

CDER Office of Neuroscience

Overview of Activities

February 18, 2026

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Who regulates Neuroscience Products at FDA?

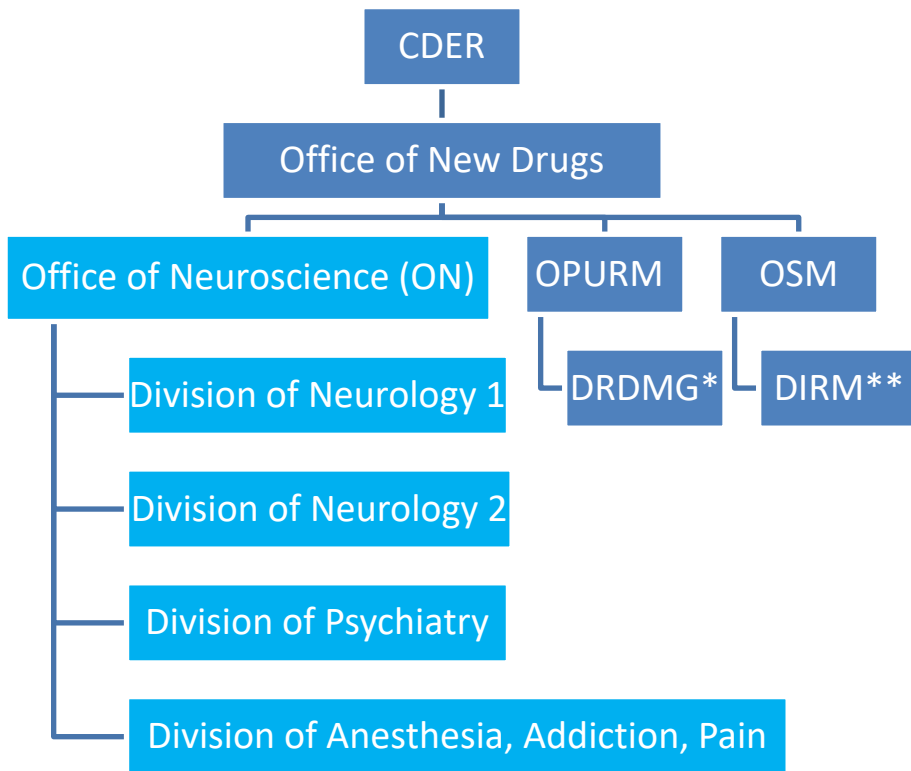


CDER: small molecules, monoclonal antibodies, proteins, diagnostic imaging agents

CBER: cell and gene therapy products, tissue products, blood products, vaccines

CDRH: therapeutic devices and diagnostics

Neuroscience products in CDER



ON has over 55 neurologists and psychiatrists and handles most neuroscience indications for new drugs in CDER, with some exceptions:

*Division of Rare Diseases and Medical Genetics (DRDMG): Products for inborn errors of metabolism

**Division of Imaging and Radiation Medicine: Imaging agents for brain conditions

ON routinely engages with other groups in FDA on neurology/psychiatry drug development issues to ensure consistency in approaches

Office of Neuroscience Structure

Office of Neuroscience Immediate Office

Division of Neurology 1

Alzheimer's disease/dementia
 Movement Disorders
 Rare neurogenetic diseases
 Neurodevelopmental diseases

Division of Neurology 2

Neuromuscular Diseases
 Epilepsy
 Stroke
 TBI
 Migraine
 Neuroimmunology

Division of Psychiatry

Depression
 Bipolar Disorder
 PTSD
 Schizophrenia
 Autism

**Division of Anesthesia,
 Addiction, Pain**

Pain
 Anesthesia
 Addiction

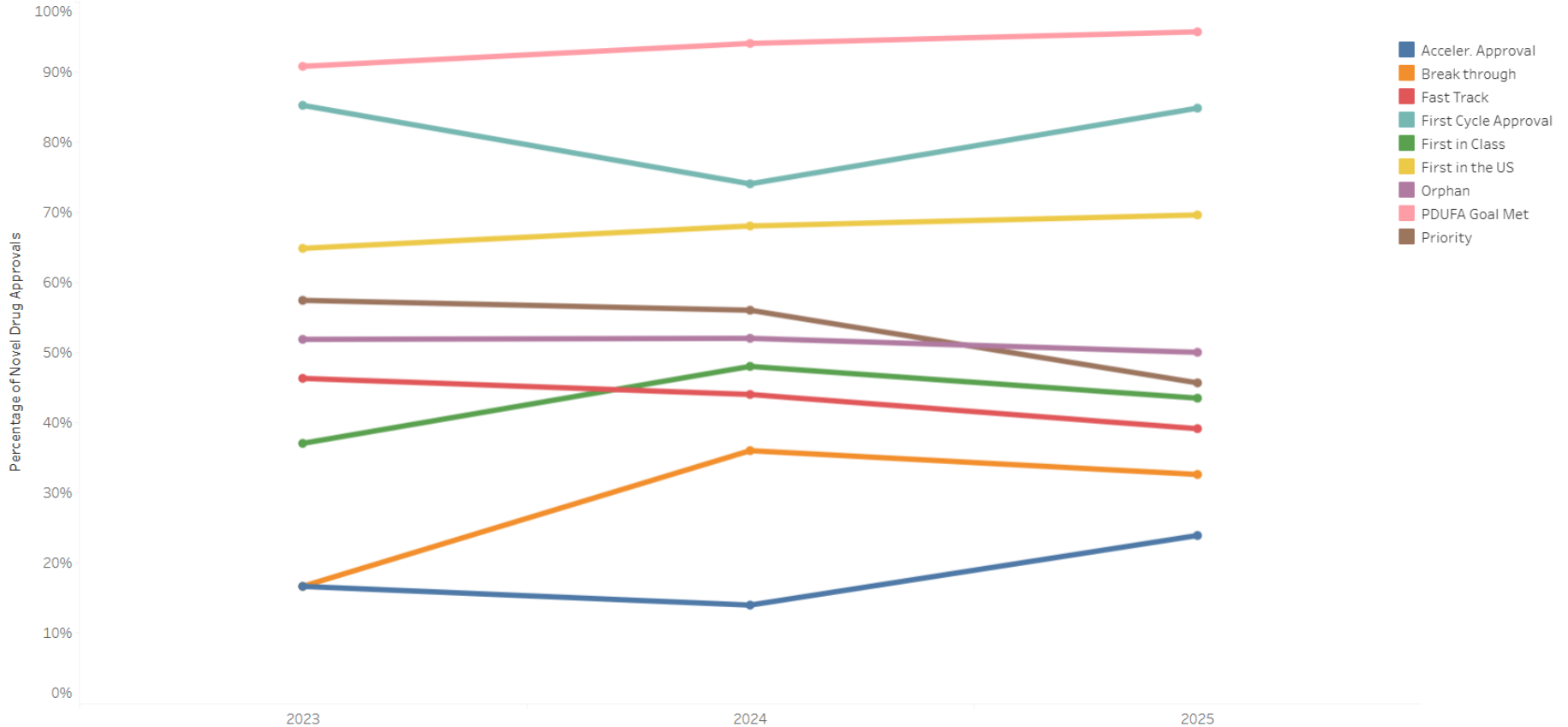
Notable ON Approvals

AA – Accelerated Approval
 PR - Priority Review
 ODD - Orphan Drug Designation
 BTD – Breakthrough Therapy Designation
 Voucher – Rare Pediatric Disease



