

2018 Medicines in Development for Neurological Disorders

A Report on Disorders of the Brain, Spinal Cord and Nerves

Alzheimer's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ABBV-8E12 (anti-tau antibody)	AbbVie North Chicago, IL	Alzheimer's disease (see also other)	Phase II www.abbvie.com
AC-1204 (glucose stimulant)	Accera Boulder, CO	mild to moderate Alzheimer's disease	Phase III www.accerapharma.com
ACI-24 (anti-Abeta vaccine)	AC Immune Lausanne, Switzerland	Alzheimer's disease	Phase I www.acimmune.com
ACI-35 (anti-pTau vaccine)	AC Immune Lausanne, Switzerland Janssen Pharmaceuticals Raritan, NJ	Alzheimer's disease	Phase I www.acimmune.com www.janssen.com
aducanumab (BIIB037) (amyloid beta mAb)	Biogen Cambridge, MA Eisai Woodcliff Lake, NJ	Alzheimer's disease (Fast Track)	Phase III www.biogen.com
AGB101 (levetiracetam low-dose)	AgeneBio Baltimore, MD	amnesic mild cognitive impairment in Alzheimer's disease	Phase II completed www.agenebio.com

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ALZ-801 (amyloid beta-protein inhibitor)	Alzheon Framingham, MA	mild Alzheimer's disease (Fast Track) (homozygous APOE4/4 genotype)	Phase II www.alzheon.com
		mild Alzheimer's disease (heterozygous APOE4 genotype)	Phase II www.alzheon.com
ALZT-OP1 (amyloid beta-protein inhibitor/ inflammation mediator inhibitor)	AZTherapies Boston, MA	Alzheimer's disease	Phase III www.aztherapies.com
AMG520 (CNP520) (BACE1 protein inhibitor)	Amgen Thousand Oaks, CA Novartis Pharmaceuticals East Hanover, NJ	Alzheimer's disease (Fast Track)	Phase II/III www.amgen.com www.novartis.com
ANAVEX™ 2-73 (M1 muscarinic receptor agonist/ intracellular sigma 1 receptor agonist)	Anavex Life Sciences New York, NY	Alzheimer's disease (see also epilepsy, genetic, Parkinson's)	Phase II www.anavex.com
AstroStem mesenchymal stem cell therapy	Nature Cell Seoul, South Korea	Alzheimer's disease	Phase I/II www.naturecell.co.kr
AUS-131 (nonhormonal estrogen receptor agonist)	Ausio Pharmaceuticals Cincinnati, OH	Alzheimer's disease	Phase I/II www.ausiopharma.com

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AVN-101 (serotonin 6 receptor antagonist)	Avineuro San Diego, CA	cognition dysfunction associated with Alzheimer's disease	Phase II www.avineuro.com
AVN-322 (serotonin 6 receptor antagonist)	Avineuro San Diego, CA	cognition dysfunction associated with Alzheimer's disease	Phase II www.avineuro.com
AVP-786 (deudextromethorphan analogue/ ultra-low does quinidine)	Avanir Pharmaceuticals Aliso Viejo, CA	agitation in Alzheimer's disease (Fast Track) (see also brain injury)	Phase III www.avanir.com
AXS-05 (bupropion/dextromethorphan)	Axsome Therapeutics New York, NY	agitation in Alzheimer's disease (Fast Track)	Phase II/III www.axsome.com
azeliragon (TTP488) (RAGE antagonist)	vTv Therapeutics High Point, NC	Alzheimer's disease (Fast Track)	Phase III www.vtvtherapeutics.com
BAN2401 (anti-amyloid beta mAb)	Biogen Cambridge, MA Eisai Woodcliff Lake, NJ	Alzheimer's disease	Phase II www.biogen.com www.eisai.com
bexarotene (RXR-selective retinoid analogue)	ReXceptor Cambridge, MA	Alzheimer's disease	Phase I completed www.rexceptor.com
BI 409306 (PDE9A inhibitor)	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	Alzheimer's disease	Phase II www.boehringer-ingelheim.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	Alzheimer's disease (see also other)	Phase I/II www.biogen.com
BIIB092 (tau protein inhibitor)	Biogen Cambridge, MA	Alzheimer's disease (see also other)	Phase I 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 (type 4 cyclic nucleotide phosphodiesterase inhibitor)	Tetra Discovery Partners Grand Rapids, MI	Alzheimer's disease	Phase I www.tetradiscovery.com
bryostatin 1 (protein kinase C stimulant)	Neurotrope BioScience New York, NY	Alzheimer's disease	Phase II www.neurotropebioscience.com
CAD106 (amilomotide) (VLP immunotherapy vaccine)	Novartis Pharmaceuticals East Hanover, NJ	Alzheimer's disease	Phase II www.novartis.com

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COR-388 (bacterial protease inhibitor)	Cortexyme South San Francisco, CA	Alzheimer's disease	Phase I www.cortexyme.com
Corplex Donepezil donepezil transdermal patch	Corium International Menlo Park, CA	Alzheimer's disease	Phase I www.coriumgroup.com
Corplex Memantine memantine transdermal patch	Corium International Menlo Park, CA	Alzheimer's disease	Phase I www.coriumgroup.com
CPC-201 (donepezil/solifenacin)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase II www.allergan.com
CPC-212 (next-generation acetylcholinesterase inhibitor)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase I completed www.allergan.com
CPC-250 (next-generation acetylcholinesterase inhibitor)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase I completed www.allergan.com
CSP-1103 (amyloid beta-protein inhibitor)	CereSpir New York, NY	mild cognitive impairment in patients at risk for Alzheimer's	Phase II www.cerespir.com

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crenezumab (anti-amyloid beta antibody)	Genentech South San Francisco, CA	Alzheimer's disease	Phase III www.gene.com
CSTC1 (BAC)	Charsire Biotechnology Tainan, Taiwan	Alzheimer's disease	Phase II www.charsire.com.tw
CT1812 (amyloid beta oligomer receptor antagonist)	Cognition Therapeutics Pittsburgh, PA	mild to moderate Alzheimer's disease (Fast Track)	Phase I www.cogrx.com
E2006 (anti-amyloid beta mAb)	Eisai Woodcliff Lake, NJ Purdue Pharma Stamford, CT	irregular sleep-wake rhythm disorder and Alzheimer's disease	Phase II www.eisai.com
E2027 (amyloid beta oligomer receptor antagonist)	Eisai Woodcliff Lake, NJ	Alzheimer's disease	Phase I www.eisai.com
E2609 (BACE1 protein inhibitor)	Biogen Cambridge, MA Eisai Woodcliff Lake, NJ	early Alzheimer's disease (Fast Track)	Phase III www.biogen.com www.eisai.com
eltoprazine (serotonin 1A/1B receptor agonist)	Amarantus BioScience San Francisco, CA	aggression in Alzheimer's disease (see also Parkinson's)	Phase II www.amarantus.com

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EVT302 (MAO-B inhibitor)	Evotec Princeton, NJ	Alzheimer's disease	Phase II www.evotec.com
gantenerumab (amyloid beta-protein inhibitor)	Genentech South San Francisco, CA	Alzheimer's disease	Phase III www.gene.com
GC021109 (purinoceptor P2Y6 agonist)	GliaCure Boston, MA	Alzheimer's disease	Phase I www.gliacure.com
GRF6019 (plasma infusions)	Alkahest San Carlos, CA	mild to moderate Alzheimer's disease	Phase I www.alkahest.com
HSRx-888 (donepezil/food-based compound)	HSRx Biopharmaceutical Tucson, AZ	Alzheimer's disease	Phase II www.hsrxbiopharmaceutical.com
immune globulin/albumin	Grifols Los Angeles, CA	Alzheimer's disease	Phase III www.grifols.com
INP102 (intranasal insulin)	Impel NeuroPharma Seattle, WA	Alzheimer's disease	Phase 0 www.impelnp.com
JNJ-54861911 (BACE inhibitor)	Janssen Research & Development Raritan, NJ	asymptomatic patients (amyloid-positive) at risk for Alzheimer's	Phase II/III www.janssen.com
JNJ-63733657 (immunomodulator)	Janssen Research & Development Raritan, NJ	Alzheimer's disease	Phase I www.janssen.com

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KPAX002 (repurposed methylphenidate)	K-PAX Pharmaceuticals Mill Valley, CA	Alzheimer's disease (see also ALS, Parkinson's)	in clinical trials www.kpaxpharmaceuticals.com
Ianabecestat (BACE inhibitor)	AstraZeneca Wilmington, DE Eli Lilly Indianapolis, IN	Alzheimer's disease (Fast Track)	Phase III www.astrazeneca.com www.lilly.com
LM11A-31 (p75 neurotrophin receptor)	Pharmatrophix Menlo Park, CA	mild to moderate Alzheimer's disease	Phase I/II 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 AF20513 (anti beta amyloid)	Lundbeck Deerfield, IL Otsuka Pharmaceutical Tokyo, Japan	mild Alzheimer's disease	Phase I www.lundbeck.com www.otsuka.com
LY3002813 (N3pG-A β mAb)	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase II www.lilly.com
LY3202626 (BACE inhibitor)	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase II www.lilly.com

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M1 agonist (selective 1 receptor agonist)	Allergan Parsippany, NJ Heptares Therapeutics Hertfordshire, United Kingdom	Alzheimer's disease	Phase I www.allergan.com
M4 agonist (muscarinic M4 receptor agonist)	Allergan Parsippany, NJ Heptares Therapeutics Hertfordshire, United Kingdom	Alzheimer's disease	Phase I www.allergan.com
MEDI1814 (A β 42 antibody)	Eli Lilly Indianapolis, IN MedImmune Gaithersburg, MD	Alzheimer's disease	Phase I www.lilly.com www.medimmune.com
MER5101 (amyloid beta protein modulator)	Mercia Pharma Washington, DC	Alzheimer's disease	Phase I www.merciapharma.com
mesenchymal stem cell therapy	Longeveron Miami, FL	Alzheimer's disease	Phase I www.longeveron.com
MP-101 (mGluR2/mGluR3 agonist)	Mediti Pharma Montreal, Canada	Alzheimer's disease	Phase II www.tvmlifescience.com
MSDC-0160 (mTOT modulator)	Metabolic Solutions Development Kalamazoo, MI	Alzheimer's disease	Phase II www.msdrx.com

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NDX-1017 (growth factor systems modifier)	M3 Biotechnology Seattle, WA	Alzheimer's disease	Phase I
neflamapimod (VX-745) (p38 mitogen-activated protein kinase inhibitor)	EIP Pharma Cambridge, MA	Alzheimer's disease	Phase II www.eippharma.com
NGP 555 (gamma secretase complex modulator)	NeuroGenetic Pharmaceuticals Rancho Santa Fe, CA	Alzheimer's disease	Phase I www.neurogeneticpharmaceuticals.com
nilvadipine (soluble amyloid reducing/ clearing agent)	Archer Pharmaceuticals Sarasota, FL NILVAD Dublin, Ireland	Alzheimer's disease	Phase III www.archerpharma.com
NP001 (macrophage activation regulator)	Neuraltus Pharmaceuticals San Bruno, CA	Alzheimer's disease (see also ALS)	Phase I www.neuraltus.com
NPT088 (GAIM Ig fusion targeting amyloid- β , tau, α -synuclein)	Proclara Biosciences Cambridge, MA	Alzheimer's disease	Phase I www.proclarabio.com
Nuedexta [®] dextromethorphan HBr and quinidine	Avanir Pharmaceuticals Aliso Viejo, CA	agitation in Alzheimer's disease (see also Parkinson's)	Phase II www.avanir.com

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Nuplazid [®] pimavanserin	ACADIA Pharmaceuticals San Diego, CA	agitation and aggression in Alzheimer's disease	Phase II www.acadia-pharm.com
piromelatine (melatonin agonist)	Neurim Pharmaceuticals Zug, Switzerland	Alzheimer's disease	Phase II www.neurin.com
Posiphen [®] R-phenserine	QR Pharma Berwyn, PA	early Alzheimer's disease	Phase I/II www.qrpharma.com
PTI-125 (microfilament protein inhibitor)	Pain Therapeutics Austin, TX	Alzheimer's disease	Phase I completed www.paintrials.com
PU-AD (HSP90 heat-shock protein inhibitor)	Samus Therapeutics Topsfield, MA	Alzheimer's disease (see also brain injury)	Phase 0 www.samustherapeutics.com
Rexulti [®] brexpiprazole	Lundbeck Deerfield, IL Otsuka Pharmaceutical Tokyo, Japan	agitation in Alzheimer's disease	Phase III www.lundbeck.com www.otsuka.com
RG6100 (tau protein inhibitor)	AC Immune Lausanne, Switzerland Genentech South San Francisco, CA	Alzheimer's disease	Phase II www.gene.com
RVT-104 (glycopyrrolate/rivastigmine combination)	Axovant Sciences New York, NY	Alzheimer's disease (see also other)	Phase I www.axovant.com

