

Neurodegeneration Portfolio Analysis

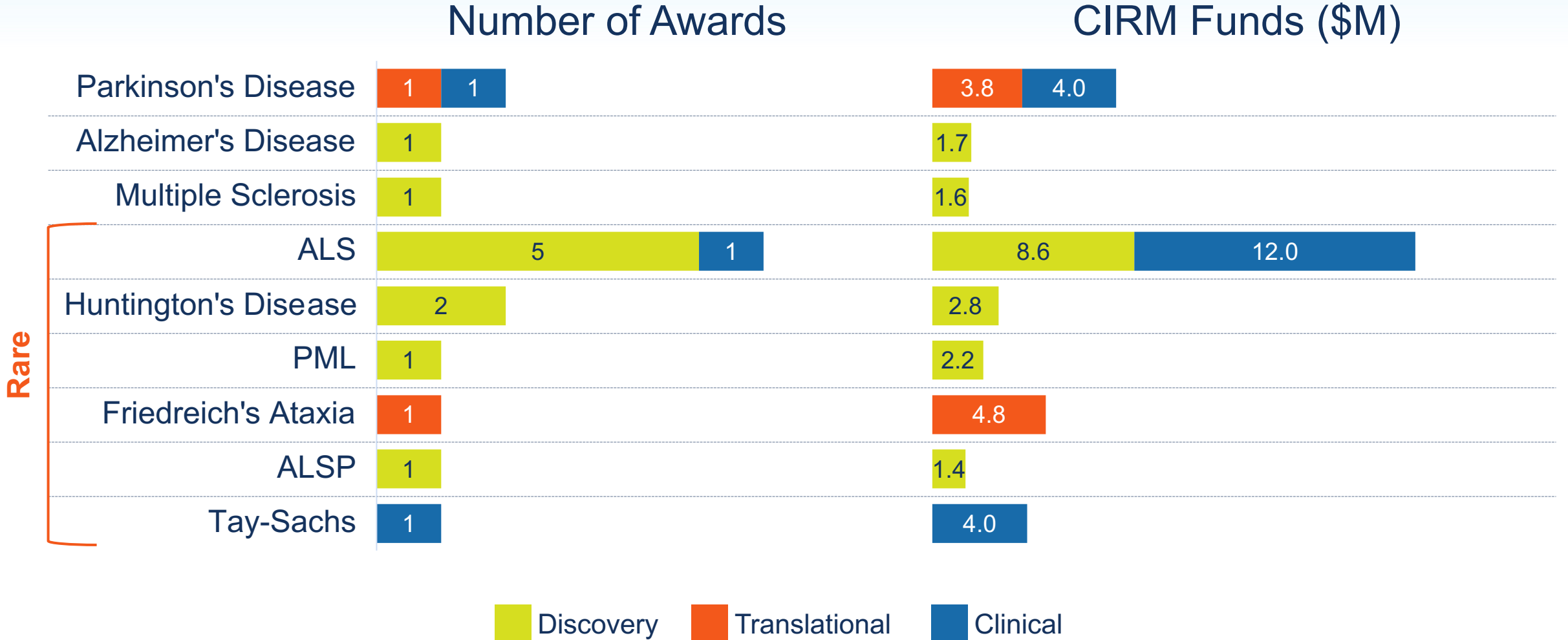
Rosa Canet-Avilés, PhD
Vice President, Scientific Programs & Education
CIRM Science Subcommittee Meeting

Neuro Task Force – March 22nd 2024



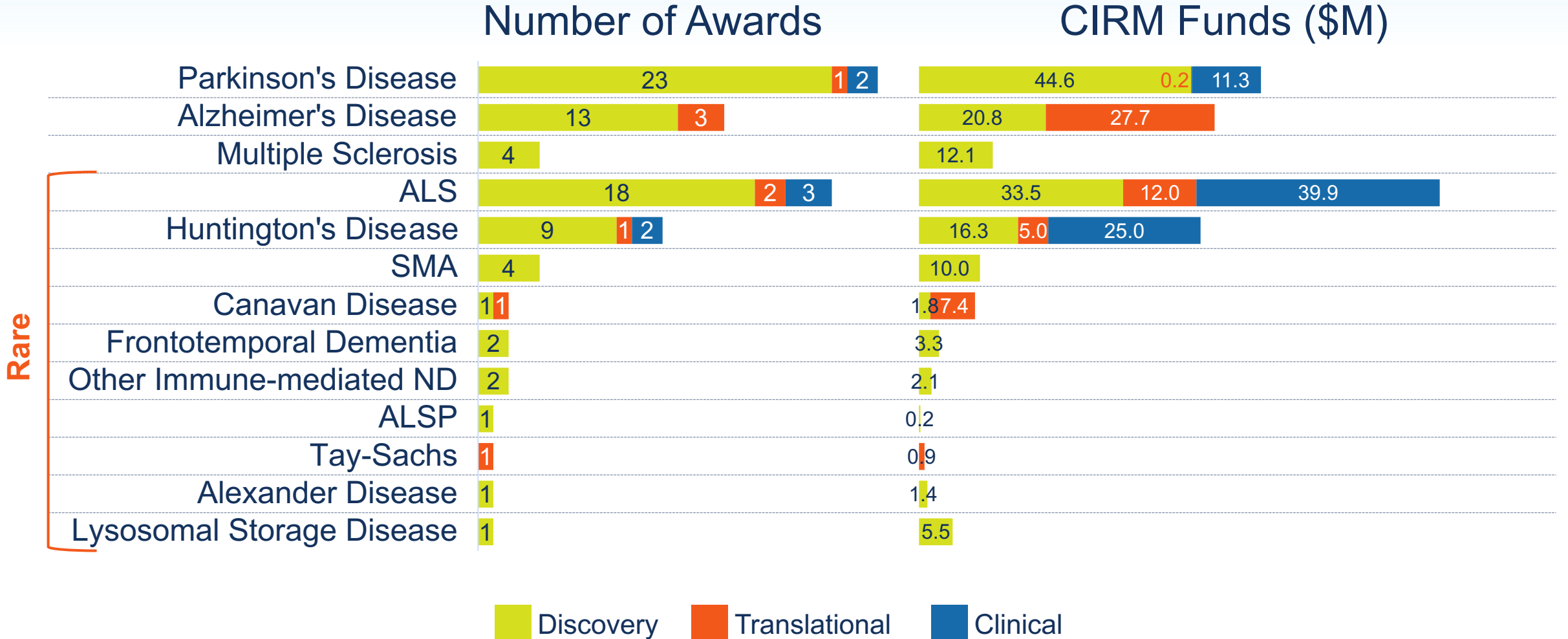
Neurodegeneration Portfolio Awards by Disease

(Prop 14 - 2021-2023)