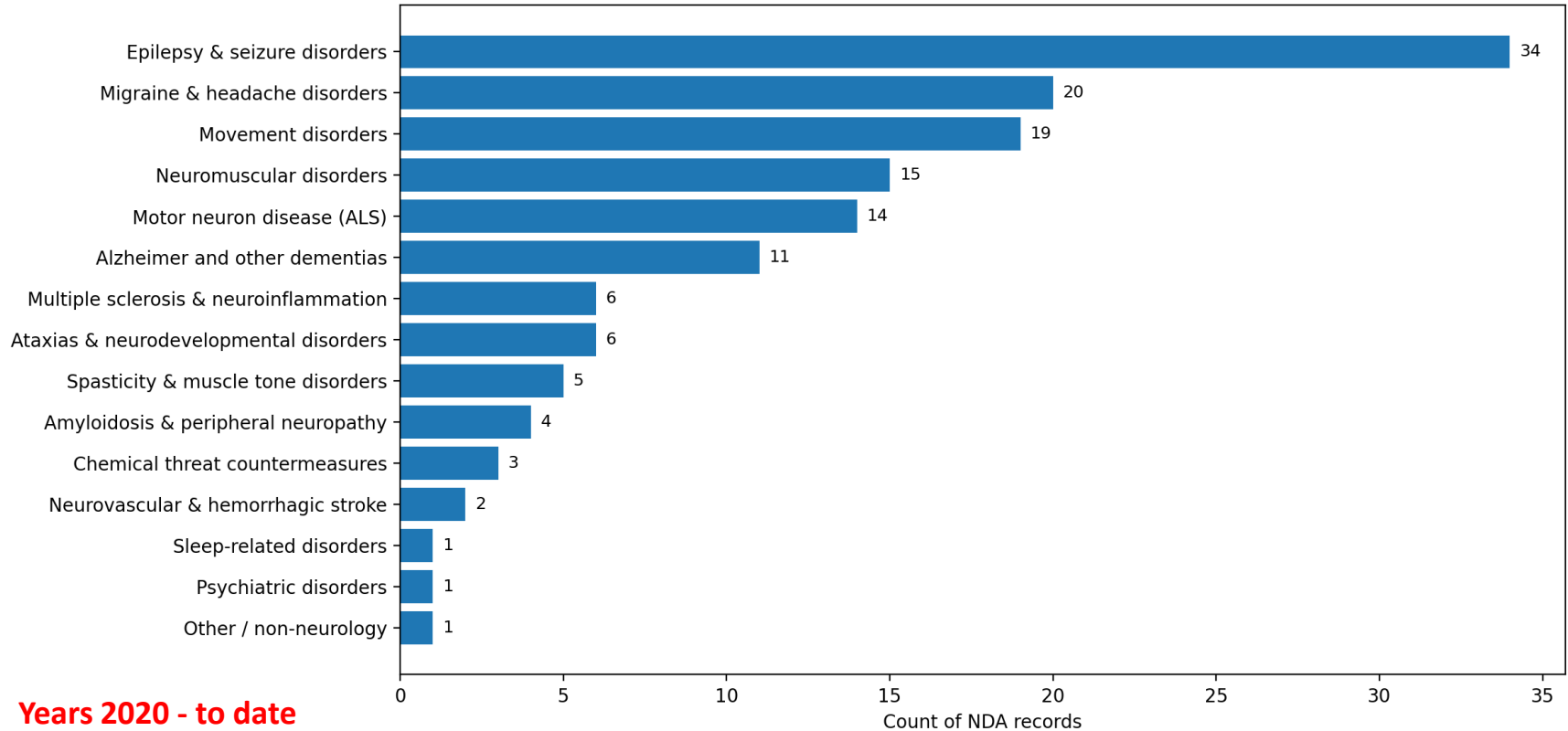
Name	Active Ingredient	Appl	Approval Date	Division	Applicant	Indication (Abbreviated)	Program
Journavx	suzetrigine	NDA	1/30/2025	DAAP	Vertex Ph, Inc.	Acute pain	PR
Imaavy	nipocalimab-aahu	BLA	4/29/2025	DN1	Janssen Bio, Inc.	AChR - MuSK myasthenia gravis	ODD, PR
Kisunla	donanemab-azbt	BLA	7/2/2024	DN1	Eli Lilly and Co	Alzheimer's disease	BTD, PR
Duvyzat	givinostat	NDA	3/21/2024	DN1	Italfarmaco SpA	Duchenne muscular dystrophy	ODD, PR, Voucher
Wainua	eplontersen	NDA	12/21/2023	DN1	Ionis Ph, Inc.	Polyneuropathy, Transthyretin-mediated amyloidosis	ODD
Agamree	vamorolone	NDA	10/26/2023	DN1	Santhera Pha Ltd.	Duchenne muscular dystrophy	ODD
Zilbrysq	zilucoplan	NDA	10/17/2023	DN1	UCB, Inc.	AChR myasthenia gravis	ODD
Rystiggo	rozanolixizumab-noli	BLA	6/26/2023	DN1	UCB, Inc.	AChR - MuSK myasthenia gravis	AA, ODD, PR
Qalsody	tofersen	NDA	4/25/2023	DN1	Biogen, Inc.	Amyotrophic lateral sclerosis	AA, ODD, PR
Daybue	trofinetide	NDA	3/10/2023	DN1	Acadia Ph, Inc.	Rett syndrome	ODD, PR, Voucher
Skyclarys	omaveloxolone	NDA	2/28/2023	DN1	Reata Ph, Inc.	Friedreich's ataxia	ODD, PR, Voucher
Leqembi	lecanemab-irmb	BLA	1/6/2023	DN1	Eisai, Inc.	Alzheimer's disease	AA, BTD, PR
Zavzpret	zavegepant	NDA	3/9/2023	DN2	Pfizer, Inc.	Migraine	
Cobenfy	xanomeline and trospium chloride	NDA	9/26/2024	DP	BMS Company	Schizophrenia	
Exxua	gepirone	NDA	9/22/2023	DP	Fabre-Kramer Phs, Inc.	Major depressive disorder	
Zurzuvae	zuranolone	NDA	8/4/2023	DP	Sage Therapeutics, Inc.	Postpartum depression	PR

