



## BRAINSTORMING NEURODEGENERATION

*Leveraging Genomics, Stem Cells, Gene Therapy and Novel Clinical Trials  
for Field-wide Advancement*

IDEATION WORKSHOP

SAN FRANCISCO AIRPORT MARRIOTT WATERFRONT

APRIL 15 – 16, 2019

### MEETING GOALS:

- Discuss novel models to accelerate therapeutic development for neurodegenerative diseases (NDs)
- Discuss proof of concept examples where genomics and large datasets have enabled progress in ND
- Prioritize elements of common utility
- Explore benefits and considerations for a neutral collective effort across NDs
- Discuss incentive structures to encourage alignment
- Propose an operational framework(s) to move from concept to reality

### MONDAY, APRIL 15

*Irvine Meeting Room*

AGENDA AT-A-GLANCE	
7:30 – 8:30 AM	BREAKFAST (60 mins)
8:30 – 9:00 AM	<b>Welcome</b> <ul style="list-style-type: none"> <li>• <i>Maria Millan</i></li> </ul> <b>Meeting Objectives</b> <ul style="list-style-type: none"> <li>• <i>Clive Svendsen</i></li> </ul>
9:00 – 9:30 AM	<b>Opening Plenary – Precision Medicine and Population Genomics (30 mins)</b> <ul style="list-style-type: none"> <li>• <i>Carlos Bustamante</i></li> </ul>
9:30 – 10:15 AM	<b>Session I: Leveraging Genomics and Big Data (45 mins)</b> <ul style="list-style-type: none"> <li>• <i>Chair: Carlos Bustamante</i></li> <li>• <i>Panelists: Joshua Stuart, Ernest Fraenkel, Howard Federoff</i></li> <li>• <i>Anchoring Questions:</i> <ul style="list-style-type: none"> <li>○ <i>What can the ND community glean from current genomics approaches?</i></li> <li>○ <i>What should the ND community look to model? What should be avoided?</i></li> <li>○ <i>Where have industry efforts failed and is there an opportunity for current data platform technologies to augment probability of success?</i></li> </ul> </li> </ul>
10:15 – 10:30 AM	BREAK (15 mins)
10:30 – 11:15 AM	<b>Session II: iPSC Models, Creating Standards, Utilizing Banks (45 mins)</b> <ul style="list-style-type: none"> <li>• <i>Chair: Lorenz Studer</i></li> <li>• <i>Panelists: Kristin Baldwin, Clive Svendsen, Genie Jones, Stuart Lipton</i></li> <li>• <i>Anchoring Questions:</i> <ul style="list-style-type: none"> <li>○ <i>Do we know enough to create standards? Can more complex models (i.e., CNS organoids, chip-based approaches) be standardized at this time?</i></li> <li>○ <i>How do we utilize existing banks and establish requirements for future banks?</i></li> <li>○ <i>Where could consolidated efforts be useful to the community?</i></li> </ul> </li> </ul>

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11:15 AM – 12:00 PM	<b>Session III: Exploring a Neurodegeneration Consortium Model (45 mins)</b> <ul style="list-style-type: none"> <li>• <i>Chair: Clive Svendsen</i></li> <li>• <i>Panelists: Lucie Bruijn, Margaret Sutherland, Leslie Thompson,</i></li> <li>• <i>Anchoring Questions:</i> <ul style="list-style-type: none"> <li>○ <i>What can be learned and broadly applied from the Answer ALS model?</i></li> <li>○ <i>What is the existing ND-specific consortium landscape? What are the features &amp; assets?</i></li> <li>○ <i>Where are the gaps left by existing consortia? How could a new combined approach address those gaps?</i></li> </ul> </li> </ul>
12:00 – 1:00 PM	LUNCH (60 mins)
1:00 – 1:30 PM	<b>Plenary – Accelerating CNS Therapy Development: Examples from the Healey Center ALS Platform Trial (30 mins)</b> <i>Merit Cudkowicz</i>
1:30 – 2:15 PM	<b>Session IV: Accelerating Drug Development Based on Patient Data (45 mins)</b> <ul style="list-style-type: none"> <li>• <i>Chair: Ernest Fraenkel</i></li> <li>• <i>Panelists: Mark Frasier, Ralph Kern, Omar Khwaja, Merit Cudkowicz, David Higgins</i></li> <li>• <i>Anchoring Questions:</i> <ul style="list-style-type: none"> <li>○ <i>How can the ND community leverage genomic and clinical data, patient-derived iPSCs, etc. to accelerate drug development? What are the challenges?</i></li> <li>○ <i>Are there current exemplars in other disease areas that the ND community should look to model?</i></li> <li>○ <i>Does industry perceive value in a consortium approach utilizing pooled data sets?</i></li> <li>○ <i>Patient perceived value in participation</i></li> </ul> </li> </ul>
2:15 – 3:00 PM	<b>Session V: Clinical Trials in Regenerative Medicine – Benefits of a Consortium (45 mins)</b> <ul style="list-style-type: none"> <li>• <i>Moderator: Abba Creasey</i></li> <li>• <i>Panelists: Malin Parmar (ESC-based), Jun Takahashi (iPSC-based), Howard Federoff (Gene Therapy)</i></li> <li>• <i>Anchoring Questions:</i> <ul style="list-style-type: none"> <li>○ <i>What could a cell or gene therapy approach offer that is different from traditional/past approaches?</i></li> <li>○ <i>Are there approaches in other disease areas that the ND community should look to model?</i></li> </ul> </li> </ul>
3:00 – 3:15 PM	BREAK (15 mins)
3:15 – 3:45 PM	<b>Plenary – Regulatory Pathway for Novel Therapies in Neurodegeneration (30 mins)</b> <ul style="list-style-type: none"> <li>• <i>Wilson Bryan</i></li> </ul>
3:45 – 4:15 PM	<b>Wrap up &amp; Close</b> <ul style="list-style-type: none"> <li>• <i>Maria Millan</i></li> <li>• <i>Kent Fitzgerald</i></li> </ul>
6:00 – 8:30 PM	DINNER at <b>Trapeze</b> (266 Lorton Ave, Burlingame, CA 94010)

