Precision Medicine in Neuroscience: Tools, Translation, and Implementation: A Workshop

Wednesday, March 4, 2026: 2:00 pm – 5:00 pm ET
Thursday, March 5, 2026: 9:00 am – 4:00 pm ET

Objectives

- Highlight case examples where precision medicine approaches have informed research, diagnosis, treatment, and disease/disorder reclassification in neuroscience.
- Examine emerging tools and technologies—including biomarkers, multi-omics, computational psychiatry, and artificial intelligence—and their applications across neuroscience research and clinical care.
- Consider barriers and opportunities for implementation, including provider education, patient engagement, payer and regulatory models, data privacy, clinical integration, and potential risks.
- Explore cross-disease/disorder and holistic frameworks, including how precision medicine approaches can address comorbidities and support a broader brain health model.

Program At-A-Glance

- **Day 1**

  Welcome & Introductory Remarks

  Workshop Overview

  Keynote Presentations

  Session 1: Precision Biology for Discovery and Defining Disease/Disorder

  Session 2: Precision Frameworks for Patient Identification and Stratification

- **Day 2**

  Welcome & Day 1 Recap

  Keynote Presentations

  Session 3: Precision Measures to Monitor Disease/Disorder and Treatment

  Session 4: Precision Strategies for Effective Clinical Trial Design

  Lunch

  Session 5: Precision Systems to Deliver Care at Scale

  Session 6: Driving Ecosystem Investment in Scalable Precision Medicine Infrastructure for Brain Health

  Session 7: Synthesis and Opportunities for Precision Medicine in Neuroscience

WEDNESDAY, MARCH 4, 2026

2:00pm **Introductory Remarks**

Frances Jensen, University of Pennsylvania, *Forum on Neuroscience and Nervous System Disorders Co-chair*

Deanna Barch, Washington University in St. Louis, *Forum on Neuroscience and Nervous System Disorders Co-chair*

2:05pm **Workshop Overview**

Rosa Canet-Avilés, California Institute for Regenerative Medicine, *Workshop Co-chair*

Gayle Wittenberg, Johnson & Johnson, *Workshop Co-chair*

2:10pm **Keynote Presentations**

Steve Hyman, Broad Institute of MIT and Harvard, *Planning Committee Member*

Jamie Heywood, Alden Scientific, PatientsLikeMe, ALS Therapy Development Institute, AOBiome Therapeutics

2:40pm **Session 1: Precision Biology for Discovery and Defining Disease/Disorder**

Objectives: This session will examine how emerging tools and technologies—including biomarkers, multi-omics, and artificial intelligence—contribute to a deeper understanding of human neurobiology in health and disease/disorder. Talks will explore the strengths and limits of these technologies in tackling the complexity of the human brain—and the challenges in distinguishing causal insight from correlation. A central theme is at what point is our biological understanding strong enough to justify moving forward?

Key discussion questions:

- What kinds of biological evidence actually shape decisions?
- When does correlation become actionable insight?
- What data are we collecting today that meaningfully shift disease/disorder definitions toward precision, and what data are we collecting that do not?

2:40pm **Session Overview**

Julie Harris, Allen Institute, *Planning Committee Member*

Dimitri Krainc, Northwestern University, *Planning Committee Member*

2:45pm **Speaker Presentations**

Daniel Geschwind (virtual), University of California, Los Angeles

Mariano Gabitto, Allen Institute

Rhoda Au, Boston University

3:20pm **Moderated Panel and Audience Q&A**

3:45pm **BREAK**

3:55pm **Session 2: Precision Frameworks for Patient Identification and Stratification**

Objectives: Positioned between discovery and development, this session explores how precision biology data are assembled into biomarker-driven and composite frameworks for patient stratification across neurological and psychiatric disorders, including threshold selection and uncertainty management, and how these choices impact regulatory relevance and downstream clinical trial design. Speakers will highlight contrasts in progress in Alzheimer's disease and Parkinson's disease vs psychiatric disorders, where stratification remains challenging.

Key discussion questions:

- Should precision frameworks be biology-first or phenotype-first and are we ready to revise diagnostic categories accordingly?
- How much uncertainty and misclassification is acceptable to ensure a framework is still usable?
- How can frameworks evolve with new biological understanding without losing regulatory confidence?

3:55pm **Session Overview**

Laura Bustamante, Washington University in St. Louis, *Planning Committee Member*

Brian Fiske, Michael J. Fox Foundation for Parkinson's Research, *Planning Committee Member*

4:00pm **Speaker Presentations**

Kathleen Poston (*virtual*), Stanford University

Frederike H. Petzschner, Brown University

Diane Stephenson, Critical Path Institute

4:35pm **Moderated Panel and Audience Q&A**

4:55pm **Day 1 Concluding Remarks**

Rosa Canet-Avilés, California Institute for Regenerative Medicine, *Workshop Co-chair*

Gayle Wittenberg, Johnson & Johnson, *Workshop Co-chair*

5:00pm **Adjourn Day 1**

THURSDAY, MARCH 5, 2026

9:00am **Review of Day 1 and Preview of Day 2**

Rosa Canet-Avilés, California Institute for Regenerative Medicine, *Workshop Co-chair*

Gayle Wittenberg, Johnson & Johnson, *Workshop Co-chair*

9:05am **Keynote Presentations**

Husseini Manji (*virtual*), UK Govt Mental Health Goals Program, Oxford University, Yale University, Duke University

Michelle Colder Carras, Carras Colder Carras LLC

9:35am **Session 3: Precision Measures to Monitor Disease/Disorder and Treatment**

Objectives: This session focuses on prognostic and longitudinal assessment, an area of particular challenge in brain disorders. Topics include predicting and assessing disease/disorder progression using imaging, biofluid, genetic, and digital markers along with clinical data. Discussion will highlight feasibility, heterogeneity and interpretability across conditions, and how these factors can influence clinical management and therapeutics.

