

2015 Medicines in Development for Neurological Disorders

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

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AC-1204 (caprylic triglyceride)	Accera <i>Broomfield, CO</i>	mild to moderate Alzheimer's disease	Phase II/III www.accerapharma.com
ACI-35 (active anti-Tau vaccine)	AC Immune <i>Lausanne, Switzerland</i> Janssen Research & Development <i>Raritan, NJ</i>	Alzheimer's disease	Phase I www.acimmune.com www.janssenrnd.com
AD-4833/TOMM40 (insulin sensitizer)	Takeda Pharmaceutical <i>Deerfield, IL</i> Zinfandel Pharmaceuticals <i>Chapel Hill, NC</i>	delay onset of mild cognitive impairment due to Alzheimer's disease	Phase III www.takeda.com
aducanumab (BIIB037) (amyloid beta-protein inhibitor)	Biogen <i>Cambridge, MA</i>	Alzheimer's disease	Phase I www.biogen.com
ALZ-801 (amyloid beta-protein inhibitor)	Alzheon <i>Framingham, MA</i>	Alzheimer's disease	Phase I www.alzheon.com
ANAVEX Plus (ANAVEX 2-73/donepezil)	Anavex Life Sciences <i>New York, NY</i>	Alzheimer's disease	Phase II www.anavex.com
ASP3662	Astellas Pharma US <i>Northbrook, IL</i>	Alzheimer's disease	Phase I www.astellas.us

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AVN-101 (serotonin 6 receptor antagonist)	AllaChem <i>Hallandale, FL</i> Avineuro Pharmaceuticals <i>San Diego, CA</i>	Alzheimer's disease	Phase II www.allachem.com
AVN-322 (serotonin 6 receptor antagonist)	AllaChem <i>Hallandale, FL</i> Avineuro Pharmaceuticals <i>San Diego, CA</i>	Alzheimer's disease	Phase II www.allachem.com
AVP-786 (dextromethorphan analogue/quinidine)	Avanir Pharmaceuticals <i>Aliso Viejo, CA</i> Concert Pharmaceuticals <i>Lexington, MA</i>	agitation in Alzheimer's disease	Phase I www.avanir.com www.concertpharma.com
AVP-923 (dextromethorphan/quinidine fixed-dose combination)	Avanir Pharmaceuticals <i>San Diego, CA</i>	agitation in Alzheimer's disease (see also headache, Parkinson's)	Phase II www.avanir.com
AZD3293 (beta-secretase inhibitor)	AstraZeneca <i>Wilmington, DE</i> Eli Lilly <i>Indianapolis, IN</i>	Alzheimer's disease	Phase II www.astrazeneca.com www.lilly.com
azeliragon (TTP488) (RAGE antagonist)	vTv Therapeutics <i>High Point, NC</i>	Alzheimer's disease (Fast Track)	Phase III www.vtvtherapeutics.com

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BACE inhibitor	CoMentis <i>South San Francisco, CA</i>	Alzheimer's disease	Phase I completed www.comentis.com
BACE inhibitor	Eli Lilly <i>Indianapolis, IN</i>	Alzheimer's disease	Phase I www.lilly.com
BACE inhibitor	Janssen Pharmaceuticals <i>Titusville, NJ</i> Shionogi <i>Osaka, Japan</i>	early-stage Alzheimer's disease	Phase II www.janssenpharmaceuticalsinc.com www.shionogi.co.jp
BAN2401 (amyloid beta-protein inhibitor)	Biogen <i>Cambridge, MA</i> Eisai <i>Woodcliff Lake, NJ</i>	Alzheimer's disease	Phase II www.biogen.com www.eisai.com
BI 409306 (phosphodiesterase 9A inhibitor)	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	Alzheimer's disease	Phase II www.boehringer-ingelheim.com
bisnorcymserine (BNC)	QR Pharma <i>Berwyn, PA</i>	advanced Alzheimer's disease	Phase I www.qrpharma.com
bryostatin-1	Neurotrope BioScience <i>Newark, NJ</i>	Alzheimer's disease	Phase II www.neurotropebioscience.com

