

Larry Goldstein, PhD
Chair, CIRM Neuro Task Force
Neuro Task Force Meeting #5
June 20, 2023





Mission Statement



OUR MISSION

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world





Aspects of CIRM's R&D Neuro Portfolio

- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden same for Tran and Clin
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions

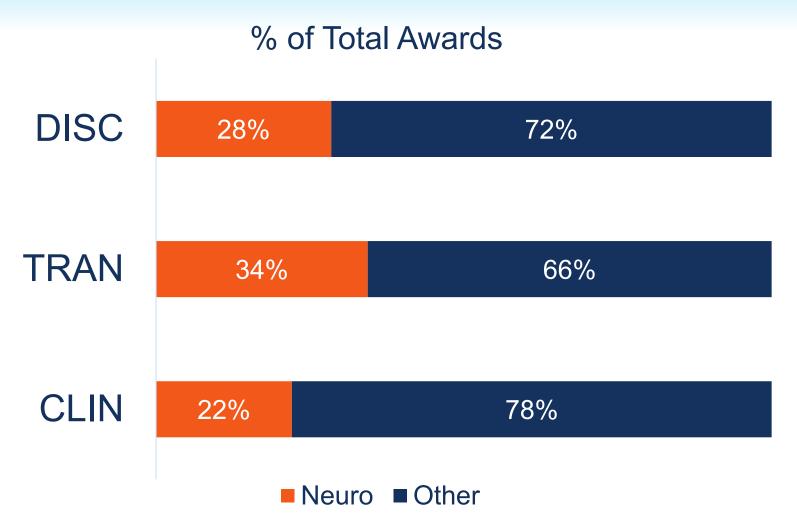


- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden same for Tran and Clin
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions



CIRM's R&D Neuro Portfolio

(2007-2023)



# Awards						
Neuro	Total					
196	689					
33	96					
30	136					

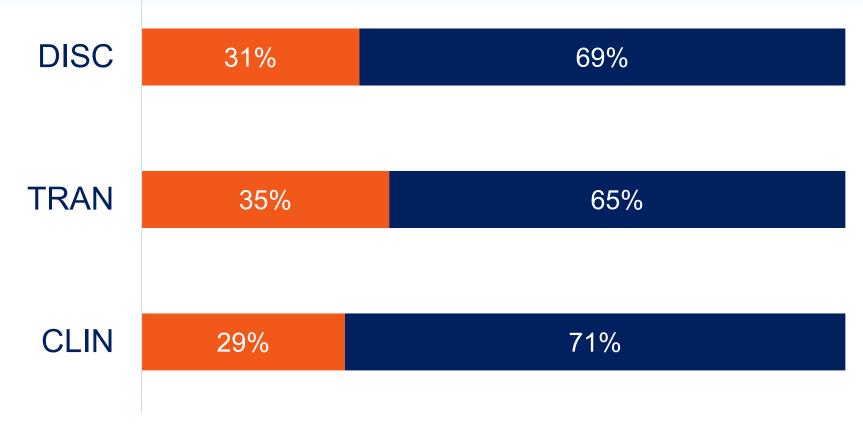
5



CIRM's R&D Neuro Portfolio

(2007-2023)

% Funds spent in Neuro/Pillar



\$ Funds (in \$M) Total Neuro 362 1.17B 581M 204 1.27B 367

■ Neuro ■ Other



- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden same for Tran and Clin
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions



Neuro Progression by Disease Area

(2007-present)