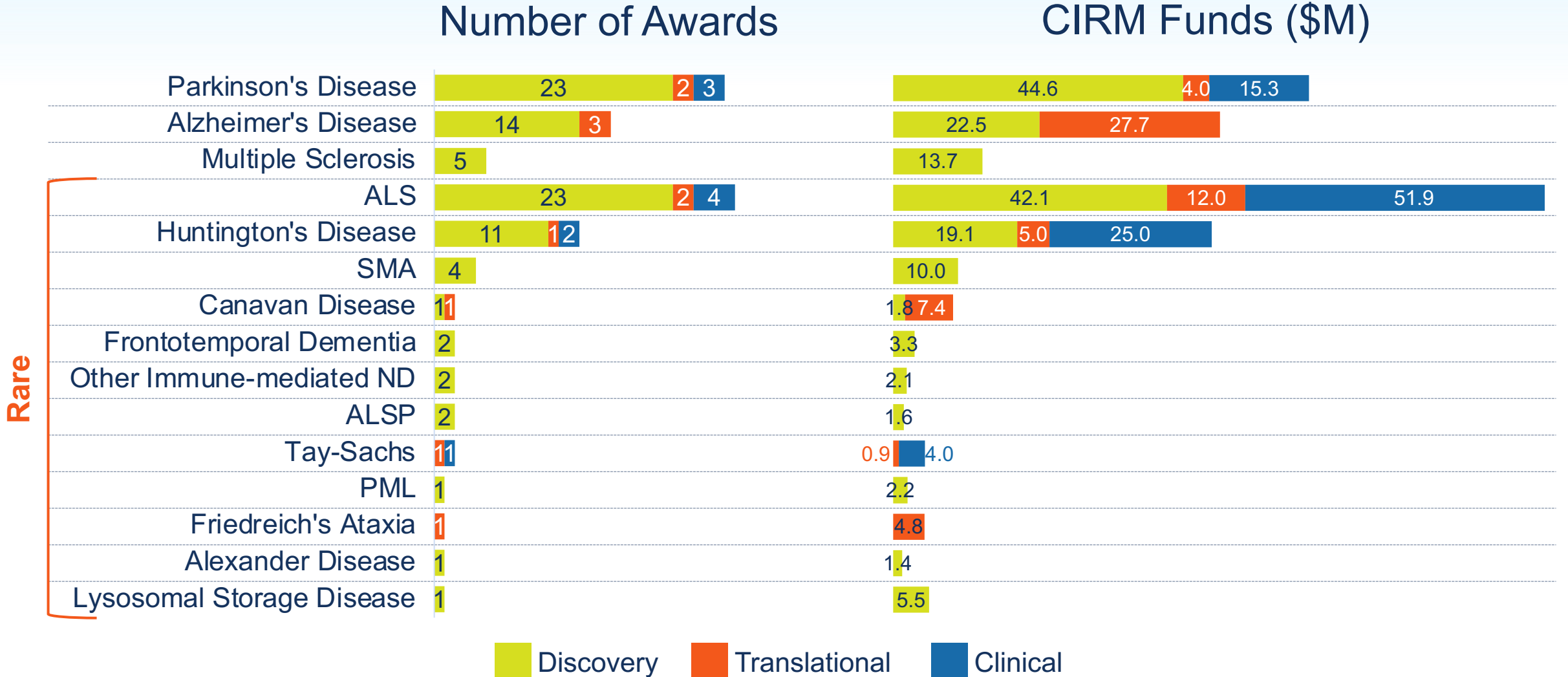
Neurodegeneration Portfolio Awards by Disease

(Prop 71 - 2007-2021)



Neurodegeneration Portfolio Awards by Disease

(Prop 71 + Prop 14 - 2007-2023)

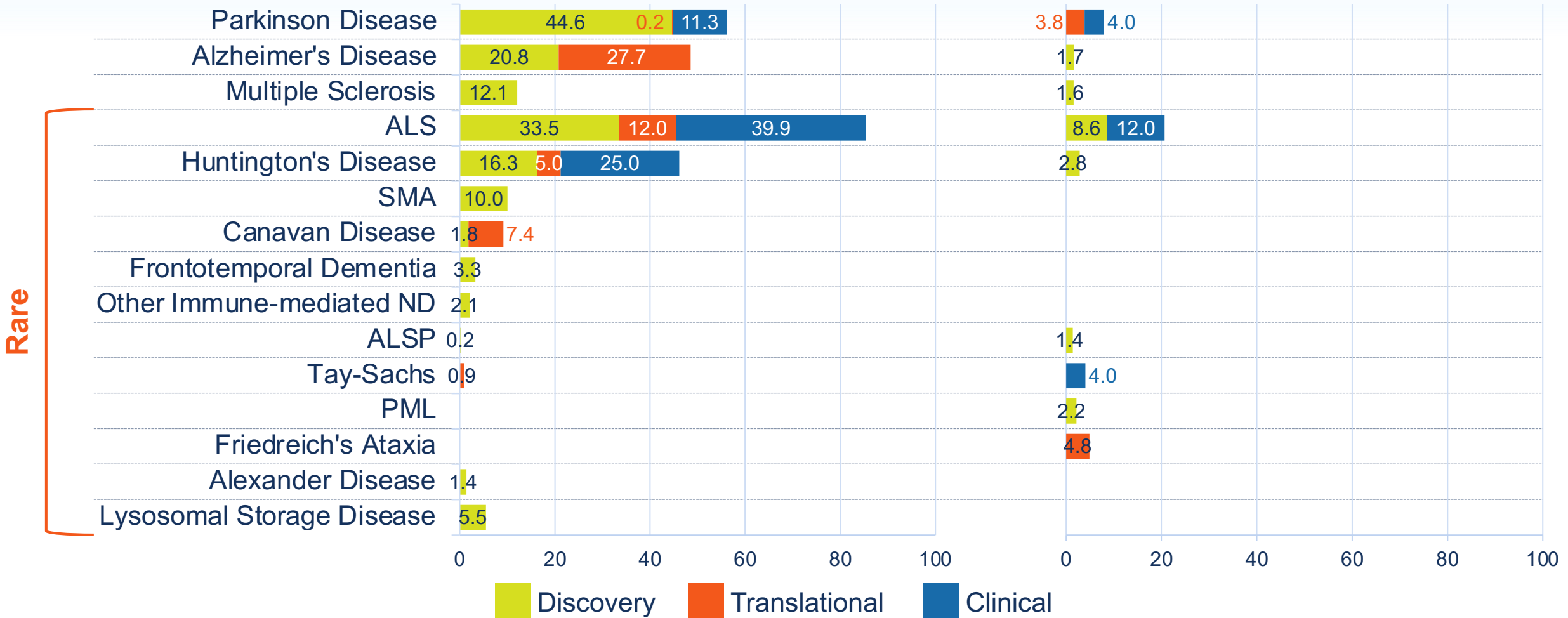


Neurodegeneration Portfolio Spending by Disease

Spending (\$M)

Prop 71 (2007-2021)