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CAD106 (beta-amyloid protein inhibitor)	GlaxoSmithKline <i>Research Triangle Park, NC</i>	Alzheimer's disease	Phase II completed www.gsk.com
CPC-201	Chase Pharmaceuticals <i>Washington, DC</i>	Alzheimer's disease	Phase II www.chasepharmaceuticals.com
crenezumab (beta-amyloid protein inhibitor)	Genentech <i>South San Francisco, CA</i>	mild to moderate Alzheimer's disease	Phase II www.gene.com
E2609 (BACE1 protein inhibitor)	Biogen <i>Cambridge, MA</i> Eisai <i>Woodcliff Lake, NJ</i>	Alzheimer's disease	Phase II www.biogen.com www.eisai.com
ELND005 (amyloid-beta protein inhibitor)	Transition Therapeutics <i>Toronto, Canada</i>	agitation/aggression in Alzheimer's disease (Fast Track)	Phase II www.transitiontherapeutics.com
encenicline (alpha-7 potentiator)	Forum Pharmaceuticals <i>Watertown, MA</i>	Alzheimer's disease	Phase III www.forumpharma.com
gantenerumab (amyloid-beta protein inhibitor)	Genentech <i>South San Francisco, CA</i>	early-stage Alzheimer's disease	Phase III www.gene.com
GC021109 (purinoceptor P2Y6 agonist)	GliaCure <i>Boston, MA</i>	mild to moderate Alzheimer's disease (Fast Track)	Phase I www.gliacure.com

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
idalopirdine (serotonin 6 receptor antagonist)	Lundbeck <i>Deerfield, IL</i> Otsuka Pharmaceutical <i>Tokyo, Japan</i>	Alzheimer's disease	Phase III www.lundbeck.com www.otsuka.co.jp
immune globulin	Grifols USA <i>Los Angeles, CA</i>	Alzheimer's disease	Phase III www.grifols.com
JNJ-54861911	Janssen Research & Development <i>Raritan, NJ</i>	Alzheimer's disease	Phase II www.janssenrnd.com
LMTX (tau protein aggregation/ TDP-43 aggregation inhibitor)	TauRx Pharmaceuticals <i>Singapore</i>	Alzheimer's disease	Phase III www.taurx.com
Lu-AF20513 (amyloid beta-protein inhibitor)	Lundbeck <i>Deerfield, IL</i> Otsuka Pharmaceutical <i>Tokyo, Japan</i>	Alzheimer's disease	Phase I www.lundbeck.com www.otsuka.co.jp
LY3002813 (N3pG-A β mAb)	Eli Lilly <i>Indianapolis, IN</i>	Alzheimer's disease	Phase I www.lilly.com
MEDI1814 (anti-amyloid beta mAb)	MedImmune <i>Gaithersburg, MD</i>	Alzheimer's disease	Phase I www.medimmune.com
mGlu2 agonist	Eli Lilly <i>Indianapolis, IN</i>	Alzheimer's disease psychosis	Phase I www.lilly.com

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MK-7622 (quinazolinone)	Merck <i>Whitehouse Station, NJ</i>	Alzheimer's disease (adjunct therapy)	Phase II www.merck.com
MK-8931 (BACE1 protein inhibitor)	Merck <i>Whitehouse Station, NJ</i>	Alzheimer's disease	Phase III www.merck.com
MSDC-0160 (mitochondrial protein stimulant)	Metabolic Solutions Development <i>Kalamazoo, MI</i>	Alzheimer's disease	Phase II completed www.msdrx.com
NIC-515 (amyloid precursor protein secretase inhibitor)	Humanetics <i>Minneapolis, MN</i>	Alzheimer's disease	Phase II www.humaneticscorp.com
PF-05212377 (SAM-760) (5HT6 antagonist)	Pfizer <i>New York, NY</i>	Alzheimer's disease	Phase II www.pfizer.com
PF-06648671	Pfizer <i>New York, NY</i>	Alzheimer's disease	Phase I www.pfizer.com
Posiphen [®] R-phenserine	QR Pharma <i>Berwyn, PA</i>	Alzheimer's disease (see also Parkinson's)	Phase II www.qrpharma.com
PTI-80 (amyloid beta/tau protein inhibitor)	ProteoTech <i>Kirkland, WA</i>	Alzheimer's disease	Phase I www.proteotech.com