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TUESDAY, APRIL 16

Irvine Meeting Room

AGENDA AT-A-GLANCE	
7:30 – 8:30 AM	BREAKFAST (60 mins)
8:30 – 9:00 AM	<b>Plenary – Federal Funding Initiatives in Neurodegeneration (30 mins)</b> <ul style="list-style-type: none"> <li>Walter Koroshetz</li> </ul>
9:00 – 9:45 AM	<b>Session VI: Taking Regenerative Medicine ND Candidates to the Clinic (45 mins)</b> <ul style="list-style-type: none"> <li>Chair: Daniela Bota</li> <li>Panelists: Wilson Bryan, Robert Pacifici, Marg Sutherland</li> <li>Anchoring Questions: <ul style="list-style-type: none"> <li>Regulatory considerations and challenges for consortium sponsored trials</li> <li>What regulatory advantages or potential challenges would a consortium model pose to the development of novel endpoints, access to expedited regulatory designations and the use of adaptive trial design for ND?"</li> <li>What are the key infrastructure gaps preventing advancement of regenerative ND candidates to the clinic?</li> <li>What solutions could be applicable (e.g., data platforms for imaging and surrogate measures, registries and data repositories, etc.)?</li> </ul> </li> </ul>
9:45 – 10:00 AM	BREAK (15 mins)
10:00 – 11:30 AM	<b>Breakout Session: Assessing the Value of a Neurodegeneration Consortium (90 mins)</b> <i>Key Questions for Consideration:</i> <ul style="list-style-type: none"> <li><b>Feasibility</b>– Is the field ready for a cross-cutting ND consortium? What common challenges do current disease-specific consortia face? Where can redundancy be minimized?</li> <li><b>Components</b> – Given the current landscape of disease-specific consortia/efforts, what elements (e.g., platforms, processes, etc.) would it make sense to pool together/consolidate? What elements should be handled by disease-specific entities?</li> <li><b>Incentives</b> – What would incentivize stakeholders to join a neutral/centralized consortium? What challenges need to be addressed?</li> </ul>
11:30 AM – 12:15 PM	<b>Report Out &amp; Discussion (45 mins)</b> <ul style="list-style-type: none"> <li>Group leads to present key discussion points</li> <li>Full conference discussion to prioritize key points for advancement</li> </ul>
12:15 – 1:15 PM	LUNCH (60 mins)
1:15 – 2:15 PM	<b>Session VII: Would an ND Consortium Accelerate Therapy Development: Considerations for a Path Forward (60 mins)</b> <ul style="list-style-type: none"> <li>Co-Chairs: Ekemini Riley and Kent Fitzgerald</li> <li>Panelists: Katja Brose, Mark Frasier, Walter Koroshetz, Maria Millan</li> <li>Anchoring Questions: Taking the breakout discussions into consideration, what could a path forward look like?</li> </ul>
2:15 – 3:00 PM	<b>Closing Remarks (45 mins)</b> <ul style="list-style-type: none"> <li>Clive Svendsen, Maria Millan, Ekemini Riley</li> </ul>
3:00 PM	DEPARTURE

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