Expedited Review Program Usage



Neurology NDAs indication grouping overview

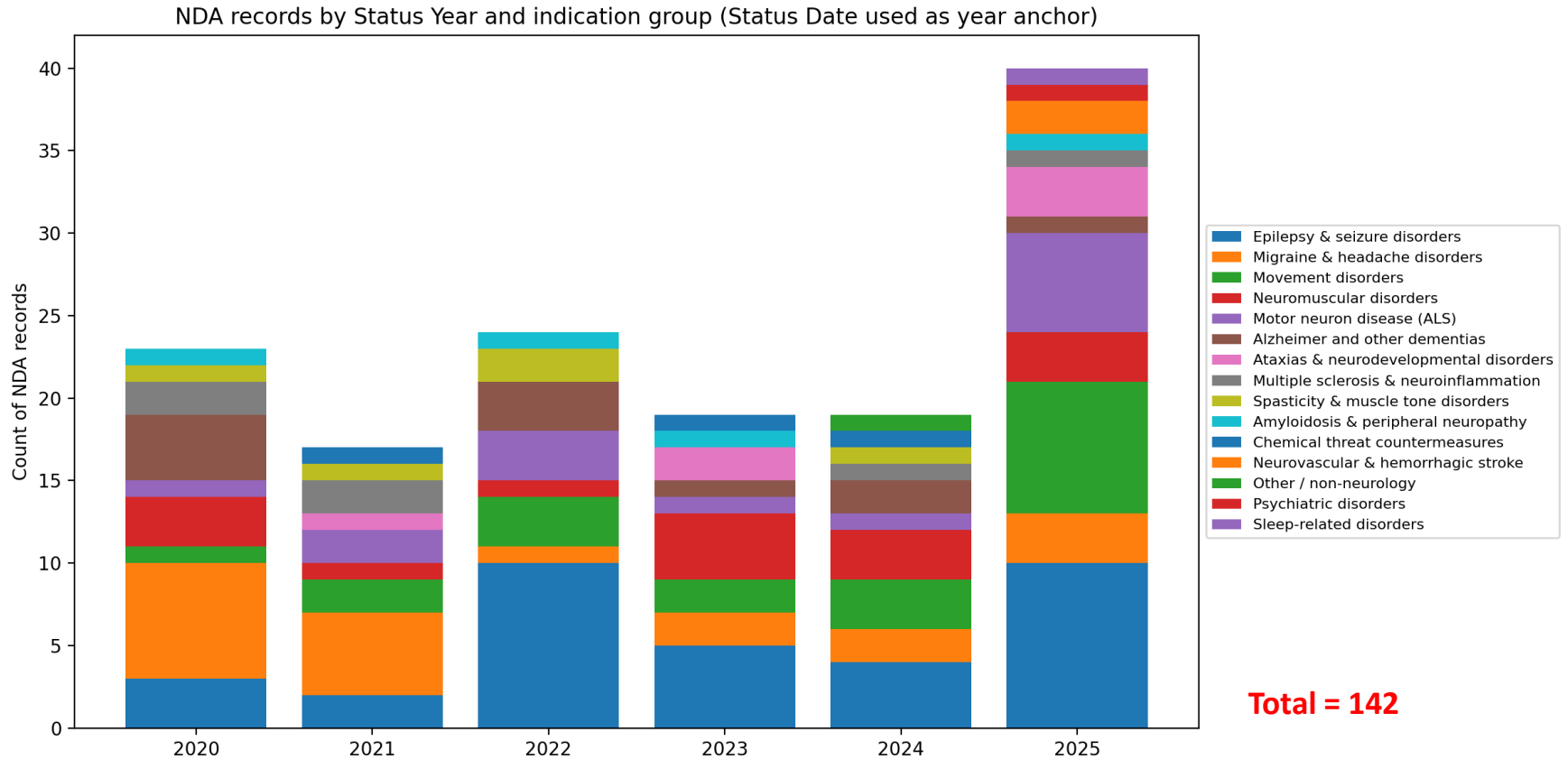
DN1 + DN2: NDA records by indication group



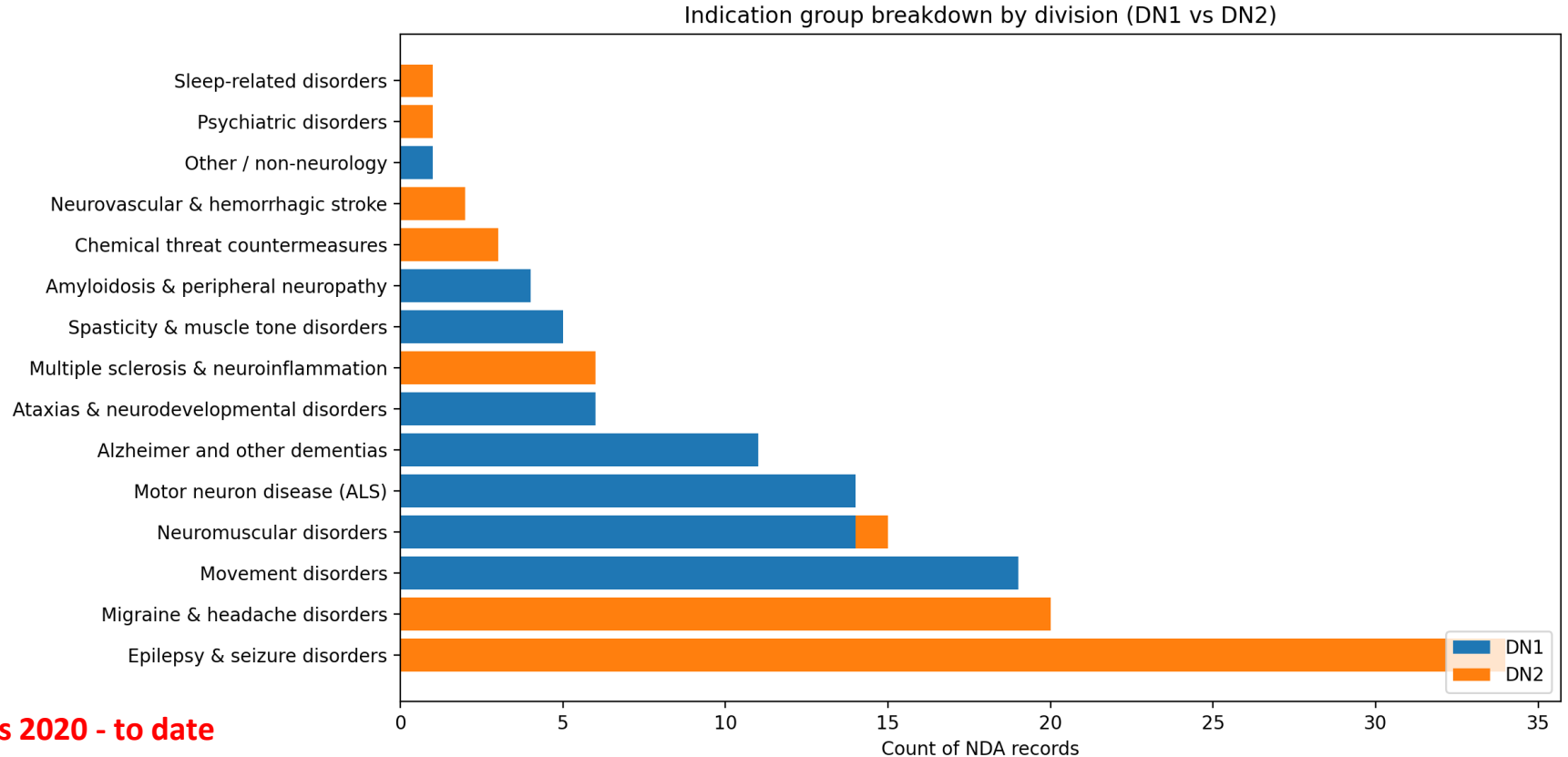
Years 2020 - to date

Total = 142

Neurology NDAs Indication groups by year



Indication groups by division (DN1 and DN2)