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<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RG1577 (MAO-B inhibitor)	Roche <i>New York, NY</i>	Alzheimer's disease	Phase II www.roche.com
RG7345 (anti-Tau mAb)	Roche <i>New York, NY</i>	Alzheimer's disease	Phase I www.roche.com
rilapladib (Lp-PLA2 inhibitor)	GlaxoSmithKline <i>Research Triangle Park, NC</i>	Alzheimer's disease	Phase II www.gsk.com
RVT-101 (5-HT6 receptor antagonist)	Axovant Sciences <i>New York, NY</i>	mild to moderate Alzheimer's disease	Phase II completed www.axovant.com
RVX208 (BET protein inhibitor)	Resverlogix <i>Calgary, Canada</i>	Alzheimer's disease	Phase I completed www.resverlogix.com
SAR228810 (anti-protofibrillar AB mAb)	Sanofi US <i>Bridgewater, NJ</i>	Alzheimer's disease	Phase I www.sanofi.com
sGC 1061 (nomethiazole)	sGC Pharma <i>Vancouver, Canada</i>	Alzheimer's disease	Phase I sgcpharma.com
solanezumab (amyloid beta protein inhibitor)	Eli Lilly <i>Indianapolis, IN</i>	Alzheimer's disease	Phase III www.lilly.com
SUVN-502 (serotonin 6 receptor antagonist)	Suven Life Sciences <i>Hyderabad, India</i>	Alzheimer's disease	Phase I www.suven.com

Alzheimer's Disease

Product Name

Sponsor

Indication

Development Phase

SUVN-G3031
(histamine H3 receptor antagonist)

Suven Life Sciences
Hyderabad, India

cognitive dysfunction in
Alzheimer's disease

Phase I
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
www.t3dtherapeutics.com

TPI 287
(abeotaxane)

Cortice Biosciences
New York, NY

mild to moderate Alzheimer's disease
(see also brain tumors, other)

Phase I
www.corticebio.com

UB-311
(amyloid beta-protein inhibitor)

United Biomedical
Hauppauge, NY

Alzheimer's disease

Phase I
www.unitedbiomedical.com

VX-745
(p38 mitogen-activated protein
kinase inhibitor)

EIP Pharma
Cambridge, MA

Alzheimer's disease

Phase II
www.eipharma.com

Amyotrophic Lateral Sclerosis

Product Name

Sponsor

Indication

Development Phase

arimoclomal
ORPHAN DRUG

ALS Foundation
Washington, DC
Orphazyme
Copenhagen, Denmark
University of Miami
Miami, FL

hereditary or familial
amyotrophic lateral sclerosis (ALS)
(Fast Track)

Phase II/III
www.orphazyme.com

CC100

Chemigen
Indianapolis, IN

ALS

Phase I completed

dexpramipexole
ORPHAN DRUG

Knopp Biosciences
Pittsburgh, PA

ALS (Fast Track)

Phase II
www.knoppbio.com

GM6
(peptide therapeutic)
ORPHAN DRUG

Genervon Biopharmaceuticals
Pasadena, CA

ALS (Fast Track)
(see also Parkinson's, stroke)

Phase II
www.genervon.com

H.P. Acthar® Gel
repository corticotropin injection

Mallinckrodt Pharmaceuticals
St. Louis, MO

ALS

Phase II
www.mallinckrodt.com

MN-166
(ibudilast)

MediciNova
La Jolla, CA

ALS
(see also multiple sclerosis)

Phase II
www.medicinova.com

neurotrophic factor-producing adult
stem cell therapy (MSC-NTF cells)
ORPHAN DRUG

BrainStorm Cell Therapeutics
Hackensack, NJ

ALS (Fast Track)

Phase II
www.brainstorm-cell.com

Amyotrophic Lateral Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NP001 (macrophage modulator)	Neuraltus Pharmaceuticals <i>Palo Alto, CA</i>	ALS	Phase II www.neuraltus.com
NSI-566 (spinal cord-derived neural stem cell therapy) ORPHAN DRUG	Neuralstem <i>Germantown, MD</i>	ALS (see also spinal cord injury, stroke)	Phase II www.neuralstem.com
ozanezumab (NOGO-A inhibitor)	GlaxoSmithKline <i>Research Triangle Park, NC</i>	ALS	Phase II www.gsk.com
tirasemtiv (troponin complex stimulant) ORPHAN DRUG	Cytokinetics <i>South San Francisco, CA</i>	ALS (Fast Track)	Phase II www.cytokinetics.com
VM202 (modified hepatocyte growth factor gene therapy)	ViroMed <i>Seoul, South Korea</i>	ALS (see also pain)	Phase I/II www.viomed.co.kr

