National Institute of
Arthritis and Musculoskeletal
and Skin Diseases

Minutes of the National Arthritis and Musculoskeletal and Skin Diseases Advisory Council Meeting

May 29, 2024

DEPARTMENT OF HEALTH AND HUMAN SERVICES
PUBLIC HEALTH SERVICE
NATIONAL ARTHRITIS AND MUSCULOSKELETAL AND SKIN DISEASES ADVISORY COUNCIL

MINUTES OF THE 114th MEETING

I. CALL TO ORDER

The 114th meeting of the National Arthritis and Musculoskeletal and Skin Diseases Advisory Council (NAMSAC) was held on May 29, 2024, in Building 31, National Institutes of Health, Bethesda, Maryland, and via videoconference. The meeting was chaired by Dr. Lindsey A. Criswell, Director, National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS).

Attendance

Council members present:
Dr. Edward Botchwey, Professor of Medicine, Wallace H. Coulter Department of Biomedical Engineering, Georgia Institute of Technology
Ms. Ann Elderkin, Consultant, American Society for Bone and Mineral Research (ASBMR)
Dr. Valentina Greco, Carolyn Walch Slayman Professor of Genetics, Yale University
Dr. Tamia Harris-Tryon, Associate Professor, Thomas L. Shields, M.D. Professorship in Dermatology, UT Southwestern, *Ad Hoc Member*
Dr. Karl Jepsen, Associate Dean for Research, Professor of Orthopaedic Surgery, Professor of Biomedical Engineering, University of Michigan
Dr. Shanthini Kasturi, Associate Professor of Rheumatology, Tufts University, *Ad Hoc Member*
Dr. Elizabeth McNally, Elizabeth J. Ward Professor of Genetic Medicine, and Director, Center for Genetic Medicine, Northwestern University Feinberg School of Medicine
Dr. Christopher Mecoli, Assistant Professor of Medicine and Director of Research Operations and Physician Lead, Myositis Precision Center of Excellence, Johns Hopkins University, *Ad Hoc Member*
Dr. Teodora Staeva, Chief Scientific Officer, Lupus Research Alliance
Dr. Maria E. Suarez-Almazor, Barnts Family Distinguished Professor, Department of Health Services Research, Cancer Prevention and Population Sciences, and Section of Rheumatology and Clinical Immunology, Department of General Internal Medicine, Division of Internal Medicine, The University of Texas MD Anderson Cancer Center
Dr. Jinoos Yazdany, Chief, Division of Rheumatology, Zuckerberg San Francisco General Hospital

Staff and Guests

The following NIAMS staff and guests attended:

NIAMS Staff

Dr. Lindsey Criswell

Dr. Robert Carter

Mr. Rick Phillips

Ms. Ryan Lombardi

Ms. Katrina Matthews

Ms. Stephanie Herndon

Dr. Susana Serrate-Sztein

Dr. Kriti Sharma

Dr. Darren Sledjeski

Dr. Gayle Lester

Dr. Mary Garcia-Cazarin

Dr. Robert Colbert

Dr. Robert Walker

Mr. Chris Nee

Ms. Robin DiLiello

Ms. Elizabeth Elliott

Ms. Dandre Thornhill

Dr. Kathy Salaita

Mr. Erik Edgerton

Ms. Melinda Nelson

Ms. Shalanda Wellington

Ms. Justine Buschman

Ms. Christa Reynolds

Dr. Stephanie Burrows

Dr. Jonelle Drugan

Dr. Justin Wilson

Dr. Linda Yang

Ms. Medha Krishnagiri

Dr. Van Nguyen

Ms. Leigh-Ann Creenan

Dr. Chiguang Feng

Dr. Archana Jha

Ms. Jade Greenhill

Dr. Sushmita Purkayastha

Dr. Emily Carifi

Dr. Aron Marquitz

Dr. Xibin Wang

Dr. Alexey Belkin

Dr. Xincheng (Ted) Zheng

Dr. Isaah Vincent

Dr. Giuseppe Pintucci

Dr. Marjorie Lindhurst

Dr. Su-Yao Mao

Dr. Kristy Nicks

Dr. Ricardo Cibotti

Dr. Peter Koch

Dr. Marie Mancini

Dr. Rebecca Lenzi

Dr. Heiyoung Park

Dr. Faye Chen

Dr. Chuck Washabaugh

Ms. Sara Myers

Mr. Jon Davis
Ms. Jana Eisenstein
Dr. Deepti Bajpai
Dr. Aneliya Velkova
Ms. Victoria Matthews
Ms. Leslie Littlejohn
Ms. Yen Thach
Ms. Sheila Simmons
Ms. Teresa Do
Ms. Pamela Beheler
Ms. Sahar Rais-Danai
Ms. Stephanie Kreider
Ms. Tanisha Brown-Caines
Mr. Tom Bond
Ms. Kristina Bushey
Mr. Mark Brown
Mr. Scott Bushman
Mr. Aychew Ghirmisso
Ms. Bonnie Kane
Mr. Patrick Hobson

A total of 207 NIH staff and members of the public watched via NIH Videocast.