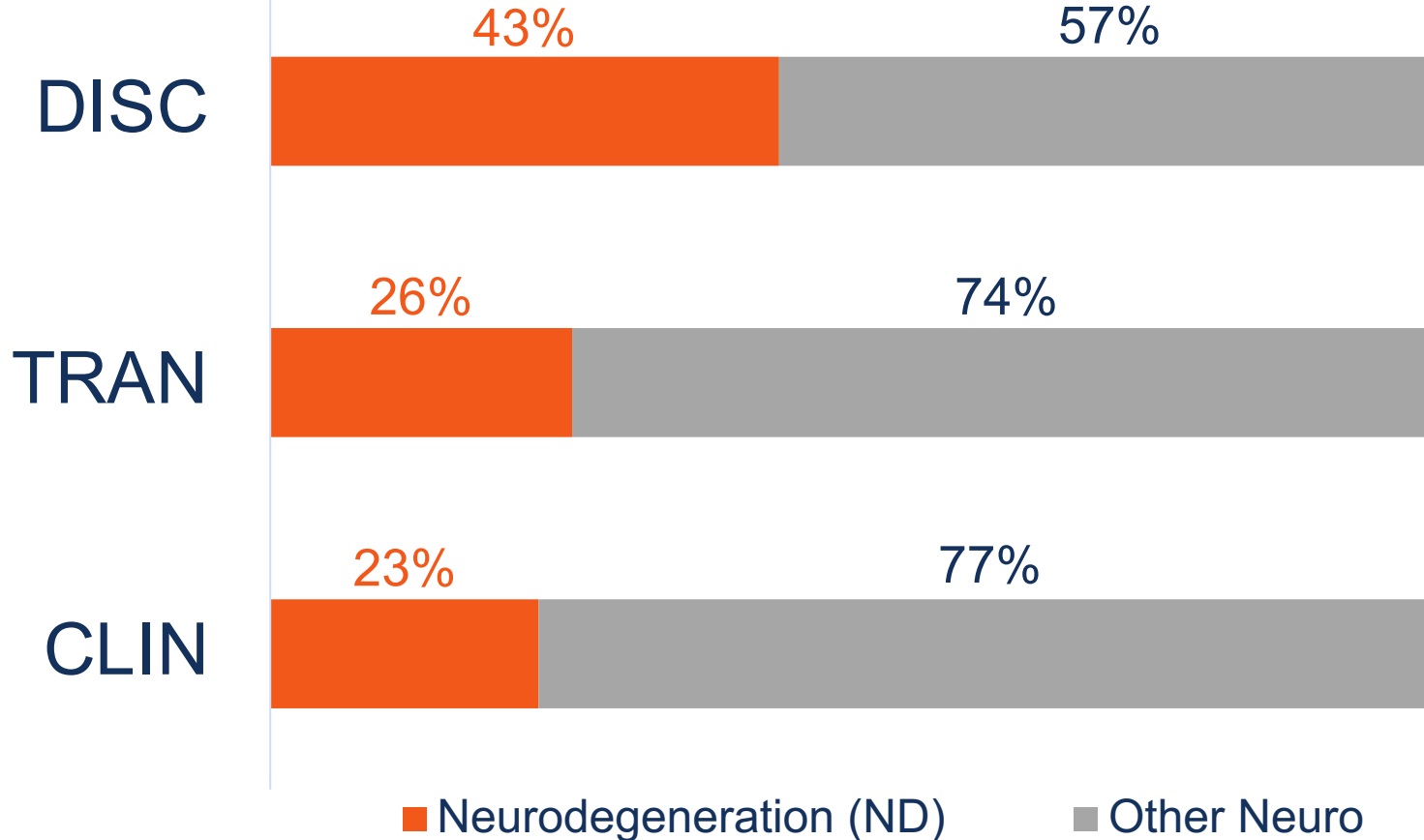
Prop 14 (2021-2023)



CIRM's R&D Neurodegeneration Portfolio

(Prop 71 + Prop 14 - 2007-2023)