PI	Institution	Indication	DISC-Idea	DISC-candidate	TRAN	CLIN1	CUN2	
Langston/Schuele	Parkinson Inst	Parkinson's Disease						_
Schaffer	UC Berkeley	Parkinson's Disease						
Loring/Aspen	Scripps	Parkinson's Disease						28 projects progressed:
Bankiewicz	UCSF	Parkinson's Disease						
Huang	Gladstone	Alzheimer's Disease						
Schubert	Salk	Alzheimer's Disease						
Huang	UC San Diego	ALS						- > 15 DISC to
Goldstein	UC San Diego	ALS						
Svendsen	Cedars Sinai	ALS						TRAN/CLIN
Thompson	UC Irvine	Huntington's Disease						
Klassen/J Cyte	UC Irvine	Retinitis Pigmentosa						> 2% of total DISC
Seiler	UC Irvine	Retinitis Pigmentosa						owerde (690)
Svendsen	Cedars Sinai	Retinitis Pigmentosa						awards (689)
Humayun	USC	Macular Degeneration						
Anderson	UC Davis	Spinal Cord Injury						_
Heilshorn	Stanford	Spinal Cord Injury						_
Tuszynski	UC San Diego	Spinal Cord Injury						Indication Coding: Neurodegenerative Eye Neurotrauma Epilepsy Other
Carmichael/Llorente	UC Los Angeles	Spinal Cord Injury						
Cummings	UC Irvine	Traumatic Brain Inujury						
Steinberg	Stanford	Stroke						
Baulch/Achayra	UC Irvine	Radiation Brain injury						
Nicholas/Kriegstein	UCSF/Neurona	epilepsy						
Gleeson	UC San Diego	epilepsy						
Muotri	UC San Diego	Rett Syndrome						
Blurton-Jones	UC Irvine	Leukoencephaly						
Shi	City of Hope	Canavan Disease						
Grikscheit	CHLA	Enteric Neuropathy						_
Farmer	UC Davis	Spina Bifida						8

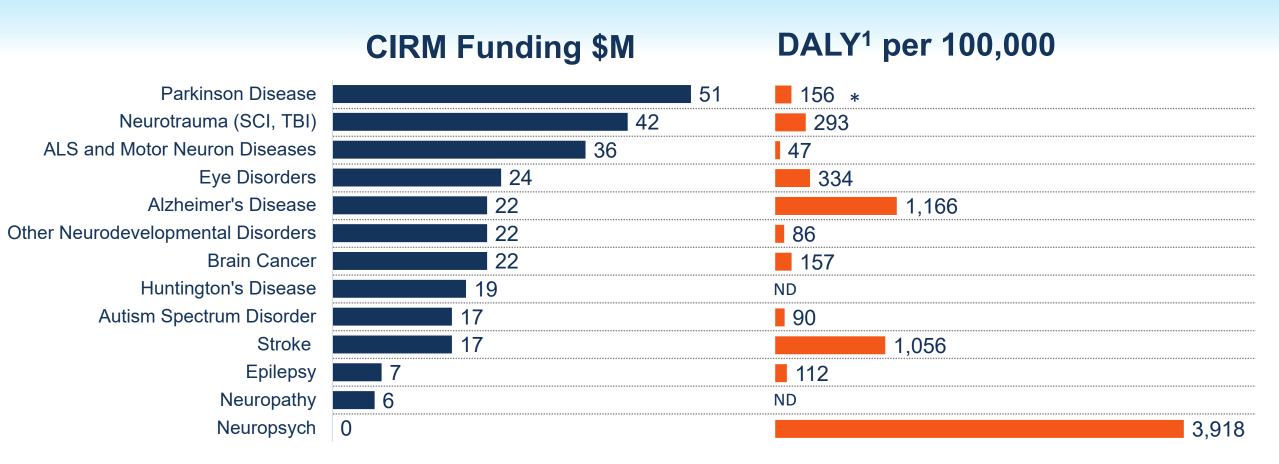


- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions



DISC R&D Neuro Portfolio by US Disease Burden

(2007-2023)



1. One disability-adjusted life year (DALY) represents the loss of the equivalent of one year of full health.

Sources: (1) CIRM portfolio data as of June 2023. (2) Global Burden of Disease Collaborative Network. Global Burden of Disease Study 2019 (GBD 2019) Reference Life Table. Seattle, United States of America: Institute for Health Metrics and Evaluation (IHME), 2021. (3) GBD 2016 Traumatic Brain Injury and Spinal Cord Injury Collaborators (2019). Global, regional, and national burden of traumatic brain injury and spinal cord injury, 1990-2016: a systematic analysis for the Global Burden of Disease Study 2016. *The Lancet. Neurology*, 18(1), 56–87.