Guests:

Dr. Joan Bathon, Director, Division of Rheumatology, Columbia University Medical Center
Dr. Joshua Jacobs, Rush University Medical Center
Ms. Amye L. Leong, President and CEO, Healthy Motivation
Dr. Peter Merkel, Chief of Rheumatology, Professor of Medicine and Epidemiology, University of Pennsylvania
Dr. Aimee Payne, Adjunct Professor of Dermatology, University of Pennsylvania
Dr. Eric Simpson, Professor of Dermatology, Oregon Health and Science University
Ms. Suzanne Schrandt, Founder, CEO, and Chief Patient Advocate, ExPPect
Dr. Kurt Spindler, Cleveland Clinic
Ms. Tiffany Westrich-Robertson, CEO, International Foundation for Autoimmune & Autoinflammatory Arthritis (AiArthritis)

II. INTRODUCTIONS/CONSIDERATION OF MINUTES/FUTURE COUNCIL MEETING DATES

Dr. Lindsey Criswell, NIAMS Director, called to order the 114th meeting of the NAMSAC and led Council members and other attendees in a round of introductions. Dr. Darren Sledjeski, NAMSAC Executive Secretary, reviewed the logistics for the hybrid meeting and reminded members of the public that they may submit written comments at the email address NIAMScouncilmail@niams.nih.gov up to 15 days following the meeting. Dr. Sledjeski reminded Council members of their conflict-of-interest obligations under federal law and notified the Council of upcoming conflict of interest reporting dates in June and October.

Dr. Sledjeski asked Council members if there were any comments or corrections on the minutes of the 113th NAMSAC meeting, which took place on January 30, 2024. There were no comments or corrections, and Dr. Sledjeski moved to approve the minutes. The motion was seconded and approved unanimously.

The dates of the next four Council meetings are September 17, 2024; January 28, 2025; June 3, 2025; and September 8, 2025.

III. NIAMS DIRECTOR'S REPORT AND DISCUSSION

Dr. Criswell opened her remarks by announcing that Dr. Ellen Gravallese has stepped down from the Council. Dr. Criswell expressed her gratitude to Dr. Gravallese for service to the Institute. She introduced and welcomed Dr. Karl Jepsen to his first meeting of his four-year term as Council member. A number of additional new Council members will be joining later this year. She also welcomed three ad hoc Council members who will be serving as Council members for this meeting: Drs. Tamia Harris-Tryon, Shanthini Kasturi, and Christopher Mecoli. These scientists were invited from the pool of NIAMS mentored career development award grantees to expose them to the work of the Council and for NIAMS to obtain input from researchers at an earlier stage in their career than typical Council members.

NIH and NIAMS Budget

Dr. Criswell updated the Council on the status of the NIH and NIAMS budgets. In March, the President signed the appropriations bill from Congress that provides funding to NIH for this fiscal year. NIH received a 0.7% decrease to its overall budget, excluding funding for the Advanced Research Projects Agency for Health (ARPA-H). NIAMS' budget remained identical to the FY 2023 funding level of approximately $687.6M. Just prior to the signing of the FY 2024 appropriations, the President released his proposed budget for FY 2025. The proposed budget calls for a 5% increase to the FY 2023 funding levels for NIH and a 0.3% increase for NIAMS over the FY 2023 budget. The Senate and House have yet to release their proposed FY 2025 appropriations, however, and those numbers are likely to differ from the President's.

Dr. Criswell presented the breakdown of NIAMS' updated funding plan based on the FY 2024 appropriations. The Institute has kept its R01 payline for early-stage investigators (ESIs) at 15%, the same level as last year. The R01 payline for most investigators has been set at the 8th percentile, which is lower than previous years. Dr. Criswell emphasized that this does not mean that the Institute will be funding fewer grants. NIAMS will be intentionally setting aside funding, known as select pay, to support applications that score above the payline but are deemed of high priority to the NIAMS mission areas. In the past, select pay decisions were made at the end of the fiscal year based on available resources; this new method allows the Institute to be more intentional in its use of select pay throughout the fiscal year. Dr. Criswell acknowledged the challenges posed by flat budgets in a time of high inflation, but conveyed NIAMS' commitment to maintain the number of grants that it awards each year to help preserve and stabilize the research workforce.

Personnel Changes

At the NIH level, NIH Director Dr. Monica Bertagnolli recently selected Dr. Sean Mooney as the new Director of the Center for Information Technology (CIT). Dr. Mooney joins NIH from the University of Washington. Dr. Kathleen Neuzil has been named Director of the Fogarty International Center and will also serve as the NIH Associate Director for International Research. Dr. Neuzil comes to NIH from the University of Maryland School of Medicine. Dr. Criswell noted that Dr. Neuzil is the first permanent female Director of the Fogarty International Center.

