HEALTH RESEARCH ALLIANCE
MEMBERS' MEETING SUMMARY

September 17-18, 2017

Sponsored by the Alzheimer's Association

Links to presentations are in the text but are also on the Members' Meeting Website

STRATEGIC PLANNING

Speaker 1: **Melissa Stevens, MBA**
Executive Director, Center for Strategic Philanthropy | Milken Institute
Title: Strategy in Action

Philanthropic investment in medical research has an outsized impact. It is 3% of total but has a much bigger impact.

- Philanthropy takes on more risk. It is much more nimble, quick, and flexible.
- Philanthropy is also patient and in it for the long haul.
- A robust strategic plan can increase success by getting all stakeholders on the same page in terms of defining and achieving commons goals

Steps to creating an effective Strategic Plan

1. Define Vision and Mission
   - Vision: Your end goal
   - Mission: How you go about achieving that goal
2. Understand the current state
   - State of the science (what do we know and what do we not know scientifically)
   - State of the system (what do we have and not have: funding, regulation, data, patients, human capital, tools)
3. Map your stakeholders
   - Talk to key opinion leaders in your space & in connected areas (patients, NIH, FDA, PPPs, Pharma, Investors, other Nonprofits, Tech, etc.)
   - Talk to industry partners downstream
   - Who else is funding in your space
   - Bring them all in the room at the same time
4. Identify unmet needs and set goals
   What are the "Gaps to Goals"
5. Find the best tool for the problem
   - What are the solutions?
     Funding *(grant or other investment solution),* Convening, Infrastructure, Policy, others
   - Consider:
     Strengths and weaknesses
     Financial resources
     Human capital
     Potential partners (leverage investment, maybe bring in others so you don't have to go it alone)
6. Measure what matters
   - Use both quantitative and qualitive metrics
   - Include patient-relevant outcomes
   - Know when to STOP
7. Walk the walk
   Actively use the plan to track progress and make decisions
   Update 3-5 years or as needed. Younger organizations should update more frequently.

Lessons learned:

Carefully message to your core constituents
Engage innovators from outside your field
Look for leverage
Be disciplined yet flexible

Speaker 2: Jackie Hausman, MPP, MPH

Program Officer for Health | Kenneth Rainin Foundation

Kenneth Rainin is a young foundation which is focused on collaboration, and getting new investigators in the field of IBD research. Their resources expanded very quickly and they needed to think strategically how they would spend these new resources which necessitated prioritizing goals and strategies. They started by asking key stakeholders where are the big gaps. Everyone they asked recommended they look at impacting the valley of death and translational science.

Kenneth Rainin's strategic planning process:

- Identify key opinion leaders (started with grantees, SAB, pharma partners)
  From there, they invited a large group (~30 people) with diverse representation of opinions and perspectives such clinical researchers, basic science researchers and other funders to participate in their process.
- They hosted a 1 day in-person retreat focused on:
  - what's currently going on
  - where are areas of need
  - what are successful models
- Jackie's key takeaways:
  - People bring their own agendas, and advocate for their own constituency. Even setting the agenda is difficult.
  - The more diverse voices are represented the greater the impact.
  - If possible, use outside facilitators.
  - Be open to the fact that the outcome may not be what you thought it should be – be open to change.

ACCELERATING TREATMENTS TO PATIENTS – CROSSING THE "VALLEY OF DEATH"

Speaker 1: Eric Schaeffer, PhD

Senior Director, Scientific Innovation, Neuroscience | Johnson and Johnson Innovation Center
Title: Accelerating Treatments to Patients: Bridging the Valley of Death

The Problem:
In 2015 there were 1.2 million medical papers but only 36% progressed to IND stage.
Phase II is the area where pharma invests the most money, because it is seen as the lowest risk for highest payoff. For pharma the bottle neck or their "valley of death" exists before Phase II, at the target validation stage, which has approximately a 1% probability of success. The low ROI means they need more targets.

J&J's solution = "J&J Innovation"

(1) Innovation Centers
(2) JLABS

1. Innovation Centers:

A high % of pipelines are external innovations. J&J is now not only focusing on taking Phase II innovations to development, but they are moving to bringing in early stage opportunities, even including preclinical.

J&J's "Innovation Centers" are the way they are accomplishing this. These are innovations hubs in San Francisco, Boston, London, and Shanghai (with satellites in San Diego, Russia, Japan, Australia, Singapore, and Israel.)
These are all looking at early stage research but in different ways.