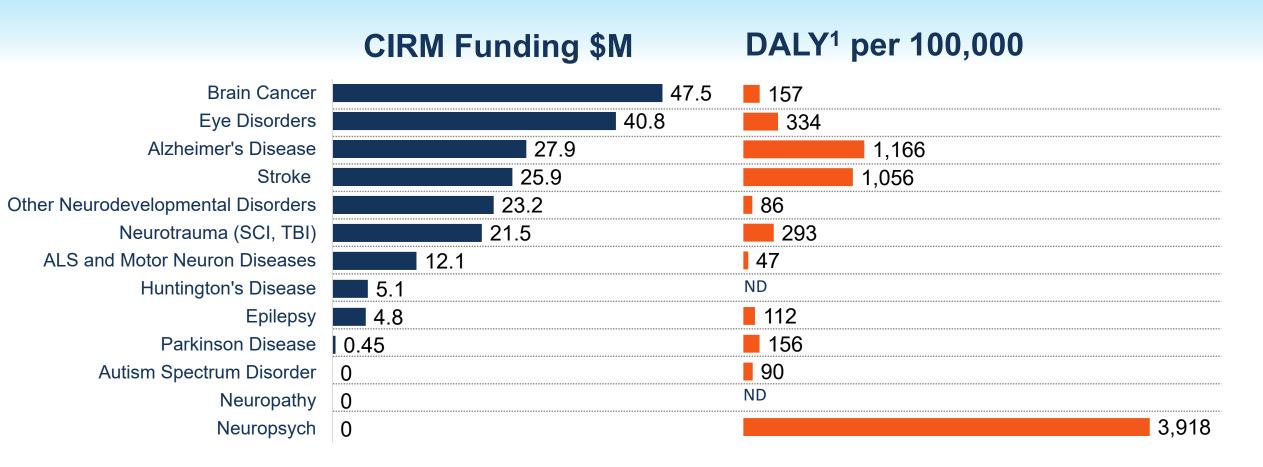


- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions



TRAN R&D Neuro Portfolio by US Disease Burden

(2007-2023)



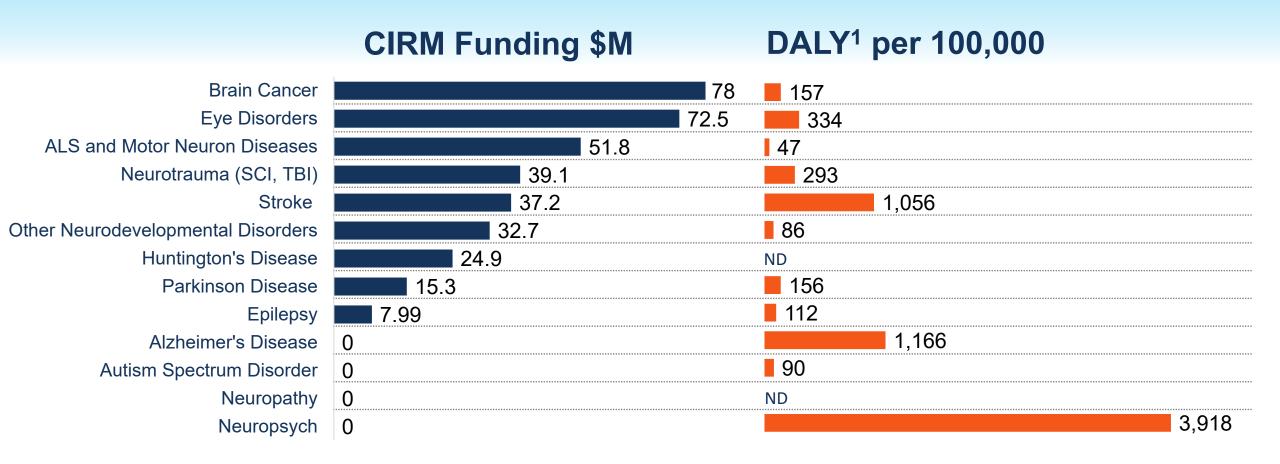
1. One disability-adjusted life year (DALY) represents the loss of the equivalent of one year of full health.