Brain Injury

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Cirara™ RP-1128	Remedy Pharmaceuticals <i>New York, NY</i>	traumatic brain injury (see also stroke)	Phase II completed www.remedypharma.com

Brain Injury

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NH001 (apomorphine subcutaneous) ORPHAN DRUG	NeuroHealing Pharmaceuticals <i>Waban, MA</i>	vegetative state (coma) or minimally conscious state following traumatic brain injury (Fast Track)	Phase II www.neurohealing.com
NNZ-2256 (cytokine inhibitor/neuropeptide receptor modulator)	Neuren Pharmaceuticals <i>Bethesda, MD</i>	traumatic brain injury (Fast Track) (see also genetic)	Phase II www.neurenpharma.com
VOLT02 (progesterone receptor agonist)	Levolta Pharmaceuticals <i>Bethlehem, PA</i>	traumatic brain injury	Phase II www.levoltapharma.com

Brain Tumors

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
2B3-101 (doxorubicin liposomal) ORPHAN DRUG	BBB Therapeutics <i>Leiden, Netherlands</i>	glioma	Phase I www.bbbtherapeutics.com
ABT-414 (monoclonal antibody-drug conjugate) ORPHAN DRUG	AbbVie <i>North Chicago, IL</i>	glioblastoma	Phase I/II www.abbvie.com
ADU-623 (therapeutic bivalent vaccine)	Aduro Biotech <i>Berkeley, CA</i>	glioblastoma	Phase I www.adoro.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

AG-120
(IDH1 inhibitor)

Agios Pharmaceuticals
Cambridge, MA

glioma

Phase I
www.agios.com

aglatimagene besadenovec
(gene therapy)
ORPHAN DRUG

Advantagene
Auburndale, MA

glioma

Phase II
www.advantagene.com

glioma (pediatric)

Phase I/II
www.advantagene.com

aldoxorubicin
(delivery-enhanced doxorubicin)

CytRx
Los Angeles, CA

glioblastoma

Phase II
www.cytrx.com

AMG 595
(anti-EGFRvIII antibody-drug conjugate)

Amgen
Thousand Oaks, CA

anaplastic astrocytoma,
recurrent glioblastoma

Phase I
www.amgen.com

ANG 1005
(peptide-paclitaxel conjugate)
ORPHAN DRUG

Angiochem
Montreal, Canada

recurrent glioblastoma (Fast Track)

Phase II
www.angiochem.com

antineoplaston A10/AS2-1
ORPHAN DRUG

Burzynski Research Institute
Houston, TX

diffuse intrinsic brainstem glioma

Phase II
www.burzynskiresearch.com

Avastin®
bevacizumab

Genentech
South San Francisco, CA

glioblastoma (1st-line)

Phase III
www.gene.com

BGJ398
(FGF-R kinase inhibitor)

Novartis Pharmaceuticals
East Hanover, NJ

recurrent glioblastoma

Phase II
www.novartis.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

buparlisib
(PI3K inhibitor)

Novartis Pharmaceuticals
East Hanover, NJ

glioblastoma

Phase I/II
www.novartis.com

Cometriq™
cabozantinib

Exelixis
South San Francisco, CA

astrocytoma, glioblastoma

Phase II completed
www.exelixis.com

Cotara®
iodine I 131 derlotuximab biotin
ORPHAN DRUG

Peregrine Pharmaceuticals
Tustin, CA

recurrent glioblastoma (Fast Track)

Phase II completed
www.peregrineinc.com

crenolanib
(PDGFR inhibitor)

Arog Pharmaceuticals
Dallas, TX

glioma (pediatric)

Phase II
www.arogpharma.com

CTO
(carboxyamidotriazole orotate)

Tactical Therapeutics
New York, NY

glioma (combination therapy)
(Fast Track)