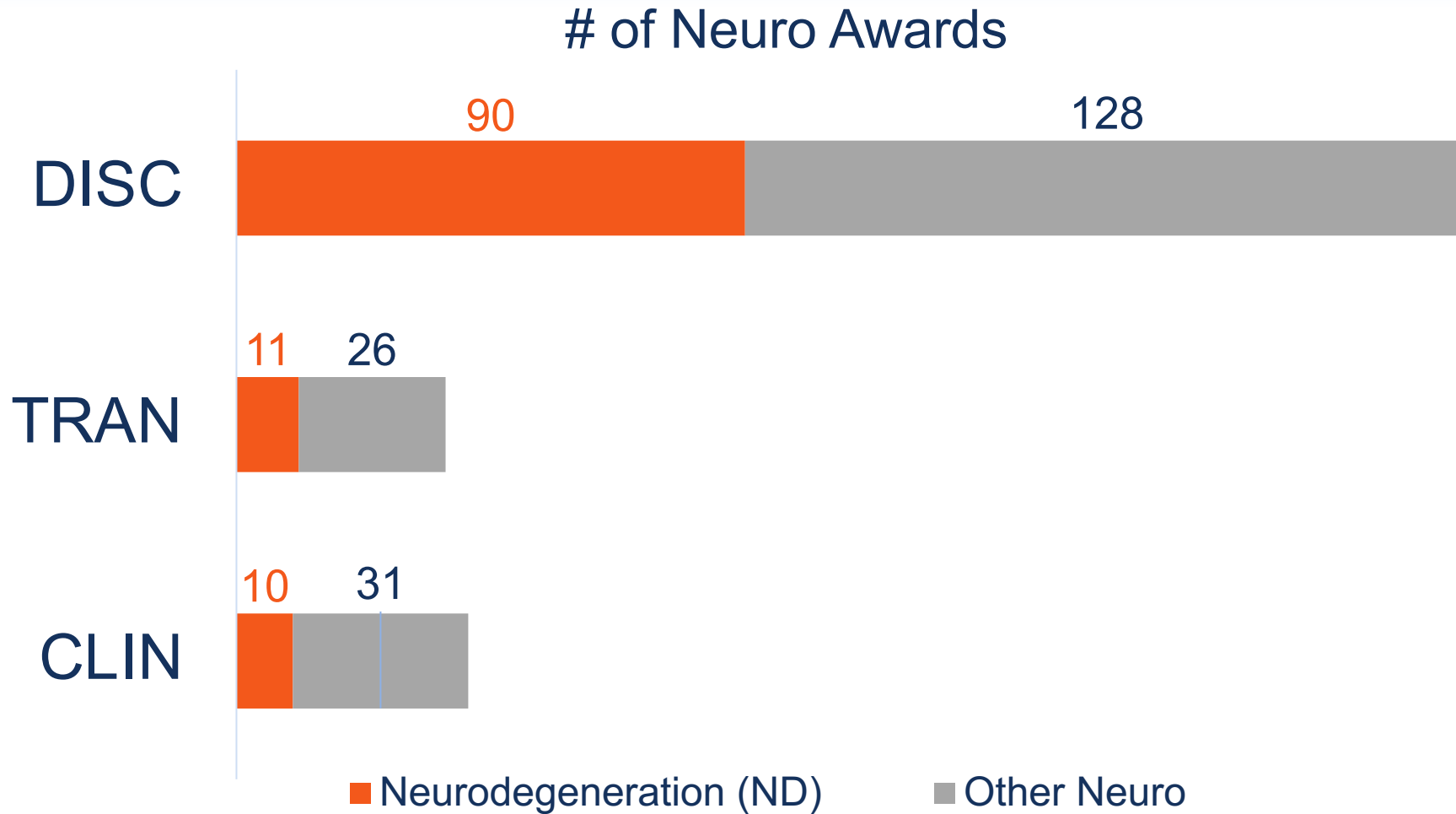
% of Neuro Spending



CIRM Funds	
ND	Total Neuro
\$169.8M	\$390.7M
\$61.8M	\$239.9M
\$96.2M	\$419.3M

CIRM's R&D Neurodegeneration Portfolio

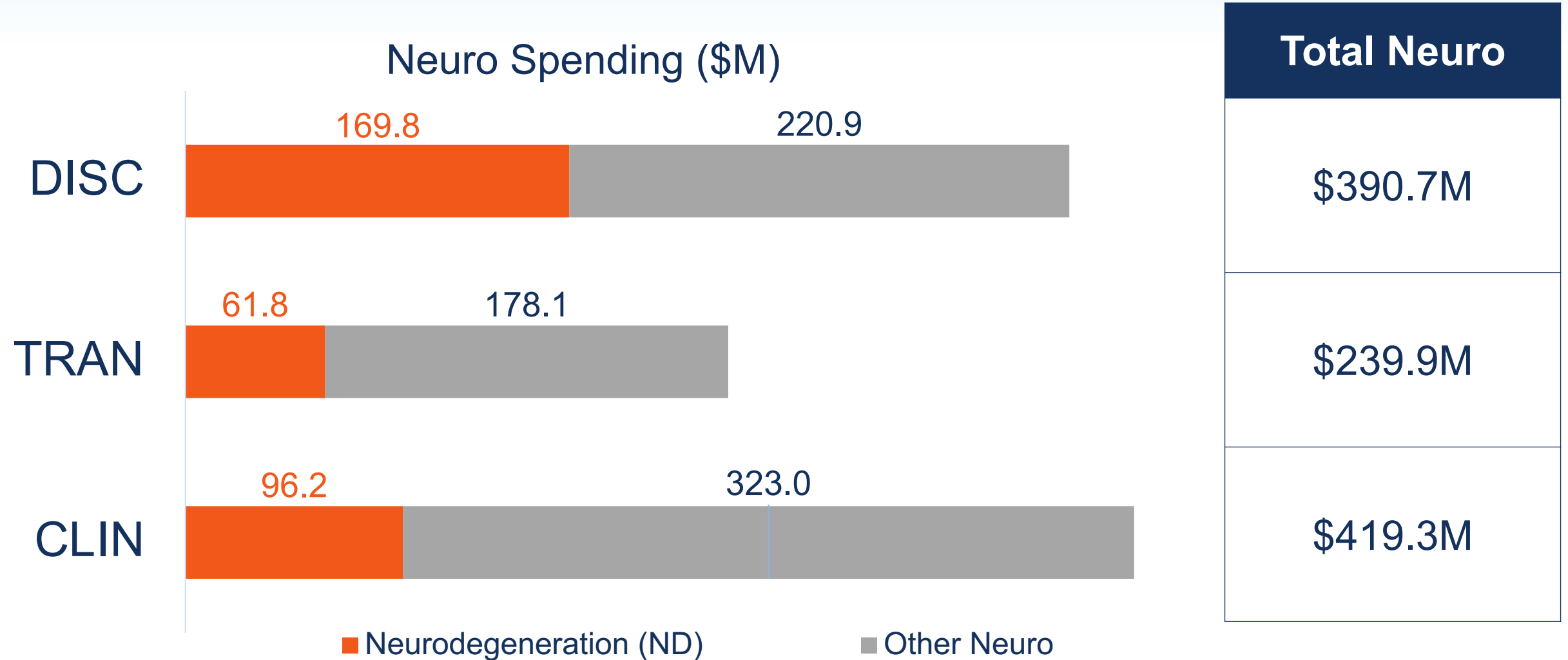
(Prop 71 + Prop 14 - 2007-2023)



Total Awards
218
37
41

CIRM's R&D Neurodegeneration Portfolio

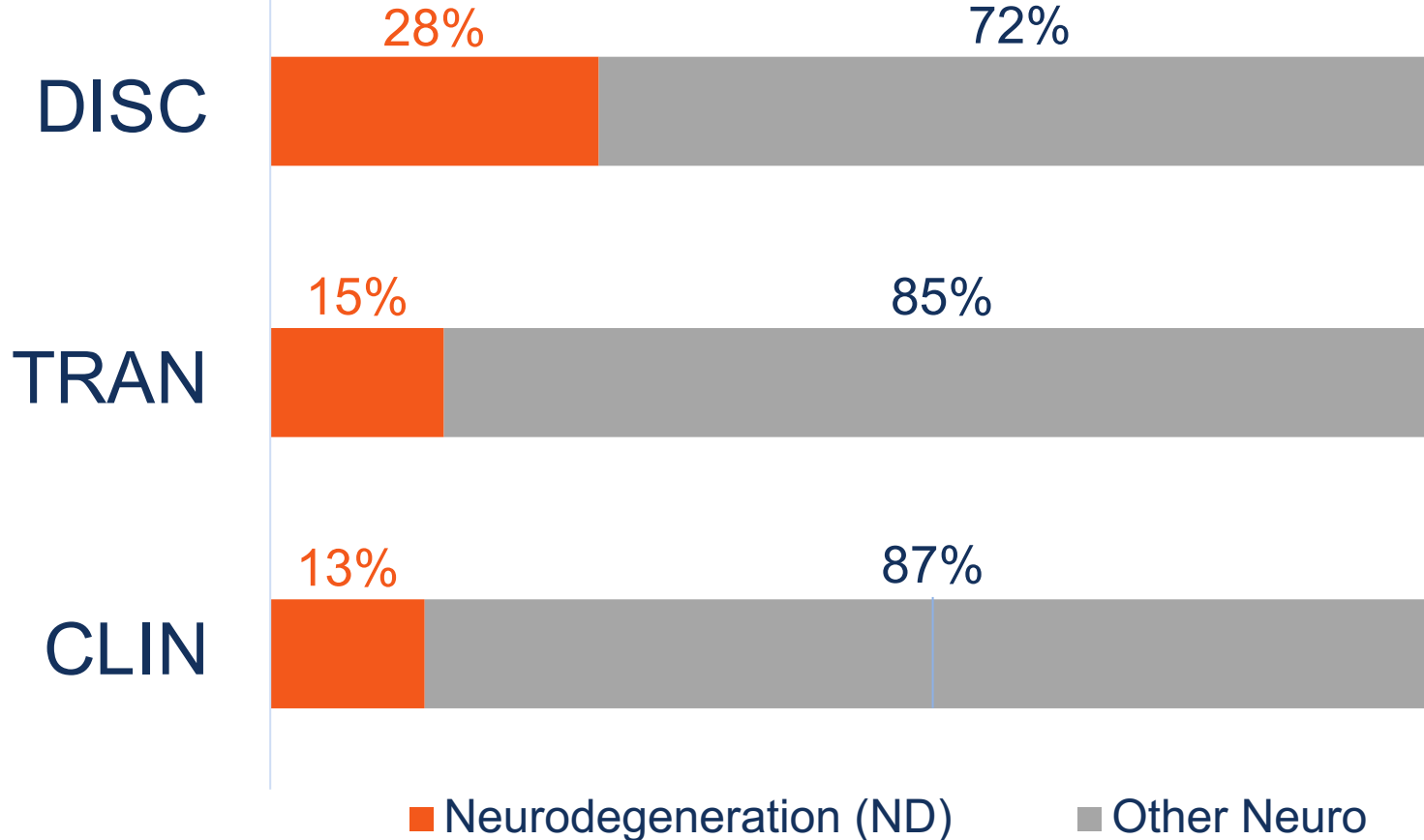
(Prop 71 + Prop 14 - 2007-2023)