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SAR228810 (anti-protofibrillar AB mAb)	Sanofi Bridgewater, NJ	Alzheimer's disease	Phase I www.sanofi.com
selective BACE 1 inhibitor	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com
solanezumab	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase III www.lilly.com
SUVN-502 (serotonin 6 receptor antagonist)	Suven Life Sciences Hyderabad, India	Alzheimer's disease	Phase II www.suven.com
SUVN-D4010 (serotonin 4 receptor agonist)	Suven Life Sciences Hyderabad, India	Alzheimer's disease	Phase I www.suven.com
SUVN-G3031 (histamine H3 receptor antagonist)	Suven Life Sciences Hyderabad, India	cognition disorder in Alzheimer's disease	Phase III www.suven.com
T-817MA (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 I/II www.t3dtherapeutics.com
TAK-071 (muscarinic M1 receptor modulator)	Takeda Pharmaceuticals Deerfield, IL	Alzheimer's disease	Phase I www.takeda.com

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tau mAb	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com
TPI-287 (next generation taxane)	Cortice Biosciences New York, NY	mild to moderate Alzheimer's disease (see also cancer, other)	Phase I www.corticebiosciences.com
UB-311 (amyloid beta protein inhibitor vaccine)	United Neuroscience Hauppauge, NY	Alzheimer's disease	Phase II www.unitedneuroscience.com
UE-2343 (11 β -HSD1 inhibitor)	Actinogen Medical Sydney, Australia	dementia in Alzheimer's disease	Phase II www.actinogen.com

Amyotrophic Lateral Sclerosis (ALS)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AMX0035 (sodium phenylbutyrate/ tauroursodeoxycholic acid) ORPHAN DRUG	Amylyx Pharmaceuticals Cambridge, MA	amyotrophic lateral sclerosis (ALS)	Phase II www.amylyx.com
AQST-117 (riluzole oral soluble film)	Aquestive Therapeutics Warren, NJ	ALS	Phase III www.aquestive.com

Amyotrophic Lateral Sclerosis (ALS)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
BHV-0223 (riluzole sublingual) ORPHAN DRUG	Biohaven Pharmaceutical New Haven, CT	ALS	Phase I www.biohavenpharma.com
BIIB067 (IONIS-SOD1 _{rx}) (superoxide dismutase inhibitor) ORPHAN DRUG	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	ALS	Phase I/II www.biogen.com
BioE-589 (NAD(P)H modulator)	BioElectron Technology Mountain View, CA Sumitomo Dainippon Pharma Osaka, Japan	ALS (see also Parkinson's)	Phase II www.bioelectron.com
CC100	Chemigen Zionsville, IN	ALS	Phase I
CK-2127107 (reldesemtiv) (fast skeletal troponin activator)	Astellas Northbrook, IL Cytokinetics South San Francisco, CA	ALS (see also genetic)	Phase II www.astellas.com www.cytokinetics.com
Cu(II)ATSM (neuron modulator)	Procypra Therapeutics Sausalito, CA	ALS (see also Parkinson's)	Phase I/II www.colmeddev.com/procypra
FLX-787 (TRP ion channel agonist)	Flex Pharma Boston, MA	cramping in ALS (Fast Track) (see also genetic, spasticity)	Phase II www.flex-pharma.com

Amyotrophic Lateral Sclerosis (ALS)

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GDC0134 (RG6000) (DLK-1 protein inhibitor)	Genentech South San Francisco, CA	ALS	Phase I www.gene.com
GM604 (motoneuronotrophic factor) ORPHAN DRUG	Genervon Biopharmaceuticals Pasadena, CA	ALS (Fast Track)	Phase II www.genervon.com
H.P. Acthar® Gel repository corticotropin injection ORPHAN DRUG	Mallinckrodt Pharmaceuticals St. Louis, MO	ALS (Fast Track)	Phase III www.mallinckrodt.com
KPAX002 (repurposed methylphenidate)	K-PAX Pharmaceuticals Mill Valley, CA	ALS (see also Alzheimer's, Parkinson's)	in clinical trials www.kpaxpharmaceuticals.com
MN-166 (ibudilast) ORPHAN DRUG	MediciNova La Jolla, CA	ALS (Fast Track) (see also multiple sclerosis)	Phase II www.medicinova.com
NP001 (macrophage activation regulator)	Neuraltus Pharmaceuticals San Bruno, CA	ALS (see also Alzheimer's)	Phase II www.neuraltus.com
NSI-566 (spinal cord-derived neural stem cell therapy) ORPHAN DRUG	Neuralstem Germantown, MD	ALS (see also spinal cord injury)	Phase II www.neuralstem.com

Amyotrophic Lateral Sclerosis (ALS)

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NurOwn® neurotrophic factor-producing mesenchymal stem cell therapy ORPHAN DRUG	Brainstorm Cell Therapeutics Hackensack, NJ	ALS (Fast Track)	Phase III www.brainstorm-cell.com
RNS60 (charge-stabilized nanostructure) ORPHAN DRUG	Revalesio Tacoma, WA	ALS (see also multiple sclerosis)	Phase I www.revalesio.com
RPI-MN (nicotinic receptor antagonist)	ReceptoPharm Plantation, FL	ALS	Phase I completed www.receptopharm.com
VM202 (donaperminogene seltoplasmid) ORPHAN DRUG	ViroMed Seoul, South Korea	ALS (Fast Track) (see also pain)	Phase II www.viomed.co.kr

Brain Injury

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AVP-786 (deudextromethorphan analogue/ ultra-low does quinidine)	Avanir Pharmaceuticals Aliso Viejo, CA	neurobehavioral disinhibition in patients with traumatic brain injury (see also Alzheimer's)	Phase II www.avanir.com
Epidiolex cannabidiol ORPHAN DRUG	GW Pharmaceuticals Carlsbad, CA	neonatal hypoxic ischemic encephalopathy (Fast Track) (see also epilepsy, genetic)	Phase I www.gwpharm.com

Brain Injury

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
PU-AD (HSP90 heat-shock protein inhibitor)	Samus Therapeutics Topsfield, MA	chronic traumatic encephalopathy, traumatic brain concussion	Phase I www.samustherapeutics.com
SB623 (modified bone marrow-derived mesenchymal stem cells)	SanBio Mountain View, CA	traumatic brain injury (see also stroke)	Phase II www.san-bio.com
trofinetide	Neuren Pharmaceuticals Camberwell, Australia	traumatic brain injury (Fast Track) (see also genetic)	Phase II www.neurenpharma.com
VOLT02 (conjugated progesterone)	Levolta Pharmaceuticals Bethlehem, PA	traumatic brain injury	Phase II completed www.levoltapharma.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
2X-111 (liposomal doxorubicin) ORPHAN DRUG	2X Oncology Cambridge, MA	brain metastases from breast cancer, recurrent glioblastoma	Phase I completed www.2xoncology.com
abemaciclib (CDK 4 and 6 inhibitor)	Eli Lilly Indianapolis, IN Dana Farber Cancer Institute Boston, MA	glioblastoma	Phase II www.lilly.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
acalabrutinib (BTK inhibitor)	Acerta Pharma Redwood City, CA	recurrent glioblastoma	Phase II www.acerta-pharma.com
AG-881 (pan-IDH inhibitor)	Agios Pharmaceuticals Cambridge, MA Celgene Summit, NJ	glioma	Phase I www.agios.com www.celgene.com
aglatimagene besadenovec ORPHAN DRUG	Advantagene Auburndale, MA	glioma	Phase II www.advantagene.com
AMG 596 (anti-EGFRvIII x anti-CD3 BiTE® antibody construct)	Amgen Thousand Oaks, CA	glioblastoma	Phase I www.amgen.com
ANG1005 (paclitaxel trevatide) ORPHAN DRUG	AngioChem Seattle, WA	glioblastoma (Fast Track)	Phase II www.angiochem.com
anti-LAG3 mAb	Bristol-Myers Squibb Princeton, NJ	glioblastoma	Phase I www.bms.com
antineoplaston therapy ORPHAN DRUG	Burzynski Research Institute Houston, TX	diffuse intrinsic pontine glioma	Phase II www.burzynskiresearch.com
APX005M (CD40 agnostic antibody)	Apexigen San Carlos, CA	recurrent refractory brain tumors	Phase I www.apexigen.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AU101 (CAR-T intracranial)	Aurora BioPharma Cambridge, MA	recurrent glioblastoma	Phase I/II www.aurora-biopharma.com
AU105 (CAR-T)	Aurora BioPharma Cambridge, MA	newly diagnosed glioblastoma	Phase I/II www.aurora-biopharma.com
Avastin® bevacizumab	Genentech South San Francisco, CA	brain metastases	Phase II www.gene.com
Azixa® verubulin ORPHAN DRUG	Cytovia Englewood Cliffs, NJ	brain metastases, glioblastoma	Phase II www.immunepharm.com
BAL101553 (tumor checkpoint controller)	Basilea Pharmaceutica Basel, Switzerland Adult Brain Tumor Consortium Baltimore, MD	newly diagnosed glioblastoma	Phase I www.basilea.com
bavituximab	Oncologie Boston, MA	glioblastoma	Phase II www.oncologie.international
BB1608 (stem cell inhibitor)	Boston Biomedical Cambridge, MA	glioblastoma	Phase I/II www.bostonbiomedical.com
BGB-290 (pamiparib) (PARP inhibitor)	BeiGene USA Cambridge, MA	glioblastoma (combination therapy)	Phase I www.beigene.com

Brain Tumors

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BMX-001 (mangano porphyrin antioxidant mimetic)	BioMimetix Greenwood Village, CO	glioma	Phase I/II
BPM 31510 (ubidecarenone)	Berg Pharma Framingham, MA	glioblastoma	Phase I www.berghealth.com
BXQ-350 (apoptosis stimulant) ORPHAN DRUG	Bexion Pharmaceuticals Covington, KY	glioblastoma	Phase I www.bexionpharma.com
CC-122 (pleiotropic pathway modulator)	Celgene Summit, NJ	glioblastoma	Phase I www.celgene.com
Cerebral EDV™ doxorubicin encapsulated nanocells ORPHAN DRUG	EnGeneIC New York, NY	glioblastoma	Phase I www.engeneic.com
CUDC-907 (HDAC/pan-PI3K inhibitor)	Curis Lexington, MA	brain tumor, neuroblastoma	Phase I www.curis.com
DCC-2618 (PDGFR α kinase switch control inhibitor) ORPHAN DRUG	Deciphera Pharmaceuticals Waltham, MA	glioblastoma, glioma	Phase I www.deciphera.com
DCVax®-L brain cancer vaccine ORPHAN DRUG	Northwest Biotherapeutics Bethesda, MD	glioblastoma	Phase III www.nwbio.com

Brain Tumors

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depatuxizumab mafodotin (anti-EGFR antibody drug conjugate) ORPHAN DRUG	AbbVie North Chicago, IL	glioblastoma	Phase III www.abbvie.com
		pediatric brain tumors	Phase I www.abbvie.com
Dicopp™ copper gluconate/disulfiram ORPHAN DRUG	Cantex Pharmaceuticals Weston, FL	recurrent glioblastoma	Phase II www.cantex.com
DM-CHOC-PEN	Dekk-Tec New Orleans, LA	primary brain cancer, brain metastases	Phase II www.dekk-tec.com
DSP-7888 (adegramotide/nelatimotide) ORPHAN DRUG	Sumitomo Dainippon Pharma Osaka, Japan	recurrent glioblastoma	Phase II www.ds-pharma.com
eflornithine oral (ornithine decarboxylase inhibitor) ORPHAN DRUG	Orbus Therapeutics Palo Alto, CA	anaplastic astrocytoma	Phase III www.orbustherapeutics.com
epacadostat (IDO1 inhibitor)	InCyte Wilmington, DE	glioblastoma	Phase I/II www.incyte.com
ERC1671 (therapeutic cancer vaccine) ORPHAN DRUG	Epitopoietic Research Pasadena, CA	glioblastoma	Phase II www.erc-immunotherapy.com

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GBM-Lamp-Vax (dendritic cell vaccine comprising CMV antigen pp65 expressed as fusion protein with LAMP)	Immunomic Therapeutics Rockville, MD Duke University Durham, NC University of Florida Gainesville, FL	glioblastoma	Phase II www.immunomix.com
Gilotrif® afatinib ORPHAN DRUG	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	glioma	Phase II completed www.boehringer-ingelheim.com
glasdegib (PI3K/mTOR inhibitor)	Pfizer New York, NY	newly diagnosed glioblastoma	Phase I www.pfizer.com
HER2.taNK (chimeric antigen receptor T-cell therapy)	NantKwest Culver City, CA	glioblastoma	Phase I www.nantkwest.com
ICT-121 (dendritic cell vaccine)	ImmunoCellular Therapeutics Westlake Village, CA	recurrent glioblastoma	Phase I www.imuc.com
ifabotuzumab (EphA3 receptor antagonist)	Humanigen Brisbane, CA Olivia Newton-John Cancer Research Institute Heidelberg, Australia	glioblastoma	Phase I www.humanigen.com