In existing models, stakeholders have nonoverlapping roles.
Academia: basic biology
Pharma: develop drugs

In J&&'s model there are 4 groups of stakeholders with roles that capitalize on each's strengths
Pharma/Biotech: Precompetitive partnerships and innovations centers
ADNI is a good model. Best people from academia and industry work together to solve a problem.
External innovation centers and TTO's at universities work to bring innovations to the drug discovery arena
Academia: Drug discovery centers, tech transfer collaborating with industry. Academia can bring in expertise from pharma to develop high through put screens. Using expertise like medicinal chemists they can find the target and validate it which de-risks it for pharma.

Government: Willing to partner and invest with industry to foster/speed translational science
NCATS has a specific program to identify and repurpose compounds deemed safe but are just sitting on a shelf.

Venture/financial: Not into early stage or high risk – but collaboration de-risks for these organizations too
Venture philanthropy plays a major part in this effort.

Examples:
A. Wellcome trust consortium for neuroinflammation in Mood and Alzheimer's (NIMA)
Engage academia and pharma to understand the role of neuroinflammation in depression and AD by leveraging previous drug discovery success against immunological disorders.
UK universities with expertise in neuroinflammation
Pharma with compounds with efficacy in inflammation
B. JHU Brain Sciences Institute fosters early stage drug discovery.
Novel mechanism discovered by academic scientists pushed investigated by J&J Tool compound then target investigation and validation by academic scientists.

2. JLABS / Company incubation:
In San Diego, Bay Area, South San Fran, Boston, Toronto, New York, Houston
Incubation labs available for researchers. Open innovation and capital-efficient model. Researchers have access to a turn-key lab space. They share common equipment and are surrounded by other small companies with valuable expertise and access to J&J. They pay rent. A community of entrepreneurs with access to experts is created.
There are no strings attached but are designed to nurture early innovation. The requirement is good science and reasonable plan to get something to market. There must be a focused problem to work on and a clear deliverable.

Where do we go from here?
- Early Education
  - Integrate training in drug discovery as part of graduate programs, since many students will consider this path
  - Provide mentoring to academic investigators seeking to develop their discoveries (SPARKS, Stanford)
- Continue to foster new models
  - Government sponsored programs (NCATS, CTSC's)
  - Academic drug discovery units (Emory, Harvard, Vanderbilt, etc)
  - Pharma investment in experimental medicine studies
  - More investment by Pharma and VCs in early biotech
- More cross-sector collaboration
  - Public Private Partnerships (focused projects)
  - Better communication across "boundaries"

Summary: Sharing expertise and removing silos is needed to make progress.

Speaker 2: **Joel Braunstein, MD, MBA**

Co-Founder, President and CEO | C2N Diagnostics

Title: Accelerating Treatments to Patients – The Experience of an Academic Spin-out

C2N Diagnostics mission: Commercialize unique technologies to better detect, monitor, and treat Alzheimer's disease and other neurodegenerative disorders.

Academic spin out: public – private partnerships was critical to accelerate treatments to patients.
Location also critical. Set up company (management and scientific team) near WashU where the discoveries were made.

Business Strategy

Business Segments:

- Diagnostics – Preclinical detection / Therapeutic monitoring
- Disease-modifying therapeutics for neurodegeneration

Business Plan:

- Large-scale clinical validation studies of SILK™/SISAQ™ Assays
- GLP to CLIA to IVD and novel biomarkers
- First in man (FIM) study for lead therapeutic

Partnerships:

- Establishing innovative partnerships with;
- Pharmaceutical companies
- Diagnostic and analytical tools companies
- Vendors within the supply chain
- Disease research foundations
- Academic centers to accelerate commercialization of technologies.

Teams and Team Construction is critical

- Models are wide-ranging (Completely virtual to fully integrated teams)
- Strong compatibility necessary between founding scientists and business partners
- Technology transfer can be optimized with (i) hiring individuals who bring continuity to the project; and (ii) independent replication of data as soon as possible
- Great project managers are necessary to manage outsourced processes
- A good lawyer engaged early can save future trouble and costs
- Experienced operators derive satisfaction from providing mentorship and advisory support

Sourcing the capital

(Research foundations, Private Capital, Federal/local grants, strategic capital?)

- What unmet medical need does your technology address?
- Who has unique interest?
- What is your timeline?
- What are your capital requirements?
- Are you building a product or a company?
- What are your own goals?
- Beyond capital, what do you need?
- What is driving your prospective investor(s) interests?