At the NIAMS level, Dr. Criswell shared that Dr. Bob Carter, NIAMS Deputy Director, has announced his intention to retire from federal service at the end of the year. Dr. Carter has served as Deputy Director since joining NIAMS in 2008 and served an extended stint as Acting Director following the passing of longtime Director Dr. Stephen Katz in 2018. Dr. Criswell highlighted Dr. Carter's central role in expanding NIAMS' participation in trans-NIH initiatives during his tenure and expressed her personal gratitude for Dr. Carter's guidance since her arrival to the Institute. Dr. Criswell also provided a brief update on NIAMS' search for its next Scientific Director following Dr. John O'Shea's decision to step down from that role. NIAMS will be inviting the top candidates to visit the Institute and meet with staff in the coming weeks.

Dr. Criswell welcomed two new NIAMS extramural program directors, Dr. Giuseppe Pintucci and Dr. Isaah Vincent. Dr. Criswell provided brief descriptions of their careers at NIH and research backgrounds. Dr. Pintucci will help oversee the Joint Biology and Diseases and Orthopaedics Program, and Dr. Vincent will manage NIAMS' training programs. Dr. Criswell also welcomed a new NIAMS Scientific Review Officer, Dr. Chiguang Feng, who joins from the National Institute of Environmental Health Sciences (NIEHS).

Activities of General Interest

In March, Dr. Criswell joined Director Bertagnolli and other NIH leaders at the White House for the signing of President Biden's Executive Order on Advancing Women's Health Research and Innovation. The aims of the executive order are to encourage the integration of women's health across the federal research portfolio, prioritize investments in women's health research, galvanize research on women's midlife health, and assess unmet needs. At the U.S. Senate, a bipartisan group of women Senators recently introduced a bill called the Advancing Menopause Care and Midlife Women's Health Act, which would authorize $275 million over five years to strengthen and expand federal research on menopause and workforce training in this area. Here at NIH, Dr. Bertagnolli recently announced the NIH-wide Strategic Plan for Research on the Health of Women 2024-2028, which outlines five goals to guide NIH-supported research on woman's health.

In other congressional and community outreach activities, Dr. Criswell recently participated in a congressional briefing sponsored by the Coalition of Skin Diseases and attended a panel at the 2024 National Lupus Advocacy Summit in Washington, D.C. Dr. Criswell emphasized NIAMS' commitment to engaging with, and listening to, patient support and community groups and incorporating their input into the Institute's programmatic decisions.

Dr. Criswell updated the Council on several programs of interest to NIAMS. The Exposome in Autoimmune Disease Collaborating Teams (EXACT) initiative, which is the inaugural program of the NIH Office of Autoimmune Disease Research, recently issued six planning awards. Rheumatoid arthritis, lupus, and psoriatic disease are included among the disease focus areas. The ARPA-H Novel Innovations for Tissue Regeneration in Osteoarthritis (NITRO) program, which was developed with significant NIAMS input, has selected its five research teams with proposals to develop breakthroughs in reversing osteoarthritis. Dr. Criswell also highlighted the Complement Animal Research in Experimentation (Complement-ARIE) program, which is an NIH Common Fund initiative that aims to accelerate the development and validation of human-based, non-animal approaches to study biological functions and human disease. These approaches are also called new approach methodologies (NAMs). The program recently announced the winners of its crowdsourcing competition for innovative ideas.

The Molecular Transducers of Physical Activity Consortium (MoTrPAC), a NIH Common Fund program that is co-managed by NIAMS, recently published results from a study of young adult rats that found that endurance exercise affects all tissues of the body, including those not usually associated with exercise. The study found differences between male and female rats that were more widespread than anticipated, which highlights the importance of including animals from both sexes in preclinical work. The data from this study is available for further use on the MoTrPAC website.

As part of NIH's data science strategy, Dr. Criswell highlighted a program called the NIH Cloud Lab that enables intramural and extramural researchers to try out commercial cloud services free for 90 days in an NIH-approved setting. The program has been newly expanded for researchers who do not yet have NIH awards.

Dr. Criswell reminded the Council that Director Bertagnolli has prioritized extending research participation opportunities among broader communities and better serving rural areas. To address these priorities, this spring the NIH Council of Councils approved a concept to establish a Clinical Research in Primary Care network. As part of this initiative, a Research Opportunity Announcement was recently published that invites existing clinical research networks to serve as pilot network research hubs as part of a planned larger infrastructure supporting research in primary care settings.