Sources: (1) CIRM portfolio data as of June 2023. (2) Global Burden of Disease Collaborative Network. Global Burden of Disease Study 2019 (GBD 2019) Reference Life Table. Seattle, United States of America: Institute for Health Metrics and Evaluation (IHME), 2021. (3) GBD 2016 Traumatic Brain Injury and Spinal Cord Injury Collaborators (2019). Global, regional, and national burden of traumatic brain injury and spinal cord injury, 1990-2016: a systematic analysis for the Global Burden of Disease Study 2016. *The Lancet. Neurology*, 18(1), 56–87.



CLIN R&D Neuro Portfolio by US Disease Burden

(2007-2023)



1. One disability-adjusted life year (DALY) represents the loss of the equivalent of one year of full health.

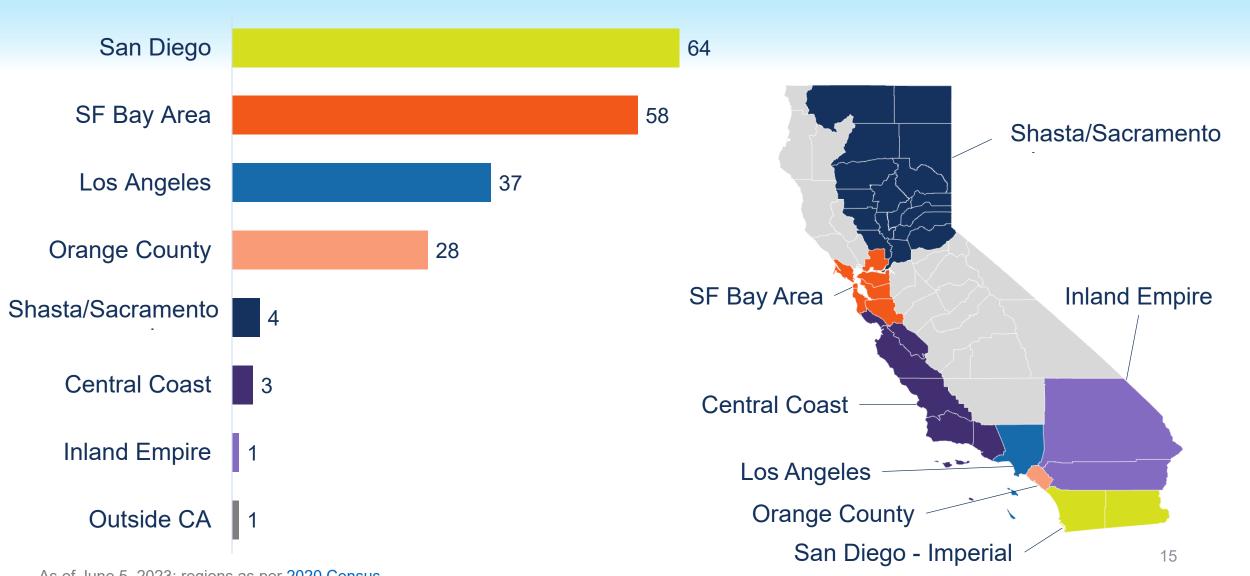
Sources: (1) CIRM portfolio data as of June 2023. (2) Global Burden of Disease Collaborative Network. Global Burden of Disease Study 2019 (GBD 2019) Reference Life Table. Seattle, United States of America: Institute for Health Metrics and Evaluation (IHME), 2021. (3) GBD 2016 Traumatic Brain Injury and Spinal Cord Injury Collaborators (2019). Global, regional, and national burden of traumatic brain injury and spinal cord injury, 1990-2016: a systematic analysis for the Global Burden of Disease Study 2016. *The Lancet. Neurology*, 18(1), 56–87.