Lessons Learned on Raising Capital

- Surround yourself with as many smart people as possible
- Know what you want before you ask investors
- Define your value milestones and assume accountability
- Be passionate and dispassionate at the same time
- Behind every investment champion, there is a critic

- Perception is reality to investors, so understand the perception
- Seek profitability as soon as possible this creates options
- Raising capital is not easy and it never really ends
- Evidence drives not only clinical adoption, but also investment and business development success

Take home: Expert Risk Management Drives Commercial Success
Consider risks in several important areas:
Technology, Market, IP, Regulatory, Financing. Management (see slides for details)

Member Speaker 1: Andrew Koemeter-Cox, PhD
Scientific Program Officer | Alzheimer's Drug Discovery Foundation
Title: ADDF ACCESS

ADDF Access: A resource for researchers to help improve and accelerate their drug discovery projects
CRO's – Contract Research Organizations can play a critical role
- Have assays and expertise
- Have pharmaceutical development expertise in drug development
- Outsourcing to CRO's can increase value of drug discovery programs to development partners

To help researchers find the appropriate CRO they developed **ADDF Access**. (www.AlzDiscovery.org/ACCESS)
A service powered by Science Exchange (www.scienceexchange.com). They had a static list initially, but partnering with SE increased power and service.

ADDF ACCESS is a dynamic list of CRO's and consultants with Drug Discovery experience powered by Science Exchange
- Matches scientists with CRO's, solicit quotes, manage project
- Library of resources including a guide to CNS drug discovery and development

Member Speaker 2: Melissa J. Nirenberg, MD, PhD, FAAN
Chief Medical Officer | The New York Stem Cell Foundation
Title: Bridge to Cure at NYSCF: (Avoiding the "Valley of Death")

NYSCF likes to use "Bridge to Cure" over the phrase "Valley of Death". Their strategies include:
- The new NYSCF Research Institute
- The NYSCF Global Stem Cell Array (robotic technology that automates and standardizes the production of stem cell lines from everyone and differentiated cell types affected by disease.)
- New CMO position (Melissa is inaugural CMO)

They use their stem cells and internal expertise to act as a catalyst to bring in expertise, funding, and institutional resources to bring therapies to clinical trial.

Member Speaker 2: Robert Sege, MD, PhD
Chief Medical Officer & Director | The Medical Foundation at Health Resources in Action
Title: Crossing the Valley of Death: Funder Options

TMF at HRiA takes 2 approaches to bridging the divide between basic research and market penetration (the "Valley of Death" or the product development stage.)

Approach 1: The Falk Foundation
Goal is to move good ideas from universities to commercialization.
- Catalyst award – 1 year 300K (benchmarks and milestones toward proof of concept)
- Transformational award – 2 year 900K each year only for successful catalyst awardees

Approach 2: The Charles H. Hood Foundation
The IRS code allows program related investments to be classified as charitable disbursement.
Hood invests in startups with child health focus – the first 3 were devices including oxygen monitoring for neonates, testing for HIV drug resistance, and inexpensive disposable neonatal incubators.

Presentation of Initial Analysis of the Inaugural Annual Member Survey

Speaker: **Maryrose Franko, PhD**
Executive Director | Health Research Alliance
Title: HRA Member Survey

The survey collection process as well as data on demographics, health research classifications and other data from 58 respondents was presented. In addition, detailed survey data about HRA members' Early Career Investigator grants (defined as research grants for junior faculty investigators to help establish their independent research programs) was presented. Of the 58 responders to the survey 36 have at least one early career investigator grants.

Alzheimer's Disease Biomarkers to Accelerate Clinical Development and to Improve Clinical Practice

Speaker: **Gil Rabinovici, MD**
Associate Professor of Neurology | UCSF Memory and Aging Center
Title: Alzheimer's Disease Biomarkers: Transforming Care and Drug Development

Dr. Rabinovici stressed the importance of the ability to measure plaques and tangles-not just during an autopsy but during life. Measuring plaques during life allows:

- Study of disease dynamics in humans
- Better diagnosis
- Early detection and intervention
- Improved drug trials

There are three strategies for measuring plaques and tangles during life:

- Fluid-Based biomarkers *(see Jim Hendrix's presentation)*
- Imaging Amyloid Plaques (PIB PET): the molecular imaging revolution
    - The A4 Study – to look at Amyloid PET in drug development.
    - IDEAS Study – to demonstrate Aβ improves short-term outcomes related to changes in management and longer-term dementia outcomes.
- Tau PET: an emerging tool
    - Study *in vivo* relationships between Aβ, Tau, and aging brain
    - Autopsy studies suggest symptoms correlate better with tangles than plaques
    - Biomarker for non-AD tauopathies (CTE, Frontotemporal dementia, Atypical parkinsonian disorders)
    - Evidence of target engagement and disease modification

Take homes:
Amyloid PET is already in the clinic
Tau PET is a powerful tool in aging-AD spectrum

Member Speaker: **James Hendrix, PhD**
Director, Global Science Initiatives | Alzheimer's Association
Title: Public/Private Partnerships in Alzheimer's' Biomarkers

Dr. Hendrix presented several significant advances in biomarker research facilitated by the Alzheimer's Association's Public / Private Partnerships.

- The IDEAS study was seeded by the studies on beta-amyloid imaging through IGRP, enabled by the FDA approval of beta-amyloid imaging agents for clinical use, and now works to ensure individuals can access beta-amyloid imaging.

- The Alzheimer's' Disease Neuroimaging Initiative (ADNI) has had many goals including validating standardizing, and optimizing biomarkers for AD trials, as well as creating a worldwide network for AD trials, and ultimately facilitating development of a surrogate biomarker outcome measure, potentially tau.
- The Global Biomarkers Standardizations Consortium (GBSC) is also led by the Alzheimer's Association and looks to define appropriate criteria for using cerebrospinal fluid biomarkers to identify and monitor the biochemical effects of a drug candidate in clinical trials, and standardize and optimize measurements across all studies.
- The Collaboration for Alzheimer's' Prevention (CAP) includes DIANTU, Alzheimer's Prevention Initiative, and TOMMORROW.

SPEAK VISUALLY: HOW TO USE VISUAL COMMUNICATION TO TELL YOUR RESEARCH STORY

Speaker: **Amy Balliett**
Co-founder & CEO | Killer Infographics
Title: Speak Visually – How to Use Visual Communication to Tell Your Research Story

Visual Communication: Defined as graphically representing information to efficiently and effectively create meaning. In other words, visual communication is graphically representing results to better share information and to engage a wide variety of audiences (i.e. engage patients, board, donors, etc).

Amy's presentation contained many statistics to bring home the points: (See her slides)
- visual information gets to the brain faster
- visuals included on press releases get more visitors
- first impressions are formed in less than 50 milliseconds

Tips:
- Use images paired with VERY limited text (a good rule to follow is ~100 – 200 words of text then break up with an image).
- Using universally understood simple images (iconography or pictograms) are best.
  See "The www.nounproject.org" you can find free and appropriate pictograms that are universally understood.
- First impressions are based on design so it's important that typography focus on a single core font (don't be messy or cluttered).
- In using stock imagery – be consistent. Use photos/realistic or artwork but use same style throughout.
- Use visuals and text – but the visuals should be able to stand alone to understand the point.
- Leave a lot of free space - be as minimal as possible.
- Don't use a white background – unless you have to print it out.

A Process that works for any budget
1. Identify visual guidelines. Produce brand guidelines and make a guideline deck to help consistency.
   a. How colors are going to be used – makes the brand cohesive.
   b. Choose only 4 primary colors to use – not more. In addition to the 4 primary colors, secondary colors can be used but only as accents.
   c. Secondary colors should be derived from the main colors.
2. Identify typography
   a. What fonts you should use. What fonts can be used and how they can be used – heading etc. Clearly define type use and hierarchy
3. Define how illustrations are going to be used. Use very simple pictograms then accessorize them to convey doctor or patient etc.
4. Use patterns to give extra emphasis.
5. Use "quantograms" to show quantities in pictures. This means using use multiples of a pictogram.
6. Identify how to use color.
7. Create simple visual metaphors.
8. Create a superset of assets that can be used in modules. Mix and match – but codify how things are used. Use "Chicklets" or boxes.

Process of visual communication design:

1. Define Content. Create a script for the infographic. Bullet the key information and create headlines and key summary paragraphs. This is the content that gets approved by key stakeholders.
   These bullet points describing the research will end up being double the length of the final infographic product.
2. Make a wireframe. Wireframes are the blueprints for the final design. Get other people to weigh in here at the wireframe stage.
3. Design. Develop many design drafts before settling on the final design

A great way to proceed it to design a work bench of assets. This workbench is a set of custom graphics and pictograms which have been custom designed that can become "vector assets" and used over and over.

