Neuro Task Force Update

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Neuro Task Force
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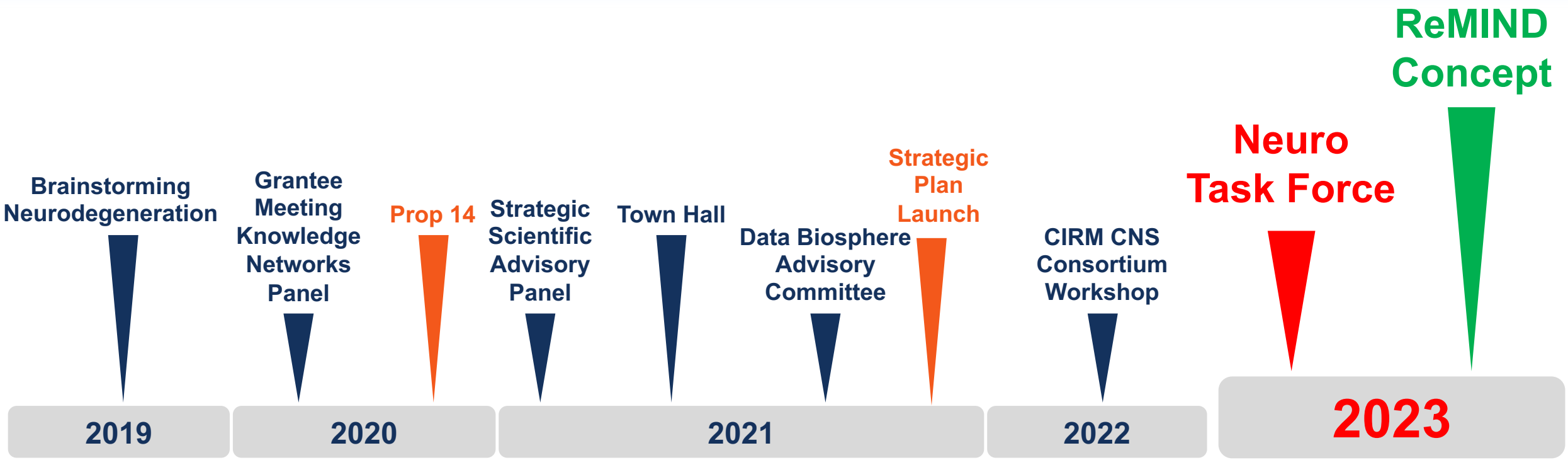
CIRM
CALIFORNIA'S STEM CELL AGENCY

Presentation Overview

1. Background
2. Neuro Task Force Progress

Previous meetings informing Neuro Strategy

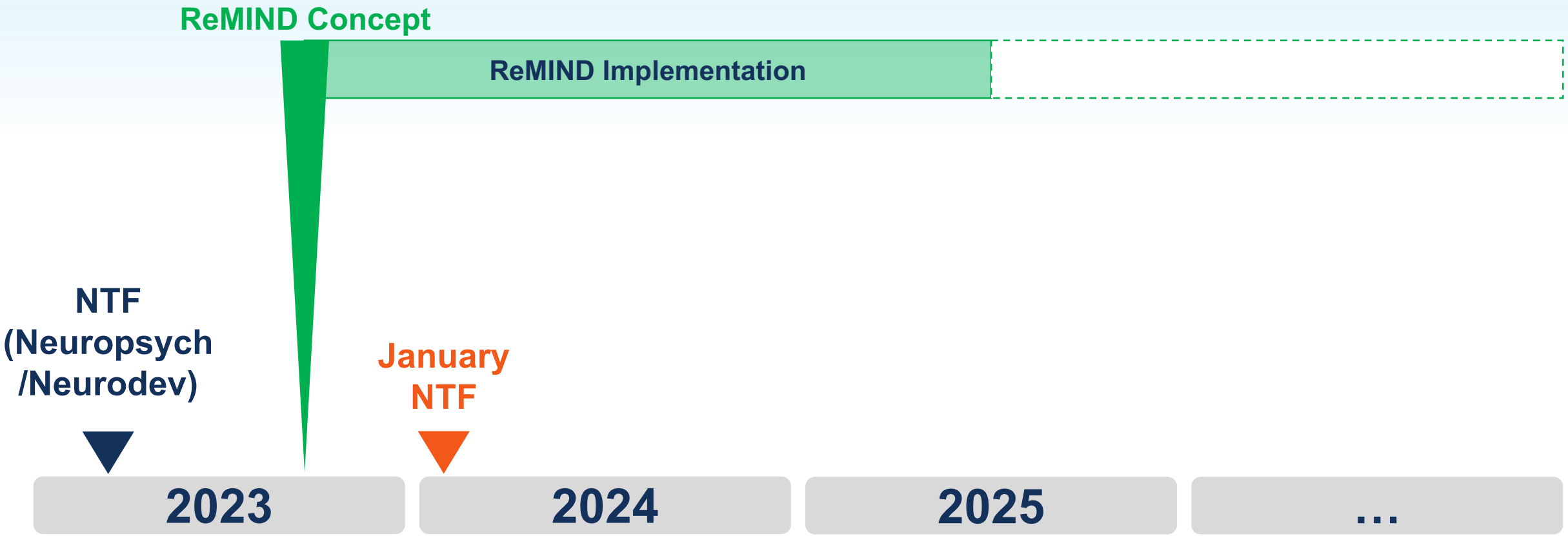


Neuro: Includes *neurodevelopmental, neurodegenerative, neuromuscular, trauma/brain injury, and neuropsychiatric diseases*

