

2021 Medicines in Development – Neurodegenerative Diseases

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAT-009 (5-HT4 partial agonist)	AskAt Nagoya, Japan	Alzheimer's disease	Phase I completed www.askat-inc.com
ABBV-8E12 (anti-tau antibody)	AbbVie North Chicago, IL C2N Diagnostics St. Louis, MO	Alzheimer's disease	Phase II www.abbvie.com
ACI-35.030 (anti-pTau vaccine)	AC Immune Lausanne, Switzerland Janssen Research & Development Raritan, NJ	early Alzheimer's disease	Phase II www.acimmune.com www.janssen.com
ACU193 (anti-amyloid-beta oligomer mAb)	Acumen Pharmaceuticals Charlottesville, VA	Alzheimer's disease	Phase I www.acumenpharm.com
AD-35 (amyloid-beta aggregation inhibitor)	Hisun USA Bridgewater, NJ	Alzheimer's disease	Phase II www.hisunusa.com
Adlarity donepezil transdermal patch	Corium International Menlo Park, CA	Alzheimer's disease	application submitted www.coriumgroup.com

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AGB101 (levetiracetam low-dose)	AgenBio Baltimore, MD	mild cognitive impairment due to Alzheimer's disease and control further progression of Alzheimer's dementia	Phase III www.agenebio.com
AKST/GRF6019 (neurogenesis stimulant)	Alkahest San Carlos, CA Grifols Barcelona, Spain	mild to moderate Alzheimer's disease, severe Alzheimer's disease	Phase II www.alkahest.com
AL002 (TREM2 protein stimulant)	AbbVie North Chicago, IL Alector South San Francisco, CA	early Alzheimer's disease	Phase II www.abbvie.com www.alector.com
AL003 (SIGLEC 3)	AbbVie North Chicago, IL Alector South San Francisco, CA	Alzheimer's disease	Phase I www.abbvie.com www.alector.com
ALX-001 (mGluR5 modulator)	Allyx Therapeutics New Haven, CT	Alzheimer's disease	Phase I www.allyxthera.com
ALZ-801 (amyloid beta-protein inhibitor)	Alzheon Framingham, MA	early Alzheimer's disease (APOE4/4 homozygotes) (Fast Track)	Phase III www.alzheon.com
		early Alzheimer's disease (APOE4 carriers)	Phase II www.alzheon.com

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ALZT-OP1 (amyloid beta-protein inhibitor/ inflammation mediator inhibitor)	AZTherapies Boston, MA	Alzheimer's disease	Phase III completed www.aztherapies.com
AMX0035 (sodium phenylbutyrate/ tauroursodeoxycholic acid)	Amylyx Pharmaceuticals Cambridge, MA	Alzheimer's disease	Phase II www.amylyx.com
ANAVEX®2-73 (blarcamesine) (muscarinic receptor agonist/ sigma-1 receptor agonist)	Anavex Life Sciences New York, NY	Alzheimer's disease	Phase II/III www.anavex.com
ANVS301 (bisnorcymserine)	Annovis Bio Berwyn, PA	advanced Alzheimer's disease	Phase I www.annovisbio.com
ANVS401 (R-phenserine)	Annovis Bio Berwyn, PA	Alzheimer's disease	Phase II www.annovisbio.com
		Alzheimer's disease in Down syndrome	Phase I www.annovisbio.com
AstroStem® mesenchymal stem cell therapy	Nature Cell Seoul, South Korea	Alzheimer's disease	Phase I/II completed www.naturecell.co.kr
ATH-1017 (HGF stimulant)	Athira Pharma Bothell, WA	Alzheimer's disease	Phase II/III www.athira.com

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atuzaginstat (COR388) (lysine gingipain inhibitor)	Cortexyme South San Francisco, CA	Alzheimer's disease	Phase III www.cortexyme.com
AUS-131 (nonhormonal estrogen receptor agonist)	Ausio Pharmaceuticals Cincinnati, OH University of Kansas Kansas City, KS	Alzheimer's disease	Phase I/II completed www.ausiopharma.com
AVP-786 (dextromethorphan analogue/ultra-low dose quinidine)	Avanir Pharmaceuticals Aliso Viejo, CA	agitation in Alzheimer's disease (Fast Track)	Phase III www.avanir.com
AXS-05 (bupropion/dextromethorphan)	Axsome Therapeutics New York, NY	agitation in Alzheimer's disease (Fast Track) (Breakthrough Therapy)	Phase III www.axsome.com
BAC	Charsire Biotechnology Tainan City, Taiwan	Alzheimer's disease	Phase II completed www.charsire.com.tw
BAN2401 (lecanemab) (anti-amyloid beta protofibril antibody)	Biogen Cambridge, MA Eisai Woodcliff Lake, NJ	early Alzheimer's disease, preclinical Alzheimer's disease (Breakthrough Therapy)	Phase III www.biogen.com www.eisai.com
beptranemab (UCB0107) (anti-tau antibody)	UCB Smyrna, GA Roche/Genentech South San Francisco, CA	Alzheimer's disease	Phase II www.ucb.com www.roche.com

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BIIB076 (anti-tau antibody)	Biogen Cambridge, MA	Alzheimer's disease	Phase I www.biogen.com
BIIB080 (IONIS-MAPT _{RX}) (tau-targeting protein)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	mild Alzheimer's disease	Phase II www.biogen.com www.ionispharma.com
BIIB092 (gosuranemab) (anti-extracellular tau antibody)	Biogen Cambridge, MA	Alzheimer's disease	Phase II www.biogen.com
BNC375 (positive allosteric modulator)	Merck Kenilworth, NJ Bionomics Thebarton, Australia	cognitive dysfunction in Alzheimer's disease	Phase I www.merck.com www.bionomics.com.au
BPN14770 (zatolmilast) (PDE4D inhibitor)	Tetra Discovery Partners Grand Rapids, MI Shionogi Florham Park, NJ	Alzheimer's disease	Phase II www.tetradiscovery.com www.shionogi.com
bryostatin 1 (protein kinase C stimulant)	Synaptogenix New York, NY	Alzheimer's disease	Phase II www.synaptogen.com
crenezumab (RG7412) (anti-amyloid beta antibody)	Roche/Genentech South San Francisco, CA	familial Alzheimer's disease in healthy people	Phase II www.roche.com