See Amy's slides for the Seattle Children's example of visual guidelines and the final products.

Resources:

- Free ebooks - Visual campaigns for Beginners (bit.ly/VizComSIC).
- www.Lynda.com Amy is an instructor on Lynda.com. *(Lynda.com is online learning platform that helps anyone learn business, software, technology and creative skills to achieve personal and professional goals. Through individual, corporate, academic and government subscriptions, members have access to the Lynda.com video library of courses taught by recognized industry experts. Public libraries often have subscriptions to Lynda)*
- Tools that are great for doing this on your own (both have great online infographic tools)
  Canva (www.canva.com)
  Visme – (www.visme.co) has custom unique assets – you can use this to make powerpoints.
- Creating slides:
  Focus on 1 piece of info per slide
  As little text as possible on each slide
  Use animations carefully – better to animate outside of PowerPoint and imbed that in the PowerPoint.

Open Science – Pre-Registration: Approaches to Ensuring Reproducibility and Transparency

Speaker 1: Deborah Zarin, MD
Director| ClinicalTrials.gov
Title: Everything You Wanted to Know About ClinicalTrials.gov*
*But were afraid to ask

Registration: the process for making key summary information about interventional studies using human volunteers accessible to the public via a web-based system, from study initiation to completion
Results Reporting: summary results information available in a structured, publicly accessible web-based database

Motivating problems:

1. Practical — Potential participants had trouble finding trials
2. Scientific — Not all trials are published (only a small subset of NIH studies published)
   Not all outcome measures are published
   Changes to protocols are not always acknowledged

ClinicalTrials.gov is an answer.

- Clinical Studies registry (launched in 2000) - Audience is the public
  - Also included observational studies
- Results database (launched in 2008). – Audience is readers of the medical literature
  - For studies that do not have publications – this is the only place to get those results.
  - Defines a minimum dataset that you would need to interpret results.

Why is Registration Important:

- Human subject protections
- Research Integrity
- Evidence based medicine
- Allocation of resources

Content of a Study Record: (minimum info required)
Sharing participant level data – is not nearly as important as prospective registration and summary results reporting.
Registration section

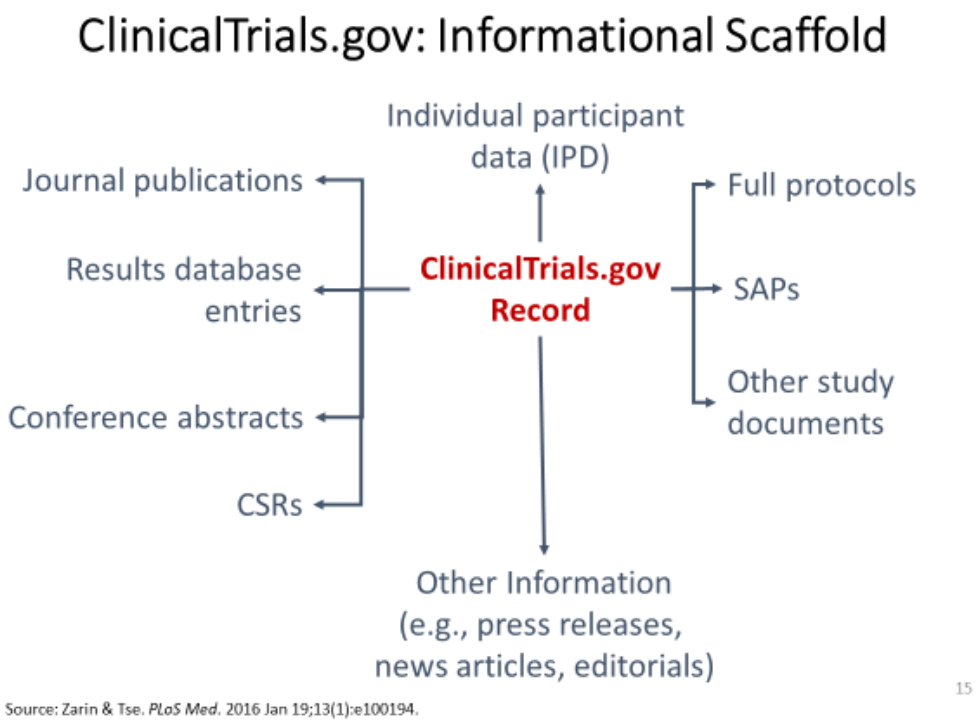
- Submitted at trial initiation
- Summarizes information from trial protocol: e.g., Condition, Interventions, Study Design
- Includes recruitment information (e.g., eligibility, locations)

