

HEALTH RESEARCH ALLIANCE MEMBERS' MEETING - MARCH 23-24, 2017 ACTION ITEMS AND TAKE AWAYS FOR HRA MEMBERS



SESSION 1: SUCCESSFUL IMPLEMENTATION OF OPEN SCIENCE AND DATA SHARING

Speakers:

[Bon Grossman, PhD](#)

Chief Research Informatics Officer | Biological Sciences Division | University of Chicago

[Salvatore La Rosa, PhD](#)

VP Research and Development | Children's Tumor Foundation

[Justin Guinney, PhD](#)

Director, Computational Oncology | Sage Bionetworks

Big picture questions:

- Why have an open data policy?
- What are data repositories and what data are they designed to contain?
- What is an open data "commons" and what data is it designed for?

Big Data Problems:

- Lack of statistical support
- Unclear how and where to share
- Accessing and reusing data

Big Data Benefits:

- Accelerates research and increases funding impact
- Can provide the critical mass of data to get required statistical power
- Supports repeatable, reproducible, and open research
- Allows for working with large datasets at a lower cost
- Broader societal impact – outcome data shared, data from 1 disease can impact other diseases
- Can build networks of researchers and build a network of networks

A "Data Commons" needs:

- Guidelines to address the tension between researchers needing data for their careers vs data getting out to accelerate research
- Governance to set up and monitor those guidelines

Action or Guidelines for funders:

- ***Remind the researcher that THEY will benefit from others' data***
- ***Implement data sharing policies – specifically require that funded researchers share data***
- ***Set clear guidelines and expectations for what is meant by data sharing***
- ***Put in place mechanisms for oversight and enforcement of data sharing practices***
- ***Provide data commons and bioinformatics support needed for data sharing***
- ***Look into "Open Commons Consortium" which can be used to set up specific data commons***
- ***Ensure that the data commons funded by or recommend by funder can interoperate or "peer"***
- ***Plan for a future when patients can contribute their own data to a commons, though this is not an issue now***

SESSION 2: DEMYSTIFYING INDIRECT COST IN FUNDER AGREEMENTS

Speakers:

[Anita Pepper, PhD](#)

VP of Development | Wistar Institute

[Sally O'Neil](#)

Director, Industrial Contract Office | Stanford University

[William Chambers, PhD](#)

Senior Vice President, Extramural Research | American Cancer Society

[Louise Perkins, PhD](#)

Chief Science Officer | Melanoma Research Alliance

Chris Percopo, MPA

Director of Grants Management | Helmsley Charitable Foundation

Big Picture of Indirect Costs:

From the university perspective, Indirect Costs are really reimbursement for expenditures. Universities only recover a portion of their annual IDCs. The rest they must make up. Stanford (who has a 57% IDC rate) uses tuition, their endowment, and gifts to make up the difference.

From the researcher perspective, since only 69 cents of the dollar goes to direct costs and 31 cents on the dollar goes to indirect costs many researchers consider this is a tax.

From the funders' perspective, there are many different IDC strategies. A very large number of foundations pay zero indirects yet a small number pay up to 55%.

Specific examples:

- The American Cancer Society pays 20% Indirect Costs (or F&A) on their R01-like grants. ACS believes that it is appropriate and important to do this because many of these costs are necessary for research. However, their awards do demand a commitment by the institution.
- The Melanoma Research Alliance does not pay IDC upfront but they do pay at the end. Thanks to other revenue streams, they have the ability to make a powerful statement to donors that 100% of their funds go to the direct conduct of research. Even administration and meetings etc. come fund the founders' money and corporate sponsorship.
- Helmsley acknowledges that there is a need to clarify and define what an institution defines as indirect direct costs as these vary by institution. Because of this, Helmsley works with institutions individually with respect to this issue.