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CT1812 (amyloid beta oligomer receptor antagonist)	Cognition Therapeutics Pittsburgh, PA	mild to moderate Alzheimer's disease (Fast Track)	Phase II www.cogrx.com
		early Alzheimer's disease	Phase I www.cogrx.com
CY6463 (sGC stimulant)	Cyclerion Therapeutics Cambridge, MA	Alzheimer's disease with vascular pathology	Phase I www.cyclerion.com
donanemab (LY3002813) (N3pG-amyloid beta antibody)	Lilly Indianapolis, IN	Alzheimer's disease (Breakthrough Therapy)	Phase III www.lilly.com
E2006 (orexin receptor antagonist)	Eisai Woodcliff Lake, NJ	Alzheimer's disease dementia	Phase II www.eisai.com
E2511 (synapse regenerant)	Eisai Woodcliff Lake, NJ	Alzheimer's disease	Phase I www.eisai.com
E2814 (anti-MTBR tau antibody)	Eisai Woodcliff Lake, NJ	Alzheimer's disease	Phase I/II www.eisai.com
EI-1071 (CSF-1R inhibitor)	Elixiron Immunotherapeutics Taipei City, Taiwan	Alzheimer's disease	Phase I www.elixiron.com
gantenerumab (RG1450) (amyloid beta-protein inhibitor)	Roche/Genentech South San Francisco, CA	Alzheimer's disease	Phase III www.roche.com

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IGC-AD1 (phytocannabinoid therapeutic)	IGC Pharma Potomac, MD	Alzheimer's disease	Phase I www.igcpharma.com
JNJ-63733657 (phospho-tau mAb inhibitor)	Janssen Research & Development Raritan, NJ	early Alzheimer's disease	Phase II www.janssen.com
JOT108 (oral capsule of trans-resveratrol)	Jupiter Orphan Therapeutics Jupiter, FL	early Alzheimer's disease	Phase II www.jupiterorphan.com
LM11A-31 (p75 neurotrophin receptor)	Pharmatrophix Menlo Park, CA	mild to moderate Alzheimer's disease	Phase I/II completed www.pharmatrophix.com
LMTX (tau protein aggregation inhibitor/ TDP-43 aggregation inhibitor)	TauRx Pharmaceuticals Singapore	mild to moderate Alzheimer's disease	Phase III www.taurx.com
Lu AF87908 (tau protein inhibitor)	Lundbeck Deerfield, IL	Alzheimer's disease	Phase I www.lundbeck.com
LX1001 (AAV-mediated gene therapy)	LEXEO Therapeutics New York, NY	APOE4-associated Alzheimer's disease (Fast Track)	Phase I www.lexeotx.com
LY3372689 (O-GlcNAcase inhibitor)	Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com

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LY3372993 (N3pG-Ab mAb)	Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com
LYN-157 (donepezil/memantine ER)	Lyndra Therapeutics Watertown, MA	Alzheimer's disease	Phase I www.lyndra.com
MEDI1814 (anti-amyloid beta mAb)	AstraZeneca Wilmington, DE	Alzheimer's disease	Phase I www.astrazeneca.com
mesenchymal stem cell therapy (allogeneic bone marrow-derived)	Longeveron Miami, FL	Alzheimer's disease	Phase I www.longeveron.com
mesenchymal stem cell therapy (ischemic-tolerant stem cells)	Stemedica Cell Technologies San Diego, CA	Alzheimer's disease	Phase II www.stemedica.com
MK-1942	Merck Whitehouse Station, NJ	Alzheimer's disease (+donepezil)	Phase I www.merck.com
NA-831 (traneurocin) (neurogenesis stimulant)	NeuroActiva San Jose, CA	Alzheimer's disease	Phase II www.neuroactiva.com
NE3107 (ERK inhibitor)	BioVie Santa Monica, CA	Alzheimer's disease	Phase III www.bioviepharma.com

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neflamapimod (VX-745) (p38 mitogen-activated protein kinase inhibitor)	EIP Pharma Cambridge, MA	Alzheimer's disease	Phase II www.eippharma.com
nilotinib (tyrosine kinase inhibitor)	KeifeRx McLean, VA	Alzheimer's disease	Phase III www.keiferx.com
NLY01-AD (GLP-1R agonist)	Neuraly Gaithersburg, MD	Alzheimer's disease	Phase II www.neuraly.com
NNI-362 (neuron regenerative therapy)	Neuronascent Clarksville, MD	Alzheimer's disease	Phase I www.neuronascent.com
NTRX-07 (cannabinoid receptor CB2 agonist)	NeuroTherapia Gates Mills, OH	Alzheimer's disease	Phase I www.neurotherapia.com
pepinemab (anti-SEMA4D mAb)	Vaccinex Rochester, NY	Alzheimer's disease	Phase I/II www.vaccinex.com
PQ912 (QC inhibitor)	Vivoryon Therapeutics Halle, Germany	early Alzheimer's disease	Phase II www.vivoryon.com
PRX005 (tau protein inhibitor)	Bristol-Myers Squibb Princeton, NJ Prothena Dublin, Ireland	Alzheimer's disease	Phase I www.bms.com

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PU-AD (icapamespib) (HSP90 heat-shock protein inhibitor)	Samus Therapeutics Topsfield, MA	Alzheimer's disease	Phase II www.samustherapeutics.com
Rexulti [®] brexpiprazole	Lundbeck Deerfield, IL Otsuka Pharmaceutical Tokyo, Japan	agitation in Alzheimer's disease (Fast Track)	Phase III www.lundbeck.com www.otsuka.com
RG6102 (brain shuttle gantenerumab)	Roche/Genentech South San Francisco, CA	Alzheimer's disease	Phase II www.roche.com
SAGE-718 (NMDA receptor modulator)	Sage Therapeutics Cambridge, MA	cognitive dysfunction in Alzheimer's disease	Phase II www.sagerx.com
semaglutide oral (GLP-1 receptor agonist)	Novo Nordisk Plainsboro, NJ	early Alzheimer's disease	Phase III www.novonordisk.com
semorinemab (RG6100) (tau protein inhibitor)	AC Immune Lausanne, Switzerland Roche/Genentech South San Francisco, CA	Alzheimer's disease	Phase II www.roche.com
SEP-380135	Sunovion Marlborough, MA	agitation in Alzheimer's disease	Phase I www.sunovion.com
simufilam (microfilament protein modulator)	Cassava Sciences Austin, TX	Alzheimer's disease	Phase II www.cassavasciences.com