CIRM's R&D Neurodegeneration Portfolio

(Prop 14 - 2021-2023)

% of Neuro Spending

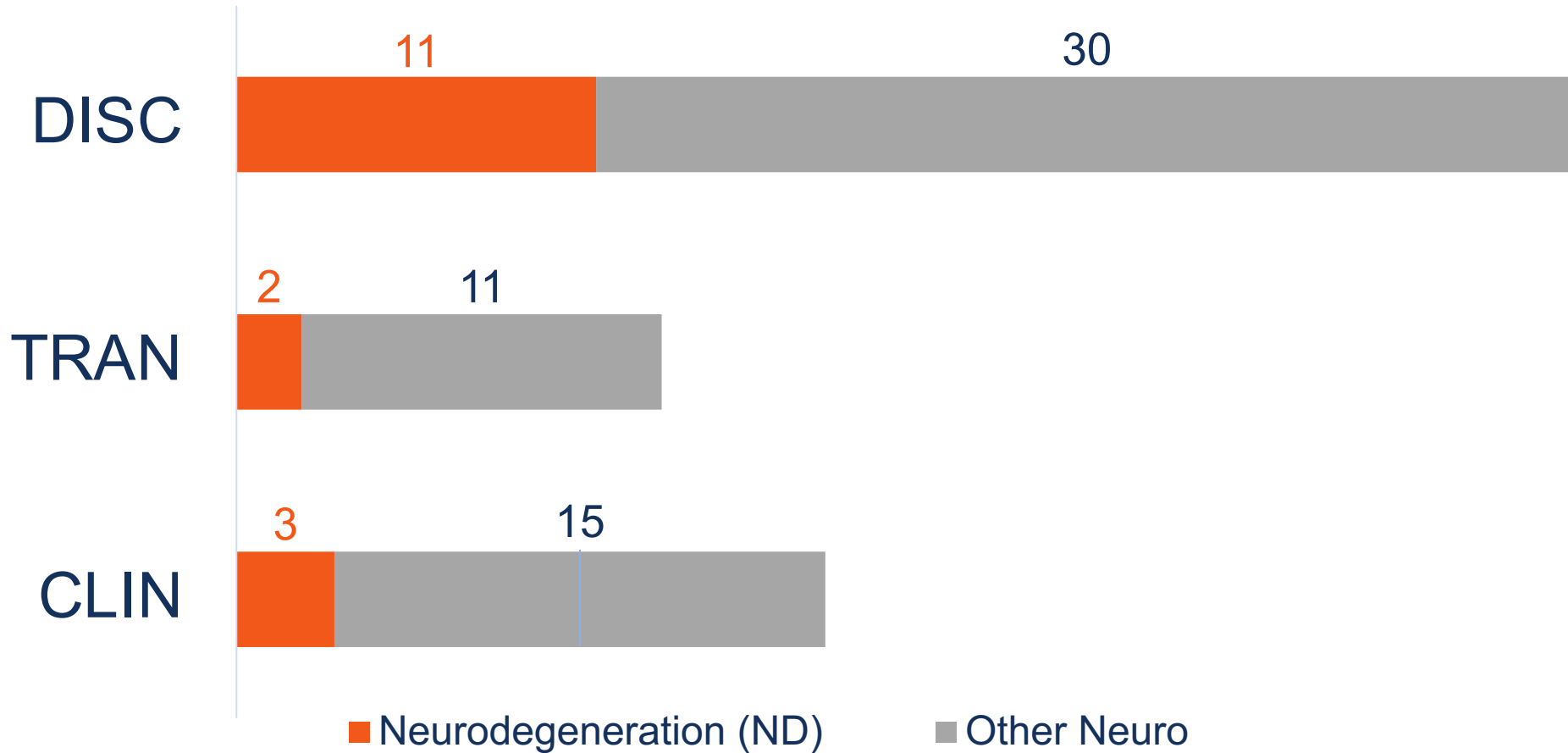


CIRM Funds	
ND	Total Neuro
\$18.3M	\$65.1M
\$8.7M	\$58.6M
\$20M	\$151.7M

CIRM's R&D Neurodegeneration Portfolio

(Prop 14 - 2021-2023)