As discussed at past meetings, NIH has undergone a process to simplify the Research Project Grant (RPG) review framework, which will go into effect for applications with due dates on or after January 25, 2025. The goal is to better facilitate the identification of the strongest and highest impact research by enabling peer reviewers to focus on the key factors for assessing the scientific and technical merit of the proposed research, while also mitigating the effect of institutional bias and reducing reviewer burden. NIH has also implemented revisions to the NIH fellowship application and review process to improve the chances that the most promising candidates will be consistently identified by focusing on the candidate's preparedness and potential, their research training plan, and the institution’s commitment to the candidate. Dr. Criswell also provided an update on the results of the NIH Center for Scientific Review's (CSR) evaluation of the immunology and respiratory systems cluster of study sections. As a result of the evaluation, most applications that were previously reviewed by the Arthritis, Connective Tissue

and Skin (ACTS) Study Section will now be handled by the new Skin and Connective Tissue Sciences (SCTS) Study Section. The remaining applications will be reviewed by the Mechanisms of Autoimmunity (MAI) and Immune Mechanisms of Hypersensitivity and Allergy (IMHA) study sections.

Since the previous Council meeting, NIH has announced pay raises of approximately 4% and 8% for predoctoral and postdoctoral scholars, respectively. These scholars will also receive increased subsidies for childcare and training-related expenses. Pay levels will be adjusted based on levels of experience, and NIH plans to increase the levels further over the next five years.

In furtherance of NIAMS' desire to support a broad portfolio of meritorious research, the Institute has announced a new funding policy in which all R01 applications from PIs with three or more active NIH R01-equivalent grants will be considered low priority. Dr. Criswell emphasized that this policy aligns with the broader NIH goal to balance, strengthen, and broaden the biomedical research workforce. This policy will take effect in FY 2025. Dr. Criswell noted several exceptions to the policy: for example, if the PI has no active NIAMS grants or if a grant will expire within a year and bring the PI's total to less than three active grants.

Finally, Dr. Criswell provided a brief update on the development of NIAMS' strategic plan for FY 2025-2029. NIAMS has issued two Requests for Information (RFIs) to solicit feedback from the community on the strategic planning process and is planning a third this summer. NIAMS staff has prepared a draft strategic plan, which has been shared with Council members for their review and will be discussed in greater detail later in the meeting.

Discussion

Dr. Elizabeth McNally commented positively about the Complement-ARIE program but felt that review panels need more education about alternatives to animal-based approaches. Dr. Criswell suggested that CSR might have a role to play in providing training as this need becomes more apparent. Dr. Carter commented that the need for education about new modalities goes beyond review panels; it can be hard to assess the strengths and weaknesses of new approaches due to their very newness. Dr. Ted Zheng, NIAMS program director, added that NIAMS and the National Institute on Aging are partnering on a Notice of Special Interest (NOSI) that encourages non-animal models, such as tissues-on-a-chip, in osteoarthritis research.

In response to a question from Dr. Maria Suarez-Almazor, Dr. Criswell expanded on NIAMS' new approach to select pay. The goal is for select pay decisions to take place over the course of the fiscal year after each Council round. NIAMS is developing a metrics team to track the impacts of the new policy and other NIAMS funding approaches. Dr. Criswell reviewed the process by which select pay decisions are made, which takes place after applications have been scored by CSR and assessed against the payline by NIAMS program officers. Program officers will then nominate applications of merit that address NIAMS priority areas but scored above the payline. Funding decisions are always difficult, particularly due to budget constraints. Dr. Criswell noted that NIAMS receives approximately 3% of all NIH R01 applications, but its budget only represents roughly 1.5% of the NIH budget. The select pay model allows NIAMS greater flexibility beyond relying solely on CSR scores. Dr. Suarez-Almazor suggested that

NIAMS publish a list of its considerations for select pay to foster greater transparency about this funding policy. Dr. Criswell said a description of the general consideration is available on the NIAMS website. Dr. Jinoos Yazdany asked what percentage of the NIAMS budget will be devoted to select pay under the new structure. Dr. Criswell replied that select pay will account for approximately 20% of the competing RPG budget. Dr. Yazdany also asked if NIAMS has determined a point in time where it will reassess the select pay model based on the metrics. Dr. Criswell said she expects that to take place internally at each Council round. Dr. Valentina Greco encouraged NIAMS to take steps to ensure select pay decisions are being made equitably, and thus ensure that those decisions match the values of NIH and NIAMS. Dr. Criswell agreed and noted that an applicant being part of an under-resourced institution is a factor that can be considered in select pay.

IV. PATIENT ENGAGEMENT PRESENTATION

Dr. Bob Carter, NIAMS Deputy Director, introduced the panel session on patient engagement. The first presenter was Ms. Suzanne Schrandt, a former NIAMS Advisory Council member and a patient engagement activist with a background in health and disability policy and law. She is founder and CEO of ExPPect and serves on the FDA's Patient Advisory Committee, and previously worked for the Patient-Centered Outcomes Research Institute (PCORI) and Arthritis Foundation in engagement leadership roles.