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SNK01 (autologous NK cell therapy)	NKGen Biotech Santa Ana, CA	Alzheimer's disease	Phase I www.nkgenbiotech.com
solanezumab (amyloid beta protein inhibitor)	Lilly Indianapolis, IN	preclinical Alzheimer's disease	Phase III www.lilly.com
SUVN-502 (masupirdine) (serotonin 6 receptor antagonist)	Suven Life Sciences Hyderabad, India	moderate Alzheimer's disease (adjunctive treatment)	Phase II completed www.suven.com
T-817MA (edonergic) (amyloid beta-protein inhibitor)	Toyama Chemical Tokyo, Japan	Alzheimer's disease	Phase II www.toyama-chemical.co.jp
T3D-959 (PPAR-delta/gamma agonist)	T3D Therapeutics Research Triangle Park, NC	Alzheimer's disease	Phase II www.t3dtherapeutics.com
TB006 (TIM-3)	TrueBinding Foster City, CA	Alzheimer's disease	Phase I www.truebinding.com
troriluzole (BHV-4157) (glutamate modulator)	Biohaven Pharmaceuticals New Haven, CT	Alzheimer's disease	Phase II www.biohavenpharma.com
UB-311 (anti-amyloid endobody vaccine)	Vaxxinity Dallas, TX	Alzheimer's disease	Phase II www.vaxxinity.com
UE-2343 (11 β -HSD1 inhibitor)	Actinogen Medical Sydney, Australia	dementia in Alzheimer's disease	Phase II www.actinogen.com

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vafidemstat (ORY-2001) (LSD1/MAOB inhibitor)	Oryzon Genomics Cambridge, MA	Alzheimer's disease	Phase II completed www.oryzon.com
XPro1595 (TNF inhibitor)	INmune Bio La Jolla, CA	Alzheimer's disease	Phase I www.inmunebio.com
zagotenemab (LY3303560) (tau deposit antibody)	Lilly Indianapolis, IN	Alzheimer's disease	Phase II www.lilly.com

Amyotrophic Lateral Sclerosis (ALS)

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
ABBV-CLS-7262 (eIF2B)	AbbVie North Chicago, IL Calico Life Sciences South San Francisco, CA	amyotrophic lateral sclerosis (ALS)	Phase I www.abbvie.com www.calicolabs.com
AIT-101	AI Therapeutics Guilford, CT	ALS	Phase II www.ai-therapeutics.com
ALS 001 (Treg cell therapy) ORPHAN DRUG	Coya Therapeutics Houston, TX	ALS	Phase II www.coyatherapeutics.com

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ALZT-OP1A (mast cell stabilizer/neuro-inflammatory microglial modulator)	AZTherapies Boston, MA	ALS	Phase II www.aztherapies.com
AMX0035 (sodium phenylbutyrate/ tauroursodeoxycholic acid) ORPHAN DRUG	Amylyx Pharmaceuticals Cambridge, MA	ALS	Phase III www.amylyx.com
ANX005 (complement C1 inhibitor)	Annexon Biosciences South San Francisco, CA	ALS	Phase II www.annexonbio.com
APB-102 (dual function AAV gene therapy) ORPHAN DRUG	Apic Bio Cambridge, MA	ALS (Fast Track)	Phase I www.apic-bio.com
AT-1501 (CD40 ligand inhibitor) ORPHAN DRUG	Eledon Pharmaceuticals Irvine, CA	ALS	Phase II www.eledon.com
BIIB067 (tofersen) (SOD1) ORPHAN DRUG	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	ALS	Phase III www.biogen.com

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BIIB078 (IONIS-C9RX) (RNA interference)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	ALS (Fast Track)	Phase I www.biogen.com
BIIB100 (XPO1 inhibitor)	Biogen Cambridge, MA	ALS	Phase I www.biogen.com
BIIB105 (ION541) (ATXN2 ASO)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	ALS	Phase I www.biogen.com
BLZ945 (CSF-1 inhibitor)	Novartis East Hanover, NJ	ALS	Phase II www.novartis.com
CNM-Au8 (nanotherapeutic)	Clene Nanomedicine Salt Lake City, UT	ALS	Phase III www.clene.com
CORT113176 (GR antagonist)	Corcept Therapeutics Menlo Park, CA	ALS	Phase I www.corcept.com
DNL343 (eIF2B stimulant)	Denali Therapeutics South San Francisco, CA	ALS	Phase I www.denalitherapeutics.com
donaperminogene seltoplasmid (gene therapy) ORPHAN DRUG	Helixmith Seoul, Korea	ALS (Fast Track)	Phase II www.helixmith.com

Amyotrophic Lateral Sclerosis (ALS)

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Empaveli® pegcetacoplan	Apellis Pharmaceuticals Waltham, MA	ALS	Phase II www.apellis.com
EPI-589 (reversible redox cofactor)	Sumitomo Dainippon Pharma Okasa, Japan	ALS	Phase II www.ds-pharma.com
GM604 (pleiotropic peptide) ORPHAN DRUG	Genervon Biopharmaceuticals Pasadena, CA	ALS (Fast Track)	Phase II www.genervon.com
ION363 (RNA interference)	Ionis Pharmaceuticals Carlsbad, CA	ALS	Phase III www.ionis.com
masitinib (tyrosine kinase inhibitor) ORPHAN DRUG	AB Science Paris, France	ALS	Phase III www.ab-science.com
MN-166 (ibudilast) (neuroprotective therapeutic) ORPHAN DRUG	MediciNova La Jolla, CA	ALS (Fast Track)	Phase III www.medicinova.com
NPT520-34 (neuroinflammation) ORPHAN DRUG	Neuropore Therapies San Diego, CA	ALS	Phase I www.neuropore.com

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NurOwn® (neurotrophic factor-producing mesenchymal stem cell therapy) ORPHAN DRUG	Brainstorm Cell Therapeutics New York, NY	ALS (Fast Track)	Phase III www.brainstorm-cell.com
pridopidine (sigma-1 receptor agonist) ORPHAN DRUG	Prilenia Therapeutics Naarden, Netherlands	ALS	Phase II www.prilenia.com
PrimeC (celecoxib/ciprofloxacin FDC) ORPHAN DRUG	NeuroSense Therapeutics Herzeliya, Israel	ALS	Phase I www.neurosense-tx.com
Radicava® edaravone (oral suspension)	Mitsubishi Tanabe Pharma America Jersey City, NJ	ALS	Phase III www.mt-pharma-america.com
RAPA-501 (autologous hybrid T-cell therapy)	Rapa Therapeutics Rockville, MD	ALS	Phase I/II
reldeesemtiv (troponin stimulant) ORPHAN DRUG	Cytokinetics South San Francisco, CA	ALS	Phase III www.cytokinetics.com
RNS60 (intracellular signaling pathway activator) ORPHAN DRUG	Revalesio Tacoma, WA	ALS (Fast Track)	Phase II www.revalesio.com

Amyotrophic Lateral Sclerosis (ALS)

