



National Institute of
Neurological Disorders
and Stroke

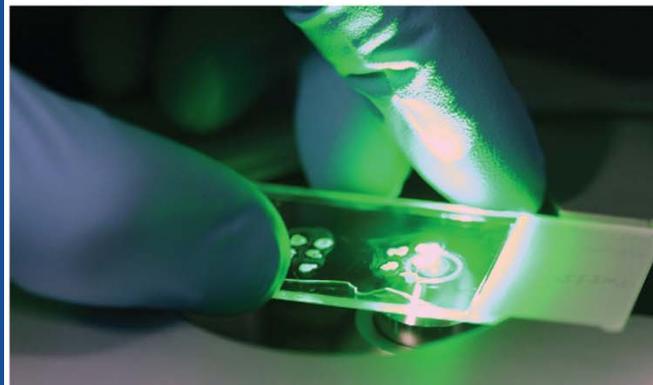
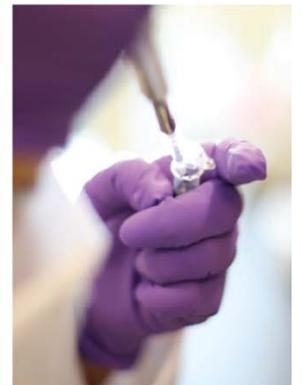
An Overview of Disease Research Planning

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Coordinating Committee

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Outline

- What is disease research planning?
- What considerations should guide planning?
- What is the general framework of a planning effort?
- MD planning by the MDCC (Heather Rieff)
- Stroke planning (Katie Pahigiannis)
- Discussion and Questions

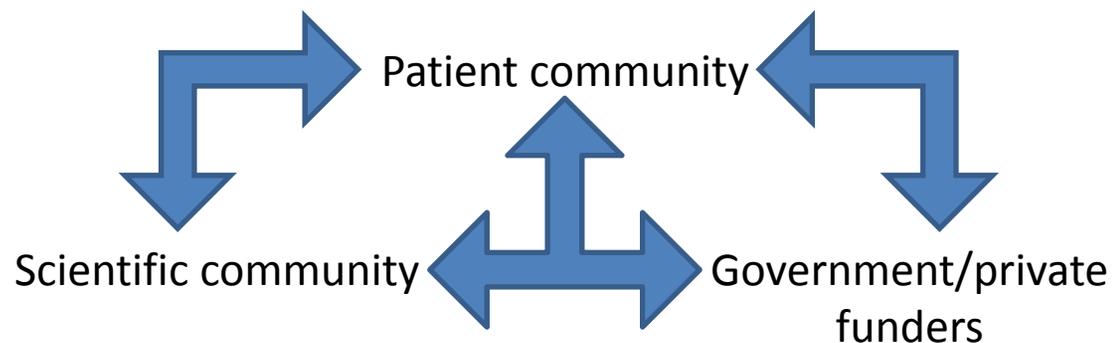
What is disease research planning?

- It is an organized, systematic effort to identify and communicate priorities for making progress in a given research area
 - Opportunities to advance an area
 - Gaps in knowledge that need to be addressed to make progress
 - Obstacles that need to be overcome to make progress
- What it is not – is NOT an attempt to prescribe how research to address such opportunities/gaps should proceed



What is disease research planning?

- Scope of planning is broad, but specific enough to be meaningful
 - Not intended to identify priorities at the level of individual projects
- At NINDS, disease research planning brings together the entire disease community to identify research priorities



- Once priorities are identified, the entire community works together to address them

Considerations for Planning: Seek broad input

- Include a wide range of scientific expertise

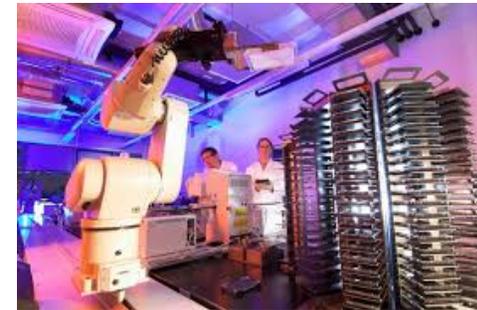
Basic researchers



Clinical researchers



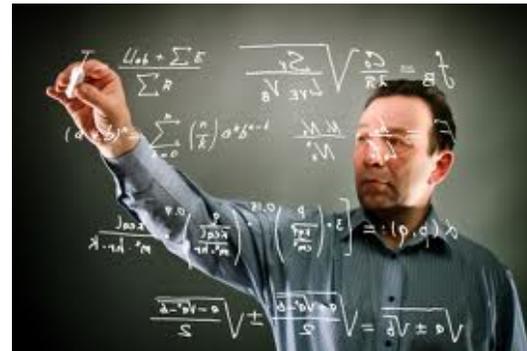
Translational researchers,
including industry