Action items for HRA and HRA Members:

- ***Be able to make the case that nongovernmental nonprofit funding has significant value beyond the money. Quantification of the value that foundation dollars bring will make universities and researchers seriously consider nonprofit dollars with low indirects when choosing between NIH and the nonprofit funder.***
- ***Increase transparency in what universities charge and what funders will pay for.***
 - ***What are the actual costs of doing research buried in the term "indirect costs"?***
 - ***Can HRA and universities work together to identify those costs?***
 - ***If so, how will that information change the initial budget, financial reporting, etc?***

SESSION 3: ADDRESSING INEFFICIENCIES TO ACCELERATE FUTURE CLINICAL TRIALS

Speakers:

[Stephen Joel Coons, PhD](#)

Executive Director | Patient-Reported Outcome Consortium Critical Path Institute

[Julie Fleshman, JD, MBBA](#)

President and CEO | Pancreatic Cancer Action Network

[Mary DeRome](#)

Director Medical Communication and Education | Multiple Myeloma Research Foundation

The Patient-Reported Outcome (PRO) Consortium

This is the Critical Path Institute's (C-PATH) approach to the qualification of clinical trial endpoint measures; established to qualify and maintain patient-reported outcome (PRO) instruments and other clinical outcome assessment (COA) tools that will be available for use in clinical trials where COA-based endpoints are used to support product labeling claims.

Goal of the consortium include:

- Enable pre-competitive collaboration that includes FDA input and expertise
- Develop and obtain FDA qualification of PRO measures and other COA tools for use in assessing primary or secondary clinical trial endpoints
- Avoid development of multiple endpoint measures for the same purpose
- Share costs of developing new endpoint measures
- Facilitate FDA's review of medical products by standardizing COA-based endpoint measures that will be publicly available

Pancreatic Cancer Action Network

PanCan has two programs that address issues with clinical trials to improve patient outcomes:

- Know Your TumorSM – precision medicine service that led to 48% actionable alterations
- Precision Promise - precision medicine platform clinical trial designed specifically for pancreatic cancer patients (Patient centricity and iterative between science and medicine are 2 key principles)

Multiple Myeloma Research Foundation

- The MMRF and its clinical consortium, the Multiple Myeloma Research Consortium (the MMRC) combine to speed new trials to MM patients, and work in collaboration with many partners in validation and approval of new clinical trial endpoints.
- MMRF uses an end-to-end Precision Medicine model to accelerate MM drug development through their translational network, innovative trial designs, and novel endpoints like MRD. MMRF is helping lead efforts for approval of Minimal Residual Disease testing as a clinical trial endpoint in MM
- MMRC includes academic centers, includes over 70 Phase I-Phase IIb trials, more than 30 different agents, I-MAP Initiative – Immune-Oncology Myeloma Accelerator Program (ongoing project on validating immune endpoints for clinical trials)

Action items for HRA and HRA Members:

- Measuring safety is important but even more important is the ability to assess patients' tolerability (especially with respect to tolerating toxic drugs) within clinical trials
- Read the recommendation from the "Second Annual [Workshop on Clinical Outcome Assessments](#) in Cancer Clinical Trials"
- Review 'Guidance for Industry and FDA Staff: qualification Process for Drug Development Tools.' Describes CDER's drug development tool qualification process; including biomarkers, animal models, and clinical outcome assessment tools.

Session 4: **CONVERGENCE: PLATFORM TECHNOLOGIES TO ACCELERATE LIFE SCIENCE DISCOVERY**

Speakers:

[Marcia McNutt, PhD](#)

President | National Academy of Sciences

[Maria Pellegrini, PhD](#)

Executive Director of Programs | W.M. Keck Foundation

[Jennifer Hall, PhD](#)

Chief, AHA Institute for Precision and Cardiovascular Medicine | American Heart Association

[Chris Martin, PhD](#)

Science Program Officer | The Kavli Foundation

Convergence research was defined as:

“The integration of engineering, physical sciences, computation, and life sciences in order to bring about profound benefits for health, energy, and environment.”

- Convergence involves solving problems not testing hypothesis.
- Yes, convergence research is “solutions-oriented” but solutions are often difficult to evaluate or review
- It can be risky and usually lacks preliminary data

Both the National Academy of Sciences National Science Foundation have prioritized convergence in research and have stated that convergence is needed to solve Grand Challenges in Engineering in 21st Century.