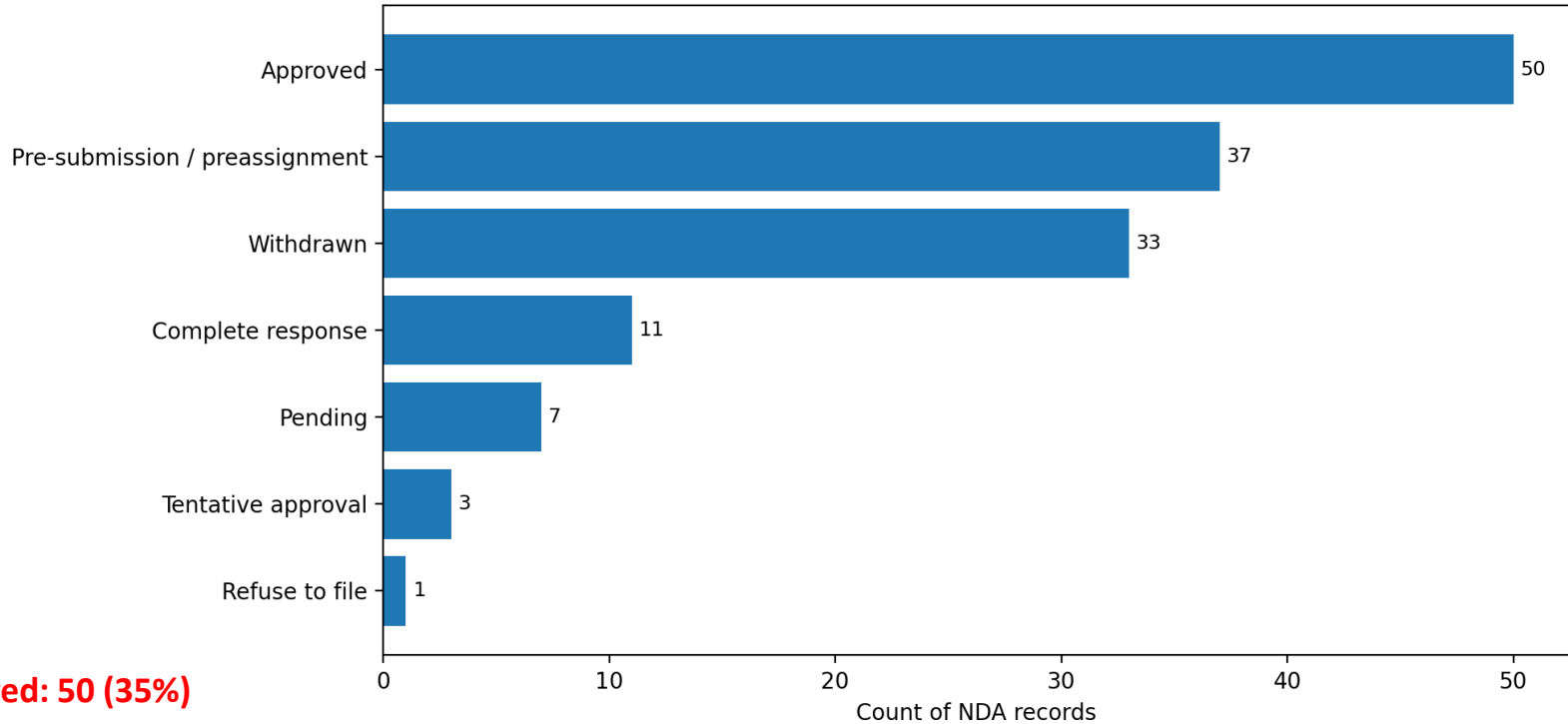


Years 2020 - to date

Total = 142

Neurology NDAs current status overview

DN1 + DN2: NDA records by current status (grouped)



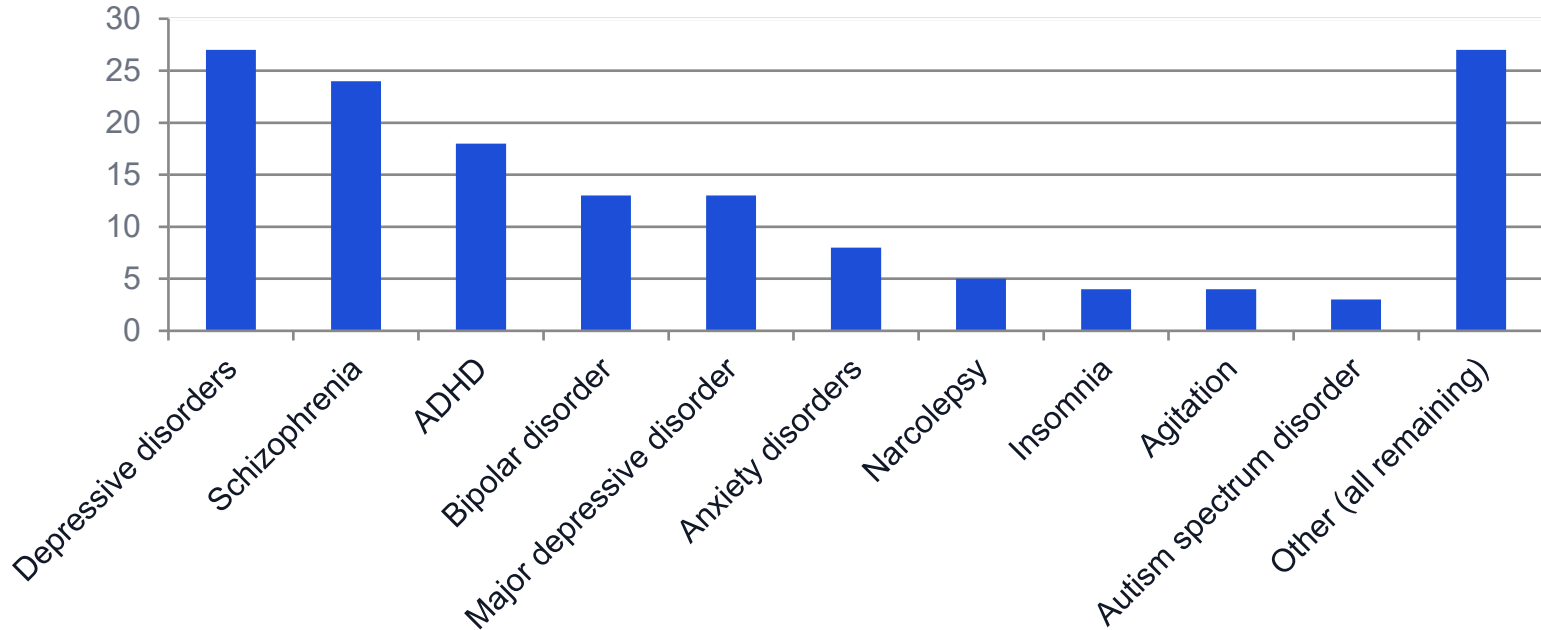
Approved: 50 (35%)

Withdrawn: 33 (23%)

Pre-submission / preassignment: 37 (26%)

**Years 2020 - to date
Total = 142**

DP - NDAs by indication



Note: An NDA can be counted under multiple indications when multiple indications are listed.
Top indications shown; remaining combined as "Other"