Promising, young
investigators



Scientists from related
fields



Considerations for Planning: Seek broad input

- Include all stakeholders, not just researchers

Disease non-profit groups



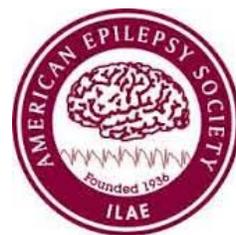
Health care systems/payers



Patients and families



Professional societies



Broader public



Considerations for Planning: Be informed

- Useful to know what research is ongoing
 - Begin with a portfolio analysis to understand what is being funded by whom



- This sets baseline for future directions
 - Allows focus on further priorities
 - Identifies potential gaps to pursue in planning

Considerations for Planning: Expect to prioritize

- Important to build in a process for prioritizing among recommendations
 - Assigning priorities and general timeframes to recommendations increases value of effort
 - Long “laundry lists” of everything that could or should be done are not as helpful
- Can be challenging to get group of people to agree on limited set of priorities
 - Consider approaches designed to build consensus



General framework of planning effort

Form organizing/steering committee



Set up working groups to develop recommendations



Hold in-person meeting to discuss/refine recommendations



Present final recommendations for approval



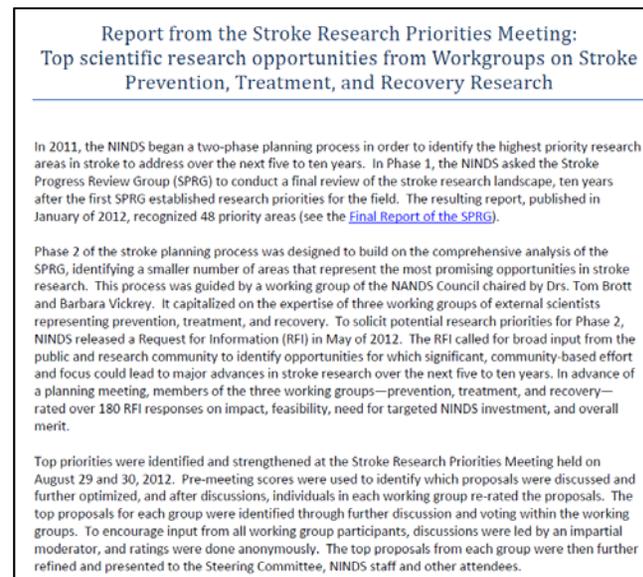
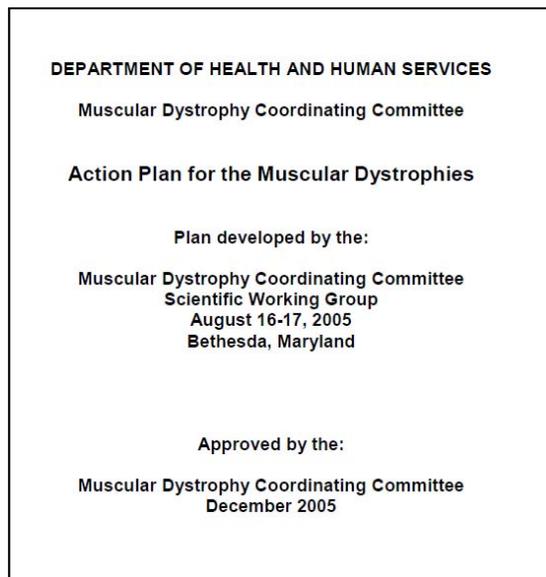
General framework of planning effort

- Patient/public input can be built in along any of these previous steps
 - Public members on working groups
 - Requests for Information (RFI) to get input from broad community
 - Public member comment at in – person meeting
 - Public comment period on draft recommendations
 - Public members serve on oversight committee
- Communicate approved recommendations to disease community and broader public



Examples of disease research planning

- Action Plan for the Muscular Dystrophies
- Stroke Research Priorities



Muscular Dystrophy Coordinating Committee (MDCC)
and
the Action Plan for the Muscular Dystrophies

Background on MDCC:



- Established by the Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2001 (*MD-CARE Act*) to coordinate activities relevant to the various forms of muscular dystrophy.
- Committee includes not more than 15 members:
 - 2/3 of members represent Federal agencies (includes 4 NIH ICs, CDC, DoD, FDA, and others)
 - 1/3 of members represent the public (patients, family members, representatives from patient voluntary organizations).