Some of the relevant NAS actions:

1. NAS has published a book entitled:

[Convergence: Facilitating Transdisciplinary Integration of Life Sciences, Physical Sciences, Engineering, and Beyond \(2014\)](#)

It contains practical strategies to support convergence including specific recommendations for funders including:

- Identify problems that would benefit from convergence
- Address barriers
- expand funding mechanisms
- Collaborative proposals review when needed

It also includes advice for other organizations, including how to avoid patchwork of isolated efforts.

2. NAS and Kavli convened the “Convergence Summit” (2015)

Take homes from that meeting:

- Thematic programs & centers facilitate exploring topics at the intersection of disciplines
- Convergence also happens spontaneously and without central planning
- Shared/core facilities can support convergence
- Seed funding and/or physical space can initiate convergence projects
- Sabbaticals can provide convergence opportunities for faculty
- As can “detailee” programs to federal agencies
- Establish regional Centers of Excellence in convergence
- Would be helpful to survey existing university investments in convergence (recruitment packages, seed funding, research facilities, cross-educated staff...)

Action items for HRA and HRA Members:

From Dr. McNutt:

Provide funding for non-project costs that promote convergence

- Fund **convergence social events** (coffee, pizza) for presentations and discussions
- Sponsor **journal clubs** to address convergence themes
- **Foster informal faculty gatherings** with shared interests in convergence problems
 - May assist discussions in advancing convergent candidates for faculty positions
- Encourage donors to establish endowed chairs that require **joint appointments** across departments/schools
- Fund **redesign of spaces** to promote convergence collaborations
- Support **executive-in-residence programs** to bring insights from practitioners in industry
- Provide infrastructure for **collaboration at a distance** for faculty from different institutions and areas of science
- Support research on **performance measures** for convergent efforts
- Support sabbaticals for **convergence curriculum development**

More from the Discussion:

- *HRA members who fund convergence should compare notes on what worked and what didn't in reviewing convergence.*
- *Funders of convergence programs should together in a workshop to compare review processes and come up with best practices for reviewing convergence proposals - for instance does anyone use a non-consensus-based review process?*
- *HRA should standardize resubmitted proposal guidelines. The barrier should be very low for someone to resubmit from one org to another.*
- *HRA members who fund convergence should sharing of reviewers in the expert areas*

Keck:

Characteristics of Convergence

- Problem focused and hypothesis not needed.
- Youth is key (Early career investigators, post-docs, graduate students)
- Dull and/or tedious experiments
- Difficult to evaluate and review
- Can be risky/lack preliminary data

Take home for HRA Members:

Process for choosing and training reviewers is critical:

- *Interdisciplinary research has consistently lower funding success*
- *Reviewers need to understand your funding priorities*
- *Divided opinions may be expected*

AHA

Convergence means:

- A world where patient data was being shared by a million people 24/7
- Data sharing is the cultural norm creating unprecedented collaborations
- New and more sophisticated tools and analytics are used to look at data

Kavli

The BRAIN Initiative (Brain Research through Advancing Innovative Neurotechnologies) is an example of a public-private partnership supporting convergent research.

- 6 federal organizations (NIH, DARPA, IARPA, FDA, NSF and DOE)
- 4 nonprofit funders (Kavli, Allen, HHMI, Simons)
- Out of the 576 FY16 NIH BRAIN awardees only ¼ were from neuroscience
- Over 160 publications have emerged from NIH-funded BRAIN grants to date

SESSION 4: PRIVATE PHILANTHROPY AND BIOMEDICAL RESEARCH: FRESH PERSPECTIVES FROM A NEW GENERATION OF DONORS

Speaker:

[Valerie Conn](#)

Vice President | Science Philanthropy Alliance

What SPA does:

The Science Philanthropy Alliance's focus is exclusively on basic research and helping to increase the basic science funding in a philanthropists portfolio of grants.

What SPA sees:

- The new science philanthropists are applying what they know from their businesses to their philanthropy and she anticipates this interdisciplinary approach will continue.
- They are investing in technology, in data collection and smart analysis to accelerate basic research.
- Philanthropists are supporting research by making traditional grants but they want to have open data to speed up research.
- They are interested in creating partnerships to advance basic research – they seek the best places for their investments, the best scientists, the best organizations, the best ideas. (Not their alma mater or their community, as in the past).