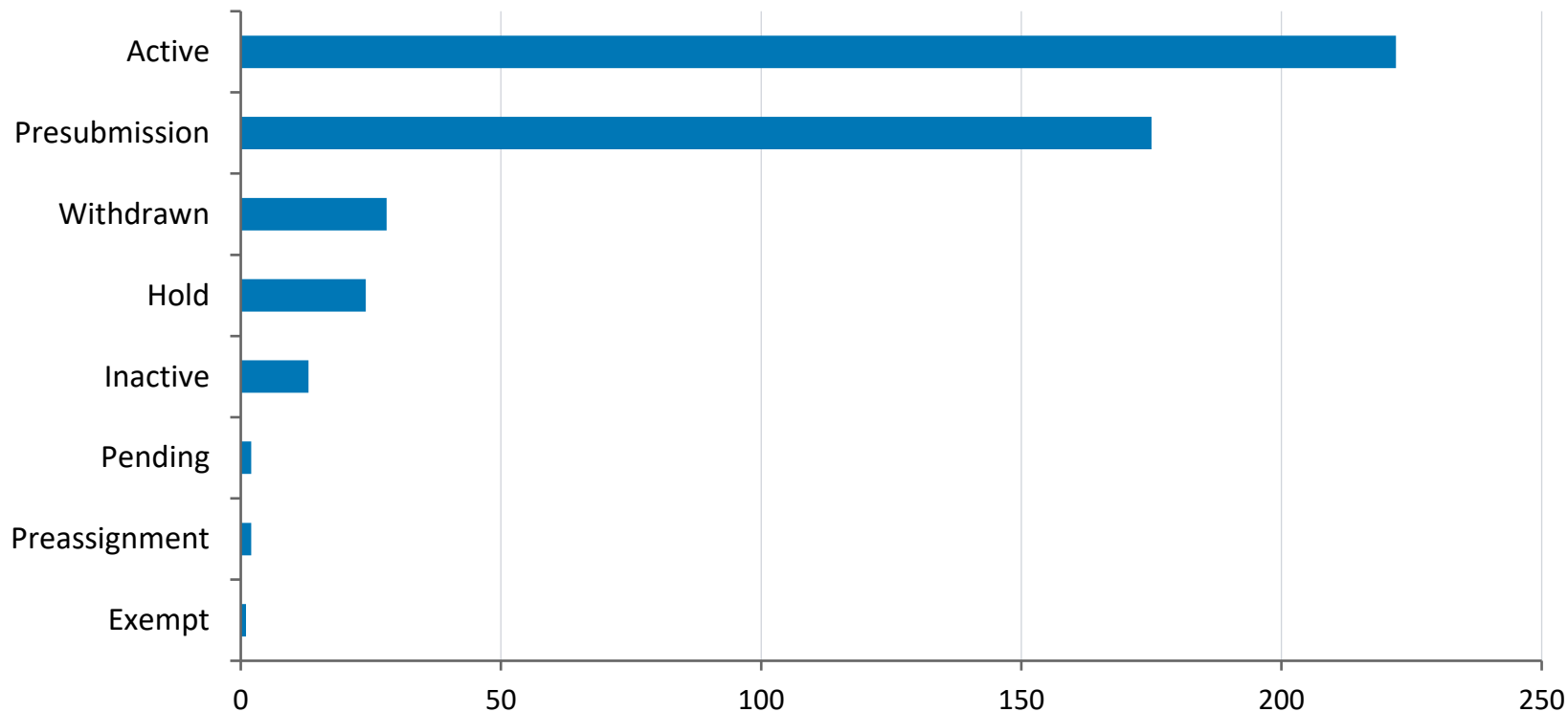
Years 2020 - to date
Total = 96



New Investigational Drugs Commercial Landscape

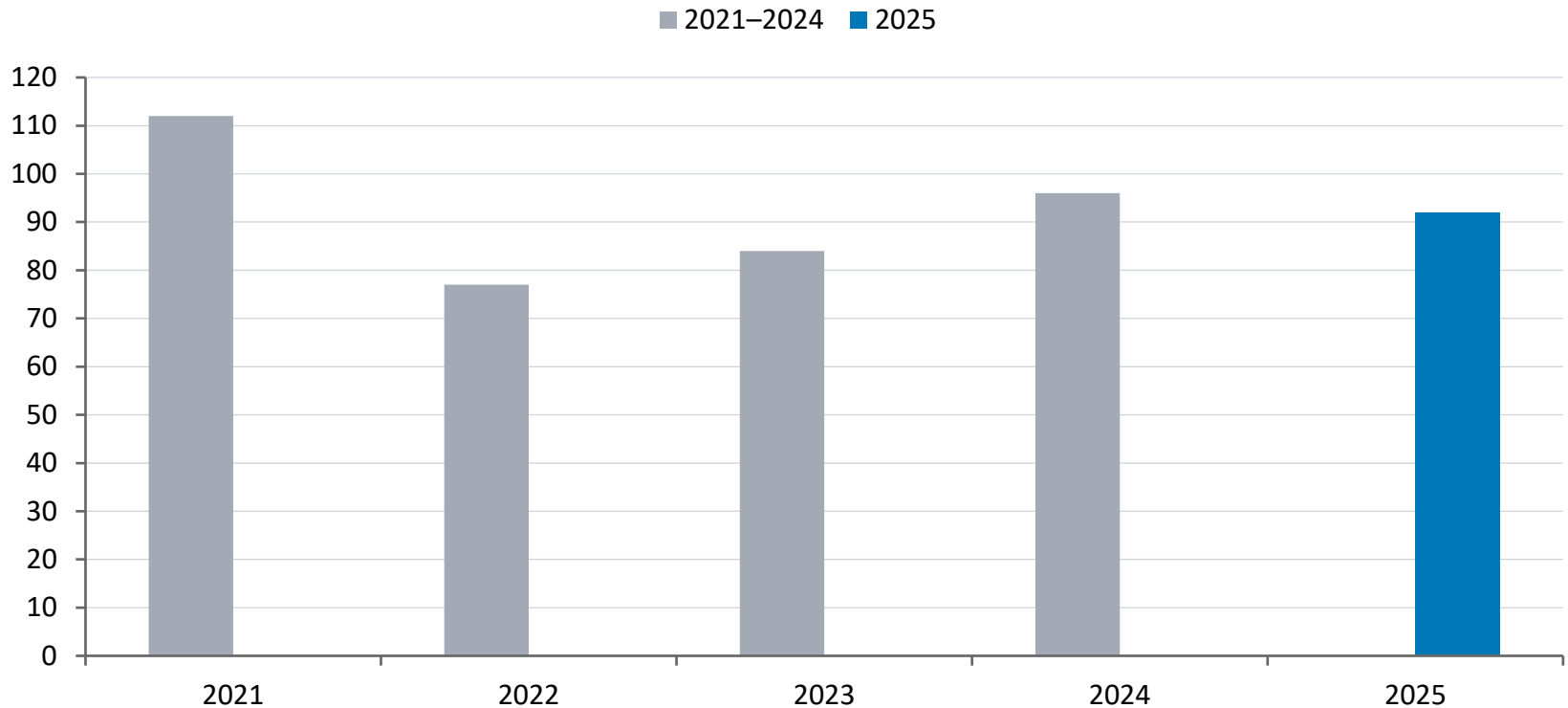
2021–2026 YTD

DN1- IND Submissions by Status (2021–2026 YTD)