Action Plan for the Muscular Dystrophies (2005)

- Identified over 70 research objectives for the entire muscular dystrophy community.
- MDCC Scientific Working Groups (24 Scientists and Physicians) :
 - *Mechanisms of Muscular Dystrophy*
 - *Diagnosis and Screening of Muscular Dystrophy*
 - *Therapy of Muscular Dystrophy*
 - *Living with Muscular Dystrophy*
 - *Research Infrastructure for Muscular Dystrophy*
- Approved by the MDCC

Current Planning Effort to Update the 2005 Action Plan:

Input on progress to date:

- Regular MDCC meetings have included updates from committee members on how their agency/organization has made progress toward areas in the Action Plan
- NIH, together with the MDCC, issued a **Request for Information (RFI)**, which invited the muscular dystrophy research, advocacy, and family communities to provide comments from their perspective on progress made toward the objectives in the Action Plan, and on remaining opportunities.

Request for Information (RFI) on the Action Plan for the Muscular Dystrophies

Notice Number: [NOT-NS-14-039](#)

Related Notices: None

Issued by: National Institute of Neurological Disorders and Stroke ([NINDS](#))

Responses requested by December 20, 2013

Information Requested

The NIH, together with the Muscular Dystrophy Coordinating Committee (MDCC), is leading an effort to update the [Action Plan for the Muscular Dystrophies](#) (PDF, 1421 kb). To help inform this planning process, the NIH and the MDCC welcome input from the public regarding accomplishments relevant to specific objectives in the Plan, remaining opportunities in the areas of the Plan, and suggestions for new research objectives. The collected comments will be reviewed and considered as the MDCC and an MDCC Scientific Working Group develop the updated Action Plan.

Similar model to 2005 Planning effort:

- Formed scientific working groups in 5 areas
 - Working group membership included at least one NIH or other agency staff person to help organize/moderate work of the group
- Working Groups interacted prior to face-to-face meeting
 - Sharepoint site to comment on/revise existing objectives and develop new objectives
 - Teleconferences to finalize proposed objectives
- Face-to-Face meeting: Working Groups and MDCC Members -- July 2014



Next Steps

NIH and Working Groups are revising objectives, writing introductory text, etc.



Draft plan will be sent to MDCC members for comment



Public Comment period



Final approval by MDCC

NINDS 2012 Stroke Research Planning Effort

- Overall goal was to develop a small, focused set of high priority research opportunities
- Built upon the Stroke Progress Review Group's work, a more comprehensive effort over a 10-yr period that assessed research gaps and opportunities
- Utilized tailored methods to facilitate consensus among experts and to garner broad support for addressing key research priorities

Planning Group Characteristics

- **Steering Committee** to help develop and oversee the process
 - chaired by 2 NINDS Council members
 - Broad expertise, some peripheral to field
 - Major advocacy and patient groups
 - International perspective
- **Three topic-based working groups** to develop the recommendations
 - Prevention, Treatment, Recovery/Rehabilitation
 - Balance of basic, translational, and clinical experts, including important subspecialties where appropriate
 - **No chairpersons**

Groundwork

Identification of Research Opportunities

- Structured input from the general public, steering committee and workgroup members