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RT001 (lipid peroxidation inhibitor)	Retrotope Los Altos, CA	ALS	Phase II www.retrotope.com
SAR443820 (DNL788) (RIPK1 inhibitor)	Denali Therapeutics South San Francisco, CA Sanofi Bridgewater, NJ	ALS	Phase I www.denalitherapeutics.com www.sanofi.com
SBT-272 (peptidomimetic)	Stealth BioTherapeutics Needham, MA	ALS	Phase I www.stealthbt.com
SLS-005 (trehalose) (protein aggregation inhibitor) ORPHAN DRUG	Seelos Therapeutics New York, NY	ALS	Phase II www.seelostherapeutics.com
Ultomiris [®] ravulizumab-cwvz	Alexion AstraZeneca Rare Disease Boston, MA	ALS	Phase III www.alexion.com
verdiperstat (oral MPO inhibitor)	Biohaven Pharmaceuticals New Haven, CT	ALS	Phase III www.biohavenpharma.com
WVE-004 (RNA interference)	Wave Life Sciences Cambridge, MA	ALS	Phase I/II www.wavelifesciences.com

Ataxia

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
CTI-1601 (frataxin protein replacement) ORPHAN DRUG	Larimar Therapeutics Bala Cynwyd, PA	Friedreich's ataxia (Fast Track)	Phase I completed www.larimartx.com
EDS-EP (dexamethasone erythrocyte encapsulated) ORPHAN DRUG	EryDel Urbino, Italy	ataxia telangiectasia	Phase III www.erydel.com
Ery-Dex (dexamethasone erythrocyte encapsulated) ORPHAN DRUG	EryDel Bresso, Italy	ataxia telangiectasia	Phase III www.erydel.com
GTX-102 (betamethasone oral mucosal spray) ORPHAN DRUG	Acasti Pharma East Brunswick, NJ	ataxia telangiectasia	Phase III www.acastipharma.com
IB1001 (N-acetyl-L-leucine) ORPHAN DRUG	IntraBio Oxfordshire, United Kingdom	ataxia telangiectasia	Phase II www.intrabio.com
JOT101 (oral capsule of trans-reversatrol) ORPHAN DRUG	Jupiter Orphan Therapeutics Jupiter, FL	Friedreich's ataxia	Phase II www.jupiterorphan.com
MIB-626 (NAD+ enhancer)	Metro International Biotech Birmingham, MI	Friedreich's ataxia	Phase II www.metrobiotech.com

Ataxia

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omaveloxolone (RTA-408) (Nrf2 stimulant) ORPHAN DRUG	Reata Pharmaceuticals Plano, TX	Friedreich's ataxia	Phase II www.reatapharma.com
RT001 (lipid peroxidation inhibitor) ORPHAN DRUG	Retrotope Los Altos, CA	Friedreich's ataxia (Fast Track)	Phase III www.retrotope.com
troriluzole (BHV4157) (glutamate modulator) ORPHAN DRUG	Biohaven Pharmaceuticals New Haven, CT	spinocerebellar ataxia (Fast Track)	Phase III www.biohavenpharma.com
vatiquinone (NQO1 modulator) ORPHAN DRUG	PTC Therapeutics South Plainfield, NJ	Friedreich's ataxia (Fast Track)	Phase III www.ptcbio.com

Dementias (Non-Alzheimer's)

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
AL001 (SORT1 inhibitor) ORPHAN DRUG	Alector South San Francisco, CA	frontotemporal dementia with a granulin mutation (Fast Track)	Phase III www.alector.com
		frontotemporal dementia with a C9orf72 mutation	Phase II www.alector.com

Dementias (Non-Alzheimer's)

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ANAVEX® 3-71 (muscarinic receptor agonist/ sigma-1 receptor agonist) ORPHAN DRUG	Anavex Life Sciences New York, NY	frontotemporal dementia	Phase I www.anavex.com
ANVS401 (R-phenserine)	Annovis Bio Berwyn, PA	chronic traumatic encephalopathy, frontotemporal dementia	Phase I www.annovisbio.com
BIIB080 (IONIS-MAPT _{RX}) (tau-targeting protein)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	frontotemporal degeneration	Phase II www.biogen.com www.ionispharma.com
bosutinib (tyrosine kinase inhibitor)	KeifeRx McLean, VA	Lewy body dementia	Phase II www.keiferx.com
E2027 (PDE9 inhibitor)	Eisai Woodcliff Lake, NJ	Lewy body dementia	Phase II www.eisai.com
GRN gene therapy	Lilly Indianapolis, IN	frontotemporal dementia with GRN mutations	Phase II www.lilly.com
mevidalen (LY1354207) (dopamine D1 receptor modulator)	Lilly Indianapolis, IN	symptomatic Lewy body dementia	Phase II www.lilly.com

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neflamapimod (VX-745) (p38 mitogen-activated protein kinase inhibitor)	EIP Pharma Cambridge, MA	Lewy body dementia (Fast Track)	Phase II www.eippharma.com
nilotinib (tyrosine kinase inhibitor)	KeifeRx McLean, VA	Lewy body dementia	Phase II www.keiferx.com
NYX-458 (NMDA receptor modulator)	Aptinyx Evanston, IL	Lewy body dementia	Phase II www.aptinyx.com
PR006 (gene therapy)	Prevail Therapeutics New York, NY	frontotemporal dementia with GRN mutation	Phase I/II www.prevailtherapeutics.com
WVE-004 (RNA interference)	Wave Life Sciences Cambridge, MA	frontotemporal dementia	Phase I/II www.wavelifesciences.com

Genetic/Inherited Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
apitegromab (SRK-015) (myostatin inhibitor) ORPHAN DRUG	Scholar Rock Cambridge, MA	spinal muscular atrophy (Fast Track)	Phase II www.scholarrock.com
AT-GTX-501 gene therapy ORPHAN DRUG	Amicus Therapeutics Philadelphia, PA	CLN6 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase I/II www.amicusrx.com

Genetic/Inherited Diseases

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AT-GTX-502 gene therapy ORPHAN DRUG	Amicus Therapeutics Philadelphia, PA	CLN3 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase I/II www.amicusrx.com
AXO-AAV-GM1 (AAV9-based gene therapy) ORPHAN DRUG	Sio Gene Therapies New York, NY	GM1 gangliosidosis	Phase I/II www.siogtx.com
AXO-AAV-GM2 (AAVrh8-based gene therapy) ORPHAN DRUG	Sio Gene Therapies New York, NY	Tay-Sachs/Sandhoff disease (GM2 gangliosidosis)	Phase I/II www.siogtx.com
BBP-671 (pantothenate kinase stimulant)	CoA Therapeutics San Francisco, CA	pantothenate kinase-associated neurodegeneration	Phase I www.coatherapeutics.com
BIIB110 (ActRIIA/B ligand trap)	Biogen Cambridge, MA	spinal muscular atrophy	Phase I www.biogen.com
branaplam (LMI070) (SMN2 RNA splicing modulator) ORPHAN DRUG	Novartis East Hanover, NJ	spinal muscular atrophy	Phase II www.novartis.com
Evrydi® risdiplam	Roche/Genentech South San Francisco, CA	spinal muscular atrophy (neonates)	Phase II www.roche.com
gavorestat (AT-007) (aldehyde reductase inhibitor)	Applied Therapeutics New York, NY	sorbitol dehydrogenase (SORD) deficiency	Phase I www.appliedtherapeutics.com

