

Request for Information on the 2021-2026 National Institutes of Neurological Disorders and Stroke Strategic Plan: Response Summary

Background: In response to the Request for Information NOT-NS-21-021, NINDS received 22 responses from professional societies, non-profit organizations, patient advocates, principal investigators, and a health care provider. Below is a summary of key themes that emerged from the submitted responses. Granular-level recommendations provided by respondents were shared with the appropriate implementation taskforce to be considered for the implementation phase of the NINDS strategic planning process. Most editorial and technical comments on the draft strategic plan were incorporated into the final strategic plan.

Patient Engagement. Respondents commended NINDS on their existing efforts to engage with the advocacy community, such as the Non-Profit Forum (NPF), and supported the addition of patient engagement as a scientific cross-cutting strategy in the NINDS Strategic Plan. Several respondents also advised NINDS to enhance their partnership with patient advocacy communities to better engage with individuals with neurological conditions and their advocates. Recommendations to accomplish this included involving individuals with neurological conditions, care providers, and advocates in research development (e.g., clinical trial design, data collection, analysis and interpretation, the dissemination of research findings), programming for training and professional development, development of research notices of funding, and the grant review process. It was suggested that NINDS consider steps to incentivize engagement with people with neurological conditions, care providers, and advocates in the planning and oversight of NINDS-funded studies. Regarding the NPF, respondents recommended that NINDS engage patient advocacy groups while planning the meeting to prioritize the needs of individuals with neurological conditions. They also suggested that NINDS expand the membership of the NPF for broader representation of organizations across the neurological disease spectrum, including groups focused on rare diseases and health disparities, and provide resources for non-profit organizations to allow them to fully participate in NINDS events and programs.

Health Equity. Many respondents were encouraged to see advancing health equity as a scientific goal in the draft NINDS Strategic Plan. Respondents acknowledged health equity as an urgent need to address across all neurologic disease areas and asked that NINDS consider the role of social determinants of health in future research in health equity. Moving forward, it was suggested that NINDS engage in community-based participatory research strategies to assure that research outcomes and endpoints are aligned with community needs and priorities, which may encourage marginalized communities to become active partners. A few respondents also highlighted the need for more inclusive research to improve the representation of underserved and minority populations in research studies. One respondent advised NINDS to research the effectiveness of tele-therapies for neurologic conditions, as telehealth may help provide equitable access to healthcare.

Diversity and Inclusion. Respondents appreciated NINDS's focus on diversity and inclusion and underlined the need for NINDS to actively promote a culture of diversity, inclusion, and equity in current and future programs and initiatives. Regarding workforce diversity, a few respondents stated their support for the inclusion of people with disabilities in this section of the NINDS Strategic Plan to prioritize the full breadth of diversity and intersectionality in the research community. A few

respondents recommended NINDS partner with professional and non-profit organizations to increase diversity and inclusion efforts in the neuroscience research community.

Biomarkers and Outcome Measures. Many respondents stated their appreciation for the growing focus on biomarkers at NINDS. Respondents underscored the urgency for biomarker development to aid in therapeutic development for neurological disorders. However, in addition to biomarkers for diagnosis, some suggested that NINDS also focus on the preclinical detection of neurological diseases to identify high risk individuals who could benefit from preventive medicine. To better address the needs of these high-risk individuals, NINDS could support additional research to better understand how environmental factors and social determinants of health affect the onset of neurological diseases and disorders. Biomarkers to track disease progression and categorize individuals into different disease subgroups should also be a priority. Regarding outcome measures, a few respondents indicated that the draft strategic plan overemphasized biological measures. They recommended the research community move towards a more patient-centered understanding of desired outcomes, such as improving functional abilities (e.g., quality of life and community integration). In addition, respondents said that NINDS should partner with the FDA to coordinate strategies for biomarker investments and outcome measures.