Results section

- Submitted after trial completion
- Summarizes trial results
    - There are 4 scientific modules (see slide 21): Participant flow, Baseline characteristics, Outcome measures (including statistical analyses), Adverse events, and "other including all cause mortality and agreements)
    - See her slide 22 for detailed "minimal results information"
- Full Protocols & SAPs

Archival Data:

- Records must be corrected or updated throughout the trial's life cycle.
- All changes are tracked on a public archive site, accessible from each record (through a "History of Changes" link).
- Both current outcomes measures and first registered outcome measures visible



Dr. Zarin included a table (slide 16) showing the Key Clinical Trial Reporting Requirements for 3 policies
Reporting Requirements include: scope, funding source, intervention type, submission timing, enforcement
Policies Include:

- ICMJE Policy
- FDAAA Final Rule (issued in 2016)
- Final NIH Policy (issued in 2016)

3 Key functions of the Trial Reporting System: (TRS) (more detail in slide 17)

- Prospective registration
- Summary Results Reporting
- IPD Sharing

General Review Criteria:

- Protocol and results must be clear and informative
- Review focuses on:
  - Logic and internal consistency
  - Apparent validity
  - Meaningful entries
  - Formatting, including appropriate use of database structure
  - Differs in important ways from peer review

Summary of Evidence of Benefits: (see slide 25 for NEJM paper with all the details)

- Reporting volume
  - ~600 new registrations/week
  - ~140 new summary results/week (50% not published)
- Journal editors depend on registration records to ensure fidelity to the study protocol
- Evidence that ClinicalTrials.gov is filling the "gaps" in the public evidence base
- Funders increasingly use ClinicalTrials.gov to inform funding decisions
- Critical database for characterizing and analyzing the clinical research enterprise

Basic uses of ClinicalTrials.gov: (see slide 28 for details)

- Identify trials of potential interest for an individual
- Track progress of a specific trial, including availability of summary results
- Identify all trials that are completed or ongoing for a specific set of conditions/interventions
- Identify investigators and/or research centers of relevance to a specific set of conditions/ interventions

For those concerned with human subject protections:

- Complete list of ongoing and completed trials of relevance
- Assurance that information about the trial of interest - is in the public domain and for some trials, results will become public

For those with medical conditions:

- Finding a trial in which to participate
- Finding an expanded access drug
- Finding a center of research for a given condition/intervention

Other ways to use ClinicalTrials.gov

- Apps: "*Clinical Trial seek"*
- Disease-Specific Tool: Example *BreastCancerTrials.org* (BCT)
  Diseased based groups take their data and curate it for their own audience as the Breast Cancer organizations did.

Other uses and details for those use cases (see slides 33-45)

- For those concerned with research integrity and methods
- For those seeking study findings/results
- For those seeking to use aggregate data
- For characterizing and analyzing the clinical research landscape (Use of Trial Registries for Systematic Reviews)

Speaker 2: **Tim Errington, PhD**

Metascience Manager | The Center for Open Science

Title: Preregistration: Increasing Reproducibility and Transparency in Biomedical Research

There are 2 modes of research:

Discovery (postdiction): Pushes knowledge into new areas/data-led discovery; finds unexpected relationships; goal is to minimize false negative; p-values meaningless; hypothesis generated to explain why data occurred

Confirmation (prediction): Traditional hypothesis testing; results held to the highest standards of rigor; goal is to minimize false positives; p-values interpretable; data used to confront the possibility that the prediction is wrong

Data can inspire a hypothesis, but you cannot use those same data to test the hypothesis.
Doing so can:

- Can lead to overconfidence in post-hoc explanations
- Inflate likelihood of believing there is evidence for a finding when there is not
- Mistaking exploratory as confirmatory increases publishability and decreases credibility of results
- Ultimately, this decreases reproducibility

Purposes of Preregistration:

1. Discoverability – so others can know that the study exists.
   Much data generated that is not "publishable" in traditional publications focused on the themes of "novel" and "complete stories." Preregistered data can be found – saving others time and effort in repeating studies.
2. Interpretability

- Distinguish exploratory and confirmatory approaches
- Clear answers require clear questions
- Exploratory research is allowed and encouraged

What elements are important in preregistration:

Time-stamped, read-only version of research plan

- Hypotheses
- Sampling plan: existing data; source, size, rationale; stopping rule
- Variables: manipulated variables; measured variables
- Design plan: type (experimental, observational); blinding; randomization; design (paired, etc)
- Analysis plan: Statistical model/test; transformations; data exclusions; missing data; inference criteria

Preregistration in practice:

- Changes to procedure during study
  - Deviations are common: Preregistration is updated if outcomes are not observed. Changes and reasons for the changes are transparently reported.
  - Allows others to assess deviations and their rationale.
  - Provides substantially greater confidence in the resulting statistical inferences.
- Many experiments
  - Specific changes to a common procedure:
    1) Preregistration as a tool to define and document changes
    2) Following optimization - test prediction by replicating experiment
  - Provides a clear understanding of conditions necessary to obtain a result
- Program of research - analysis plan must be defined blind to the research outcomes, but also all outcomes of that analysis plan must be reported in order to avoid the problem of selective reporting. This increases understanding of generalizability of results.
  - Multiple different experiments:
    1) Preregistration of all experiments does not necessary increase confidence

2) Challenge of multiple comparisons or selective reporting
    - To achieve benefit all preregistrations and results need to be permanently preserved and accessible
- Few a priori expectations
- Data are pre-existing
- Longitudinal studies; large, multivariate data
- Assumption violations during analysis
- Competing predictions

Advancing Opportunities for Preregistering research:

Registry infrastructure

- Domain-specific (i.e. ClinicalTrials.gov)
- Domain-general (i.e. osf.io/registries)
- Specific OSF infrastructure exits with flexible and extensive functionality:
    - The Open Science Framework hosts **"OSF Registries" at** https://osf.io/registries

  OSF Registries provide tools for communities to create and manage their own registry. It allows them to devote resources to community building and standards creation.
    - OSF registry can be community specific – "branded" by funder, institution, etc.

Education about the value and the availability

- Webinars (see osf.io)
- Instructional guides (se osf.io)

Incentivize use

- Funder/Journal policy and encouragement
- Preregistration challenge sponsored by the OSF (1st 1000 scientists win $1,000 for publishing results of their preregistered research)

Recommendations for HRA members and other funders:

- Increase awareness
    - Send grantees OSF Newsletters and connect with OSF training webinars
    - Send them the very detailed word template https://osf.io/jea94/ that walks through preregistration – *(including sampling plan, variables, design plan, analysis plan, etc.)* Also linked here.
    - Publicize Registries and the Preregistration Challenge
- Encourage
    - include preregistration identifier and an optional field in grant applications
    - Acknowledge preregistration as an interim research product in progress reports
- Require
    - As a condition of distributing funds
- Training
    - Dissertation proposals for students – great way to train the next generation of scientists

Intersection of Policy and Science: Advocating for Funding to Advance Research

Speaker 1: **Ellie Dehoney, MPH**
Vice President, Policy and Advocacy | Research!America
Title: Intersection of Policy and Science - Advocating for Funding to advance Research

Affecting change in Federal Policy:
- Direct
- Indirect
- Even more indirect but important

Advocacy vs Lobbying (Research!America does both)
Advocate: champion a cause
Lobbying: communications intended to influence legislation

Engagement and advocacy by scientists has never been more important:
Opportunities: champions for medical research on both side of aisle
job creation, economic growth and maintaining competitiveness high priority
Challenges: tight budgets
Potential anti-science climate
Health care costs and coverage issues overpower research issues

Advocacy basics:
- Build relationships
- Engage the heart (you can't use facts to change feelings)
- Engage the mind (use facts that are appealing)

Do your homework and follow-up:
- Research your policy maker (statements, positions, committee assignments, events).
- Be ready to like the staffer you work with and be ready to provide them anything they need.
- Find something to thank them and something good that they have done.
- Is funding for medical research addressed on the congressman's website. That's a good place to start.

Meetings:
Try to meet with a health legislative assistant. They are all listed on their website - get in touch with them directly.
Keep it short and simple
Expect tough questions ("If NIH needs more money – what should we cut")

Phone calls:
Ask to speak with a legislative assistant
Ask the staffer to share your conversation (name, town, comments) with policymaker
Keep it short
Ask for a written response

Does and Don'ts
DO: Send a thank you email. Even better, write a handwritten note sign it and email the pdf
Leave a 1-page document
Make a clear ask during the meeting

DON'T Use jargon or acronyms
Worry if you can't answer a question. It's an opportunity to follow up.

Affecting change without advocating for it.