Phase I/II
www.tacticaltherapeutics.com

DCVax®-L
dendritic cell-based vaccine
ORPHAN DRUG

Northwest Biotherapeutics
Bethesda, MD

glioblastoma

Phase III
www.nwbio.com

DM-CHOC-PEN
(non-neurotoxic derivative of
penclomidine)

Dekk-Tek
New Orleans, LA

brain tumors

Phase II
www.dekk-tec.com

DNX-2401
(oncolytic virus)
ORPHAN DRUG

DNatrix
Houston, TX

glioblastoma (Fast Track)

Phase I
www.dnatrix.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

E7050
(golvatinib)

Eisai
Woodcliff Lake, NJ

recurrent glioblastoma

Phase I/II
www.eisai.com

ERC1671
(therapeutic vaccine)
ORPHAN DRUG

Epitopoietic Research
Pasadena, CA
University of California
Irvine, CA

grade IV glioblastoma

Phase II
www.ercbelgium.com

ETS2101
(dexanabinol)

e-Therapeutics
Oxfordshire, United Kingdom
Moores UCSD Cancer Center
La Jolla, CA

glioma

Phase I
www.etherapeutics.co.uk

galunisertib
(TGF-beta 1 kinase inhibitor)

Eli Lilly
Indianapolis, IN

glioblastoma

Phase II
www.lilly.com

glioma

Phase I/II
www.lilly.com

Gilotrif®
afatinib

Boehringer Ingelheim Pharmaceuticals
Ridgefield, CT

recurrent glioma

Phase II
www.boehringer-ingelheim.com

heat shock protein vaccine
(Prophage Series G-200)
ORPHAN DRUG

Agenus
Lexington, MA

recurrent or newly diagnosed
glioblastoma

Phase II
www.agenus.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

ICT-107
(dendritic cell-based vaccine)
ORPHAN DRUG

ImmunoCellular Therapeutics
Calabasas, CA

newly diagnosed glioblastoma

Phase II
www.imuc.com

ICT-121
(dendritic cell-based vaccine)

ImmunoCellular Therapeutics
Calabasas, CA

recurrent glioblastoma

Phase I
www.imuc.com

indoximod
(IDO inhibitor)

NewLink Genetics
Ames, IA

primary malignant brain tumors

Phase I/II
www.newlinkgenetics.com

INXN-2001
(Ad-RTS-IL-12 gene therapy)

ZIOPHARM Oncology
Boston, MA

glioblastoma, anaplastic
oligoastrocytoma

Phase I/II
www.ziopharm.com

Jevtana®
cabazitaxel

Sanofi US
Bridgewater, NJ

malignant central nervous system
tumors

Phase I/II
www.sanofi.com

KX02
(Src kinase/pre-tubulin inhibitor)
ORPHAN DRUG

Kinex Pharmaceuticals
Buffalo, NY

glioma

Phase I
www.kinexpharma.com

LDE225
(SMO protein inhibitor)

Novartis Pharmaceuticals
East Hanover, NJ

medulloblastoma

Phase II
www.novartis.com

macitentan
(endothelin A/B receptor antagonist)

Actelion Pharmaceuticals
South San Francisco, CA

glioblastoma

Phase I
www.actelion.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

marizomib
(proteasome inhibitor)

Triphase Accelerator
San Diego, CA

glioblastoma

Phase I
www.triphaseco.com

mibefradil
(T-type calcium channel antagonist)
ORPHAN DRUG

Cavion
Charlottesville, VA

high-grade glioma

Phase I
www.cavionpharma.com

mipsagargin
(thapsigargin analogue)

GenSpera
San Antonio, TX

glioblastoma

Phase II
www.genspera.com

MM-398
(encapsulated irinotecan
nanotherapeutic)

Merrimack Pharmaceuticals
Cambridge, MA

glioma

Phase I
www.merrimackpharma.com

NEO-100
(highly purified perillyl alcohol)
ORPHAN DRUG

NEONC Technologies
Los Angeles, CA

recurrent glioblastoma

Phase I
www.neonctech.com

NVX-108
(radiation-sensitizing agent)

NuvOx Pharma
Tucson, AZ
National Cancer Institute
Bethesda, MD

glioblastoma

Phase I/II
www.nuvoxpharma.com

Opdivo®
nivolumab

Bristol-Myers Squibb
Princeton, NJ

glioblastoma

Phase III
www.bms.com

PEPIDH1M
(dendritic cell vaccine)