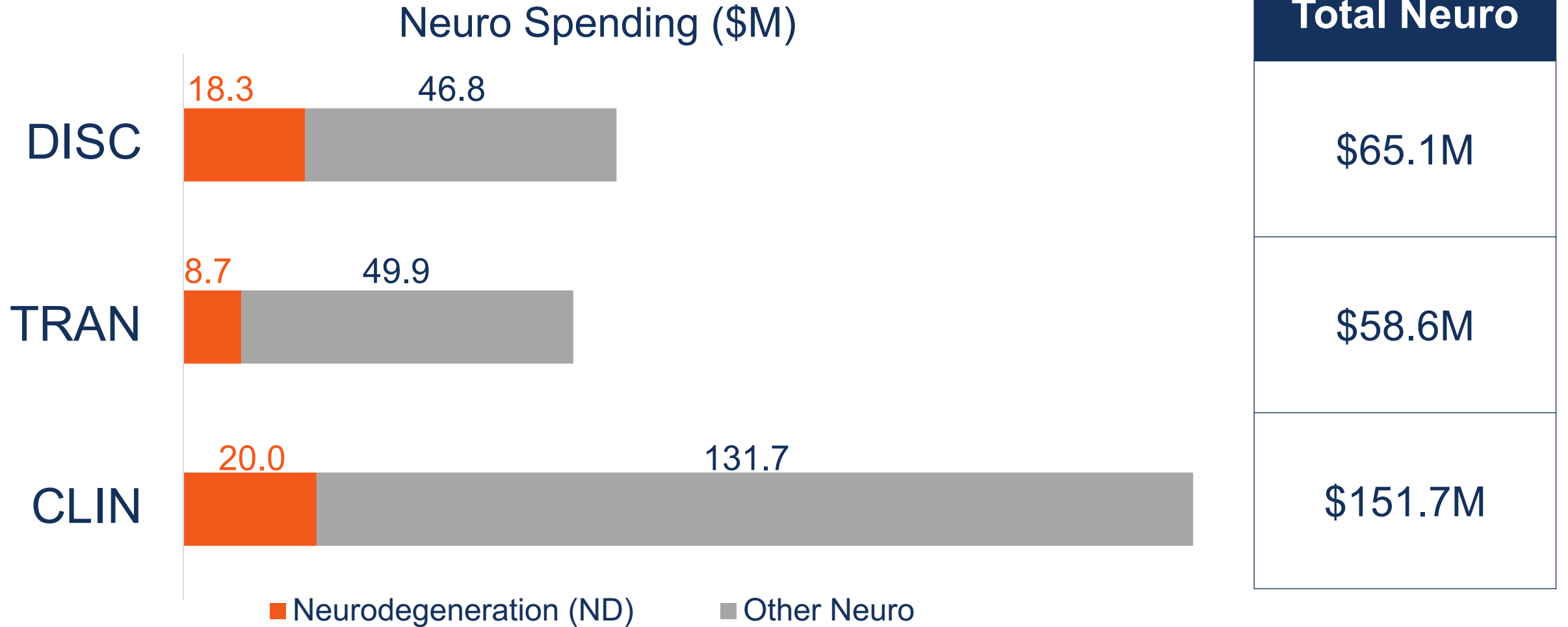
of Neuro Awards



Total Awards
41
13
18

CIRM's R&D Neurodegeneration Portfolio

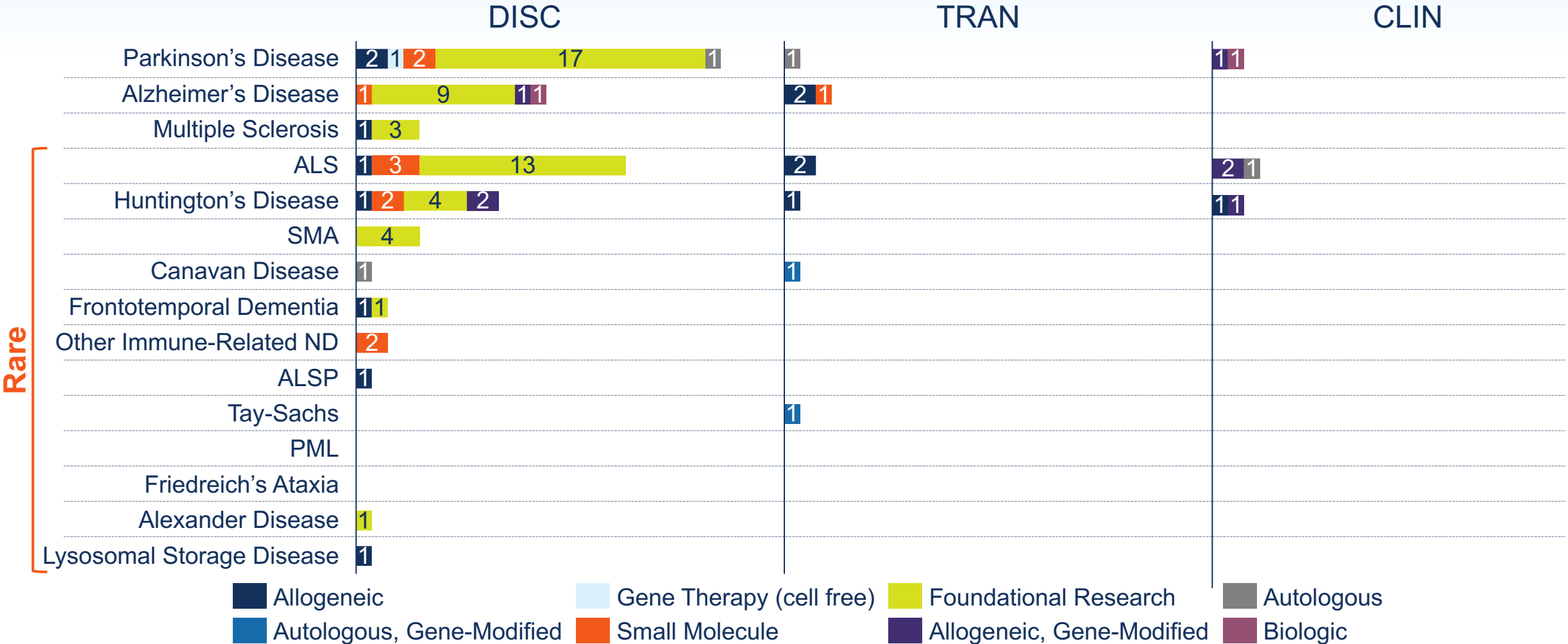
(Prop 14 - 2021-2023)



Neurodegeneration Portfolio Awards by Approach

(Prop 71 - 2007-2021)

