of the cancer registry as a component of the cancer survivor registry. The weakest link involves disparities and underserved populations. He invited the NCRA members to offer feedback to help identify an individual to lead this effort. In answer to a question from Ms. Leach, Dr. Croyle said there would be representation for pediatric cancer on the search committee.

- Mr. Arons asked about areas of the Cancer Moonshot that advocacy groups can get "excited about" in order to support and amplify its goals. Dr. Lowy said that the Cancer Moonshot allows NCI to support research that might not be strongly supported otherwise, such as highly selective imaging technology for patients with extensive prostate cancer. He also noted that NCI wants to ensure that Congress makes a distinction between regular appropriations and the Cancer Moonshot, so that regular appropriations are not decreased.
- Dr. Vidaver asked about the lack of geographic diversity for funding and whether expansion in that area is anticipated. Dr. Lowy said there is a potential for change in that area, but it will depend on the quality of the applications and proposals.
- Ms. Battles asked if the Next Generation Research Initiative has a strategy to include more community physicians and younger researchers. Dr. Lowy said that while it is important to engage these individuals in research, this is not the focus of the Next Generation Research Initiative. NCI does not have direct access to the age of applicants; NIH de-identifies that information. NCI is considering increasing the amount of time used to identify early-stage investigators. The primary focus is on PhDs versus MDs; the definition of "early stage" differs for each and is a greater issue for PhDs than MDs.
- Mr. Arons asked about the business strategy for the MATCH trial. Dr. Lowy said that this will depend on the arm of the trial and the particular treatments. He noted opportunities for adding immunotherapy as an alternative for non-responsive patients. The trial was successful because of extraordinary planning with cooperative groups and the engagement of the pharmaceutical companies in providing drugs. Costs would be prohibitive for companies to do such trials individually. The public-private partnership is cost-effective.
- Dr. Friedman asked if any efforts are being made to match patients to other trials if they are not eligible for a particular trial. Dr. Lowy said that while this is not a systematic effort, patients are informed about other known trials.
- Dr. Vargas asked about efforts to encourage and support emerging investigators, particularly women and ethnic minorities, and to have clinical trials proportionally reflect the country's population. Dr. Lowy said that while no firm decisions have been made about addressing under-represented minorities, NCI is considering these issues very seriously and trying to find the right balance to be as fair as possible to applicants. Dr. McKoy said that while it is important to keep the field fair, the science should stand on its own. In answer to Dr. Vidaver's question about using a blinded letter of intent, Dr. Lowy said that this has been considered for pilot studies. He added that new applications are funded at the same rate for early-stage and experienced investigators, and NCI is considering an even larger boost for early-stage

investigators. Renewals come mainly from experienced investigators and fare better than brand new proposals. However, most NCI awards are new awards.

Wrap-Up
Mr. Arons, Ms. Williams

Mr. Arons commended Dr. Lowy on his leadership and reviewed the progress the country has made in the fight against cancer during his tenure:

- Precision medicine initiative: launch of the adult and pediatric MATCH trials
- Cancer Moonshot: leadership and organization of the Blue Ribbon Panel, implementation of the recommendations, and public and patient advocacy engagement
- Big data: launch and expansion of the Genomic Data Commons
- Health disparities: launch of the largest ever study of breast cancer genetics in black women, and launch of a ground-breaking new study of African American cancer survivors
- Increase in NCI's efforts in immunotherapy
- Continued commitment to early career investigators
- Improving lives through cancer research
- Increases in federal funding and federal relations

Mr. Arons also thanked Dr. Lowy for his efforts in opening his door to advocates, calling him the "Patients' NCI Director." Several members joined in to thank Dr. Lowy for his availability and support for advocates, his support for survivorship efforts, and his openness to public-private partnerships.

Ms. Williams thanked Mr. Arons for planning the meeting and thanked Dr. Lowy, the speakers and the members for their participation.

Adjournment

The meeting adjourned at 3:40 p.m.