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IB1001 (N-acetyl-L-leucine) ORPHAN DRUG	IntraBio Oxfordshire, United Kingdom	Sandhoff disease (Fast Track), Tay-Sachs disease (Fast Track), Neumann-Pick type C	Phase II www.intrabio.com
Lenti-D™ elivaldogene autotemcel ORPHAN DRUG	bluebird bio Cambridge, MA	cerebral adrenoleukodystrophy (adolescents, children, infants, neonates) (Breakthrough Therapy)	Phase III www.bluebirdbio.com
leriglitazone (MIN-102) (PPAR gamma agonist) ORPHAN DRUG	Minoryx Therapeutics Barcelona, Spain	cerebral adrenoleucodystrophy (Fast Track)	Phase II/III www.minoryx.com
LX1004 (AAV-mediated gene therapy) ORPHAN DRUG	LEXEO Therapeutics New York, NY	CLN2 Batten disease (neuronal ceroid lipofuscinoses)	Phase I/II www.lexeotx.com
mirdametinib (MEK 1/2 inhibitor) ORPHAN DRUG	SpringWorks Therapeutics Stamford, CT	neurofibromatosis 1 (Fast Track)	Phase II www.springworkstx.com
NFX-179 topical gel (MEK inhibitor) ORPHAN DRUG	NFlection Therapeutics Wayne, PA	neurofibromatosis 1	Phase II completed www.nflectionrx.com
OAV101 (SMN gene therapy)	Novartis Gene Therapies Bannockburn, IL	spinal muscular atrophy type 2/3 (IT formulation)	Phase II www.novartis.com

Genetic/Inherited Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
PLX-200 (TFEB upregulator) ORPHAN DRUG	Polaryx Therapeutics Paramus, NJ	CLN3 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase II www.polaryx.com
PTX3003 (baclofen/naltrexone/sorbitol) ORPHAN DRUG	Pharnext Paris, France	Charcot-Marie-Tooth disease (Fast Track)	Phase III www.pharnext.com
rAAV Olig001 ASPA gene therapy	Cure Rare Disease Boston, MA	Canavan disease	Phase I/II www.curerareisease.org
REC-2282 (HDAC inhibitor)	Recursion Pharmaceuticals Salt Lake City, UT	neurofibromatosis 2	Phase I www.recursion.com
RT001 (lipid peroxidation inhibitor) ORPHAN DRUG	Retrotope Los Altos, CA	infantile neuroaxonal dystrophy	Phase II/III www.retrotope.com
TSHA-118 (AAV9-based gene therapy)	Taysha Gene Therapies Dallas, TX	CLN1 Batton disease (neuronal ceroid lipofuscinoses)	Phase I/II www.tayshagtx.com
TSHA-120 (AAV9-based gene therapy) ORPHAN DRUG	Taysha Gene Therapies Dallas, TX	giant axonal neuropathy	Phase I/II www.tayshagtx.com
VK-0214 (TR beta agonist) ORPHAN DRUG	Viking Therapeutics San Diego, CA	X-linked adrenoleucodystrophy	Phase I www.vikingtherapeutics.com

Huntington's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
AMT-130 (AAV5 gene therapy) ORPHAN DRUG	uniQure Biopharma Lexington, MA	Huntington's disease (Fast Track)	Phase I/II www.uniqure.com
ANX005 (complement C1 inhibitor)	Annexon Biosciences South San Francisco, CA	Huntington's disease	Phase II www.annexonbio.com
branaplam (LMI070) (SMN2 RNA splicing modulator) ORPHAN DRUG	Novartis East Hanover, NJ	Huntington's disease	Phase I www.novartis.com
Ingrezza® valbenazine	Neurocrine Biosciences San Diego, CA	chorea in Huntington's disease	Phase III www.neurocrine.com
MP101 (brain penetrant mitochondrial uncoupler) ORPHAN DRUG	Mitochon Pharmaceuticals Blue Bell, PA	Huntington's disease	Phase I www.mitochonpharma.com
neflamapimod (VX-745) (p38 mitogen-activated protein kinase inhibitor)	EIP Pharma Cambridge, MA	Huntington's disease	Phase II www.eippharma.com
nilotinib (tyrosine kinase inhibitor)	KeifeRx McLean, VA	Huntington's disease	Phase I www.keiferx.com

Huntington's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
pepinemab (anti-SEMA4D mAb) ORPHAN DRUG	Vaccinex Rochester, NY	Huntington's disease (Fast Track)	Phase II completed www.vaccinex.com
pridopidine (sigma-1 receptor agonist) ORPHAN DRUG	Prilenia Therapeutics Naarden, Netherlands	Huntington's disease	Phase III www.prilenia.com
PTC518 (HD protein inhibitor)	PTC Therapeutics South Plainfield, NJ	Huntington's disease	Phase I www.ptcbio.com
RG6042 (tominersen) (RNA interference) ORPHAN DRUG	Ionis Pharmaceuticals Carlsbad, CA Roche/Genentech South San Francisco, CA	Huntington's disease	Phase III www.roche.com
SAGE-718 (NMDA receptor modulator)	Sage Therapeutics Cambridge, MA	cognitive dysfunction in Huntington's disease	Phase II www.sagerx.com
T3D-959 (PPAR-delta/gamma agonist)	T3D Therapeutics Research Triangle Park, NC	Huntington's disease	Phase I www.t3dtherapeutics.com
WVE-003 (RNA interference)	Wave Life Sciences Cambridge, MA	Huntington's disease	Phase I/II www.wavelifesciences.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
ANK-700 (immunomodulator)	Anokion Ecublens, Switzerland	relapsing remitting multiple sclerosis	Phase I www.anokion.com
arbaclofen ER	Osmotica Pharmaceutical Bridgewater, NJ	multiple sclerosis spasticity	Phase III www.osmotica.com
ATA188 (T-cell immunotherapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis	Phase II www.atarabio.com
BIIB061 (orla remyelination)	Biogen Cambridge, MA	multiple sclerosis	Phase I www.biogen.com
BIIB091 (BTK inhibitor)	Biogen Cambridge, MA	multiple sclerosis	Phase I www.biogen.com
BIIB107 (integrin alpha4beta1 antagonist)	Biogen Cambridge, MA	multiple sclerosis	Phase I www.biogen.com
CNM-Au8 (nanotherapeutic)	Clene Nanomedicine Salt Lake City, UT	multiple sclerosis	Phase II www.clene.com
EHP-101 (PPAR gamma modulator/ CB2 receptor agonist)	Emerald Health Pharmaceuticals San Diego, CA	multiple sclerosis	Phase II www.emeraldpharma.life