Ms. Schrandt began her presentation by homing in on the definition of patient engagement. She posited a dual definition that includes both personal engagement, which is the more commonly understood definition, and system-level engagement. Personal engagement refers to patients being engaged in their own care and/or participation in clinical trials, whereas system-level engagement is patients being engaged at an organizational level to improve diagnosis, care, and research generally. Both types of engagement are important and intertwined; system-level engagement is necessary to widely implement personal patient engagement practices.

To help answer the question of why patient engagement is important, Ms. Schrandt highlighted a study from 2018 which showed that compared to an investment of $100,000 in patient engagement, the net and expected present value can exceed 500-fold the investment. Patient engagement led to these savings by reducing protocol amendments and preventing trial failure due to lack of recruitment and retention, among other factors. Patient engagement and involving patients in study teams can lead to improvements and efficiencies throughout the clinical trial cycle and translation to the clinic. Ms. Schrandt discussed some of the key aspects of incorporating patient engagement best practices into research, such as training of staff and patients in engagement basics, training of patients in science and research basics, incorporating an engagement facilitation framework into trials, and evaluating the impact of engagement throughout the trial. She closed her remarks by pointing to PCORI's Foundational Expectations for Partnerships patient engagement rubric for those interested in further guidance.

Dr. Carter then introduced Dr. Peter Merkel, a rheumatologist and researcher from the University of Pennsylvania. Dr. Merkel is the PI of the Vasculitis Clinical Research Consortium (VCRC)

and the Vasculitis Patient-Power Research Network (VPPRN). He focused his presentation on the investigator's perspective regarding successful patient engagement in clinical research.

Dr. Merkel opened by noting that the importance of patient engagement has become much more widely accepted in recent decades, and the definition of engagement has expanded. Engagement has moved beyond engaging to improve recruitment to fully including the patient perspective in research prioritization, study design and implementation, data analysis, and dissemination of results. Dr. Merkel believes that engaging patients in research improves the quality of the results. He highlighted the important role that PCORI has played in changing the paradigm around patient engagement in recent decades through its outreach, guidance, resources, and direct research funding support.

Dr. Merkel discussed the evolution of his patient engagement-focused research in vasculitis, which dates to grants he received from PCORI in 2012 and was fully launched in 2014 when the VPPRN was established. One important lesson is that patient engagement does not take place overnight. Dr. Merkel stressed the importance of setting reasonable expectations about the level of engagement and the speed of research progress and clarifying roles. Researchers should avoid taking a one-size-fits-all approach but should tailor their engagement strategy based on the nature of the study. Dr. Merkel discussed a qualitative analysis that he produced from 2019 that supported the feasibility and benefits of direct engagement of patients as partners in clinical research. He also noted that several patients and patient advocates were co-authors in this study.

Dr. Merkel provided some examples of research efforts with successful patient engagement, including a study that resulted in the first validated patient-reported outcome in vasculitis and the ongoing research partnership between the VPPRN, VCRC, and the Vasculitis Foundation. Dr. Merkel highlighted their practice of allowing the patient community to select the patient research partner (PRP) for each study. Thus far, the VPPRN has completed 32 studies and produced 27 publications, each of which has a PRP as co-author.

Dr. Carter next welcomed Ms. Amye Leong, an internationally recognized patient advocate, health communications specialist, patient engagement leader, and president and CEO of Healthy Motivation. Ms. Leong is a former NIAMS Advisory Council member, and has been diagnosed with rheumatoid arthritis, Sjögren's disease, and osteoporosis. Among other career activities and achievements, she has served as an advisor for many federal boards and programs and was awarded the President's Service Award by President George W. Bush.

Ms. Leong focused her presentation on current patient engagement implementation strategies. Like the other speakers, she emphasized the importance of PCORI as the primary federally funded agency focused on patient-centered outcomes and their requirement that patient engagement be included in their funded awards. Investigators must determine how to identify and educate patient partners so that this relationship can be beneficial for all parties. Ms. Leong also highlighted the FDA Center for Devices and Radiological Health's (CDRH) Patient Engagement Advisory Committee and international patient engagement models, most notably OMERACT and EULAR/ACR. National nonprofits—such as the National Health Council, FasterCures, and the Center for Patient Advocacy Leaders—also provide valuable patient engagement guidance and resources. Ms. Leong also noted that for-profit health consulting firms

are starting to play a role in this space, and preliminary work is underway that could one day lead to a formalized certification for patient engagement practices.

Ms. Leong also discussed opportunities to systemically adopt patient engagement strategies within NIAMS programs. As Dr. Merkel indicated, engagement strategies have to be tailored to specific studies based on the disease, patient population, geographical region, and other factors. NIAMS has a role to play in fostering bidirectional training and education for both investigators and patients.