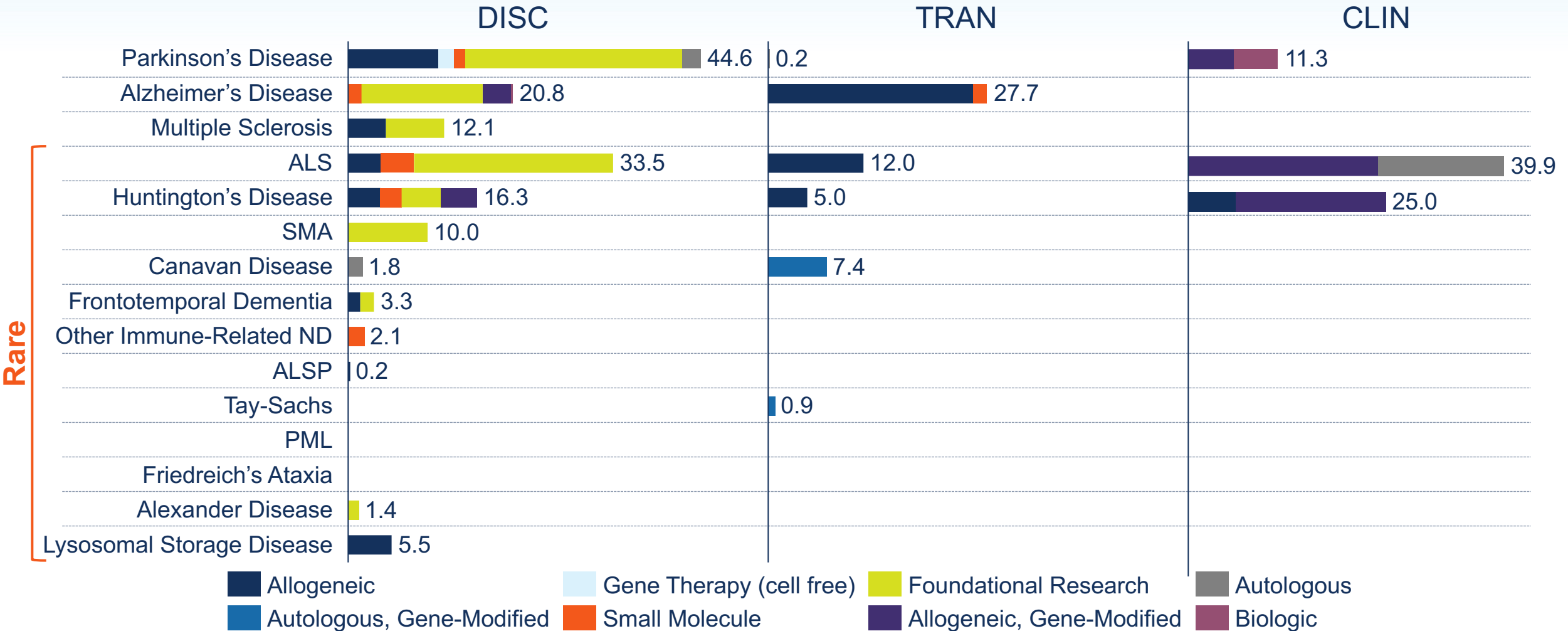
Number of Awards



Neurodegeneration Portfolio Spending by Approach

(Prop 71 - 2007-2021)

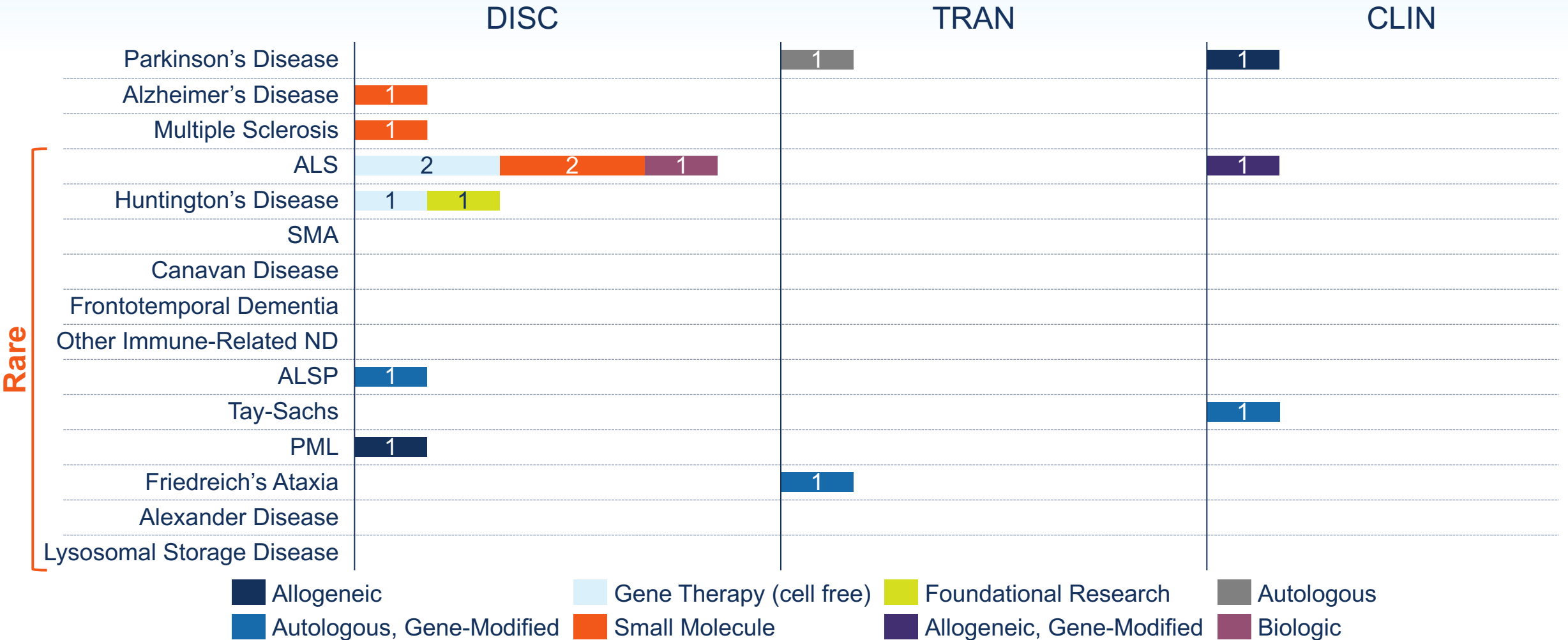
CIRM Funds (\$M)



Neurodegeneration Portfolio Awards by Approach

(Prop 14 - 2021-2023)