Brain Tumors

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INC280 (proto-oncogene protein c-Met inhibitor)	Novartis Pharmaceuticals East Hanover, NJ	glioblastoma (combination therapy)	Phase I www.novartis.com
indoximod + temozolomide	NewLink Genetics Ames, IA	malignant brain tumors	Phase I www.newlinkgenetics.com
INO-5401 (T-cell activating DNA immunotherapy)	Inovio Pharmaceuticals Plymouth Meeting, PA Regeneron Pharmaceuticals Tarrytown, NY	glioblastoma (with cemiplimab)	Phase II www.inovio.com
INXN 2001 (Ad-RTS-hIL-12 / DNA-based therapy) ORPHAN DRUG	ZIOPHARM Oncology Boston, MA	glioblastoma	Phase I www.ziopharm.com
larotrectinib (TRK inhibitor)	Array BioPharma Boulder, CO Loxo Oncology Stamford, CT	primary CNS tumors with TRK fusions (adults and adolescents)	Phase II www.loxooncology.com
		primary CNS tumors with TRK fusions (pediatric)	Phase I/II www.loxooncology.com
marizomib (proteasome inhibitor) ORPHAN DRUG	Celgene Summit, NJ	glioblastoma	Phase II www.celgene.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
MB-101 (CAR-T cell therapy)	Mustang Bio New York, NY	glioblastoma	Phase I www.mustangbio.com
MDNA55 (interleukin-4 superkine) ORPHAN DRUG	Medicenna Therapeutics Toronto, Canada	glioblastoma (Fast Track)	Phase II www.medicenna.com
MNX-100 (nifurtimox)	MetronomX Houston, TX	medulloblastoma	Phase II www.metronomx.com
MTX110 (nano-inclusion product)	Midatech Pharma US Raleigh, NC	diffuse interstitial pontine glioma	Phase I www.midatechpharma.com
MV-NIS (cancer vaccine)	Vyriad Rochester, MN University of California-San Francisco San Francisco, CA	medulloblastoma	Phase I www.vyriad.com
NEO100 (perillyl alcohol intranasal) ORPHAN DRUG	NEONC Technologies Los Angeles, CA	recurrent malignant glioma (Fast Track)	Phase I/II www.neonctech.com
NVX-108 (perflenapent nanoparticles) ORPHAN DRUG	NuvOx Pharma Tucson, AZ	glioblastoma (combination therapy)	Phase I www.nuvoxpharma.com
Odomzo [®] sonidegib	Novartis Pharmaceuticals East Hanover, NJ	medulloblastoma	Phase II completed www.novartis.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
OKN007 (disufenton sodium) ORPHAN DRUG	Oblato (GtreeBNT) Oklahoma City, OK	glioblastoma	Phase I www.gtreebnt.com
omburtamab I-124 (B7-H3 inhibitor)	Y-mAbs Therapeutics New York, NY	diffuse intrinsic pontine glioma	Phase I/II www.ymabs.com
omburtamab I-131 (B7-H3 inhibitor) ORPHAN DRUG	Y-mAbs Therapeutics New York, NY	CNS/leptomeningeal metastasis from neuroblastoma	Phase III www.ymabs.com
ONC201 (dopamine receptor D2/3 antagonist) ORPHAN DRUG	Oncocyteics Philadelphia, PA	high-grade gliomas (adults)	Phase II www.oncoceutics.com
		high-grade gliomas (pediatric)	Phase I www.oncoceutics.com
Onzeald™ etirinotecan pegol	Nektar San Francisco, CA	brain metastases from breast cancer (Fast Track)	Phase III www.nektar.com
Opdivo® nivolumab	Bristol-Myers Squibb Princeton, NJ	glioblastoma	Phase III www.bms.com
Opdivo® nivolumab + Yervoy® ipilimumab	Bristol-Myers Squibb Princeton, NJ	high grade primary CNS malignancies	Phase II www.bms.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
OT-101 (TGF-beta antisense) ORPHAN DRUG	Autotelic Costa Mesa, CA Oncotelic Agoura Hills, CA	glioblastoma	Phase II/III www.oncotelic.com
p28 cell penetrating peptide	CDG Therapeutics Chicago, IL	recurrent or progressive CNS tumors (pediatric)	Phase I www.cdgti.com
PAC-1 (VO-100) (caspase 3 stimulant)	Vanquish Oncology Champaign, IL	anaplastic astrocytoma, glioblastoma	Phase I www.vanquishoncology.com
PDT with Photofrin® porfimer sodium	Pinnacle Biologics Chicago, IL	brain cancer	Phase II www.pinnaclebiologics.com
PEPIDH1M (dendritic cell vaccine)	Annias Immunotherapeutics Chapel Hill, NC	glioblastoma	Phase I/II www.anniasimmuno.com
PF-06840003 (IDO1 inhibitor)	iTeos Therapeutics Gosselies, Belgium Pfizer New York, NY	glioma	Phase I www.pfizer.com
Poly-ICLC (biological response modifier)	Oncovir Washington, DC	glioblastoma	Phase I/II completed www.oncovir.com
Pomalyst® pomalidomide	Celgene Summit, NJ	recurrent or progressive primary brain tumors	Phase II www.celgene.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
pratumumab (human IgG antibody) ORPHAN DRUG	Nascent Biotech Vero Beach, FL	glioma	Phase II www.nascentbiotech.com
Prophage™ vitespen ORPHAN DRUG	Agenus Lexington, MA	newly diagnosed glioblastoma	Phase II www.agenusbio.com
PT2385 (HIF-2 alpha inhibitor)	Peloton Therapeutics Dallas, TX	recurrent glioblastoma	Phase II www.pelotontherapeutics.com
RRx-001 (free radical stimulant)	EpicentRx San Diego, CA	anaplastic glioma, newly diagnosed glioblastoma (combination therapy)	Phase I www.epicentrx.com
selinexor oral (nuclear export inhibitor)	Karyopharm Therapeutics Newton, MA	glioblastoma	Phase II www.karyopharm.com
SGT-53 (gene therapy)	SynerGene Therapeutics Potomac, MD	recurrent glioblastoma	Phase II
SL-701 (immunotherapeutic) ORPHAN DRUG	Stemline Therapeutics New York, NY	glioblastoma (adult)	Phase II www.stemline.com
SurVaxM BIRC5 protein inhibitor vaccine	MimiVax Buffalo, NY	glioblastoma	Phase II www.mimivax.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
Sym-004 (EGFR antagonist)	Symphogen Somerville, NJ	glioblastoma	Phase II www.symphogen.com
Tafinlar® + Mekinist® dabrafenib and trametinib	Novartis Pharmaceuticals East Hanover, NJ	BRAF V600 mutation positive relapsed or refractory high grade glioma	Phase II www.novartis.com
tasadenoturev (DNA-2401) (DNA vaccine) ORPHAN DRUG	DNAtrix Houston, TX	recurrent glioblastoma (Fast Track) (monotherapy and combination)	Phase II www.dnatrix.com
		newly diagnosed diffuse intrinsic pontine glioma	Phase I www.dnatrix.com
tazemetostat (EZH2 enzyme inhibitor)	Epizyme Cambridge, MA National Cancer Institute Bethesda, MD	CNS tumors (pediatric)	Phase II www.epizyme.com
TB-403 (anti-PIGF mAb)	BioInvent International Lund, Sweden Oncurious Leuven, Belgium	medulloblastoma	Phase I/II www.bioinvent.com
tesevatinib (protein tyrosine kinase inhibitor)	Kadmon Pharmaceuticals New York, NY	recurrent glioblastoma	Phase II www.kadmon.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
TG02 (multi-kinase inhibitor)	Tragana Pharmaceuticals Carlsbad, CA	anaplastic astrocytoma, glioblastoma	Phase I/II www.traganapharma.com
Toca 511 & Toca FC (vocimagene amiretrorepvec flucytosine gene therapy) ORPHAN DRUG	Tocagen San Diego, CA	recurrent high-grade glioma (Fast Track)	Phase III www.tocagen.com
TPI-287 (next generation taxane)	Cortice Biosciences New York, NY	recurrent glioblastoma (see also Alzheimer's, other)	Phase II www.corticebiosciences.com
		secondary brain metastases	Phase I www.corticebiosciences.com
trans-sodium crocetin ORPHAN DRUG	Diffusion Pharmaceuticals Charlottesville, VA	glioblastoma	Phase II www.diffusionpharma.com
		brain metastases	Phase I www.diffusionpharma.com
TRC 102 (methoxyamine hydrochloride)	TRACON Pharmaceuticals San Diego, CA	glioblastoma	Phase II www.traconpharma.com
TRC 105 (carotuximab)	TRACON Pharmaceuticals San Diego, CA	glioblastoma (combination therapy)	Phase II www.traconpharma.com

Brain Tumors

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
TVB-2640 (FASN inhibitor)	3-V Biosciences Menlo Park, CA	astrocytoma	Phase II www.3vbio.com
TVI-Brain-1 cancer vaccine ORPHAN DRUG	TVAX Biomedical Olathe, KS	brain cancer	Phase II completed www.tvaxbiomedical.com
VAL-083 (dianhydrogalactitol) ORPHAN DRUG	DelMar Pharmaceuticals Menlo Park, CA	glioblastoma (Fast Track)	Phase III www.delmarpharma.com
varlilumab (CD27 antigen inhibitor)	Celldex Therapeutics Hampton, NJ	glioblastoma (combination therapy)	Phase II www.celldex.com
VB-111 (ofranergene obadenovec) ORPHAN DRUG	VBL Therapeutics Modi'in, Israel	recurrent glioblastoma (Fast Track)	Phase III www.vblrx.com
VBI-1901 (bivalent cancer vaccine)	VBI Vaccines Cambridge, MA	glioblastoma	Phase I/II www.vbivaccines.com
WP1066 (STAT3 transcription factor inhibitor)	Moleculin Biotech Houston, TX	brain cancer, CNS tumors	Phase I www.moleculin.com

Epilepsy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ADS-4101 (lacosamide extended release)	Adamas Pharmaceuticals Emeryville, CA	partial onset seizures	Phase I www.adamaspharma.com
ADX71149 (mGluR2 modulator)	Addex Therapeutics Geneva, Switzerland Janssen Raritan, NJ	epilepsy	Phase I www.addextherapeutics.com
ANAVEX™ 2-73 (M1 muscarinic receptor agonist/ intracellular sigma 1 receptor agonist) ORPHAN DRUG	Anavex Life Sciences New York, NY	infantile spasms (see also Alzheimer's, genetic, Parkinson's)	Phase I www.anavex.com
AQST-120 (clobazam oral soluble film)	Aquestive Therapeutics Warren, NJ	Lennox-Gastaut syndrome	application submitted www.aquestive.com
AQST-203 (diazepam oral soluble film) ORPHAN DRUG	Aquestive Therapeutics Warren, NJ	acute repetitive seizures	Phase III www.aquestive.com
BIS-001 (huperzine A)	Biscayne Neurotherapeutics Miami, FL	refractory epilepsy	Phase I www.biscayneneuro.com
brexanolone (GABA-A receptor modulator) ORPHAN DRUG	Sage Therapeutics Cambridge, MA	status epilepticus (Fast Track) (see also other)	Phase III completed www.sagerx.com

Epilepsy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
Briviact® brivaracetam	UCB Smyrna, GA	partial onset seizures (children ≥4 years to <17 years of age)	Phase III www.ucb.com
cannabidiol oral solution ORPHAN DRUG	INSYS Therapeutics Chandler, AZ	infantile spasm	Phase III www.insysrx.com
		childhood absence epilepsy	Phase II www.insysrx.com
carisbamate (YKP509) (neurotransmitter modulator)	SK Life Science Fair Lawn, NJ	orphan epilepsy	Phase I www.sklifescienceinc.com
cenobamate (YKP3089)	SK Life Science Fair Lawn, NJ	partial onset seizures	Phase III www.sklifescienceinc.com
CPP-115 (GABA-AT inhibitor) ORPHAN DRUG	Catalyst Pharmaceutical Coral Gables, FL	infantile spasms (see also tic disorders)	Phase I www.catalystpharma.com
CX-8998 (Cav3 modulator)	Cavion Charlottesville, VA	epilepsy (see also Parkinson's, other)	Phase II www.cavion.com
E2730 (synaptic transmission modulator)	Eisai Woodcliff Lake, NJ	epilepsy	Phase I www.eisai.com