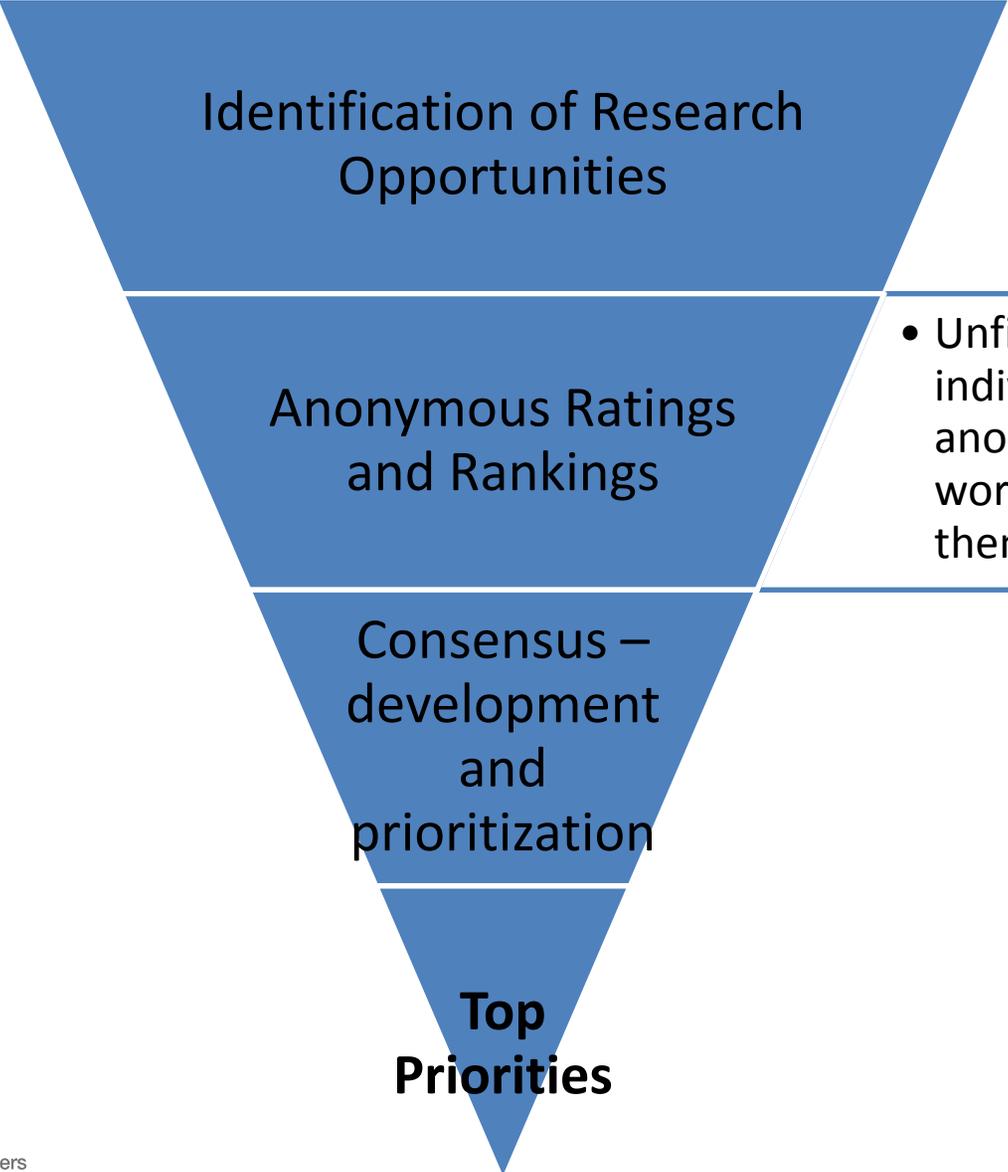
Anonymous Ratings and Grouped Rankings

Consensus – Development and Prioritization

9 Top Priorities

Identification of Research Opportunities

- Request for Information to solicit ideas from the public
 - The *only* way to get ideas into the mix for consideration
 - Broad call for input, major dissemination effort
 - Template requested comments on 5 defined elements: description, rationale, readiness, impact, approach



Pre-meeting activities

- Unfiltered topics rated individually and anonymously by the workgroup members and then ranked by NINDS staff



Anonymous Pre-meeting Ratings of Unfiltered Set of Ideas

- Workgroup members rated each research opportunity *individually, without group discussion*, using the online tool SurveyMonkey
- Scored each opportunity on: impact, feasibility, need for targeted focus, overall assessment
- NINDS staff ranked the topics based on the results and grouped by scientific theme to facilitate meeting discussions

Identification of Research Opportunities

Anonymous Ratings and Rankings

Consensus – development and prioritization

**Top
Priorities**

*2-day meeting in
Bethesda: breakout
sessions*

- Moderated workgroup discussions based on the results/rankings from first-found ratings

Moderated discussions and prioritization of the best ideas

- Results provided to workgroup members prior to in-person meeting
- **Moderated discussions** within workgroups focused on high and moderate scoring proposals within each theme
- **Anonymous re-ratings using automated system** (with clickers) in an iterative process to narrow down the list. Ended with forced choice voting round to facilitate agreement around 2-3 top ideas

Identification of Research Opportunities

Anonymous Ratings and Rankings

Consensus – development and prioritization

Top Priorities

- Cross-group discussion and voting
- Steering committee consideration
- Presentation to NINDS leadership and Council

2-day meeting in Bethesda: final session

follow-up discussions



Results & Lessons

- **9 research priorities** - www.ninds.nih.gov/strokepriorities
 - 3 prevention (VCI, imaging biomarkers, CER)
 - 3 treatment (trial infrastructure, reperfusion therapies, neuroprotection)
 - 2 recovery (brain-machine interfaces, early recovery)
 - 1 cross-cutting (predictive value of preclinical studies)
- **Feedback and Lessons from the process**
 - Overall, participants liked this new model and felt it led to high quality debate / discussion
 - Suggestions for how to improve the process next time
 - Not sure how to get around the tendency for omnibus priorities

Closing thoughts

- Overview and MD/stroke models not meant to be cookie cutter templates – each effort is different; your approach may be a hybrid of the models we have discussed
- What comes out of planning
 - Guides research community in terms of priorities
 - Informs public and private funding organizations
 - At NINDS, planning has lead to further focused workshops, new trans-IC collaborations, funding initiatives