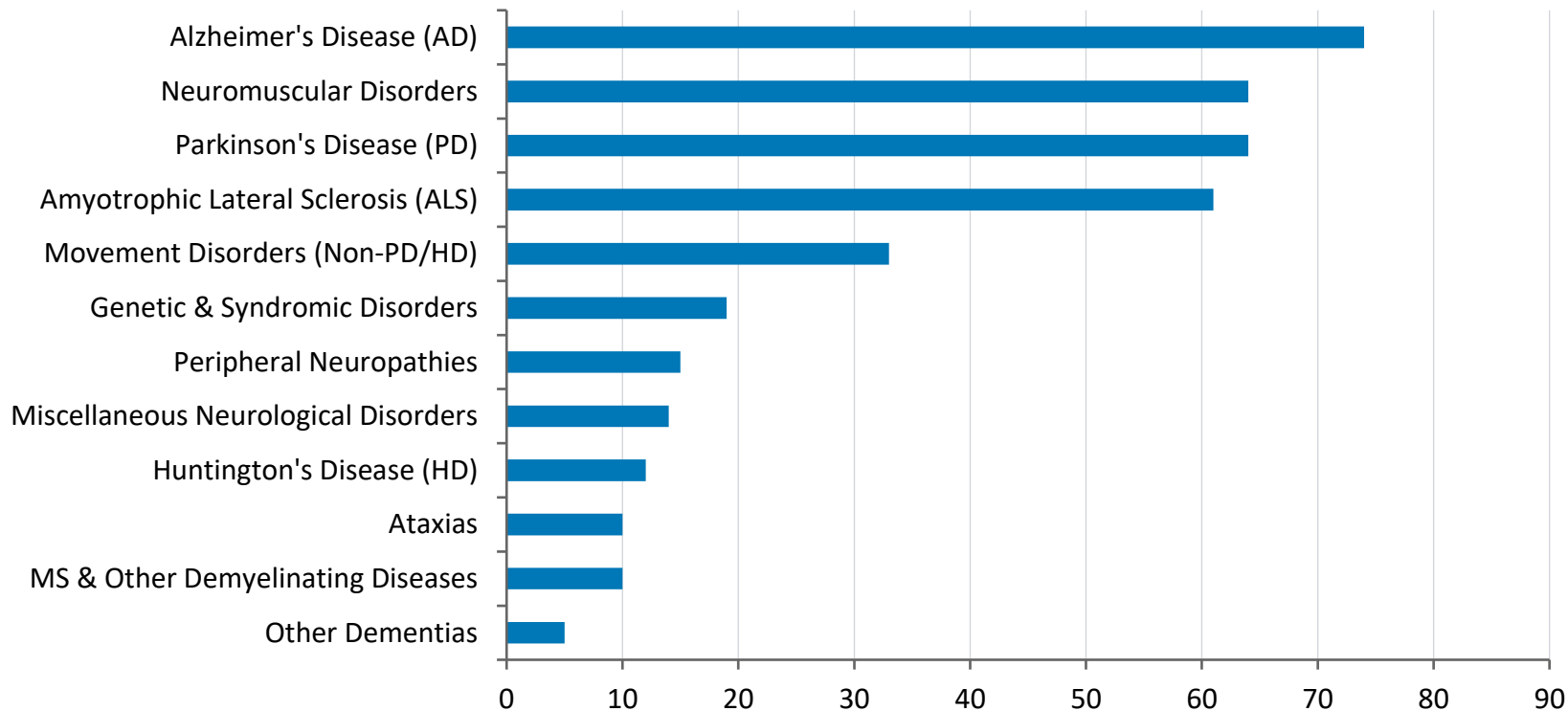
Total (2021–2026 YTD): 467

DN1 - IND Submissions by Year (2021–2025)



Total (2021–2025): 461 | 2026 YTD: 6 (excluded)

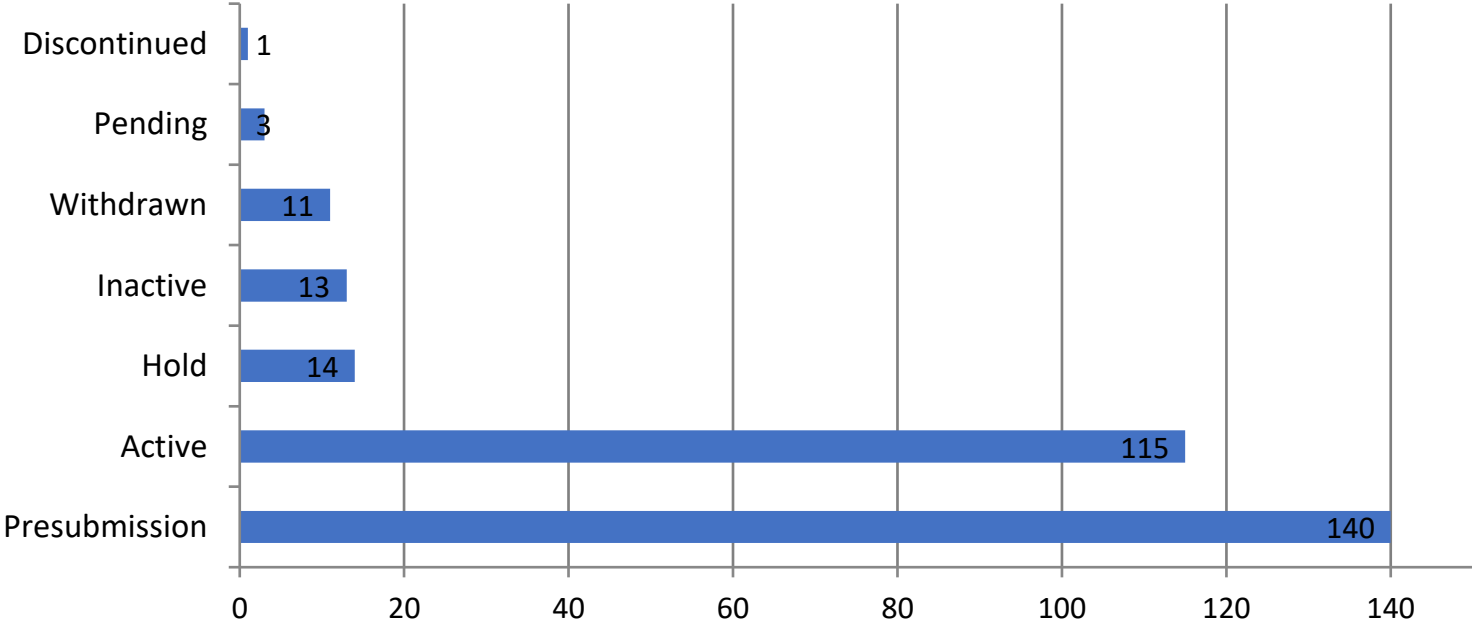
DN1 - IND Submissions by Indication Area (2021–2026 YTD)



Grouping per 'Grouping of DN1 indications' definitions. Note: 'Uncategorized' (n=86) omitted; records not mapped to a grouping.

DN2 - IND submissions by status (2021–2026 YTD)

Key Insight: Presubmissions (47%) and active INDs (39%) make up most records.