Epilepsy

Drug Name

Sponsor

Indication

Phase of Development

Epidiolex cannabidiol ORPHAN DRUG	GW Pharmaceuticals Carlsbad, CA	Dravet syndrome (Fast Track), Lennox-Gastaut syndrome (see also brain injury, genetic)	application submitted www.gwpharm.com
		infantile spasms	Phase III www.gwpharm.com
EPX-100 (serotonin receptor modulator) ORPHAN DRUG	Epygenix Therapeutics Paramus, NJ	Dravet syndrome	Phase I www.epygenix.com
EPX-200 (serotonin receptor modulator) ORPHAN DRUG	Epygenix Therapeutics Paramus, NJ	Dravet syndrome	Phase II www.epygenix.com
EPX-300 (serotonin receptor modulator) ORPHAN DRUG	Epygenix Therapeutics Paramus, NJ		Phase II www.epygenix.com
fosphenytoin (Captisol-enabled™)	Sedor Pharmaceuticals Paoli, PA	status epilepticus seizures	Phase III www.sedorpharmaceuticals.com
Fycompa® perampanel	Eisai Woodcliff Lake, NJ	epilepsy (pediatric), Lennox-Gastaut syndrome	Phase III www.eisai.com

Epilepsy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ganaxolone (GABA A receptor agonist) ORPHAN DRUG	Marinus Pharmaceuticals Radnor, PA	pediatric refractory epilepsy (CDKL5 deficiency disorder, PCDH19 epilepsy, Lennox Gastaut syndrome), status epilepticus	Phase II www.marinuspharma.com
Lyrica® pregabalin	Pfizer New York, NY	tonic-clonic seizures, partial onset seizures (1 month - 16 years)	Phase III www.pfizer.com
NRL-1 (diazepam intranasal) ORPHAN DRUG	Neurelis San Diego, CA	intermittent use to control bouts of seizure activity (seizure emergencies) (Fast Track)	Phase III www.neurelis.com
padsevoniil (UCB0942)	UCB Smyrna, GA	drug-resistant epilepsy, focal-onset seizures	Phase II www.ucb.com
PRAX-330 (Nav1.6 voltage-gated sodium channel inhibitor)	Praxis Precision Medicines Cambridge, MA	epilepsy	Phase I
radiprodil (UCB3491) (NMDA receptor antagonist)	UCB Smyrna, GA	drug-resistant infantile epilepsy	Phase I www.ucb.com
SHP615 (midazolam buccal)	Shire Lexington, MA	convulsive seizures	Phase II www.shire.com
Staccato® alprazolam inhaled alprazolam	Engage Therapeutics Summit, NJ	epilepsy	Phase II www.engagetherapeutics.com

Epilepsy

Drug Name

Sponsor

Indication

Phase of Development

TAK-935 (OV935) (CH24H inhibitor) ORPHAN DRUG	Ovid Therapeutics New York, NY Takeda Pharmaceuticals Deerfield, IL	Dravet syndrome, Lennox-Gastaut syndrome (see also genetic)	Phase II www.ovidrx.com www.takeda.com
Translarna® ataluren	PTC Therapeutics South Plainfield, NJ	CDKL5 disorder, Dravet syndrome (see also muscular dystrophy)	Phase II www.ptcbio.com
Tysarbi® natalizumab	Biogen Cambridge, MA	focal epilepsy (see also stroke)	Phase II www.biogen.com
USL261 (intranasal midazolam) ORPHAN DRUG	Upsher Smith Maple Grove, MN	seizure clusters (Fast Track)	Phase III www.upsher-smith.com
Vimpat® lacosamide	UCB Smyrna, GA	partial onset seizures (>1 month to <4 years), tonic clonic seizures (oral)	Phase III www.ucb.com
		epilepsy (intravenous)	Phase II/III www.ucb.com
		generalized seizures (pediatric/tablet)	Phase II www.ucb.com
XeriJect™ diazepam rescue pen ORPHAN DRUG	Xeris Pharmaceuticals Chicago, IL	acute repetitive seizures	Phase I www.xerispharma.com

Epilepsy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ZX008 (fenfluramine low dose) ORPHAN DRUG	Zogenix Emeryville, CA	Dravet syndrome (Fast Track), Lennox-Gastaut syndrome	Phase III www.zogenix.com
ZYN002-CBD gel (cannabidiol patch)	Zynerba Pharmaceuticals Devon, PA	epileptic encephalopathies, adult refractory focal seizures	Phase II www.zynerba.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ACE-083 (recombinant fusion protein)	Acceleron Pharma Cambridge, MA	Charcot-Marie-Tooth disease (see also muscular dystrophy)	Phase II www.acceleronpharma.com
ANAVEX™ 2-73 (M1 muscarinic receptor agonist/ intracellular sigma 1 receptor agonist) ORPHAN DRUG	Anavex Life Sciences New York, NY	Rett syndrome (see also Alzheimer's, epilepsy, Parkinson's)	Phase I www.anavex.com
AT132 (gene therapy) ORPHAN DRUG	Audentes Therapeutics San Francisco, CA	X-linked myotubular myopathy (Fast Track)	Phase I/II www.audentestx.com
AVXS-101 (gene therapy) ORPHAN DRUG	AveXis Bannockburn, IL	spinal muscular atrophy type 1 (Fast Track)	Phase III www.avexis.com
		spinal muscular atrophy type 2	Phase I www.avexis.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
BioE-743 (NQO1 modulator) ORPHAN DRUG	BioElectron Technology Mountain View, CA	Leigh syndrome, RARS2 syndrome	Phase II www.bioelectron.com
BHV-5000 (NMDA receptor antagonist) ORPHAN DRUG	Biohaven Pharmaceuticals New Haven, CT	Rett syndrome	Phase I www.biohavenpharma.com
BXCL101 (proprietary version of bortezomib) ORPHAN DRUG	BioXcel Branford, CT	neurofibromatosis 2	Phase I/II www.bioxcel.com
CAD-1833 (SK channel modulator)	Cadent Therapeutics Cambridge, MA	spinocerebellar ataxia (see also other)	Phase I www.cadenttx.com
CK-2127107 (reldesemtiv) (fast skeletal troponin activator) ORPHAN DRUG	Astellas Northbrook, IL Cytokinetics South San Francisco, CA	spinal muscular atrophy (see also ALS)	Phase II www.astellas.com www.cytokinetics.com
EDS-EP (dexamethasone erythrocyte encapsulated) ORPHAN DRUG	EryDel Urbino, Italy	ataxia telangiectasia	Phase III www.erydel.com
epicatechin (mitochondrial protein modulator)	Cardero Therapeutics Los Altos Hills, CA	Friedreich's ataxia (see also muscular dystrophy)	Phase I/II www.carderorx.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
Epidiolex cannabidiol ORPHAN DRUG	GW Pharmaceuticals Carlsbad, CA	tuberous sclerosis complex (see also brain injury, epilepsy)	Phase III www.gwpharm.com
Ferriprox® deferiprone	ApoPharma Toronto, Canada	pantothenate kinase-associated neurodegeneration	Phase III www.apopharma.com
Firdapse® amifampridine ORPHAN DRUG	Catalyst Pharmaceuticals Coral Gables, FL	congenital myasthenic syndrome (see also other)	Phase III www.catalystpharma.com
		spinal muscular atrophy (type 3)	Phase I www.catalystpharma.com
FLX-787 (TRP ion channel agonist)	Flex Pharma Boston, MA	cramping in Charcot-Marie-Tooth (see also ALS, spasticity)	Phase II www.flex-pharma.com
fosmetpantotate (phosphopantothenate prodrug) ORPHAN DRUG	Retrophin San Diego, CA	pantothenate kinase-associated neurodegeneration (Fast Track)	Phase III www.retrophin.com
inotersen (antisense RNA modulator) ORPHAN DRUG	Ionis Pharmaceuticals Carlsbad, CA	transthyretin familial amyloid polyneuropathy (Fast Track)	application submitted www.ionispharma.com
JOT101 (trans-resveratrol oral) ORPHAN DRUG	Jupiter Orphan Therapeutics Jupiter, FL	Friedreich's ataxia	Phase II www.jupiterorphan.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
Lenti-D™ elivaldogene tavalentivec ORPHAN DRUG	bluebird bio Cambridge, MA	cerebral adrenoleukodystrophy	Phase II/III www.bluebirdbio.com
LMI070 (branaplam) (SMN2 protein modulator)	Novartis Pharmaceuticals East Hanover, NJ	spinal muscular atrophy	Phase III www.novartis.com
omaveloxolone (NF-E2-related factor 2 stimulant) ORPHAN DRUG	Reata Pharmaceuticals Irving, TX	Friedreich's ataxia	Phase II www.reatapharma.com
OV101 (gaboxadol oral) ORPHAN DRUG	Ovid Therapeutics New York, NY	Angelman syndrome (Fast Track)	Phase II www.ovidrx.com
pexidartinib	Plexikon Berkeley, CA	neurofibromatosis, type 1 (pediatric)	Phase I/II www.plexikon.com
PXT3003 (baclofen/naltrexone/sorbitol) ORPHAN DRUG	Pharnext Issy-les-Moulineaux, France	Charcot-Marie-Tooth disease	Phase III www.pharnext.com
RG7916 (SMN3 protein modulator) ORPHAN DRUG	PTC Therapeutics South Plainfield, NJ Roche Basel, Switzerland	spinal muscular atrophy (Fast Track)	Phase II www.ptcbio.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
RT001 (Di-deuterated linoleic acid ethyl ester) ORPHAN DRUG	Retrope Los Altos, CA	Friedreich's ataxia	Phase I/II completed www.retrope.com
sarizotan (dopamine D2 receptor partial agonist, serotonin 1A receptor agonist) ORPHAN DRUG	Newron Pharmaceuticals Milan, Italy	Rett syndrome	Phase II/III www.newron.com
SOM1201	SOM Biotech Cambridge, MA	adrenoleukodystrophy	Phase I www.sombiotech.com
TAK-831 (DAAO inhibitor)	Takeda Pharmaceuticals Deerfield, IL	Friedreich's ataxia	Phase II www.takeda.com
TAK-935 (OV935) (CH24H inhibitor)	Ovid Therapeutics New York, NY Takeda Pharmaceuticals Deerfield, IL	tuberous sclerosis (see also epilepsy)	Phase II www.ovidrx.com www.takeda.com
trigriluzole (riluzole prodrug) ORPHAN DRUG	Biohaven Pharmaceuticals New Haven, CT	hereditary spinocerebellar ataxia (Fast Track)	Phase III www.biohavenpharma.com
triheptanoin (UX007) ORPHAN DRUG	Ultragenyx Pharmaceutical Novato, CA	glucose transporter type 1 deficiency syndrome (GLUT1)	Phase III www.ultragenyx.com

Genetic Disorders

Drug Name

Sponsor

Indication

Phase of Development

trofinetide
ORPHAN DRUG

Neuren Pharmaceuticals
Camberwell, Australia

Rett syndrome (Fast Track)
(see also brain injury)

Phase II
www.neurenpharma.com

Vyndaqel®
tafamidis meglumine
ORPHAN DRUG

Pfizer
New York, NY

transthyretin familial amyloid
polyneuropathy (Fast Track)

application submitted
www.pfizer.com

Headache

Drug Name

Sponsor

Indication

Phase of Development

Aimovig™
erenumab

Amgen
Thousand Oaks, CA
Novartis Pharmaceuticals
East Hanover, NJ

prevention of migraine

application submitted
www.amgen.com
www.novartis.com

ALLOD-2

Allodynic Therapeutics
North Miami, FL

acute migraine, migraine prevention
(see also pain)

Phase II
www.allodynic.com

AMG 301
(PAC1 inhibitor)

Amgen
Thousand Oaks, CA
Novartis Pharmaceuticals
East Hanover, NJ

migraine prevention

Phase II
www.amgen.com
www.novartis.com

atogepant
(CGRP antagonist)

Allergan
Parsippany, NJ

migraine prevention

Phase II/III
www.allergan.com

Headache

Drug Name

Sponsor

Indication

Phase of Development

Botox® onabotulinumtoxinA	Allergan Parsippany, NJ	migraine prevention (pediatric)	Phase III completed www.allergan.com
CVT-427 (zolmitriptan inhalation)	Acorda Therapeutics Ardsley, NY	acute treatment of migraine	Phase I completed www.acorda.com
DFN-02 (sumatriptan intranasal)	Promius Pharma Princeton, NJ	acute treatment of migraine	Phase III www.promiuspharma.com
DFN-15 (rapid acting non-triptan oral solution of celecoxib)	Promius Pharma Princeton, NJ	migraine	Phase III www.promiuspharma.com
Dorolac® Nasal Solution doxepin intranasal	Winston Pharmaceuticals Mount Vernon, IL	chronic migraine	Phase II www.winstonlabs.com
eptinezumab (CGRP inhibitor)	Alder Biopharmaceuticals Bothell, WA	prevention of frequent episodic migraine, prevention of chronic migraine	Phase III www.alderbio.com