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
elezanumab (RGMA protein inhibitor)	AbbVie North Chicago, IL	multiple sclerosis	Phase II www.abbvie.com
evobrutinib (BTK inhibitor)	EMD Serono Rockland, MA	relapsing multiple sclerosis	Phase III www.emdserono.com
fenebrutinib (RG7845) (BTK inhibitor)	Roche/Genentech South San Francisco, CA	primary progressive multiple sclerosis, relapsing multiple sclerosis	Phase III www.roche.com
foralumab (regulatory T-lymphocyte stimulant)	Tiziana Life Sciences New York, NY	secondary progressive multiple sclerosis	Phase I www.tizianalifesciences.com
GA depot (glatiramer acetate controlled release)	Mapi Pharma Ness Ziona, Israel	relapsing remitting multiple sclerosis	Phase III www.mapi-pharma.com
IMS110 (mesenchymal stem cell therapy)	ImStem Biotechnology Farmington, CT	multiple sclerosis	Phase I www.imstem.com
Lu AG06466 (MAGL inhibitor)	Lundbeck Deerfield, IL	multiple sclerosis spasticity	Phase I www.lundbeck.com
Mayzent [®] spinonimod	Novartis East Hanover, NJ	secondary progressive multiple sclerosis (pediatric)	Phase III www.novartis.com
MN-166 (ibudilast) (neuroprotective therapeutic)	MediciNova La Jolla, CA	progressive multiple sclerosis (Fast Track)	Phase II www.medicinova.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
natalizimab biosimilar	Polpharma Biologics Gdansk, Poland Sandoz Princeton, NJ	relapsing-remitting multiple sclerosis	Phase III www.polpharmabiologics.com www.sandoz.com
NeuroVax (immunostimulant vaccine) ORPHAN DRUG	Immune Response BioPharma Atlantic City, NJ	multiple sclerosis (Fast Track)	Phase II/III www.immuneresponsebiopharma.com
NurOwn® (neurotrophic factor-producing mesenchymal stem cell therapy)	Brainstorm Cell Therapeutics New York, NY	progressive multiple sclerosis	Phase II www.brainstorm-cell.com
Ocrevus® obinutuzumab (high dose)	Roche/Genentech South San Francisco, CA	relapsing multiple sclerosis and primary progressive multiple sclerosis	Phase III www.roche.com
orelabrutinib	InnoCare Pharma Beijing, China	relapsing remitting multiple sclerosis	Phase II www.innocarepharma.com
PIPE-307 (M1R antagonist)	Pipeline Therapeutics San Diego, CA	multiple sclerosis (myelin restoration)	Phase I www.pipelinetherapeutics.com
Plegridy® peginterferon beta-1a	Biogen Cambridge, MA	multiple sclerosis (pediatric)	Phase III www.biogen.com
RNS60 (intracellular signaling pathway activator)	Revalesio Tacoma, WA	multiple sclerosis	Phase I completed www.revalesio.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
RO7121932	Roche/Genentech South San Francisco, CA	multiple sclerosis	Phase I www.roche.com
SAR441344 (anti-CD40L mAb)	Immunext Lebanon, NH Sanofi Bridgewater, NJ	multiple sclerosis	Phase II www.sanofi.com
STAT-203 (noroxymorphone analog)	Statera BioPharma Fort Collins, CO	multiple sclerosis	Phase I www.staterabiopharma.com
Tecfidera [®] dimethyl fumarate	Biogen Cambridge, MA	relapsing remitting multiple sclerosis (pediatric)	Phase III www.biogen.com
tolebrutinib (BTK inhibitor)	Sanofi Bridgewater, NJ	primary progressive multiple sclerosis, relapsing multiple sclerosis, secondary progressive multiple sclerosis	Phase III www.sanofi.com
Tysarbi [®] natalizumab (subcutaneous formulation)	Biogen Cambridge, MA	relapsing multiple sclerosis	application submitted www.biogen.com
ublituximab (rituximab biobetter)	TG Therapeutics New York, NY	relapsing multiple sclerosis	Phase III www.tgtherapeutics.com
ZD03	Shanxi Zhendong Pharmaceutical Beijing, China	multiple sclerosis	Phase I completed www.zhendongpharm.com