- Weigh-in with policy influencers
- Connect people
- Supply stories, data, examples
- Interact with policymakers as a constituent, without agenda
- Help cultivate advocates

Member Speaker 1: Laurie Whitsel, PhD

Director of Policy Research | American Heart Association

Title: Translating Science into Policy: Putting Research into Action

There are 4 anchors of advocacy:

- Legislative & Regulatory Lobbying
- Policy Research
- Media Advocacy
- Grassroots Mobilization

Components to Policy Research:

- Policy development
- Policy Evaluation
- Policy statements
- Review of Legislation/Regulatory Comments
- Managing External Relationships/Coalitions Relevant to our Work
- Partnering with other organizations in policy development/research
- Fact Sheets
- Reviewers for policy-related journals
- Reviewers for national surveillance systems (esp. CDC)
- Development of strategic policy agenda

Critical to AHA's Evidence-based Policy Making Strategies is their Policy Checklist:
A rigorous policy for choosing priorities

- Evidence Assessment
- Strategic Alignment
- Health Impact
- Feasibility
- Ability to Address SDOH
- Positioning
- Grassroot/Volunteer Engagement
- Level of Risk
- Internal Will
- Resource Commitment
- Likelihood of Success

The result is AHA's Policy Report and peer reviewed policy statements.

- Guide advocacy and inform policy makers, practitioners, health care professionals, researchers, media, public, etc
- Cited by Surgeon General, WHO, CDC, EU, Publications, and others
- Result in "wins" (federal wins include increased budgets for NIH and CDC Division for Heart Disease and Stroke prevention, Preservation of ACA, stronger nutrition standards in schools, and others. Many state wins also.)
- Voices for Healthy Kids (partnered with Robert Wood Johnson Foundation) a successful model for impacting policy
- To help increase impact – AHA provides many resources for state and local issue-advocacy campaigns.

Member Speaker 2: Jon Retzlaff, MBA, MPA

Chief Policy Officer, Vice President, Science Policy Government Affairs | AACR

Title: Intersection of Policy and Science: Advocating for Funding to Advance Research

American Association for Cancer Research has a large Science Policy and Government Affairs Committee composed of well-known researchers, as well as topic-specific subcommittees.

- Serve as an authoritative voice for important science and public policy issues
- Advocate for increased funding for cancer research and biomedical science
- Formulate policy recommendations for Congress and federal agencies (NIH, FDA, CMS, PCORI)
- Inform policymakers, scientists, public via workshops, special briefings and conferences, and advocacy publications
- Identify regulatory science and policy areas where the AACR has the potential to stimulate positive change
- Build strong, productive alliances with research advocacy and patient advocacy groups

Advocacy actions and responses to events (such as recent budget proposals decreasing NIH budget) include:

- Special events at the Annual Meeting
- Capitol Hill Days for AACR leaders, patient advocates, early career scientists
- Local advocacy events with Congressional supporters and cancer centers
- Online tools, action alerts and social media engagement
- Rapid response in the media and in press statements
- Educational briefings to inspire support on Capitol Hill
- 5th Annual Rally for Medical Research! (and other "Hill Days")
- Target specific Senators – Roy Blunt (R-MO) 2-time cancer survivor and Chairman of the Senate Appropriations Subcommittee on Labor-Health and Human Services-Education.
- Hosted Congressional Briefing on National Cancer Moonshot Initiative (Now Beau Biden Cancer Moonshot)
- AACR Cancer Progress Report (annual)
- AACR Scientist – Survivor Program. Builds bridges between scientific, cancer survivor, and patient advocacy communities. Address survivorship, quality of life, policy issues, clinical trial input, facilitate access to information.

Member Speaker 3: Matthew Ellsworth, MFA

Vice President, Communications | The Flinn Foundation

Title: Arizona's Bioscience Roadmap A Model of Engagement for a 501(c)(3) Foundation

Origin of the Roadmap - "To improve the quality of life in Arizona, to benefit future generations" (1965). Then 10-year bio funding commitment (2000) and finally the Arizona's Bioscience Roadmap was developed (2002).

Goals and Metrics:

- Battelle consultancy
- Multi-sector endorsement
- Existing & emerging strengths
- Regular convening & reporting

Form Steering committee that has:

- Multi-sector membership
- C-level leaders
- Convenes quarterly
- Education, message alignment, advocacy functions

Progress

- Reaching Policy Makers – ("77 potential actions", Annual elected officials reception, Biennial candidate forums)
- Cornerstone organizations
- Research funding & activity
- Industry & employment
- Public-sector investment