Number of Awards

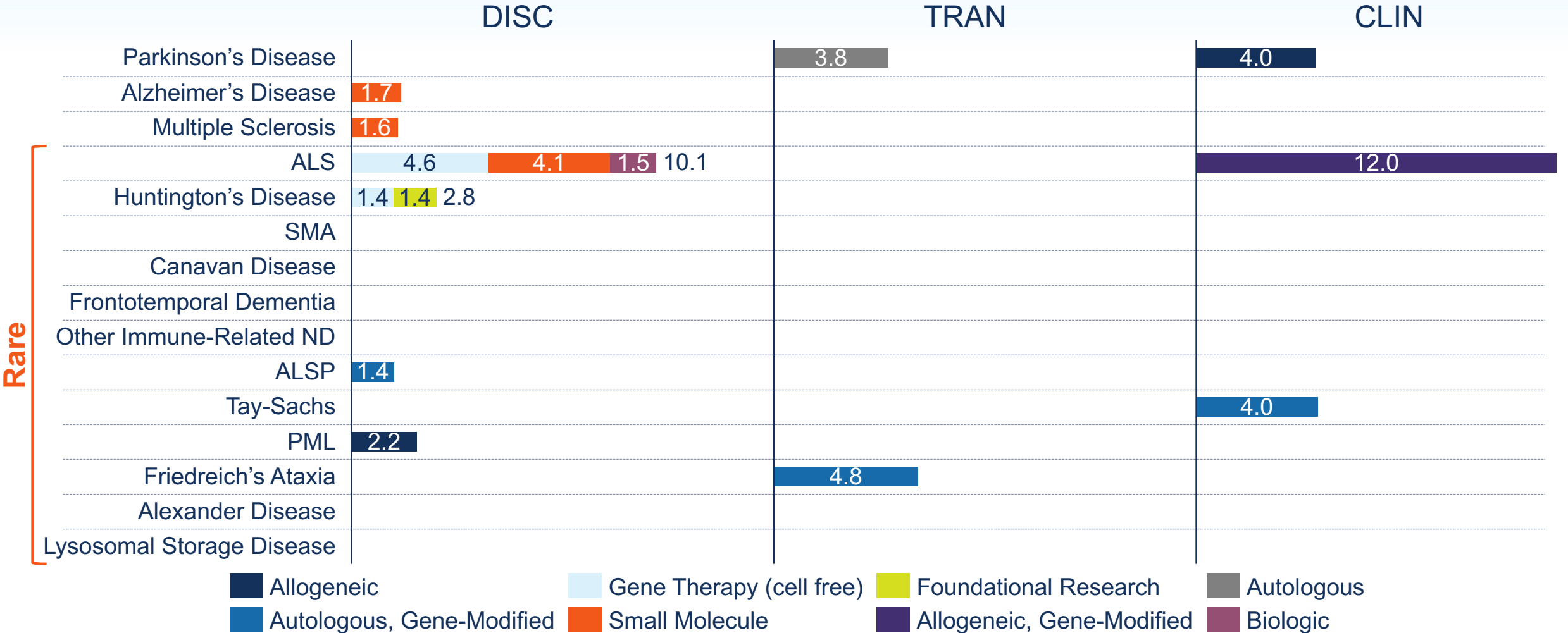


Data as of February 2024; ALS: Amyotrophic Lateral Sclerosis; SMA: Spinal Muscular Atrophy; ND: Neurodegeneration; ALSP: Adult-onset Leukoencephalopathy With Axonal Spheroids and Pigmented Glia; PML: Progressive Multifocal Leukoencephalopathy

Neurodegeneration Portfolio Spending by Approach

(Prop 14 - 2021-2023)

CIRM Funds (\$M)



Data as of February 2024; ALS: Amyotrophic Lateral Sclerosis; SMA: Spinal Muscular Atrophy; ND: Neurodegeneration; ALSP: Adult-onset Leukoencephalopathy With Axonal Spheroids and Pigmented Glia; PML: Progressive Multifocal Leukoencephalopathy

Neuro Progression Events (2007-2023)

PI	Institution	Approach	Indication	DISC - Idea	DISC - Candidate	TRAN	CLIN1	CLIN2
Langston/Schuele	Parkinson Inst	Gene therapy (cell free)	Parkinson's Disease					
Blurton-Jones/Ghandi	UC Irvine/Novoglia	Allogeneic	ALSP					
Gleeson/Kim-McManus	UC San Diego	Gene therapy (cell free)	Epilepsy/SCN2A					
Huang	UC San Diego	Cell Therapy	ALS					
Muotri	UC San Diego	Small Molecule	Rett Syndrome					
Schaffer	UC Berkeley	Allogeneic	Parkinson's Disease					
Heilshorn	Stanford	Autologous	Spinal cord injury					
Zholudeva	Gladstone	Allogeneic	Spinal cord injury					
Baulch/Achayra	UC Irvine	Biologic	Radiation brain injury					
Blurton-Jones	UC Irvine	Autologous, Gene-Modified	Sanfilippo Syndrome					
Carmichael/Llorente	UC Los Angeles/PAVIR	Allogeneic	Stroke					
Cummings	UC Irvine	Allogeneic	Traumatic brain injury					
Grikscheit	CHLA	Autologous	Enteric neuropathy					
Huang	Gladstone	Cell Therapy	Alzheimer's Disease					
Shi	City of Hope	Autologous	Canavan Disease					
Schubert	Salk	Small Molecule	Alzheimer's Disease					
Goldstein	UC San Diego	Allogeneic	ALS					
Anderson	UC Irvine	Allogeneic	Spinal cord injury					
Loring/Aspen	Scripps	Autologous	Parkinson's Disease					
Seiler	UC Irvine	Allogeneic	Retinitis Pigmentosa					
Thompson	UC Irvine	Allogeneic	Huntington's Disease					
Tuszynski	UC San Diego	Allogeneic	Spinal cord injury					
Nicholas/Kriegstein	UCSF/Neurona	Allogeneic	Epilepsy					
Klassen/J Cyte	UC Irvine	Allogeneic	Retinitis Pigmentosa					
Steinberg	Stanford	Allogeneic	Stroke					
Anderson	UC Davis	Autologous, Gene-Modified	Tay-Sachs					
Lebkowski	Regenerative Patch Technologies	Allogeneic	Geographic atrophy					
Farmer	UC Davis	Allogeneic	Spina bifida					
Svendsen	Cedars Sinai	Allogeneic, Gene-Modified	ALS					
Svendsen	Cedars Sinai	Allogeneic	Retinitis Pigmentosa					
Svendsen/Bankiewicz	UCSF	Allogeneic, Gene-Modified	Parkinson's Disease					
Humayun	USC	Allogeneic	Macular Degeneration					

Neurodegeneration Progression Events (2007-2023)

PI	Institution	Approach	Indication	DISC - Idea	DISC - Candidate	TRAN	CLIN1	CLIN2
Langston/Schuele	Parkinson Inst	Gene therapy (Cell Free)	Parkinson's Disease					
Schaffer	UC Berkeley	Allogeneic	Parkinson's Disease					
Loring/Bratt-Leal/Wirth	Scripps/Aspen	Autologous	Parkinson's Disease					
Schubert	Salk	Small Molecule	Alzheimer's Disease					
Huang	Gladstone	Cell Therapy	Alzheimer's Disease					
Huang	UC San Diego	Cell Therapy	ALS					
Goldstein	UC San Diego	Allogeneic	ALS					
Blurton-Jones/Gandhi	UC Irvine/Novoglia	Autologous	ALSP					
Shi	City of Hope	Autologous	Canavan Disease					
Thompson	UC Irvine	Allogeneic	Huntington's Disease					
Anderson	UC Davis	Autologous, Gene-Modified	Tay-Sachs					
Svendsen	Cedars Sinai	Autologous, Gene- Modified	ALS					
Svendsen/Bankiewicz	UCSF	Allogeneic, Gene-Modified	Parkinson's Disease					

Rare

Neurodegeneration Partnering Activities (2007-2024)

Pillar	Approach	Indication	Partnership Activity
Discovery	Foundational Research	ALS & SMA	Locana Bio Spinout raised \$155M VC before closing
	Foundational Research	Parkinson's Disease	Acurex, Inc. Spinout grant-funded, clinical trials ongoing
	Cell Therapy	Parkinson's Disease	Accurexa Spinout (status unknown) Clearpoint Neuro Licensed
	Autologous Cell Therapy	Parkinson's Disease	Aspen Neuroscience Spinout raised \$200M+ Series A & B, clinical trials ongoing
	Allogeneic Cell Therapy	ALSP	NovoGlia Spinout awarded its own DISC2
Translation	Gene-Modified Cell Therapy	Friedreich's Ataxia	Papillon Therapeutics Spinout
Clinical	Allogeneic Cell Therapy	Huntington's Disease	Unnamed Startup Spinout in collaboration with AgeX
	Allogeneic Cell Therapy	Parkinson's Disease	Kenai Therapeutics Awardee (formerly Ryne Bio) raised \$82 Series A Financing
	Autologous Cell Therapy	ALS & SMA	Brainstorm Awardee raised additional funding after award ended, clinical trials ongoing
	Biologic	Parkinson's Disease	Brain Neurotherapy Bio Awardee acquired by Bayer