Multiple System Atrophy

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
AAV2 GDNF gene therapy	Brain Neurotherapy Bio Columbus, OH	multiple system atrophy	Phase I www.brainneubio.com
BIIB101 (ION464) (SNCA ASO)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	multiple system atrophy	Phase I www.biogen.com
Lu AF82422 (alpha-synuclein mAb)	Lundbeck Deerfield, IL	multiple system atrophy	Phase I www.lundbeck.com
verdiperstat (oral MPO inhibitor) ORPHAN DRUG	Biohaven Pharmaceuticals New Haven, CT	multiple system atrophy (Fast Track)	Phase III www.biohavenpharma.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
AAV2 GDNF gene therapy	Brain Neurotherapy Bio Columbus, OH	Parkinson's disease	Phase I www.brainneubio.com
ABBV-0805 (alpha-synuclein)	AbbVie North Chicago, IL	Parkinson's disease	Phase I www.abbvie.com
ABBV-951 (dopamine receptor agonist)	AbbVie North Chicago, IL	Parkinson's disease	Phase III www.abbvie.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
AKST4290 (CCR3 inhibitor)	Alkahest San Carlos, CA	Parkinson's disease	Phase II www.alkahest.com
AKST/GRF6021 (neurogenesis stimulant)	Alkahest San Carlos, CA	Parkinson's disease with cognitive impairment	Phase II www.alkahest.com
ANAVEX® 2-73 (muscarinic receptor agonist/ sigma-1 receptor agonist)	Anavex Life Sciences New York, NY	Parkinson's disease dementia	Phase II www.anavex.com
ANVS401 (R-phenserine)	Annovis Bio Berwyn, PA	Parkinson's disease	Phase II www.annovisbio.com
Apokyn® apomorphine infusion pump	Supernus Pharmaceuticals Rockville, MD	on-off episodes in Parkinson's	application submitted www.supernus.com
AV101 + oral probenecid (NMDA receptor modulator)	VistaGen Therapeutics South San Francisco, CA	levodopa-induced dyskinesia in Parkinson's disease	Phase I www.vistagen.com
AXO-Lenti-PD (OXB-102) (lentivirus gene therapy)	Sio Gene Therapies New York, NY	Parkinson's disease	Phase II www.sioctx.com
BIIB094 (ION859) (LRRK2 ASO)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	Parkinson's disease	Phase I www.biogen.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
BIIB118 (CK1 inhibitor)	Biogen Cambridge, MA	irregular sleep wake rhythm disorder in Parkinson's disease	Phase I www.biogen.com
BIIB122 (DNL151) (LRRK2 SM)	Biogen Cambridge, MA Denali Therapeutics South San Francisco, CA	Parkinson's disease	Phase I www.biogen.com
CNM-Au8 (nanotherapeutic)	Clene Nanomedicine Salt Lake City, UT	Parkinson's disease	Phase II www.clene.com
CTC-413 (dopamine D2/D3 receptor agonist)	Chase Therapeutics Washington, DC	Parkinson's disease	Phase I www.chasetherapeutics.com
CVN424 (dopamine D2 receptor modulator)	Cerevance Boston, MA	Parkinson's disease	Phase II www.cerevance.com
DA01 (induced pluripotent stem cell therapy)	BlueRock Therapeutics Cambridge, MA	Parkinson's disease (Fast Track)	Phase I www.bluerocktx.com
DA-9805 (botanical)	Dong-A ST Seoul, South Korea	Parkinson's disease	Phase II completed en.donga-st.com
dipraglurant (mGluR5 modulator) ORPHAN DRUG	Addex Pharmaceuticals San Francisco, CA	levodopa-induced dyskinesia associated with Parkinson's disease	Phase III www.addextherapeutics.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
DopaFuse® levodopa/carbidopa continuous release	SynAgile Wilson, WY	Parkinson's disease	Phase II www.synagile.com
E2027 (PDE9 inhibitor)	Eisai Woodcliff Lake, NJ	Parkinson's disease dementia	Phase II www.eisai.com
ENT-01 (alpha-synuclein inhibitor)	Enterin Philadelphia, PA	Parkinson's disease	Phase II www.enterininc.com
EPI-589 (reversible redox cofactor)	Sumitomo Dainippon Pharma Okasa, Japan	Parkinson's disease	Phase II www.ds-pharma.com
ESB1609 (S1P5 agonist)	Escape Bio South San Francisco, CA	Parkinson's disease	Phase I www.escapebio.com
FB-101 (c-Abl inhibitor)	1ST Biotherapeutics Gyeonggi-do, South Korea	Parkinson's disease	Phase I www.1stbio.com
GM608 (pleiotropic peptide)	Genervon Biopharmaceuticals Pasadena, CA	Parkinson's disease	Phase II www.genervon.com
HB-adMSCs (autologous adipose-derived mesenchymal stem cell therapy)	Hope Biosciences Sugar Land, TX	Parkinson's disease	Phase II www.hope.bio

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
IkT-148009 (Bcr-abl tyrosine kinase inhibitor)	Inhibikase Therapeutics Atlanta, GA	Parkinson's disease	Phase I www.inhibikase.com
IPX203 (carbidopa/levodopa extended-release FDC)	Amneal Pharmaceuticals Bridgewater, NJ	Parkinson's disease	Phase III www.amneal.com
INP107 (carbidopa/levodopa intranasal)	Impel NeuroPharma Seattle, WA	morning OFF episodes in Parkinson's disease	Phase I completed www.impelnp.com
ISC-hpNSC[®] parthenogenetic stem cell-derived neural stem cells	International Stem Cell Carlsbad, CA	Parkinson's disease	Phase I completed www.internationalstemcell.com
JM-010 (buspirone/zolmitriptan)	Bukwang Pharmaceutical Seoul, South Korea	drug-induced dyskinesia in Parkinson's disease	Phase II www.bukwangpharm.com
JZP385 (Cav3 modulator)	Jazz Pharmaceuticals Dublin, Ireland	essential tremor in Parkinson's disease	Phase II www.jazzpharma.com
K102	Kashiv BioSciences Piscataway, NJ	Parkinson's disease	Phase I www.kashivbiosciences.com
lenrispodun (ITI-124) (PDE inhibitor)	Intra-Cellular Therapies New York, NY	Parkinson's disease	Phase II www.intracellulartherapies.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
Lu AF28996 (D1/D2 agonist)	Lundbeck Deerfield, IL	Parkinson's disease	Phase I www.lundbeck.com
LY03003 (rotigotine extended release)	Luye Pharma Shanghai, China	Parkinson's disease	Phase III www.luye.cn
mesdopetam (dopamine D3 receptor antagonist)	Integrative Research Laboratories Göteborg, Sweden Ipsen Paris, France	levodopa-induced dyskinesia in Parkinson's disease	Phase II www.irlab.se
mesocarb (dopamine uptake inhibitor)	Melior Pharmaceuticals Exton, PA	Parkinson's disease	Phase I www.meliorpharmaceuticals.com
NBTX-001 (NMDA receptor antagonist)	Nobilis Therapeutics Portland, OR	Parkinson's disease	Phase I www.nobilistx.com
ND0612 (levodopa/carbidopa subcutaneous)	NeuroDerm Rehovot, IL	Parkinson's disease	Phase III www.neuroderm.com
NE3107 (NF- κ B/ERK1/2/MAPK3/1 inhibitor)	BioVie Santa Monica, CA	Parkinson's disease	Phase I www.bioviepharma.com
nilotinib (tyrosine kinase inhibitor)	KeifeRx McLean, VA	Parkinson's disease	Phase III www.keiferx.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
NLX-P101 (AAV-GAD gene therapy)	MeiraGTx New York, NY	Parkinson's disease (Fast Track)	Phase I/II www.meiragtx.com
NLY01-PD (GLP-1R agonist)	Neuraly Gaithersburg, MD	early Parkinson's disease	Phase II www.neuraly.com
NPT520-34 (neuroinflammation)	Neuropore Therapies San Diego, CA	Parkinson's disease	Phase I www.neuropore.com
NYX-458 (NMDA receptor modulator)	Aptinyx Evanston, IL	Parkinson's disease-associated cognition disorders	Phase II www.aptinyx.com
P2B001 (pramipexole/rasagiline)	Pharma Two B Rehovot, Israel	early Parkinson's disease	Phase III www.pharma2b.com
PR001 (GBA1 gene therapy)	Lilly Indianapolis, IN Prevail Therapeutics New York, NY	Parkinson's disease with GBA1 mutation (Fast Track)	Phase II www.prevailtherapeutics.com
PTC857 (15-lipoxygenase inhibitor)	PTC Therapeutics South Plainfield, NJ	Parkinson's disease with GBA mutation	Phase I www.ptcbio.com
RG7935 (prasinezumab) (alpha-synuclein inhibitor)	Roche/Genentech South San Francisco, CA	Parkinson's disease	Phase II www.roche.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
SAGE-324 (BIIB124) (GABA A receptor modulator)	Biogen Cambridge, MA Sage Therapeutics Cambridge, MA	Parkinson's disease	Phase I www.sagerx.com
SAGE-718 (NMDA receptor modulator)	Sage Therapeutics Cambridge, MA	cognitive dysfunction in Parkinson's disease	Phase II www.sagerx.com
SPN-830 (apomorphine subcutaneous)	Supernus Pharmaceuticals Rockville, MD	hypomobility in Parkinson's disease	application submitted www.supernus.com
Staccato® apomorphine (inhalation)	Alexza Pharmaceuticals Mountain View, CA	Parkinson's disease (acute treatment of OFF episodes)	Phase I www.alexza.com
SUN-K0706 (Bcr-abl tyrosine kinase inhibitor)	Sun Pharma Advanced Research Mumbai, India	early Parkinson's disease	Phase II www.sunpharma.com
TAK-071 (muscarinic M1 receptor modulator)	Takeda Deerfield, IL	Parkinson's disease	Phase I www.takeda.com
TAK-341/MEDI1341 (alpha-synuclein inhibitor)	AstraZeneca Bridgewater, NJ Takeda Deerfield, IL	Parkinson's disease	Phase I www.astrazeneca.com www.takeda.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
tavapadon (D1/D5 receptor agonist)	Cerevel Therapeutics Cambridge, MA	early Parkinson's disease, late Parkinson's disease (adjunct with L-dopa)	Phase III www.cerevel.com
UCB0022	UCB Smyrna, GA	Parkinson's disease	Phase I www.neuropore.com
UCB0599 (α -synuclein oligomerization inhibitor)	Neuropore Therapies San Diego, CA UCB Biopharma Smyrna, GA	early Parkinson's disease	Phase II www.neuropore.com
UB-312 (alpha-synuclein inhibitor)	Vaxxinity Dallas, TX	Parkinson's disease	Phase I www.vaxxinity.com
XC-130 (dopamine receptor agonist)	Xoc Pharmaceuticals Los Gatos, CA	Parkinson's disease	Phase I www.xocpharma.com
YTX-7739 (SCD inhibitor)	Yumanity Therapeutics Boston, MA	Parkinson's disease	Phase I www.yumanity.com