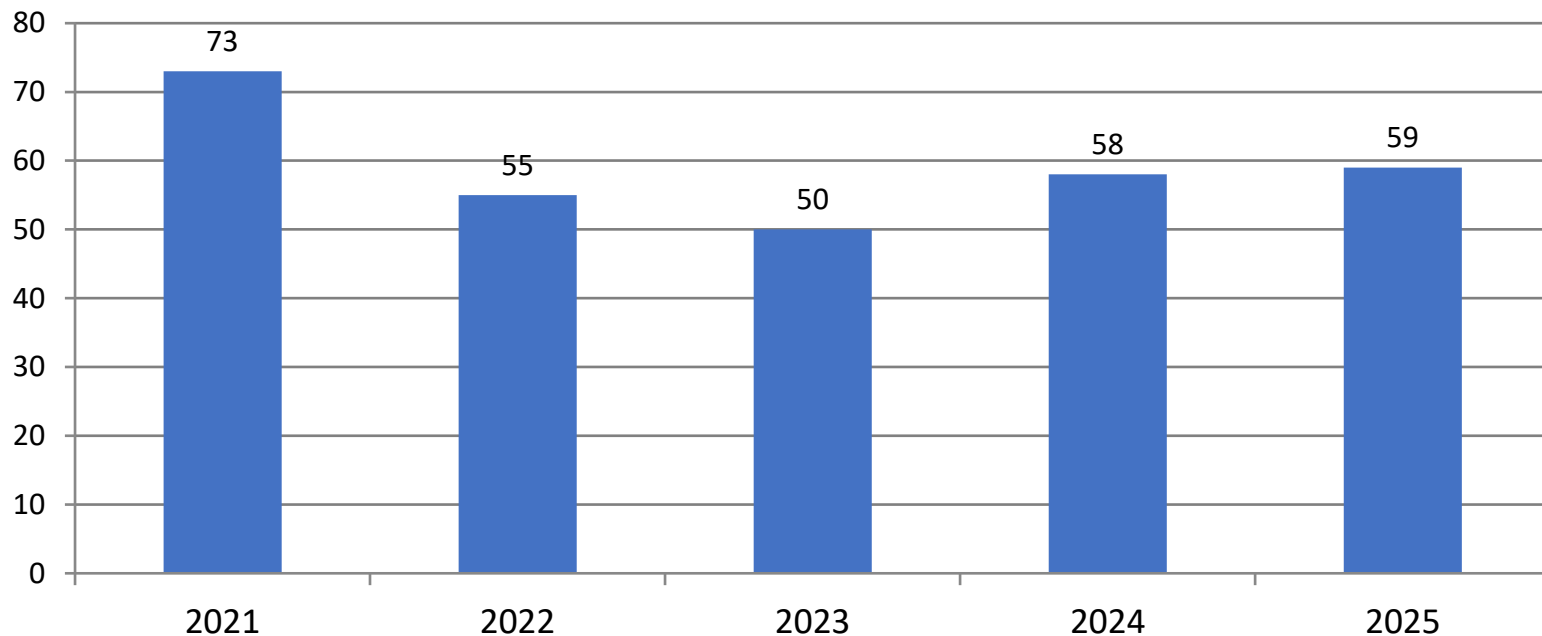


Counts include all 297 submissions (any status) with Earliest Create Time in 2021–2026.

DN2 - IND submissions by year (exclude 2026)

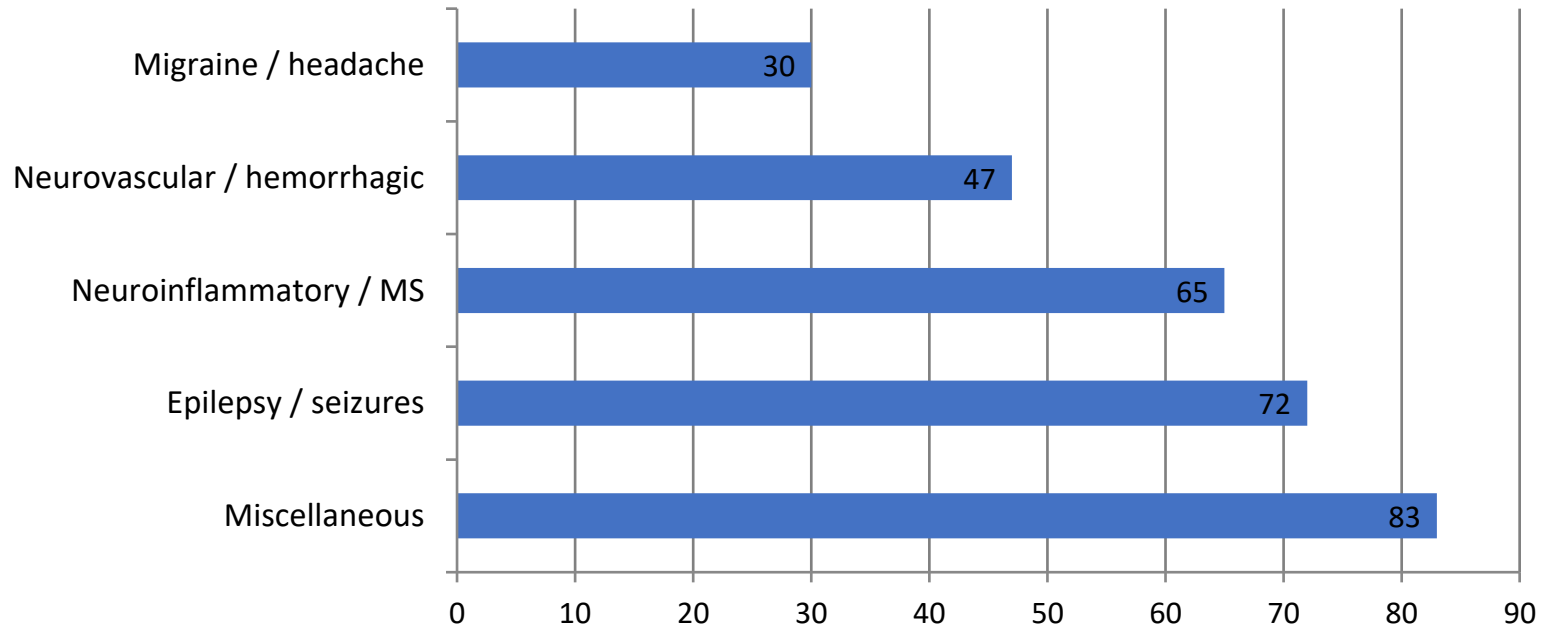


Key Insight: Volume peaks in 2021 (73) and remains 50–59 annually in 2022–2025.



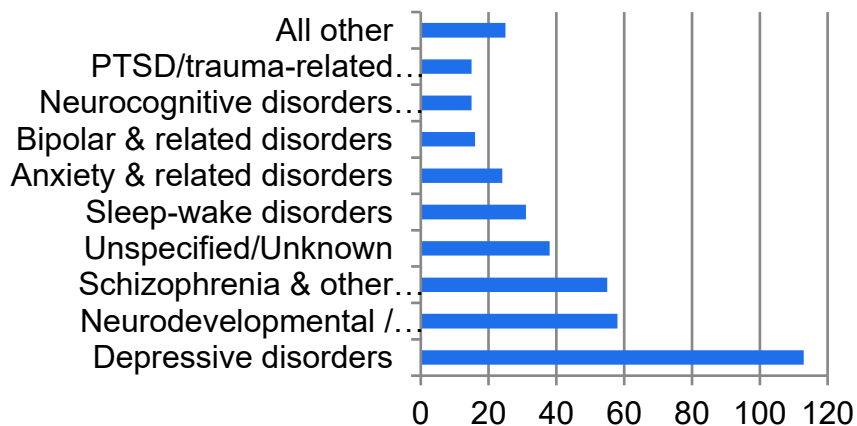
DN2 - IND submissions by disease area (2021–2026 YTD)

Key Insight: Miscellaneous, epilepsy/seizures, and neuroinflammatory/MS are the most common areas.