Annias Immunotherapeutics
Chapel Hill, NC

glioblastoma

Phase I/II
www.anniasimmuno.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

PLX-3397
(CSF-1R inhibitor)

Plexxikon
Berkeley, CA

glioblastoma

Phase II
www.plexxikon.com

Poly-ICLC
ORPHAN DRUG

Oncovir
Washington, DC

glioblastoma

Phase II
www.oncovir.com

Reolysin[®]
pelareorep
ORPHAN DRUG

Oncolytics Biotech
Calgary, Canada

high grade glioma (pediatrics)

Phase I
www.oncolyticsbiotech.com

rindopepimut
(EGFR antagonist)
ORPHAN DRUG

Celldex Therapeutics
Hampton, NJ

glioblastoma (1st-line) (Fast Track)
(Fast Track)

Phase III
www.celldex.com

recurrent glioblastoma
(Fast Track)

Phase II
www.celldex.com

Seprehvir[®]
HSV1716

Virttu Biologics
Glasgow, United Kingdom
National Cancer Institute
Bethesda, MD

glioblastoma

Phase I completed
www.virttu.com

selinexor (oral)
(exportin-1 protein inhibitor/
nuclear protein inhibitor)

Karyopharm Therapeutics
Newton, MA

glioblastoma

Phase II
www.karyopharm.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

SL-701
(immunotherapy)
ORPHAN DRUG

Stemline Therapeutics
New York, NY

glioblastoma, glioma

Phase I/II
www.stemline.com

SurVaxM
(BIRC5 protein inhibitor)

MimiVax
Buffalo, NY

malignant glioma

Phase I
www.mimivax.com

TH-302
(evofosfamide)

Threshold Pharmaceuticals
South San Francisco, CA
University of Texas
San Antonio, TX

high grade glioma

Phase I/II
www.thresholdpharm.com

Toca 511 + Toca FC
(gene therapy)

Tocagen
San Diego, CA

anaplastic astrocytoma,
glioblastoma

Phase II/III
www.tocagen.com

TPI 287
(abeotaxane)

Cortice Biosciences
New York, NY

glioblastoma
(see also Alzheimer's, other)

Phase II
www.corticebio.com

TRC105
(ENG protein inhibitor)

TRACON Pharmaceuticals
San Diego, CA
National Cancer Institute
Bethesda, MD

glioblastoma

Phase II
www.traconpharma.com

TSC
(trans-sodium crocetinate)
ORPHAN DRUG

Diffusion Pharmaceuticals
Charlottesville, VA

glioblastoma

Phase I/II
www.diffusionpharma.com

Brain Tumors

Product Name

Sponsor

Indication

Development Phase

TVI-Brain-1
(immunotherapy)
ORPHAN DRUG

TVAX Biomedical
Lenexa, KS

glioma

Phase II completed
www.tvaxbiomedical.com

VAL-083
(N7-alkylating agent)
ORPHAN DRUG

DelMar Pharmaceuticals
Vancouver, Canada

glioblastoma

Phase I/II
www.delmarpharma.com

VB 111
(targeted anti-angiogenic agent)
ORPHAN DRUG

VBL Therapeutics
Tel Aviv, Israel

recurrent glioblastoma (Fast Track)

Phase II
www.vblrx.com

veliparib

AbbVie
North Chicago, IL

brain metastasis

Phase II
www.abbvie.com

Epilepsy

Product Name

Sponsor

Indication

Development Phase

Afinitor®
everolimus

Novartis Pharmaceuticals
East Hanover, NJ

tuberous sclerosis complex seizures

Phase III
www.novartis.com

Optiom®
eslicarbazepine acetate

Sunovion Pharmaceuticals
Marlborough, MA

epilepsy in adults (monotherapy)

application submitted
www.sunovion.com

AZ-002
(inhalation alprazolam)