Headache

Drug Name

Sponsor

Indication

Phase of Development

fremanezumab (anti-CGRP)	Teva Pharmaceuticals USA North Wales, PA	chronic migraine, episodic migraine	application submitted www.tevapharm.com
		cluster headache (Fast Track)	Phase III www.tevapharma.com
		post-traumatic headache	Phase II www.tevapharma.com
galcanezumab (CGRP mAb)	Eli Lilly Indianapolis, IN	migraine prevention	application submitted www.lilly.com
		cluster headache (Fast Track)	Phase III www.lilly.com
INP104 (dihydroergotamine)	Impel NeuroPharma Seattle, WA	migraine	Phase I www.impelnp.com
ketoprofen topical (lipoxygenase-cyclooxygenase inhibitor)	Achelios Therapeutics Chapel Hill, NC	migraine (see also pain)	Phase II www.achelios.com
lasmiditan (5-HT1F receptor agonist)	Eli Lilly Indianapolis, IN	migraine	Phase III www.lilly.com
M207 (zolmitriptan transdermal)	Zosano Pharma Fremont, CA	migraine	Phase III www.zosanopharma.com

Headache

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NXN-188 (nNOS inhibitor + serotonin agonist)	NeurAxon Pharma Mississauga, Canada	migraine	Phase II www.neuraxon.com
Onzetra® Xsail® sumatriptan nasal powder	Avanir Pharmaceuticals Aliso Viejo, CA	migraine (adolescents)	Phase III www.avanir.com
promethazine/sumatriptan fixed-dose combination (fast dissolving)	Charleston Laboratories Jupiter, FL	migraine, migraine induced nausea and vomiting (MINV)	Phase II www.charlestonlabs.com
rimegepant (BHV-3000) (CGRP receptor antagonist)	Biohaven Pharmaceutical New Haven, CT	migraine	Phase III www.biohavenpharma.com
Rizaport® rizatriptan oral film	IntelGenx Quebec, Canada	migraine	application submitted www.intelgenx.com
Semprana dihydroergotamine mesilate inhalation	Allergan Parsippany, NJ	migraine	application submitted www.allergan.com
SOM230 (pasireotide)	Novartis Pharmaceuticals East Hanover, NJ	cluster headache	Phase II www.novartis.com
TI-001 (oxytocin intranasal)	Trigemina Moraga, CA	migraine	Phase II completed www.trigemina.com

Headache

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
TRV250 (delta receptor modulator)	Trevena Chesterbrook, PA	migraine	Phase I www.trevena.com
ubrogepant (CGRP receptor antagonist)	Allergan Parsippany, NJ	acute treatment of migraine	Phase III www.allergan.com
Zomig® zolmitriptan nasal spray	Impax Pharmaceuticals Hayward, CA	migraine (6-11 years)	Phase III www.impaxlabs.com

Huntington's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
IONIS-HTT _{rx} (RG6042) (antisense HD protein inhibitor) ORPHAN DRUG	Ionis Pharmaceuticals Carlsbad, CA Roche Basel, Switzerland	Huntington's disease	Phase II www.ionispharma.com
laquinimod ORPHAN DRUG	Teva Pharmaceuticals USA North Wales, PA	Huntington's disease (see also multiple sclerosis)	Phase II www.tevapharm.com
OMS824 (PDE10 inhibitor) ORPHAN DRUG	Omeros Seattle, WA	Huntington's disease (Fast Track)	Phase II www.omeros.com

Huntington's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
PBT2 (chelating agent) ORPHAN DRUG	Prana Biotechnology Melbourne, Australia	Huntington's disease	Phase II completed www.pranabio.com
pridopidine (dopamine D2 receptor antagonist) ORPHAN DRUG	Teva Pharmaceuticals USA North Wales, PA	Huntington's disease	Phase II www.tevapharm.com
SBT-20 (tetrapeptide)	Stealth BioTherapeutics Newton, MA	Huntington's disease	Phase I/II www.stealthbt.com
SOM3355 (VMAT inhibitor)	SOM Biotech Cambridge, MA	Huntington's disease (see also other)	Phase II www.sombiotech.com
VX15 (CD100 antigen inhibitor) ORPHAN DRUG	Vaccinex Rochester, NY	Huntington's disease (Fast Track)	Phase II www.vaccinex.com
WVE-120101 (RNA interference targeting mHTT SNP1) ORPHAN DRUG	WaVe Life Sciences Cambridge, MA	Huntington's disease	Phase I/II www.wavelifesciences.com
WVE-120102 (RNA interference targeting mHTT SNP2)	WaVe Life Sciences Cambridge, MA	Huntington's disease	Phase I/II www.wavelifesciences.com

Multiple Sclerosis

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ACT-101 (recombinant alpha fetoprotein)	Alpha Cancer Technologies Toronto, Canada	multiple sclerosis (see also other)	Phase I www.alpha-cancer.com
ARN-6039 (BOS172767) (RORyt inverse agonist)	Arrien Pharmaceuticals Salt Lake City, UT Boston Pharmaceuticals Cambridge, MA	multiple sclerosis	Phase I completed www.arrienpharma.com
ATA188 (allogeneic T-cell activated therapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis, relapsing remitting multiple sclerosis	Phase I www.atarabio.com
ATA190 (autologous Epstein-Barr virus specific T-cell therapy)	Atara Biotherapeutics South San Francisco, CA	multiple sclerosis	Phase I www.atarabio.com
Aubagio [®] teriflunomide	Sanofi Genzyme Cambridge, MA	relapsing multiple sclerosis (pediatric)	Phase III www.sanofigenzyme.com
BIIB-098 (monomethyl fumarate prodrug)	Biogen Cambridge, MA	multiple sclerosis	Phase III www.biogen.com
cladribine tablets (lymphocyte targeting agent)	EMD Serono Rockland, MA	relapsing multiple sclerosis	Phase III www.emdserono.com
elezanumab (ABT-555) (RGMa inhibitor)	AbbVie North Chicago, IL	multiple sclerosis (see also spinal cord injury)	Phase I www.abbvie.com

Multiple Sclerosis

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
evobrutinib (BTK inhibitor)	EMD Serono Rockland, MA	multiple sclerosis	Phase II www.emdserono.com
freeze-dried bee venom (intradermal)	Apimeds Gyeonggi-do, South Korea	multiple sclerosis	Phase III www.apimeds.com
Gilenya [®] fingolimod	Novartis Pharmaceuticals East Hanover, NJ	multiple sclerosis (pediatric)	application submitted www.novartis.com
Gocovri [™] amantadine extended release	Adamas Pharmaceuticals Emeryville, CA	multiple sclerosis walking	Phase III www.adamaspharma.com
laquinimod	Teva Pharmaceuticals USA North Wales, PA	progressive forms of multiple sclerosis (Fast Track) (see also Huntington's)	Phase II www.tevapharm.com
Lemtrada [®] alemtuzumab	Sanofi Genzyme Cambridge, MA	relapsing remitting multiple sclerosis (pediatric)	Phase III www.sanofigenzyme.com
MD1003 (NF-Kappa B inhibitor)	MedDay Pharmaceuticals Paris, France	progressive multiple sclerosis	Phase III www.medday-pharma.com
MN-166 (ibudilast)	MediciNova La Jolla, CA	progressive multiple sclerosis (Fast Track) (see also ALS)	Phase II www.medicinova.com

Multiple Sclerosis

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NeuroVax™ immunostimulant vaccine ORPHAN DRUG	Immune Response BioPharma Atlantic City, NJ	secondary progressive multiple sclerosis (Fast Track)	Phase II www.immuneresponsebiopharma.com
ofatumumab (anti-CD20 mAb)	Novartis Pharmaceuticals East Hanover, NJ	relapsing remitting multiple sclerosis	Phase III www.novartis.com
opicinumab (BIIB033) (LINGO-1 protein inhibitor)	Biogen Cambridge, MA	multiple sclerosis	Phase II www.biogen.com
ozanimod (S1P1 modulator)	Receptos San Diego, CA	relapsing multiple sclerosis	application submitted www.receptos.com
ponesimod (S1P1 receptor agonist)	Actelion Pharmaceuticals South San Francisco, CA	multiple sclerosis	Phase III www.actelion.com
PRN2246 (SAR442168) (BTK inhibitor)	Principia Biopharma South San Francisco, CA Sanofi Bridgewater, NJ	multiple sclerosis	Phase I www.principiabio.com www.sanofi.com
rHlgM22 (remyelinating antibody)	Acorda Therapeutics Ardsley, NY	multiple sclerosis	Phase I completed www.acorda.com
RNS60 (charge-stabilized nanostructure)	Revalerio Tacoma, WA	multiple sclerosis (see also ALS)	Phase II www.revalerio.com