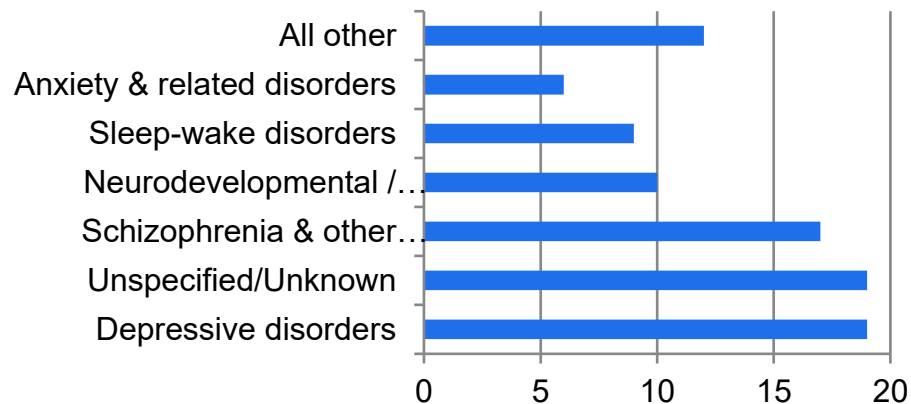


DP IND submissions by disease area

(2021–2026 YTD)



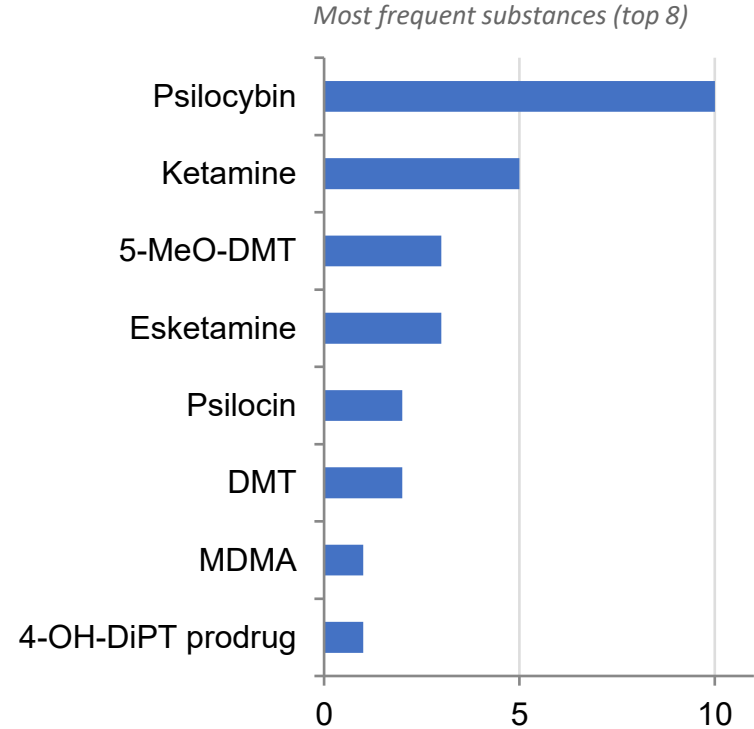
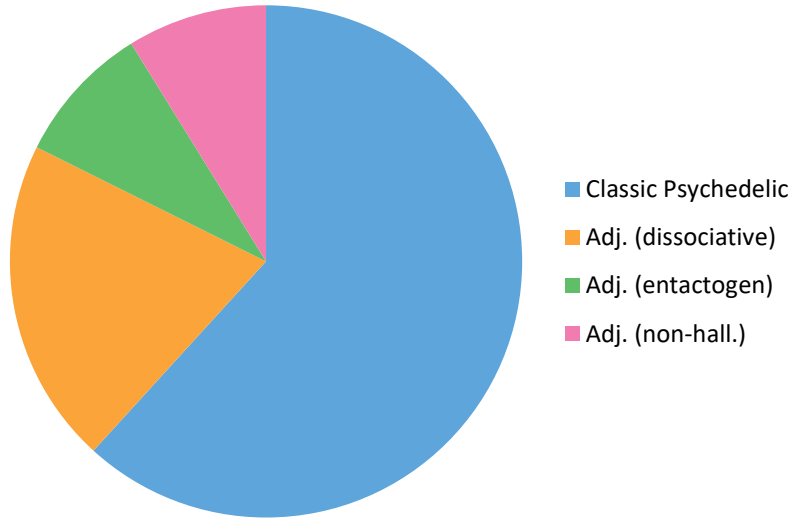
(2025)



All statuses included (n=390)

Commercial psychedelic IND landscape

Key insight: Psychedelic-related pipeline (2021–Jan 30, 2026): 34 submissions (8.7% of all DP INDs; N=390); classic psychedelics = 21 (62%). Most frequent substance label: Psilocybin (10 submissions).



Novel Outcomes

- Supporting development of staging systems to reflect underlying biology of disease
- Working to optimize PD endpoints to reflect current and future drug development
- Examining endpoints in HD

Biomarkers

- Markers of amyloid and tau in AD
- Letter of Support for alpha synuclein as enrichment biomarker
- Emerging fluid and imaging biomarkers of neuroinflammation, neurodegeneration, etc.

Digital Health Technologies and AI



- Multi-fold increase in the last decade of DHT and AI in IND submissions
- ON open and supportive of the potential use of DHTs and AI
 - Movement, Neuromuscular
- Engage with us early to discuss the potential use and determine what evidence is needed

Engagement

- Patient-Focused Drug Development Meetings and Patient Listening Sessions
 - AD, Insomnia, Schizophrenia, MDD, HD, DEEs
- CDER Committees
 - RD4, RDEA, DDT, DHT, PPI ICH, PED Methods
- Mandated Committees
 - NAPA, Federal Partners PDRD, Act for ALS

Engagement

- Critical Path Institute
 - AD, PD, HD, DMD, Ataxias, RND, RDCA-DAP, COA
- FNIH
 - AMP ALS, AMP ADRD , AMP PDRD, AMP Schizophrenia
 - Depression biomarker/NH study
 - Biomarker Consortium
- NIH
 - IMPACT-AD, Various NIA AD Workshops, Muscular Dystrophy Group

Engagement

- Research Roundtables
 - ISCTM, ASCP, ACNP, ECNP
 - Alzheimer's Association, ACT-AD, Us Against Alzheimer's
 - Epilepsy Foundation
 - ALS Association
 - Lewy Body Dementia Association
 - Association for Frontotemporal Degeneration
 - Combined Brain

Final Thoughts

- Exciting time in Neuroscience with current advancements
- However, unmet needs still exist
- Collaboration and partnership is needed to continue to advance the field
- The Office of Neuroscience is a partner in this advancement



THANK YOU !



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