Improving Treatments. Many respondents were appreciative of NINDS's emphasis on improving treatments and the focus on disease-modifying approaches in the draft plan. Several commended NINDS on the development of the Ultra-rare Gene-based Therapy (URGenT) Network and view the URGenT Network as an opportunity to standardize and harmonize best practices and protocols to advance gene-based therapies for ultra-rare diseases. Respondents advised that NINDS make necessary investments across the research spectrum (e.g., basic, translational, and clinical research) and coordinate with other federal agencies to overcome barriers in therapy development to deliver safe and equitable access to future treatments. In regards to disease prevention, it was recommended that NINDS emphasize the promotion of overall, optimal brain health as well. A respondent also highlighted the importance of implementation science to fill the gap between therapy development and demonstration of a therapy's efficacy and implementation.

Partnerships & Collaborations. A few respondents were pleased with NINDS's emphasis on partnerships and collaborations in the draft plan. They encouraged NINDS to partner and collaborate with other federal agencies (e.g., FDA and CMS), industry, academia, professional societies, and non-profit organizations to move research forward. To the extent possible, one respondent requested that NINDS be more explicit on how NINDS plans to collaborate and interact with federal agencies.

Suggested Research Priorities. Many respondents urged NINDS to enhance research efforts in several areas, including pediatric neurology, cerebral palsy, rehabilitation, enhancing quality of life, functional cognition, pain management, behavioral health disorders, health inequities, epilepsy, Parkinson's disease, amyotrophic lateral sclerosis, neuro-palliative care, basic research, cognitive assessment, neurodevelopmental disorders, vascular dementia, and the acute and long-term neurological effects of COVID-19.

Training and Mentorship. A few respondents commented that NINDS must continue and expand funding for research training, career development, and mentorship to support the next generation of researchers. A respondent mentioned the need for more attention on developing and supporting early career scientists in the strategic plan as well as address the impact of COVID-19 in this group,

particularly for women and those from underrepresented backgrounds. Furthermore, a few respondents also focused on the need to support physician-scientists at all career levels to increase recruitment and retainment of this group. In addition to scientific training, it was suggested that researchers also receive training in patient engagement.

Communications. The inclusion of communications as a goal in the strategic plan was welcomed by respondents. They encouraged NINDS to develop a more active outreach strategy. A few respondents offered to assist NINDS in strengthening their communication efforts by acting as conduits to external NINDS stakeholder groups. Respondents also encouraged NINDS to focus more on implementation science and implementation networks as part of their dissemination, education, and communication plans.

Data Sharing and Data Science. A few respondents stressed the importance of data sharing and data science for future neuroscience research. To promote data sharing within the neuroscience research community, NINDS will need to create and implement the necessary policies, improve data infrastructure, define data quality standards, and develop data interoperability standards that facilitate data sharing and aggregation. Furthermore, respondents recommended that NINDS deliberate the ethical considerations with data sharing (e.g., data privacy and artificial intelligence/machine learning) moving forward.

Team Science. A few respondents encouraged NINDS to enhance their support of team science to enable more transformative research.

Rigor and Transparency. A couple of respondents commended NINDS's ongoing efforts in rigor and transparency and applauded NINDS for its leadership in this field. They were supportive of the development of an online resource to train researchers about good, rigorous scientific practices.

Neuroethics. Respondents strongly supported NINDS's ongoing efforts in neuroethics considering growing bioethical issues in neuroscience research including placebo-controlled clinical trials, gene therapy testing and access, brain death and consciousness, brain-computer interface, and cognitive enhancement. However, it was recommended that NINDS expand ethics funding opportunities beyond the BRAIN Initiative and suggested NINDS consider developing a strategic plan in this area. In particular, one respondent requested NINDS provide additional neuroethics resources towards the bioethical study of gene therapies.

Workforce Culture. In addition to diversity and inclusion in the workplace, a respondent also recommended NINDS consider concerns about burnout and development and support of family-friendly grant policies and structures.