Examples:

At the Chan Zuckerberg Initiative: they recently hired a Chief Technology Officer, former Amazon executive Brian Pinkerton. Brian is helping CZI support engineers and data scientists who will work hand in hand with the scientists.

At the Allen Brain Institute: they invested to scale up the production of large data sets. ABI generates large scale open data sets – their brain map -- for sharing data that are produced beyond the magnitude of previous other research institutes.

Recommendations for HRA and HRA Members:

- Offer an easy way for them to partner with you, to establish a collaboration
- Think long-term with your initiatives, and the role that basic science can play.
- Getting to know other colleagues; the Health Research Alliance is a great way for you to create partnerships that will facilitate co-investments in technology and data sharing
- They want to fund the best, so HRA members have a real opportunity to appeal to these philanthropists, but you need to tell your story in a compelling way
- Have a basic science story - Don't be afraid to say that you are working hard to solve problems
- Check out the [SPA website](#)
- Sign-up for SPA's enewsletter

SESSION 5: PUTTING GRANTS DATA TO WORK: PRACTICAL APPROACHES TO EVALUATION

Speakers:

[Katie Hickling](#)

Product Manager | PLoS

[Micah Moughon](#)

Research Information Manager | American Heart Association

[Kari Wojtanik, PhD](#)

Sr. Manager, Evaluation & Outcomes | Susan G. Komen

[Rachel Witsamen MPH, PMP](#)

Program Officer | PCORI

PLoS Article Level Metrics (ALMs):

Provide metrics and data about research articles on the article level. ALMs enable you to evaluate levels of attention- both traditional and otherwise. ALMs include views and downloads, several citation counters, social media trackers including twitter and facebook, Wikipedia mentions, Mendeley saves and f1000 recommends, research blogs, and more.

Qualitative data is also made available on every article via a news coverage feed, post publication commenting and PLoS's tweetstream. PLoS also makes their database available to anyone via an open API and via our reporting application, alm reports.

ALMs give funder (and others) more information earlier (before traditional citations). They give granular information which is clearer than scores, roll ups or averages. Multiple sources show where attention is gaining

Best to use in conjunction to qualitative factors, such as peer review

Data should be available from metrics providers for analysis

AHA:

Uses Elsevier products Pure, SciVal, and Scopus to curate publications and utilized natural language processing to match publication abstracts to project summaries.

Two golden rules of evaluating Research Output:

- Always use qualitative and quantitative metrics.
- Always use at least two quantitative metrics together.

Research Output Metrics used by AHA:

Publications

Citations

Field Weighted Citation Impact

Collaboration

Subsequent NIH Funding

Patents

Komen:

Uses an Internal Classification/Tracking System

Classifies grants by:

1. Product potential
 - a. target names
 - b. associated research resources
2. Stage in research pipeline – (basic, preclinical, clinical, FDA review, approved)

They will use the data to answer:

- How are we progressing towards reaching their goal?
 - How many projects have moved from Basic or Preclinical Research to Clinical Trials?
 - Are Komen-funded “products” in clinical use and contributing to reductions in mortality?
- Identify promising technologies, treatment strategies, etc. that have immediate potential for further development or commercialization?
- Share progress and metrics with stakeholders

PCORI:

Measures along a continuum: From Dissemination to Use to Impact

Dissemination

- Results reported to study participants
- Access to PCORI Study Report
- Presentations (scientific and lay)
- Bibliometrics (pubs, time to publication, impact factor, citations)
- Alternative metrics for specific groups (#downloads, #bookmarks, media coverage, social media)

Uptake and use

- Adoption of study findings in the study setting
- Incorporation into:
 - Patient and consumer education materials
 - Grad Medical Education or Continuing Medical Education
 - Practice guidelines
 - Decision Making infrastructure (electronic decision aids, clinical reference tools)
 - Payer policies
 - Institutional, local, state and national policy

Impact on Health Decisions or care and Outcomes (changes in the metrics below):

- Health Decisions
- Health care (quality of care, practice patterns and variation, disparities in care)
- Health Outcomes (functional status, morbidity, health-related quality of life, mortality)