Discussion

Dr. Carter opened the floor to the Council and invited members to consider how NIAMS can encourage the inclusion of patient engagement practices in NIAMS-funded clinical research, e.g., through writing such expectations into grant awards. Dr. Teodora Staeva argued that the expectation should be included in NIAMS grant applications to ensure engagement is front-and-center and not an afterthought. She believes that researchers have sufficient resources available to them now, through the sources mentioned by the panel and elsewhere, that this would not create an undue burden. Ms. Ann Elderkin agreed and added that NIAMS could play a leading role in the training realm. She would also like to see more analysis of what concrete engagement activities are successful and, just as important, what approaches do not work. Dr. Criswell expressed her agreement that NIAMS should proactively express expectations on this topic, but also stressed her belief that there should be robust support to minimize burden and facilitate ease of adoption for research teams. As one idea, NIAMS could attempt to gather and distill existing best practices, guidance, and resources. Dr. Merkel commented that it might not be appropriate to make patient engagement a blanket requirement because there are study types where it might be of limited utility. Ms. Leong suggested that NIAMS could develop a pool of patients who are interested in serving as patient research partners based on research interests, geographic areas, etc. Ms. Schrandt recommended that NIAMS start with a patient engagement-focused pilot grant and use lessons learned from that to inform future expansion.

Dr. Karl Jepsen noted that making engagement a requirement could negatively impact institutions with limited resources. He asked the panelists to comment on the patient engagement resource burden and what NIH can do to ameliorate that. Ms. Schrandt said that PCORI incorporates engagement costs into their grants and provides education and assistance regarding how to reach patients.

V. CLINICAL TRIALS WORKING GROUP PRESENTATION

Dr. Carter invited Dr. Joan Bathon to present the findings and recommendations of the Council's Clinical Trials Working Group (CTWG). The CTWG was co-chaired by Dr. Bathon (a former Council member) and Dr. Stuart Goodman (a current Council member) and met 18 times. The working group was charged with providing feedback to Council on how NIAMS can optimally solicit and fund the highest impact clinical trials across the Institute's mission areas. Dr. Criswell also asked the working group to consider a number of questions related to trial impact, investigator and community engagement, and trial budget and size considerations.

The CTWG delivered interim recommendations to improve existing trial funding mechanisms to the Council in August 2023, which the Institute has responded to by developing and publishing a new R61 small exploratory trials Notice of Funding Opportunity (NOFO) and phased clinical trials UG3/UH3 NOFO. Dr. Bathon presented the working group's final recommendations, starting with the topic of trial impact. The working group recommended to Council that NIAMS consider shifting its strategy from funding a maximum number of trials to funding the most impactful trials. Additionally, NIAMS should prioritize trials that emphasize the impact of an intervention, taking effect size and disease burden into consideration, at both the individual and population levels. Thirdly, NIAMS should solicit input from relevant professional and patient communities on what they see as the biggest unmet needs. The CTWG also strongly felt that NIAMS should consider formally engaging the Advisory Council as a second level of review to assess trial impact, since study section review is primarily focused on scoring the science and methodology. The working group suggested that Council review occur for trial applications of greater than $500,000 per year and take place at closed sessions of Council meetings.

The working group was asked to consider NIAMS' niche in the broader clinical trials landscape. The CTWG felt that NIAMS' niche, given its relatively small budget, should be focused on disease areas not significantly targeted by industry. They also recommended that the Institute prioritize trials that aim to influence clinical management and guidelines and should consider a selection process with specific criteria for game-changing research in its NOFOs.

On the topic of engagement with the extramural community, the CTWG believed that NIAMS should seek input from stakeholders on research topics to prioritize, identify and publish areas of interest, and support investigators pursuing novel approaches to confront research gaps. Dr. Bathon acknowledged that NIAMS does many of these activities already, but the working group believes it would be beneficial for them to be more formalized.

The CTWG also developed findings in response to the question of how NIAMS can better support applicants for clinical trial grants. These include NIAMS developing and promoting educational initiatives, encouraging communication with NIAMS program officers, holding informational webinars for new clinical trial NOFOs, and providing resources such as data tools and guidance on funding partnerships to investigators.

The working group also recommended that NIAMS expand the scope of the AMS Clinical Trials Review Committee to include other study mechanisms and to define and expand the role of program staff in clinical trials oversight. NIAMS should also continually work to evaluate its trials portfolio to identify the most impactful trial designs.

Dr. Bathon then presented the CTWG's recommendations regarding trial budget considerations. The working group agreed that NIAMS should continue to fund a broad mix of clinical trial mechanisms. The working group recommended to Council that NIAMS consider re-issuing a clinical trial planning grant that focuses on stages earlier than the UG3 mechanism and includes potential impact on clinical care or public health as a review criterion. The CTWG also discussed in depth recommendations regarding whether NIAMS should explore establishing a clinical trials network. While the working group agreed that efficiencies could be gained by such a network, they were cognizant of the significant costs and the Institute's current budget situation. Thus, the

working group recommended that NIAMS consider adopting components of existing networks that would provide some economy of scale benefits to investigators.