Multiple Sclerosis

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
RPI-78M (nicotinic receptor antagonist) ORPHAN DRUG	ReceptoPharm Plantation, FL	multiple sclerosis	Phase I www.receptopharm.com
RTL1000 (T lymphocyte modulator) ORPHAN DRUG	Artielle ImmunoTherapeutics San Mateo, CA	multiple sclerosis	Phase I www.artielle.com
siponimod (sphingosine-1 phosphate receptor modulator)	Novartis Pharmaceuticals East Hanover, NJ	secondary progressive multiple sclerosis	Phase III www.novartis.com
Tecfidera® dimethyl fumarate	Biogen Cambridge, MA	relapsing remitting multiple sclerosis (pediatric)	Phase III www.biogen.com
ublituximab (anti-CD20 mAb)	TG Therapeutics New York, NY	multiple sclerosis	Phase II www.tgtherapeutics.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AAV1-FS344 (follistatin gene therapy) ORPHAN DRUG	Milo Biotechnology Cleveland, OH	Becker muscular dystrophy, Duchenne muscular dystrophy (see also other)	Phase I/II www.milobiotechnology.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ACE-083 (recombinant fusion protein)	Acceleron Pharma Cambridge, MA	facioscapulohumeral muscular dystrophy (see also genetic)	Phase II www.acceleronpharma.com
AMO-02 (glycogen synthase kinase 3 beta inhibitor) ORPHAN DRUG	AMO Pharma Berwyn, PA	myotonic dystrophy, type 1	Phase II www.amo-pharma.com
ARM210 (calcium channel stabilizer) ORPHAN DRUG	ARMGO Pharma Tarrytown, NY	Duchenne muscular dystrophy	Phase I www.armgo.com
ATYR 1940 (resokine pathway stimulator) ORPHAN DRUG	aTyr Pharma San Diego, CA	facioscapulohumeral muscular dystrophy (Fast Track), limb girdle muscular dystrophy (Fast Track)	Phase I/II www.atyrpharma.com
CAP-1002 (allogeneic cardiosphere-derived stem cell therapy) ORPHAN DRUG	Capricor Therapeutics Beverly Hills, CA	Duchenne muscular dystrophy	Phase I/II www.capricor.com
Carmeseal-MD™ poloxamer 188 NF ORPHAN DRUG	Phrixus Pharmaceuticals Ann Arbor, MI	Duchenne muscular dystrophy	Phase I www.phrixuspharmaceuticals.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
domagrozumab (myostatin inhibitor) ORPHAN DRUG	Pfizer New York, NY	Duchenne muscular dystrophy (Fast Track)	Phase II www.pfizer.com
DT-200 (selective androgen receptor modulator)	Akashi Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase I completed www.akashirx.com
edasalonexent (NF-kappa B inhibitor) ORPHAN DRUG	Catabasis Pharmaceuticals Cambridge, MA	Duchenne muscular dystrophy (Fast Track)	Phase II www.catabasis.com
epicatechin (mitochondrial protein modulator)	Cardero Therapeutics Los Altos Hills, CA	Becker muscular dystrophy, Duchenne muscular dystrophy (see also genetic)	Phase I/II www.carderorx.com
ezutromid (utrophin activator) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA Summit Therapeutics Oxfordshire, United Kingdom	Duchenne muscular dystrophy (Fast Track)	Phase II www.sarepta.com
GALGT2 gene therapy	Sarepta Therapeutics Cambridge, MA Nationwide Children's Hospital Columbus, OH	Duchenne muscular dystrophy	Phase I/II www.sarepta.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
givinostat (HDAC inhibitor)	Italfarmaco Milan, Italy	Duchenne muscular dystrophy	Phase III www.italfarmaco.com
HT-100 (delayed-release halofuginone) ORPHAN DRUG	Akashi Therapeutics Cambridge, MA	Duchenne muscular dystrophy (Fast Track)	Phase I/II www.akashirx.com
MA-0211	Astellas Northbrook, IL Mitobridge Cambridge, MA	Duchenne muscular dystrophy	Phase I www.mitobridge.com
micro-dystrophin gene therapy	Nationwide Children's Hospital Columbus, OH Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase I www.sarepta.com
MNK-1411 (cosyntropin injection) ORPHAN DRUG	Mallinckrodt St. Louis, MO	Duchenne muscular dystrophy (Fast Track)	Phase II www.mallinckrodt.com
MYO-102 (gene therapy)	Myonex Therapeutics New Albany, OH	limb girdle muscular dystrophy	Phase I/II www.myonexustx.com
MYO-201 (gene therapy)	Myonex Therapeutics New Albany, OH	limb girdle muscular dystrophy	Phase I www.mtonexustx.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NS-065 (dystrophin expression stimulant) ORPHAN DRUG	Nippon Shinyaku Kyoto, Japan	Duchenne muscular dystrophy (Fast Track)	Phase II www.nippon-shinyaku.co.jp
omigapil ORPHAN DRUG	Santhera Pharmaceuticals Burlington, MA	congenital muscular dystrophy (Fast Track)	Phase I www.santhera.com
pamrevlumab (anti-CTGF antibody)	FibroGen San Francisco, CA	Duchenne muscular dystrophy	Phase II www.fibrogen.com
PF-06939926 (minidystrophin) ORPHAN DRUG	Pfizer New York, NY	Duchenne muscular dystrophy	Phase I www.pfizer.com
Raxone [®] idebenone ORPHAN DRUG	Santhera Pharmaceuticals Burlington, MA	Duchenne muscular dystrophy (Fast Track)	Phase III www.santhera.com
RG6206 (anti-myostatin adnectin IgG1-Fc fusion protein)	Roche Basel, Switzerland	Duchenne muscular dystrophy	Phase II/III www.roche.com
SGT-001 (gene therapy) ORPHAN DRUG	Solid Biosciences Cambridge, MA	Duchenne muscular dystrophy	Phase I/II www.solidbio.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
SRP-4045 (casimersen) (RNA-targeted therapy and phosphorodiamidate morpholino oligomer)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase III www.sarepta.com
SRP-4053 (golodirsen) (RNA-targeted therapy)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase III www.sarepta.com
SRP-5051 (RNA-targeted therapy)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase I www.sarepta.com
Translarna® ataluren ORPHAN DRUG	PTC Therapeutics South Plainfield, NJ	Duchenne muscular dystrophy (Fast Track) (see also epilepsy)	application submitted www.ptcbio.com
trehalose ORPHAN DRUG	Bioblast Pharma Tel Aviv, Israel	oculopharyngeal muscular dystrophy (Fast Track)	Phase II www.bioblastpharma.com
vamorolone (glucocorticoid receptor agonist) ORPHAN DRUG	ReveraGen BioPharma Rockville, MD	Duchenne muscular dystrophy	Phase II www.reveragen.com
WVE-210201 (dystrophin expression modulator)	WaVe Life Sciences Cambridge, MA	Duchenne muscular dystrophy	Phase I www.wavelifesciences.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
3VM1001 topical cream	CDA Research Group Pittsburgh, PA	postherpetic neuralgia	Phase I/II
ABX-1431 (MGLL inhibitor)	Abide Therapeutics San Diego, CA	neuropathic pain (see also tic disorders)	Phase I www.abidetx.com
ALLOD-2	Allodynic Therapeutics North Miami, FL	chronic low back pain (see also migraine)	Phase II www.allodynic.com
AMG0103 (NF-kappa-B decoy oligonucleotide)	AnGes Osaka, Japan	chronic discogenic lumbar back pain	Phase I
AmiKet™ amitriptyline/ketamine ORPHAN DRUG	Maxim Pharmaceuticals Englewood Cliffs, NJ	neuropathic pain (Fast Track)	Phase II completed www.immunepharma.com
APD371 (cannabinoid receptor CB2 agonist)	Arena Pharmaceuticals San Diego, CA	Crohn's pain	Phase II www.arenapharm.com
ASP0819 (calcium-activated potassium channel opener)	Astellas Northbrook, IL	fibromyalgia	Phase II www.astellas.com
ASP8062 (GABA-B receptor positive allosteric modulator)	Astellas Northbrook, IL	fibromyalgia	Phase II www.astellas.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AXS-02 (oral zoledronic acid) ORPHAN DRUG	Axsome Therapeutics New York, NY	knee osteoarthritis pain with bone marrow lesions (Fast Track), chronic lower back pain	Phase III www.axsome.com
AXS-06 (esomeprazole/meloxicam fixed dose combination)	Axsome Therapeutics New York, NY	osteoarthritis's pain, rheumatoid arthritis pain	Phase I www.axsome.com
BIIB074 (vixotrigine) (Nav1.7 voltage gated sodium channel inhibitor) ORPHAN DRUG	Biogen Cambridge, MA	trigeminal neuralgia	Phase II www.biogen.com
brivoligide (AYX1) (EGR1 1 inhibitor)	Adynxx San Francisco, CA	prevent transition to persistent or chronic pain (Fast Track)	Phase II www.adynxx.com
CA-011 (TRPV-1 receptor agonist)	Concentric Analgesics Los Altos, CA	chronic pain associated from moderate to severe osteoarthritis	Phase I www.concentricanalgesics.com
CAM2038 (buprenorphine depot)	Braeburn Pharmaceuticals Princeton, NJ	chronic pain	Phase III www.braeburnpharmaceuticals.com
CC8464 (ASP1807)	Astellas Northbrook, IL Chromocell North Brunswick, NJ	pain associated with idiopathic small fiber neuropathy (Fast Track)	Phase I www.chromocell.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
cibinetide (erythropoietin receptor agonist) ORPHAN DRUG	Araim Pharmaceuticals Tarrytown, NY	sarcoidosis pain (Fast Track)	Phase II completed www.araimpharma.net
		diabetic neuropathic pain	Phase I completed www.araimpharma.com
CMX-020 (arachidonic acid analog)	Cmxtwenty Wauwatosa, WI	neuropathic pain	Phase II www.cmxtwenty.com
CNTX-0290 (SSTR4 agonist)	Centrexion Therapeutics Boston, MA	chronic pain	Phase I www.centrexion.com
CNTX-4975 (TRPV1 receptor agonist) ORPHAN DRUG	Centrexion Therapeutics Boston, MA	osteoarthritis pain	Phase III www.centrexion.com
		Morton's neuroma pain (Fast Track)	Phase II www.centrexion.com
CNTX-6970 (CCR2 receptor antagonist)	Centrexion Therapeutics Boston, MA	inflammatory pain	Phase I www.centrexion.com
COL-195 (abuse-resistant hydrocodone)	Collegium Pharmaceutical Canton, MA	chronic pain	Phase I/II www.collegiumpharma.com
CR845 (difelikefalin)	Cara Therapeutics Stamford, CT	chronic pain	Phase II www.caratherapeutics.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
dapansutrile (NLRP3 inflammasome inhibitor)	Olatec Therapeutics New York, NY	chronic musculoskeletal pain, acute gout flares	Phase II www.olatec.com
dexmedetomidine (intranasal)	Recro Pharma Malvern, PA	breakthrough cancer pain (intranasal)	Phase II www.recropharma.com
		severe intractable pain (sublingual)	Phase II www.recropharma.com
DSP-2230 (voltage-gated sodium channel Nav1.7/Nav1.8 inhibitor)	Sunovion Pharmaceuticals Marlborough, MA	neuropathic pain	Phase I www.sunovion.com
EB-001 (botulinum toxin E)	Bonti Newport Beach, CA	musculoskeletal pain	Phase II www.bonti.com
Egalet-002 (abuse-deterrent extended- release oxycodone)	Egalet Wayne, PA	chronic pain	Phase III www.egalet.com
Egalet-004 (abuse-deterrent extended- release hydrocodone)	Egalet Wayne, PA	chronic pain	Phase I www.egalet.com
EMA401 (angiotensin II type 2 receptor antagonist)	Novartis Pharmaceuticals East Hanover, NJ	peripheral neuropathic pain	Phase II www.novartis.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
fadolmidine (alpha-2 adrenergic receptor agonist)	Recro Pharma Malvern, PA	neuropathic pain	Phase II completed www.recropharma.com
fasinumab (nerve growth factor inhibitor)	Regeneron Pharmaceuticals Tarrytown, NY Teva Pharmaceuticals USA North Wales, PA	osteoarthritis pain, chronic lower back pain	Phase III www.regeneron.com
fentanyl transdermal (JNJ-35685)	Janssen Raritan, NJ	chronic pain	Phase I completed www.janssen.com
Gamunex-C® immune globulin 10%	Grifols USA Los Angeles, CA	diabetic neuropathy (see also other)	Phase II www.grifolsusa.com
GDC0310 (RG6029) (Nav1.7 voltage gated sodium channel inhibitor)	Genentech South San Francisco, CA Xenon Pharmaceuticals Burnaby, Canada	chronic pain	Phase I www.gene.com
HX-100 (TRPA1 protein inhibitor)	Hydra Biosciences Cambridge, MA	painful diabetic neuropathy	Phase I completed www.hydrabiosciences.com
hydromorphone intrathecal	Piramal Enterprises Mumbai, India	chronic pain	Phase III www.piramal.com
IMC-1 (celecoxib/famciclovir fixed-dose combination)	Innovative Med Concepts Tuscaloosa, AL	fibromyalgia (Fast Track)	Phase II www.innovativemedconcepts.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ketoprofen topical (lipoygenase-cyclooxygenase inhibitor)	Achelios Therapeutics Chapel Hill, NC	osteoarthritis pain (see also headache)	Phase I www.achelios.com
KIT-302 (amlodipine/celecoxib fixed-dose combination)	Kitov Pharmaceuticals Tel Aviv, Israel	osteoarthritis pain	application submitted www.kitovpharma.com
LTX-03 (immediate-release hydrocodone bitartrate/abuse-resistant)	Acura Pharmaceuticals Palatine, IL	moderate to severe pain	Phase II www.acurapharm.com
LTX-04 (immediate-release hydromorphone/ abuse-resistant)	Acura Pharmaceuticals Palatine, IL	pain (Fast Track)	Phase I www.acurapharm.com
LX9211 (AAK1 inhibitor)	Lexicon Pharmaceuticals The Woodlands, TX	neuropathic pain	Phase I www.lexiconpharma.com
MEDI7352 (NGF/TNF bispecific mAb)	MedImmune Gaithersburg, MA	osteoarthritis pain	Phase I www.medimmune.com
mirogabalin (DS-5565) (CACNA2D1 protein modulator)	Daiichi Sankyo Basking Ridge, NJ	fibromyalgia pain	Phase III www.dsi.com
neridronate intravenous ORPHAN DRUG	Grünenthal Morristown, NJ	complex regional pain syndrome (Fast Track)	Phase III www.grunenthal.us

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NKTR-181 (opioid mu receptor agonist)	Nektar Therapeutics San Francisco, CA	moderate to severe chronic low back pain (Fast Track)	Phase III www.nektar.com
NYX-2925 (NMDA receptor modulator)	Aptinyx Evanston, IL	diabetic peripheral neuropathy (Fast Track), fibromyalgia	Phase II www.aptinyx.com
Omnitram O-desmethyltramadol	Syntrix Biosystems Auburn, WA	chronic pain	Phase II www.syntrixbio.org
oxycodone ER (abuse-resistant) ORPHAN DRUG	Intellipharma International Toronto, Canada	moderate to severe chronic pain (Fast Track)	application submitted www.intellipharma.com
palmitoylethanolamide (micro-PEA)	Prismic Pharmaceuticals Worcester, MA	fibromyalgia (with pregabalin)	Phase II www.prismicpharma.com
		severe chronic pain (with targeted opioids)	Phase I www.prismicpharma.com
PDA-002 (human placental-derived cell therapy)	Celularity Warren, NJ	diabetic peripheral neuropathy	Phase II www.celularity.com
PF329 (abuse-resistant hydromorphone)	Ensysce Biosciences San Diego, CA	chronic pain	Phase I www.ensysce.com
PF614 (abuse-resistant oxycodone extended release)	Ensysce Biosciences San Diego, CA	chronic pain (Fast Track)	Phase I www.ensysce.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
REL-1015 (extended release, abuse-resistant levorphanol)	Relmada Therapeutics New York, NY	chronic pain	Phase II www.relmada.com
REL-1017 (d-methadone)	Relmada Therapeutics New York, NY	neuropathic pain	Phase I www.relmada.com
REL-1028 (buprenorphine oral)	Relmada Therapeutics New York, NY	chronic pain	Phase I www.relmada.com
relugolix (LHRH receptor antagonist)	Myovant Sciences Brisbane, CA	endometriosis-related pain	Phase III www.myovant.com
Remoxy® ER oxycodone controlled release (abuse-resistant)	Pain Therapeutics Austin, TX	moderate to severe chronic pain	application submitted www.paintrials.com
resiniferatoxin (RTX) (TRPV1 receptor agonist) ORPHAN DRUG	Sorrento Therapeutics San Diego, CA	intractable cancer pain, osteoarthritis pain	Phase I www.sorrentotherapeutics.com
RGN-352 injectable (thymosin beta-4)	RegeneRx Biopharmaceuticals Rockville, MD	peripheral neuropathy (see also stroke)	Phase I completed www.regenerx.com
RTA-901 (Hsp90 heat shock protein modulator)	Reata Pharmaceuticals Irving, TX	diabetic neuropathy	Phase I www.reatapharma.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
SequestOx™ oxycodone/naltrexone (abuse-resistant formulation)	Elite Pharmaceuticals Northvale, NJ	moderate to severe chronic pain	application submitted www.elitepharma.com
SoluMatrix® naproxen	Iroko Pharmaceuticals Philadelphia, PA	osteoarthritis pain	Phase II www.iroko.com
SP-102 (non-opioid epidural steroid injectable gel)	Semnur Pharmaceuticals Mountain View, CA	sciatica/lumbar radicular pain (Fast Track)	Phase III www.semnurpharma.com
SWT06101 (topical analgesic)	SWITCH Biotech Conway, AR	fibromyalgia	Phase I completed www.switch-biotech.com
T-121 (zoledronic acid oral) ORPHAN DRUG	Grünenthal USA Morristown, NJ	complex regional pain syndrome	Phase III www.grunenthal.us
tanezumab (nerve growth factor inhibitor)	Eli Lilly Indianapolis, IN Pfizer New York, NY	cancer pain, chronic lower back pain, osteoarthritis pain (Fast Track)	Phase III www.lilly.com
tetrodotoxin (TTX) (sodium channel antagonist)	WEX Pharmaceuticals Vancouver, Canada	chemotherapy-induced neuropathy	Phase II www.wextech.ca
TRV734 (mu receptor agonist)	Trevena Chesterbrook, PA	moderate to severe chronic pain	Phase I www.trevena.com