Neuro Task Force – Main outcomes to date

- Recommended increasing investment in Neuropsychiatric disorders
- The Task Force approved a pilot program (ReMIND) that uses a subset of funds to develop multidisciplinary teams to tackle disease mechanisms in neuropsychiatric disorders

NTF Process Timeline



January NTF – Discussion outcomes

1. **Expert Educational Sessions (March-May):** Overview neurodegenerative research, spotlighting innovative approaches and under-explored areas
   - **Aim**: Gain unbiased insights from a mix of experts highlighting novel approaches and identifying areas ripe for exploration
2. **Review and Prioritization:** Identify and prioritize research areas that hold the most promise for groundbreaking discoveries, especially on those not already well-funded
3. **Funding Mechanism and Structure:** Implement a funding model based on the REMIND program to foster cross-disciplinary work and groundbreaking research, allocating a large budget portion to priority projects
4. **Budget and Funding Allocation:** Ensure a balanced investment across various aspects of neuro research

March NTF Outcomes

Design Brief | Intent

1. Identify the bottlenecks/knowledge gaps that would uniquely benefit from multidisciplinary solutions and knowledge sharing
2. Cross-Disease Analysis – discuss how insights from stem cell and genetic research in one ND disease can be applied to others
3. Discuss how insights and innovative tools and techniques can be applied across diseases
4. Discuss a potential role for CIRM in addressing the above points

Design Brief | Key Knowledge Gaps

Prioritize 3 key knowledge gaps that need to be addressed within your field (i.e., disease area within ND)	Outline how CIRM could help address those gaps
1	
2	
3	

Design Brief | Guiding Questions

General questions for all sessions:

1. What are the three critical knowledge gaps in the current understanding and treatment of [specific ND disease]?	1. 2. 3.
2. What are the most important bottlenecks in the development process for your therapeutic programs and what kinds of innovations could be most impactful?	•
3. How will your presentation today contribute to closing these gaps?	•
4. Which new technologies that were developed in other fields can now be adapted to answer questions in ND?	•
5. Which new conceptual framework would you propose to be tested?	•
6. How can collaboration between academia, industry, and clinical practice be improved to address these knowledge gaps more effectively?	•
7. Are there particular types of public data or biological resource infrastructure that would benefit from added investment in CA?	•

March NTF – Expert educational sessions in PD and ALS

Lorenz Studer, MD
Director, Center for Stem Cell Biology at Sloan Kettering Institute for Cancer Research
Location: New York, NY United States

Jeffrey Rothstein MD, PhD
Professor Of Neurology and Neuroscience;
Director, Brain Science Institute
Johns Hopkins, School of Medicine

Parkinson's Disease (PD) | Key Knowledge Gaps

Prioritize 3 key knowledge gaps that need to be addressed within your field (i.e., disease area within ND)	Outline how CIRM programs could address those gaps
1. **Non-Dopaminergic Symptoms in PD**: Non-motor symptoms like cognitive decline and GI issues are not well-managed by current therapies	Launch interdisciplinary research to explore these non-dopaminergic pathways, aiming to develop novel therapeutic strategies
2. **Patient Stratification:** Patient stratification is needed to tailor treatments more effectively	Focus on patient stratification, leveraging advances in machine learning and patient-derived models to develop biomarkers for predicting treatment responses
3. **Grafted Cell Delivery and Survival:** Critical to success is the integration and survival of grafted cells post-transplantation, avoiding adverse effects and ensuring safe and reproducible delivery	Translational bottleneck program focus

ALS | Key Knowledge Gaps

Prioritize 3 key knowledge gaps that need to be addressed within your field (i.e., disease area within ND)	Outline how CIRM programs could address those gaps
1. **Sporadic ALS and Pathway Analysis**: The majority of ALS cases are sporadic with no clear genetic cause	Support studies utilizing iPSC lines from large cohorts of ALS patients to discover new pathways involved in the disease, which could lead to novel therapeutic approaches.
2. **Variability in ALS Progression**: ALS progression is highly variable, with a few patients surviving 30 years with the disease	Fund research into genetic, molecular, or environmental factors contributing to this variability, potentially through comprehensive patient-derived iPSC libraries or biomarker development
3. **Beyond Motor Neurons**: Other cell types, such as glial cells, also degenerate and contribute to the disease's clinical syndrome	Broaden the focus of ALS to these other cell types, which could lead to a more comprehensive understanding of the disease and potentially new therapeutic targets.

Today – Experts educational sessions in AD and HD

Alison M. Goate, PhD
Jean C. and James W. Crystal Professor of Genomics, Chair of the Department of Genetics and Genomic Sciences, Professor of Neuroscience and Neurology Icahn School of Medicine at Mount Sinai

James F. Gusella, PhD
Bullard Professor of Neurogenetics in the Department of Genetics, Harvard Medical School
Research Staff, Massachusetts General Hospital