Progressive Supranuclear Palsy

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
bepranemab (UCB0107) (tau protein inhibitor)	UCB Smyrna, GA	progressive supranuclear palsy	Phase I www.ucb.com

Progressive Supranuclear Palsy

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
fasudil (rho kinase inhibitor) ORPHAN DRUG	Woolsey Pharmaceuticals New York, NY	progressive supranuclear palsy	Phase II www.woolseypharma.com
NIO752 (tau antagonist)	Novartis East Hanover, NJ	progressive supranuclear palsy	Phase I www.novartis.com
RT001 (lipid peroxidation inhibitor) ORPHAN DRUG	Retrotope Los Altos, CA	progressive supranuclear palsy	Phase II www.retrotope.com

Other

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
CST-2032 (beta-2 adrenoceptor agonist)	CuraSen Therapeutics San Carlos, CA	neurodegenerative diseases	Phase I www.curasen.com
fasudil (rho kinase inhibitor) ORPHAN DRUG	Woolsey Pharmaceuticals New York, NY	corticobasal degeneration	Phase II www.woolseypharma.com
ION373 (GFAP inhibitor, RNA interference) ORPHAN DRUG	Ionis Pharmaceuticals Carlsbad, CA	Alexander disease (leukodystrophy)	Phase III www.ionis.com

Other

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Status</u>
OS870	Osmotica Pharmaceutical Bridgewater, NJ	neurodegenerative diseases	Phase I www.osmotica.com
PNT001 (tau protein inhibitor)	Pinteon Therapeutics Newton, MA	neurodegenerative diseases	Phase I completed www.pinteon.com
RG6182	Roche/Genentech South San Francisco, CA	neurodegenerative diseases	Phase I www.roche.com

The content of this report has been obtained through public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. Report current as of **September 9, 2021**. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States and abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. Some products may not be in active clinical trials. The information may not be comprehensive. For more, specific information about a particular product, contact the individual company directly or go to www.clinicaltrials.gov. The entire series of *Medicines in Development* is available on PhRMA's website: www.phrma.org.

Definitions

Application Submitted—An application for marketing has been submitted by the company to the U.S. Food and Drug Administration (FDA).

Breakthrough Therapy—Upon request by a sponsor, the FDA can grant this designation to expedite the development and review of a drug or biologic intended, alone or in combination with one or more other drugs, to treat a serious or life threatening disease or condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically-significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug or biologic is designated as a Breakthrough Therapy, the FDA will expedite the development and review. With this designation, all Fast Track features convey to the medicine.

Fast Track—Upon request by a sponsor, the FDA can grant this designation to facilitate the development and expedite the review of a drug or biologic to treat a serious condition and fill an unmet medical need. When considering a biopharmaceutical company's request for Fast Track designation for an investigational drug or biologic, the FDA evaluates whether it will affect factors such as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one, and whether a condition can be adequately addressed by available therapy. With Fast Track designation, early and frequent communication between the FDA and the biopharmaceutical company is encouraged throughout the entire drug development and review process to help to quickly resolve any questions or issues that arise, potentially leading to an earlier approval and access by patients.

Definitions

Orphan Designation—Upon request by a sponsor, the FDA can grant special status (“orphan status”) to a drug or biologic to treat a rare disease or condition. In order to receive an orphan designation, a qualifying drug or biologic must be intended for the treatment, diagnosis, or prevention of a rare disease or condition that affects usually fewer than 200,000 people in the United States.

Phase I—Researchers test the investigational drug or biologic in a small group of people, usually between 20 and 100 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range, and identify potential side effects.

Phase II—The investigational drug or biologic is given to volunteer patients, usually between 100 and 500, to determine whether it is effective, identify an optimal dose, and to further evaluate its short-term safety.

Phase III—The investigational drug or biologic is given to a larger, more diverse patient population, often involving between 1,000 and 5,000 patients (but sometimes many more), to generate statistically significant evidence to confirm its safety and effectiveness. Phase III studies are the longest studies and usually take place in multiple sites around the world.