Pain (Chronic Pain and Chronic Pain Conditions)

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
TV1001SR (slow release oral sodium nitrite)	Soin Neuroscience Dayton, OH TheraVasc Cleveland, OH	painful diabetic neuropathy	Phase II www.soinneuroscience.com www.theravasc.com
V120083 (S120083)	Purdue Pharma Stamford, CT Shionogi Florham Park, NJ	chronic pain due to osteoarthritis of the knee	Phase II www.shionogi.com
VBY-129 (cathepsin S inhibitor)	Virobay Menlo Park, CA	neuropathic pain	Phase I www.virobayinc.com
VM202 (donaperminogene seltoplasmid)	ViroMed Seoul, South Korea	diabetic peripheral neuropathy (see also ALS)	Phase III www.viromed.co.kr
VX-128 (NaV1.8 sodium channel inhibitor)	Vertex Pharmaceuticals Boston, MA	pain	Phase I www.vrtx.com
VX-150 (NaV1.8 sodium channel inhibitor)	Vertex Pharmaceuticals Boston, MA	neuropathic pain	Phase II www.vrtx.com
WST-057 (muscarinic M1 receptor antagonist)	WinSanTor San Diego, CA	diabetic peripheral neuropathy	Phase I www.winsantor.com

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ABBV-951 (levodopa/carbidopa)	AbbVie North Chicago, IL	Parkinson's disease	Phase I www.abbvie.com
ANAVEX™ 2-73 (M1 muscarinic receptor agonist/ intracellular sigma 1 receptor agonist)	Anavex Life Sciences New York, NY	Parkinson's disease (see also Alzheimer's, epilepsy, genetic)	Phase I www.anavex.com
AP-CDLD (levodopa/carbidopa controlled release)	Intec Pharma Jerusalem, Israel	advanced Parkinson's disease	Phase III www.intecpharma.com
APL-130277 (apomorphine sublingual film)	Aquestive Therapeutics Warren, NJ Sunovion Pharmaceuticals Marlborough, MA	off episodes associated with Parkinson's disease (Fast Track)	Phase III www.sunovion.com
apomorphine infusion	US WorldMeds Louisville, KY	idiopathic Parkinson's disease	Phase III www.usworldmeds.com
BIIB054 (alpha-synuclein inhibitor)	Biogen Cambridge, MA	Parkinson's disease	Phase II www.biogen.com
BioE-589 (NAD(P)H modulator)	BioElectron Technology Mountain View, CA Sumitomo Dainippon Pharma Osaka, Japan	Parkinson's disease (see also ALS)	Phase II www.bioelectron.com
Cu(II)ATSM (neuron modulator)	Procypra Therapeutics Sausalito, CA	Parkinson's disease (see also ALS)	Phase I www.colmeddev.com/procypra

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
CX-8998 (Cav3 modulator)	Cavion Charlottesville, VA	Parkinson disease tremors (see also epilepsy, other)	Phase II www.cavion.com
dasatinib (abelson kinase inhibitor)	Inhibikase Therapeutics Atlanta, GA	Parkinson's disease	Phase II www.inhibikase.com
dipraglurant (mGluR5 negative allosteric modulator) ORPHAN DRUG	Addex Pharmaceuticals Geneva, Switzerland	levodopa-induced dyskinesia in Parkinson's disease	Phase II www.addextherapeutics.com
DNL151 (LRRK2 protein inhibitor)	Denali Therapeutics South San Francisco, CA	Parkinson's disease	Phase I www.denalitherapeutics.com
DNL201 (LRRK2 protein inhibitor)	Denali Therapeutics South San Francisco, CA	Parkinson's disease	Phase I www.denalitherapeutics.com
DopaFuse™ levodopa/carbidopa continuous infusion	SynAgile Wilson, WY	advanced Parkinson's disease	Phase II www.synagile.com
DSP-6745 (serotonin receptor antagonist)	Sunovion Pharmaceuticals Marlborough, MA	Parkinson's disease psychosis	Phase I www.sunovion.com
eltoprazine (serotonin 1A/1B receptor agonist) ORPHAN DRUG	Amarantus Bioscience San Francisco, CA	levodopa-induced dyskinesia in Parkinson's disease (see also Alzheimer's)	Phase II www.amarantus.com

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
foliglurax	Lundbeck Deerfield, IL	Parkinson's disease	Phase II www.lundbeck.com
GM608 (motoneuronotrophic factor)	Genervon Biopharmaceuticals Pasadena, CA	mild to moderate Parkinson's disease	Phase II www.genervon.com
Inbrija [®] levodopa inhalation powder	Acorda Therapeutics Ardsley, NY	Parkinson's disease	application submitted www.acorda.com
INP103 (levodopa powder drug-device)	Impel NeuroPharma Seattle, WA	Parkinson's disease (OFF episodes)	Phase II www.impelnp.com
IPX-203 (carbidopa/levodopa fixed-dose combination)	Impax Pharmaceuticals Hayward, CA	Parkinson's disease	Phase II www.impaxlabs.com
ISC-hpNSC [®] neural stem cell therapy	International Stem Cell Carlsbad, CA	Parkinson's disease	Phase I www.internationalstemcell.com
istradefylline (adenosine A2A receptor antagonist)	Kyowa Hakko Kirin Princeton, NJ	Parkinson's disease	Phase III completed www.kyowa-kirin.com
ITI-214 (PDE1 inhibitor)	Intra-Cellular Therapies New York, NY	Parkinson's disease	Phase I/II www.intracellulartherapies.com

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
KPAX002 (repurposed methylphenidate)	K-PAX Pharmaceuticals Mill Valley, CA	Parkinson's disease (see also ALS, Alzheimer's)	in clinical trials www.kpaxpharmaceuticals.com
LTI-291 (glucocerebrosidase stimulant)	Allergan Parsippany, NJ Lysomal Therapeutics Cambridge, MA	Parkinson's disease with GBA1 mutation	Phase II www.allergan.com
LY03003 (rotigotine extended release microspheres)	Luye Pharma Shanghai, China	Parkinson's disease	Phase I www.luye-pharm.com
LY3154207 (D1 potentiator)	Eli Lilly Indianapolis, IN	Parkinson's disease dementia	Phase II www.lilly.com
MEDI1341 (alpha-synuclein inhibitor)	MedImmune Gaithersburg, MD	Parkinson's disease	Phase I www.medimmune.com
ND0612 (levodopa/carbidopa subcutaneous)	Mitsubishi Tanabe Pharma America Jersey City, NJ	Parkinson's disease	Phase II www.mt-pharma-america.com
NPT-200-11 (alpha synuclein inhibitor)	Neuropore Therapies San Diego, CA UCB Smyrna, GA	Parkinson's disease (see also other)	Phase I www.neuropore.com

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
Nuedexta® dextromethorphan HBr and quinidine	Avanir Pharmaceuticals Aliso Viejo, CA	levodopa-induced dyskinesia in Parkinson's disease (see also Alzheimer's)	Phase II www.avanir.com
opicapone (COMT inhibitor)	Neurocrine Biosciences San Diego, CA	Parkinson's disease	Phase III www.neurocrine.com
P2B001 (pramipexole/rasagiline - low dose, sustained release combination)	Pharma Two B Rehovot, Israel	Parkinson's disease	Phase III www.pharma2b.com
PRX002 (RG7935) (alpha-synuclein inhibitor)	Prothena South San Francisco, CA Roche Basel, Switzerland	Parkinson's disease	Phase II www.prothena.com www.roche.com
ropinirole subdermal implant (dopamine D2 receptor antagonist)	Titan Pharmaceuticals South San Francisco, CA	Parkinson's disease	Phase I/II www.titanpharm.com
SAGE-217 (GABA-A receptor modulator)	SAGE Therapeutics Cambridge, MA	Parkinson's disease (see also other)	Phase II www.sagerx.com
SEP-363856 (serotonin 1A receptor agonist, trace amine-associated receptor 1 agonist)	Sunovion Pharmaceuticals Marlborough, MA	Parkinson's disease psychosis	Phase II www.sunovion.com

Parkinson's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
SER-214 (rotigotine controlled release)	Serina Therapeutics Huntsville, AL	Parkinson's disease	Phase I www.serinatherapeutics.com
SUN-K0706 (tyrosine kinase inhibitor)	Sun Pharma Advanced Research Mumbai, India	Parkinson's disease	Phase I www.sunpharma.in
SYN120 (landipirdine)	Acorda Therapeutics Ardsley, NY	Parkinson's disease	Phase II www.acorda.com
TPU-002 (7-day rasagiline transdermal patch)	Teikoku Pharma USA San Jose, CA	Parkinson's disease	Phase I www.teikokuusa.com
UCB0599	UCB Smyrna, GA	Parkinson's disease	Phase I www.ucb.com
venglustat (GCS inhibitor)	Sanofi Genzyme Cambridge, MA	Gaucher-related Parkinson's disease (heterozygous carriers with GBA mutation)	Phase II www.sanofigenzyme.com
VY-AADC01 gene therapy	Voyager Therapeutics Cambridge, MA	advanced Parkinson's disease	Phase I www.voyagertherapeutics.com
YKP10461	SK Life Science Fair Lawn, NJ	Parkinson's disease	Phase I www.sklifescienceinc.com

Spasticity

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
FLX-787 (TRP ion channel agonist)	Flex Pharma Boston, MA	muscle spasticity in multiple sclerosis (see also ALS, genetic)	Phase II www.flex-pharma.com
MedChew-1401 (cannabidiol/tetrahydrocannabinol)	AXIM Biotechnologies New York, NY	muscle spasticity in multiple sclerosis	Phase I www.aximbiotech.com
OS-440 (arbaclofen extended release)	Osmotica Pharmaceutical Bridgewater, NJ	muscle spasticity in multiple sclerosis	application submitted www.osmotica.com
Sativex® nabiximols	GW Pharmaceuticals Carlsbad, CA	muscle spasticity in multiple sclerosis	Phase III www.gwpharm.com
tolperisone (neuromuscular blocking agent)	Neurana Pharmaceuticals San Diego, CA	muscle spasticity associated with spinal cord injury	Phase I www.neuranapharma.com

Spinal Cord Injury

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AST-OPC1 (hESC-derived cell therapy) ORPHAN DRUG	Asterias Biotherapeutics Fremont, CA	subacute spinal cord injury	Phase I/II www.asteriasbiotherapeutics.com
CX717 (AMPA receptor agonist)	RespireRx Pharmaceuticals Glen Rock, NJ	spinal cord injury	Phase II www.respirerx.com

Spinal Cord Injury

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
elezanumab (ABT-555) (RGMa inhibitor)	AbbVie North Chicago, IL	spinal cord injury (see also multiple sclerosis)	Phase I www.abbvie.com
NSI-566 (spinal cord-derived neural stem cell therapy)	Neuralstem Germantown, MD	chronic spinal cord injury (see also ALS)	Phase I www.neuralstem.com
Privigen® 10% intravenous immunoglobulin	CSL Behring King of Prussia, PA	acute traumatic spinal cord injury (see also other)	Phase I www.cslbehring.com
trospium intravesicular	TARIS Biomedical Lexington, MA	neurogenic detrusor overactivity due to spinal cord injury	Phase I www.tarisbiomedical.com
VX-210 (Rho GTP-binding protein inhibitor) ORPHAN DRUG	Vertex Pharmaceuticals Boston, MA	acute spinal cord injury (Fast Track)	Phase II/III www.vrtx.com