Dr. Bathon detailed the working group's recommendations related to increasing diversity in clinical trial enrollments, which included developing and maintaining a resource of research sites with community engagement experience for grantees to partner with, providing training to Scientific Review Officers on how to help reviewers assess applications on this level, and publishing guidance and best practices for the research community. The CTWG also made recommendations calling for increased engagement of the patient community in trial design, implementation, and dissemination of results.

Discussion

Dr. Criswell thanked the working group members for their hard work on this important undertaking and for considering NIAMS' budgetary circumstances, which will make the recommendations more actionable. She discussed her participation on the NIH Clinical Trials Stewardship Task Force, which recommended that NIH develop centralized clinical trial support resources to support smaller Institutes and Centers (ICs). Dr. Bertagnolli's interest in establishing an NIH primary care clinical research network could also provide opportunities for segments of NIAMS' mission areas. Dr. Carter said that the CTWG's recommendation to re-issue the planning grant is the most actionable of the recommendations. In his opinion, adopting the recommendations calling for community engagement in the design of FOAs and for a second-level Council review of applications would lead to big changes with uncertain outcomes.

Dr. Susana Serrate-Sztein, NIAMS Associate Director for Strategic Initiatives, asked for the CTWG members to comment on how to assess the potential impact of clinical trial applications, which the Institute has found to be challenging. Dr. Bathon acknowledged the challenge but noted reviewers should be looking for the trial's potential for changing clinical practice and treatment guidelines and any direct link to improvements in patient outcomes, among other factors. The recommendation for Council review of larger grants is intended to assist in the assessment of impact. Dr. Staeva said that the lupus patient community is interested in studies of lifestyle interventions, which could be a fruitful area for partnerships, for example, with the National Center for Complementary and Integrative Health (NCCIH). The Council discussed further with NIAMS leadership the importance of collaboration and co-funding. Dr. Criswell said a crucial component of this is to make sure investigators do not feel like they must do all the work to find partners by themselves. Dr. Shanthini Kasturi mentioned the challenges she is facing in recruiting diverse participants in a single-site pilot study she is currently running and said the recommendations about providing diverse enrollment resources would be very useful for investigators in similar circumstances.

VI. CONCEPT PRESENTATION

Dr. Sledjeski reminded the Council that concept clearance is the planning process by which NIAMS receives feedback on the merits of a proposed concept and approval from the Advisory Council.

Dr. Mary Garcia-Cazarin, Deputy Director, Division of Extramural Research, presented one concept to the Council titled Resource-based Centers for Rheumatic, Skin, and Musculoskeletal Disease Areas. The goal of the program is to promote cooperative interactions among investigators and improve access to shared administrative and scientific resources cores. The program also supports pilot and feasibility projects of up to three years in duration. This concept is a reissuance, and Dr. Garcia-Cazarin highlighted the program's success in facilitating team science. Dr. Christopher Mecoli said he strongly supports the P30 mechanism.

The Advisory Council voted unanimously to approve the concept.

VII. NIAMS STRATEGIC PLAN

Dr. Stephanie Burrows, Chief, Scientific Planning, Policy, and Analysis Branch, presented to the Council on NIAMS' progress thus far and next steps in the development of its FY 2025-2029 Strategic Plan. Every NIH IC is required by statute to maintain a strategic plan, which is the Institute's public statement of interest and serves as an internal reference for staff and a guidepost for the extramural research community. Dr. Burrows noted that the FY 2025-2029 plan will be the first issued under Dr. Criswell's tenure and will therefore be of particular interest to the community.

Dr. Burrows provided a brief overview of the strategic planning timeline, which began in mid-2022 and will culminate in the presentation of the final plan to Council at the September meeting later this year. The process began with the issuance of a Request for Information (RFI) in July 2022 that sought feedback on cross-cutting themes and bold aspirations for the Institute to pursue in the coming years. The RFI received 46 responses, primarily from individuals in academia and from professional and voluntary organizations. Last September, NIAMS held a multi-day idea generation meeting with stakeholders from across the community to crowdsource additional suggestions for cross-cutting themes and discuss those topics in greater depth. NIAMS subsequently identified nine themes with corresponding bold aspirations and issued a second RFI for feedback on those themes, which received 25 responses.

NIAMS has delineated four overarching goals for the draft strategic plan: identifying and testing interventions, developing resources, communicating advances, and supporting infrastructure, team science, and a diverse workforce. The draft plan articulates six strategic research priorities based on feedback from the stakeholder community: molecular and cellular mechanisms in health and disease; regenerative medicine; data science, artificial intelligence/machine learning, and computational biology; behaviors and environmental exposures; translating findings into interventions; and health disparities and health equity. The plan also identifies several capacity-building and operational priorities related to research infrastructure, interdisciplinary research, workforce training, and communication.