Alexza Pharmaceuticals
Mountain View, CA

acute repetitive seizures

Phase II
www.alexza.com

Epilepsy

Product Name

Sponsor

Indication

Development Phase

brivaracetam

UCB
Smyrna, GA

epilepsy (adjunctive treatment)

application submitted
www.ucb.com

Carbella™
carbamazepine intravenous
ORPHAN DRUG

Lundbeck
Deerfield, IL

epilepsy

application submitted
www.lundbeck.com

cannabidiol oral solution

INSYS Therapeutics
Chandler, AZ

treatment resistant seizures

Phase I/II
www.insysrx.com

CPP-115
(4-aminobutyrate transaminase inhibitor)
ORPHAN DRUG

Catalyst Pharmaceuticals
Coral Gables, FL

complex partial seizures,
infantile spasms
(see also Tourette's)

Phase I
www.catalystpharma.com

Epidiolex®
cannabidiol
ORPHAN DRUG

GW Pharmaceuticals
Cambridge, United Kingdom

Dravet syndrome (Fast Track)
(pediatric)

Phase III
www.gwpharm.com

Lennox-Gastaut syndrome

Phase II
www.gwpharm.com

Fycompa®
perampanel

Eisai
Woodcliff Lake, NJ

primary generalized tonic-clonic
seizures

application submitted
www.eisai.com

partial-onset seizures (pediatric)

Phase II
www.eisai.com

Epilepsy

Product Name

Sponsor

Indication

Development Phase

ganaxolone
(GABA-A receptor agonist)
ORPHAN DRUG

Marinus Pharmaceuticals
Radnor, PA

focal onset seizures (adults)

Phase III
www.marinuspharma.com

PCDH19 female epilepsy (pediatric)

Phase II
www.marinuspharma.com

INS-001
(synthetic huperzine)

Insero Health
Miami Beach, FL

epilepsy

Phase I completed
www.insero.co

IPX218

Impax Laboratories
Hayward, CA

epilepsy

Phase II completed
www.impaxlabs.com

Lyrica®
pregabalin

Pfizer
New York, NY

generalized tonic-clonic seizures,
partial onset seizures (pediatric)
(see also pain)

Phase III
www.pfizer.com

MP-102

Marathon Pharmaceuticals
Northbrook, IL

infantile spasms

Phase III
www.marathonpharma.com

Onfi®
clobazam

Lundbeck
Deerfield, IL

Dravet syndrome (pediatric)

Phase III
www.lundbeck.com

Plumiaz™
diazepam nasal spray
ORPHAN DRUG

Acorda Therapeutics
Ardsley, NY

cluster seizures

Phase III
www.acorda.com

Epilepsy

Product Name

Sponsor

Indication

Development Phase

SAGE-547
(GABA_A synaptic and extra-synaptic
receptor modulator)
ORPHAN DRUG

SAGE Therapeutics
Cambridge, MA

super refractory status epilepticus
(Fast Track)

Phase III
www.sagerx.com

essential tremors

Phase II
www.sagerx.com

topiramate IV
ORPHAN DRUG

CURx Pharmaceuticals
San Diego, CA

epilepsy in patients unable to take
oral therapy

Phase I completed
www.curxpharma.com

USL261
(intranasal midazolam)
ORPHAN DRUG

Upsher-Smith Laboratories
Maple Grove, MN

seizure clusters (Fast Track)

Phase III
www.upsher-smith.com

Vimpat®
lacosamide

UCB
Smyrna, GA

partial onset seizures (pediatric)

Phase III
www.ucb.com

primary generalized tonic clonic
seizures

Phase II
www.ucb.com

YKP3089

SK Life Science
Fair Lawn, NJ

partial onset seizures

Phase II
www.skbp.com

ZX008
(serotonin receptor agonist)
ORPHAN DRUG

Zogenix
San Diego, CA

Dravet syndrome (pediatric)