Stroke

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
3K3A-APC (recombinant human wild-type activated protein C)	ZZ Biotech Houston, TX	moderate to severe ischemic stroke	Phase II www.zzbiotech.com

Stroke

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
BIB093 (glibenclamide IV) ORPHAN DRUG	Biogen Cambridge, MA	large hemispheric infarction (severe form of ischemic stroke) (Fast Track)	Phase II www.biogen.com
Brillinta [®] ticagrelor	AstraZeneca Wilmington, DE	acute ischemic stroke or transient ischemic stroke	Phase III www.astrazeneca.com
BVI-007 (thrombopoietin inhibitor)	BioVascular San Diego, CA	prevention of thrombotic stroke in patients who have had a previous cardiovascular event	Phase I www.biovascularinc.com
clazosentan (endothelin receptor antagonist) ORPHAN DRUG	Idorsia Pharmaceuticals Allschwil, Switzerland	cerebral vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase III www.idorsia.com
CN-105 (apolipoprotein E agonist)	AegisCN Raleigh, NC	intracerebral hemorrhage	Phase II
DM199 (recombinant human kallikrein-1)	DiaMedica Therapeutics Minneapolis, MN	acute ischemic stroke	Phase II www.diamedica.com
DS-1040 (TAFI-alpha inhibitor)	Daiichi Sankyo Basking Ridge, NJ	acute ischemic stroke	Phase I www.dsi.com

Stroke

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
EG-1962 (nimodipine microparticles) ORPHAN DRUG	Edge Therapeutics Berkeley Heights, NJ	aneurysmal subarachnoid hemorrhage (intraventricular delivery) (Fast Track)	Phase III www.edgetherapeutics.com
		aneurysmal subarachnoid hemorrhage (intracisternal delivery)	Phase I www.edgetherapeutics.com
GM602 (motoneuronotrophic factor)	Genervon Biopharmaceuticals Pasadena, CA	ischemic stroke	Phase II completed www.genervon.com
LT-3001 (antioxidant/free radical scavenger)	Lumosa Therapeutics Taipei, Taiwan	acute ischemic stroke	Phase I www.lumosa.com.tw
MAA868 (anti-FXI mAb)	Novartis Pharmaceuticals East Hanover, NJ	stroke prevention in atrial fibrillation	Phase II www.novartis.com
mesenchymal stem cell therapy (ischemic-tolerant stem cells)	Stemedica Cell Technologies San Diego, CA	ischemic stroke	Phase II www.stemedica.com
MP-124 (PARP inhibitor)	Mitsubishi Tanabe Pharma America Jersey City, NJ	stroke	Phase I www.mt-pharma-america.com
MultiStem [®] allogeneic stem cell therapy	Athersys Cleveland, OH	ischemic stroke (Fast Track)	Phase II www.athersys.com
NA-1 (PSD-95 antagonist)	NoNO Toronto, Canada	stroke with endovascular intervention	Phase III www.nonoinc.ca

Stroke

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NVX-208 (dodecafluoropentane nanoemulsion)	NuvOx Pharma Tucson, AZ	acute ischemic stroke	Phase I/II www.nuvoxpharma.com
PF-05230907 (factor Xa protein replacement) ORPHAN DRUG	Pfizer New York, NY	intracerebral hemorrhage	Phase I www.pfizer.com
Pradaxa [®] dabigatran etexilate	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	secondary stroke prevention in patients with embolic stroke of undetermined source	Phase III www.boehringer-ingelheim.com
RGN-352 injectable (thymosin beta-4)	RegeneRx Biopharmaceuticals Rockville, MD	stroke (see also pain)	Phase I completed www.regenerx.com
salvinorin A (opioid kappa receptor agonist)	Neurokappa Therapeutics Philadelphia, PA	cerebral vasospasm	Phase I
Sanguinate [™] PEGylated hemoglobin and carbon monoxide	Prolong Pharmaceuticals South Plainfield, NJ	delayed cerebral ischemia after acute subarachnoid hemorrhage	Phase II completed www.prolongpharma.com
SB623 (modified bone marrow derived mesenchymal stem cells)	SanBio Mountain View, CA Sunovion Pharmaceuticals Marlborough, MA	chronic stroke (see also brain injury)	Phase II www.sanbio.com www.sunovion.com
stroke therapeutic	HSRx Biopharmaceutical Tucson, AZ	stroke	Phase I www.hsrxbiopharmaceutical.com

Stroke

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
TF0023 (aspirin pro-drug)	Techfields Pharma Dover, DE	ischemic stroke	Phase II www.tfpharma.com
Tysarbi® natalizumab	Biogen Cambridge, MA	acute ischemic stroke (see also epilepsy)	Phase II www.biogen.com

Tic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
ABX-1431 (MGLL inhibitor)	Abide Therapeutics San Diego, CA	Tourette's syndrome and chronic motor Tic disorder (see also pain)	Phase I www.abidetx.com
Austedo® deutetrabenazine ORPHAN DRUG	Teva Pharmaceuticals USA North Wales, PA	Tourette's syndrome	Phase III www.tevapharm.com
CPP-115 (GABA-AT inhibitor)	Catalyst Pharmaceutical Coral Gables, FL	Tourette's syndrome (see also epilepsy)	Phase II www.catalystpharma.com
ecopipam (dopamine D1 receptor antagonist)	Psyadon Pharmaceuticals Germantown, MD	Tourette's syndrome	Phase II www.psyadonrx.com
Ingrezza® valbenazine ORPHAN DRUG	Neurocrine Biosciences San Diego, CA	Tourette's syndrome	Phase II www.neurocrine.com

Tic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
THX-TS01 (cannabinoid receptor modulator)	Therapix Biosciences Givatayim, Israel	Tourette's syndrome	Phase III www.therapixbio.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
AAV1-FS344 (follistatin gene therapy) ORPHAN DRUG	Milo Biotechnology Cleveland, OH	inclusion body myositis (see also muscular dystrophy)	Phase I/II www.milobiotechnology.com
ABBV-8E12 (anti-tau antibody) ORPHAN DRUG	AbbVie North Chicago, IL	progressive supranuclear palsy (see also Alzheimer's)	Phase II www.abbvie.com
ACT-101 (recombinant alpha fetoprotein) ORPHAN DRUG	Alpha Cancer Technologies Toronto, Canada	myasthenia gravis (see also multiple sclerosis)	Phase I www.alpha-cancer.com
ANX005 (C1q protein complex inhibitor)	Annexon Biosciences South San Francisco, CA	neurodegenerative disorders	Phase I www.annexonbio.com
ARGX-113 (efgartigimod) ORPHAN DRUG	Argenx Breda, Netherlands	myasthenia gravis	Phase II www.argenx.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
arimoclomol ORPHAN DRUG	Orphazyme Copenhagen, Denmark	inclusion body myositis	Phase II/III www.orphazyme.com
BIIB080 (IONIS-MAPT _{rx}) (tau-targeting protein)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	frontotemporal dementia (see also Alzheimer's)	Phase I/II www.biogen.com
BIIB092 (tau protein inhibitor) ORPHAN DRUG	Biogen Cambridge, MA	progressive supranuclear palsy (see also Alzheimer's)	Phase II www.biogen.com
brexanolone (GABA-A receptor modulator)	Sage Therapeutics Cambridge, MA	essential tremor (see also epilepsy)	Phase II completed www.sagerx.com
BTD-001 (GABA antagonist)	Balance Therapeutics San Bruno, CA	narcolepsy, type 2	Phase II www.balance-therapeutics.com
BYM338 (bimagrumab) (activin type 2 receptor inhibitor)	Novartis Pharmaceuticals East Hanover, NJ	muscular atrophy after hip fracture recovery	Phase II www.novartis.com
CAD-1833	Cadent Therapeutics Cambridge, MA	essential tremor (see also genetic)	Phase I www.cadenttx.com
CFZ533 (CD40 antigen inhibitor)	Novartis Pharmaceuticals East Hanover, NJ	myasthenia gravis	Phase II completed www.novartis.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
CNM-Au8 (nanocrystalline gold suspension)	Clene Nanomedicine Salt Lake City, UT	neurodegenerative disorders	Phase I www.clene.com
CX1789 (AMPA receptor agonist)	RespireRx Pharmaceuticals Glen Rock, NJ	central sleep apnea	Phase II www.respirerx.com
CX-8998 (Cav3 modulator)	Cavion Charlottesville, VA	essential tremor (see also epilepsy, Parkinson's)	Phase II www.cavion.com
elamipretide (mitochondria-targeting short peptide)	Stealth BioTherapeutics Newton, MA	mitochondrial myopathy (Fast Track)	Phase III www.stealthbt.com
Firdapse® amifampridine ORPHAN DRUG	Catalyst Pharmaceuticals Coral Gables, FL	Lambert-Eaton myasthenic syndrome (see also genetic)	Phase III www.catalystpharma.com
		MuSK myasthenia gravis	Phase II www.catalystpharma.com
Gamunex-C® immune globulin 10%	Grifols USA Los Angeles, CA	myasthenia gravis (see also pain)	Phase II www.grifolsusa.com
GTP-004 (acetylcholinesterase inhibitor)	GT Biopharma Washington, DC	myasthenia gravis	Phase I www.gtbiopharma.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
inebilizumab (CD19 antigen inhibitor) ORPHAN DRUG	MedImmune Gaithersburg, MD	neuromyelitis optica	Phase II www.medimmune.com
Injectafer® ferric carboxymaltose	Luitpold Pharmaceuticals Shirley, NY	restless leg syndrome	Phase III www.luitpold.com
MK 8719 (OGA inhibitor)	Alectos Therapeutics Vancouver, Canada Merck Whitehouse Station, NJ	progressive supranuclear palsy	Phase I completed www.alectos.com www.merck.com
MRX-4TZT (tizanidine transdermal patch)	Cipla USA Miami, FL MEDRx Kagawa, Japan	spastic paralysis	Phase I www.ciplausa.com www.medrx.co.jp
NBI-640756 (nervous system modulator)	Neurocrine Biosciences San Diego, CA	essential tremor	Phase I www.neurocrine.com
nelotanserin (5HT2A receptor agonist)	Axovant Sciences New York, NY	Lewy body dementia (Fast Track)	Phase II www.axovant.com
Niagen® nicotinamide riboside	ChromaDex Irvine, CA	mitochondrial myopathy	Phase I www.chromadex.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
NPT-200-11 (alpha synuclein inhibitor)	Neuropore Therapies San Diego, CA UCB Smyrna, GA	multiple system atrophy (see also Parkinson's)	Phase I www.neuropore.com
PF-06755347	Pfizer New York, NY	chronic inflammatory demyelinating polyneuropathy	Phase I www.pfizer.com
Privigen® 10% intravenous immunoglobulin	CSL Behring King of Prussia, PA	chronic inflammatory demyelinating polyneuropathy (see also spinal cord injury)	application submitted www.cslbehring.com
QPI-1007 (RNA interference) ORPHAN DRUG	Quark Pharmaceuticals Fremont, CA	non-arteritic anterior ischemic optic neuropathy (optic nerve stroke)	Phase III www.quarkpharma.com
RA101495 (complement C5 inhibitor)	Ra Pharmaceuticals Cambridge, MA	myasthenia gravis	Phase II www.rapharma.com
rozanolixizumab (Fc receptor antagonist)	UCB Smyrna, GA	myasthenia gravis	Phase II www.ucb.com
RVT-104 (glycopyrrolate/rivastigmine combination)	Axovant Sciences New York, NY	Lewy body dementia (see also Alzheimer's)	Phase I www.axovant.com

Other Neurological Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Phase of Development</u>
SA237 (RG6168) (interleukin-6 receptor antagonist)	Chugai Pharma USA Berkeley Heights, NJ Roche Basel, Switzerland	neuromyelitis optica	Phase III www.chugai-pharm.com
SAGE-217 (GABA-A receptor modulator)	SAGE Therapeutics Cambridge, MA	essential tremor (see also Parkinson's)	Phase II completed www.sagerx.com
SHP680 (d-amphetamine prodrug)	Shire Lexington, MA	neurologic conditions	Phase I www.shire.com
Soliris® eculizumab ORPHAN DRUG	Alexion Pharmaceuticals New Haven, CT	neuromyelitis optica spectrum disorder	Phase III www.alexion.com
SOM3355 (VMAT inhibitor)	SOM Biotech Cambridge, MA	tardive dyskinesia (see also Huntington's)	Phase I www.sombiotech.com
TPI-287 (next generation taxane) ORPHAN DRUG	Cortice Biosciences New York, NY	progressive supranuclear palsy (see also Alzheimer's, cancer)	Phase I www.corticebiosciences.com
xenon (gas for inhalation) ORPHAN DRUG	Mallinckrodt St. Louis, MO NeuroproteXeon Orchard Park, NY	chronic brain damage due to cardiac arrest	Phase III www.mallinckrodt.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 **March 21, 2018**. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States 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).

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.

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.