NIAMS will be issuing a third RFI this summer to solicit additional comments on the draft strategic plan, which will incorporate Council member feedback. This version will also include two appendices on accomplishments related to the previous strategic plan and an overview of the

Institute's implementation plans. The final FY 2025-2029 Strategic Plan is on track to be released by the end of the calendar year, following its review by the Council in September.

Discussion

Dr. Criswell thanked NIAMS staff for their great work throughout the strategic planning process, and particularly lauded their efforts to gather and incorporate feedback from the broader NIAMS community. Dr. Jepsen said he would like to see a greater emphasis on personalized diagnostics and treatments, particularly in a way that is more proactive than reactive. Dr. Criswell suggested this might be a comment that could be reflected in the plan's bold aspirations. In response to a question from Dr. Edward Botchwey, Dr. Criswell affirmed that the plan's research priority on health disparities will take into account research into social determinants of health, environmental stressors, and related factors. Dr. Tamia Harris-Tryon said she was pleased to see behavioral and environmental exposures included as priorities in the plan. Dr. Criswell said that was one of the prominent themes from the idea generation meeting, along with the need for research infrastructure support.

Dr. Staeva asked if the Institute's ambitions to increase patient engagement and the recommendations from the CTWG will be formally included in the strategic plan. Dr. Burrows said staff will work to incorporate Council comments prior to the release of the draft plan in conjunction with the third RFI. Dr. Aimee Payne, University of Pennsylvania, encouraged NIAMS to explore ways to co-invest in training opportunities for clinical investigators with industry and other government partners. She also cautioned the Institute to not become too overly proscriptive in its assessments of human relevance because significant scientific discoveries can often come from unexpected places.

VIII. OPEN DISCUSSION

Dr. Criswell opened the floor for the remainder of the open session for discussion of topics of the Council's choosing. Dr. Greco returned to the topic of the Council having a larger role in evaluating grant applications and assessing impact of proposed clinical trials. She said NIAMS would have to consider what the appropriate composition of the Council would be to perform the task if the Council were to take up that responsibility. The size of the Council is set by congress. Dr. Criswell noted that membership levels have been constrained in recent years due to approval backlogs at the departmental level. This situation is improving, however, and NIAMS expects up to seven new Council members to formally join by the September meeting. A great deal of thought is put into making sure the Council is comprised of representatives from a broad and diverse array of the community. Dr. Carter added that the more granular assessment of the science is performed at the study section level; the purpose of a Council-level review would be to assess the broader impact, and thus would not necessarily require finer-grained expertise. Dr. Criswell described the Council review as participating in the assessment process that is already being performed by NIAMS leadership in conjunction with program officers following study section review and scoring. Dr. McNally commented that the Council rarely has members with experience in industry, which might be an important perspective to have in this context. Dr. Yazdany suggested that the Institute test out the Council review in a pilot manner to see how it

goes in practice before committing to the change. Dr. Staeva suggested the Institute also consider establishing a patient advisory board to provide input on impact assessments.

Dr. Botchwey encouraged NIAMS to maintain its emphasis on rare disease research, conditions that often lack attention and funding. Dr. Criswell acknowledged NIAMS' history and reputation for being supportive of rare disease research, and that will continue to be a focus for the Institute, particularly in the Intramural Research Program.

IX. CLOSED SESSION

This portion of the meeting was closed to the public in accordance with the determination that it concerned matters exempt from mandatory disclosure under Section 552b(c)(4), Title 5, U.S. Code and Section 10(d) of the Federal Advisory Committee Act, as amended. Members exited the meeting during the discussion and voting process on applications from their own institutions or other applications that presented a potential conflict of interest, real or apparent. Members signed a statement to this effect.

X. REVIEW OF APPLICATIONS

The 114th National Arthritis and Musculoskeletal and Skin Diseases Advisory Council met on May 29, 2024. Six Council members and three Ad-Hoc Council members attended in person and two attended virtually. En bloc concurrence was unanimously approved for 844 primary and 170 secondary applications. The total cost requested in year -01 for all applications was $445,216,943.

XI. ADJOURNMENT

The 114th National Arthritis and Musculoskeletal and Skin Diseases Advisory Council adjourned at 4:00 p.m. Proceedings of the public portion of this meeting are recorded in this summary.

I hereby certify that, to the best of my knowledge, the foregoing summary and attachments are accurate and complete.

Darren D. Sledjeski -S
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Date: 2024.07.22 13:12:56 -04'00'

Darren Sledjeski, Ph.D.
Executive Secretary, National Arthritis and Musculoskeletal and Skin Diseases Advisory Council
Director, Division of Extramural Activities
National Institute of Arthritis and Musculoskeletal and Skin Diseases

Lindsey A. Criswell -S
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Date: 2024.07.22 14:28:11 -04'00'

Lindsey A. Criswell, M.D., M.P.H., D.Sc.
Chair, National Arthritis and Musculoskeletal and Skin Diseases Advisory Council
Director, National Institute of Arthritis and Musculoskeletal and Skin Diseases