Key discussion questions:

- How can biomarker, clinical, and other data be integrated for prognosis and prediction of progression?
- What do biomarker changes over time tell us about the biology of disease/disorder?
- How can initial and longitudinal biomarker profiles inform therapeutic interventions, including initiation, dose changes, discontinuation, or switching?

9:35am **Session Overview**

Linda Brady, National Institute of Mental Health, *Planning Committee Member*

9:40am **Speaker Presentations**

Reisa Sperling, Harvard Medical School

Kenneth Marek, Institute of Neurodegenerative Disorders

Scott Woods, Yale University

10:15am **Moderated Panel and Audience Q&A**

10:45am **BREAK**

11:00am **Session 4: Precision Strategies for Effective Clinical Trial Design**

Objectives: This session will explore how biological insight informs drug development and therapeutic strategy. Discussion would address patient stratification and enrichment approaches, trial design, endpoint choice, and biomarker translational medicine strategy. The emphasis is on how precision approaches influence decisions about which intervention is appropriate for which patient, and under what evidentiary conditions.

- Given a biological hypothesis, what intervention do we choose for which patient population?
- How is precision medicine incorporated across the clinical development stages
- How do we ensure generalizability of biomarker results from studies?

11:00am **Session Overview**

Michael Irizarry, Eisai, *Planning Committee Member*

11:05am **Speaker Presentations**

Toby Ferguson, Alnylam Therapeutics

Hugh Marston, Boehringer Ingelheim

Larisa Reyderman, Eisai

Valentina Mantua, Food and Drug Administration

11:50am **Moderated Panel and Audience Q&A**

12:20pm **LUNCH**

1:00pm **Session 5: Precision Systems to Deliver Care at Scale**

Objectives: This session examines what happens when precision medicine approaches move from trials and centers of excellence into routine clinical care. Discussion will focus on the practical constraints that shape real-world delivery (clinical workflows, workforce capacity, infrastructure, reimbursement, ethical obligations, and health-system readiness) and how these factors determine whether precision approaches can be scaled, sustained, and deliver value to patients and health systems.

Key discussion questions:

- What breaks when precision medicine moves into real-world practice?
- How can implementation barriers be overcome?
- How do health systems decide what is worth adopting and sustaining?
- Where do ethical, economic, and clinical considerations collide?
- Why do some precision approaches stall despite strong biology?

1:00pm **Session Overview**

Bruce Korf (*virtual*), University of Alabama, Birmingham, *Planning Committee Member*

1:05pm **Speaker Presentations**

Nilufer Ertekin-Taner (*virtual*), Mayo Clinic, Florida

Soeren Mattke (*virtual*), University of Southern California

Nita Limdi (*virtual*), University of Alabama at Birmingham

1:40pm **Moderated Panel and Audience Q&A**

2:05pm **BREAK**

2:15pm **Session 6: Driving Ecosystem Investment in Scalable Precision Medicine Infrastructure for Brain Health**

Objectives: This session will explore perspectives on the precision medicine infrastructure needed to advance brain care, including shared platforms that support translation, reduce risk, and improve patient outcomes. The discussion will examine structural scientific, regulatory, financial, and governance challenges that limit scale, interoperability, and long-term sustainability and cannot be addressed by individual institutions alone. Participants will also consider public–private partnership and investment approaches and what factors may inform future action-oriented efforts to move precision medicine tools from research assets toward broader clinical and public health impact over the next several years.

Key Discussion Questions:

- What shared platforms (e.g., biomarkers, biorepositories, brain banks, AI and real-world data systems) are most critical to accelerate translation, de-risk innovation, and improve patient outcomes, and what cross-cutting scientific, regulatory, financial, and governance constraints limit their interoperability, adoption, scalability, and long-term sustainability—particularly those that cannot be addressed by individual institutions or stakeholders alone?
- What lessons can be drawn from existing partnership and investment models (e.g., ARPA-H, IHI, industry consortia) to mobilize coordinated national investment and leverage international collaboration, share risk, and ensure tools are built as durable, accessible infrastructure rather than fragmented pilots?
- What elements would be important to include in an actionable roadmap that outlines priority investments, partnership structures, key institutions, and policy levers required over the next 3–5 years to scale precision medicine tools from research assets into routine clinical and public health impact?

2:15pm **Session Overview**

Magali Haas, Cohen Veterans Bioscience, Psilera, *Planning Committee Member*

Elisabetta Vaudano (virtual), Innovative Health Initiative

2:20pm **Speaker Presentations**

Martien Kas (virtual), University of Groningen

Tristan Glatard, Centre for Addiction and Mental Health

Julian Tillmann, Roche

Nathaniel Mohatt, Advanced Research Projects Agency for Health

3:00pm **Moderated Panel and Audience Q&A**

3:25pm **Session 7: Synthesis and Opportunities for Precision Medicine in Neuroscience**

Objectives: Examine the core themes that have been highlighted during the workshop. Consider next steps to advance precision medicine research and care for brain disorders.

3:25pm **Session Overview**

Rosa Canet-Avilés, California Institute for Regenerative Medicine, *Workshop Co-chair*

Gayle Wittenberg, Johnson & Johnson, *Workshop Co-chair*

3:30pm **Themes & Future Opportunities Discussion**

Session Moderators

3:55pm **Concluding Remarks**

Rosa Canet-Avilés, California Institute for Regenerative Medicine, *Workshop Co-chair*

Gayle Wittenberg, Johnson & Johnson, *Workshop Co-chair*

4:00pm **Adjourn Day 2**