- 1. Percent of Disc, Tran and Clin awards that are Neuro
- 2. Percent of Neuro Disc awards that progressed to Tran or Clin
- 3. Percent of Disc awards that are Neuro relative to disease burden
- 4. Percent of Tran and Clin awards relative to disease burden in population
- 5. Distribution of Neuro awards relative to geography of academic and medical research institutions

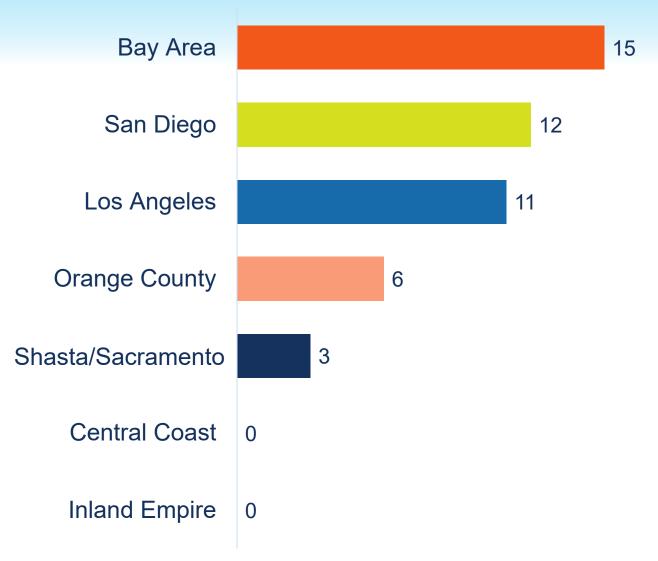


DISC R&D Neuro Awards by Region





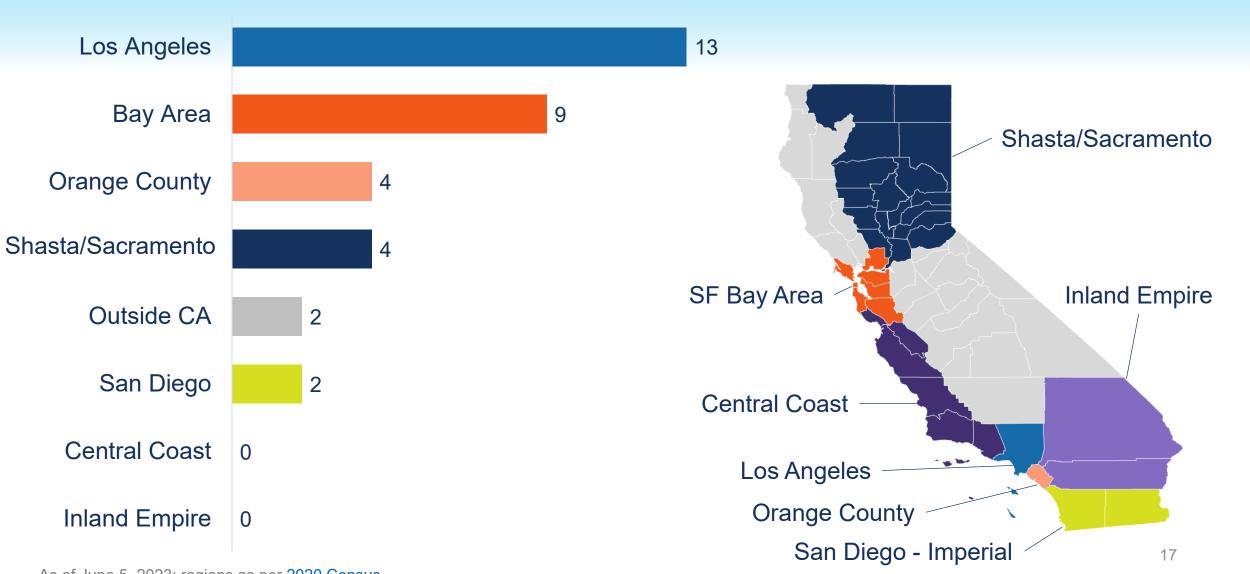
TRAN R&D Neuro Awards by Region







CLIN R&D Neuro Awards by Region





Questions for the Task Force (June 2023)

- 1. What percentage of \$1.5B+ does task force want to make recommendations for?
- 2. What percentage of \$1.5B+ should be allocated based on quality/novelty of ideas and approach as assessed by grants working group as opposed to centralized planning?
- 3. Are there any important areas of Neuro research that we are missing?