Phase II completed
www.skbp.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABT-RTA 408 (NF/E2 related factor 2 stimulant)	AbbVie <i>North Chicago, IL</i> Reata Pharmaceuticals <i>Irving, TX</i>	Friedrich's ataxia (see also other)	Phase II www.abbvie.com www.reatapharma.com
Actimmune® interferon gamma-1b ORPHAN DRUG	Horizon Pharma <i>Deerfield, IL</i>	Friedreich's ataxia (Fast Track)	Phase II www.horizonpharma.com
BMN 190 (rhTPP1 enzyme replacement therapy) ORPHAN DRUG	BioMarin Pharmaceutical <i>San Rafael, CA</i>	late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease	Phase I/II www.bmrn.com
BVS857	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	spinal and bulbar muscular atrophy	Phase II www.novartis.com
EPI-743 (vatiquinone) ORPHAN DRUG	Edison Pharmaceuticals <i>Mountain View, CA</i>	Friedrich's ataxia (Fast Track), Leigh syndrome, Rett syndrome	Phase II www.edisonpharma.com
EryDex dexamethasone erythrocyte (encapsulated)	EryDel <i>Urbino, Italy</i>	ataxia telangiectasia (see also muscular dystrophy)	Phase I www.erydel.com
Ferriprox® deferiprone	ApoPharma <i>Toronto, Canada</i>	pantothenate kinase-associated neurodegeneration	Phase III www.apopharma.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ISIS-SMNRx (antisense) ORPHAN DRUG	Biogen <i>Cambridge, MA</i> Isis Pharmaceuticals <i>Carlsbad, CA</i>	spinal muscular atrophy (Fast Track) (pediatric)	Phase III www.biogen.com www.isispharm.com
ISIS-TTRRx (antisense) ORPHAN DRUG	GlaxoSmithKline <i>Research Triangle Park, NC</i> Isis Pharmaceuticals <i>Carlsbad, CA</i>	familial amyloid polyneuropathy (Fast Track)	Phase III www.gsk.com www.isispharm.com
Lenti-D™ adrenoleucodystrophy gene therapy ORPHAN DRUG	Bluebird Bio <i>Cambridge, MA</i>	cerebral adrenoleucodystrophy (pediatric)	Phase II/III www.bluebirdbio.com
NLX-101 (serotonin 1A receptor agonist) ORPHAN DRUG	Neurolix <i>Dana Point, CA</i>	Rett syndrome	Phase I www.neurolix.com
NNZ-2256 (cytokine inhibitors/neuropeptide receptor modulator) ORPHAN DRUG	Neuren Pharmaceuticals <i>Bethesda, MD</i>	Rett syndrome (Fast Track) (see also brain injury)	Phase II www.neurenpharma.com
patisiran (ALN-TTRO2) (siRNA inhibitor targeting TTR)	Alnylam Pharmaceuticals <i>Cambridge, MA</i> Genzyme <i>Cambridge, MA</i>	familial amyloid polyneuropathy (Fast Track)	Phase III www.alnylam.com www.genzyme.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RE-024 (phosphopantothenate replacement) ORPHAN DRUG	Retrophin <i>San Diego, CA</i>	pantothenate kinase-associated neurodegeneration (Fast Track)	Phase III www.retrophin.com
RG3039 (DcpS inhibitor) ORPHAN DRUG	Repligen <i>Waltham, MA</i>	spinal muscular atrophy (Fast Track)	Phase I www.repligen.com
RG7800 (splicing of the SMN2 gene modifier)	PTC Therapeutics <i>South Plainfield, NJ</i> Roche <i>New York, NY</i>	spinal muscular atrophy	Phase I www.ptcbio.com www.roche.com
RP103 (cysteamine bitartrate delayed release)	Raptor Pharmaceuticals <i>Novato, CA</i>	Leigh syndrome and other mitochondrial diseases (see also Huntington's)	Phase II www.raptorpharma.com
RT001 (fatty acid replacement)	Retrotope <i>Los Altos, CA</i>	Friedreich's ataxia	Phase I/II www.retrotope.com
SHP622 (indolepropionic acid)	Shire ViroPharma <i>Exton, PA</i>	Friedreich's ataxia	Phase I www.shire.com
tafamidis meglumine (transthyretin dissociation inhibitor) ORPHAN DRUG	Pfizer <i>New York, NY</i>	transthyretin familial amyloid polyneuropathy (Fast Track)	application submitted www.pfizer.com

Genetic Disorders

Product Name

Sponsor

Indication

Development Phase

WTX101
(bis-choline tetrathiomolybdate)
ORPHAN DRUG

Wilson Therapeutics
Burlington, MA

Wilson's disease
(hepatolenticular degeneration)

Phase II
www.wilsontherapeutics.com

Headache

Product Name

Sponsor

Indication

Development Phase

ALD403
(anti-CGRP mAb)

Alder Biopharmaceuticals
Bothell, WA

migraine prevention

Phase II
www.alderbio.com

AMG 301
(mAb)

Amgen
Thousand Oaks, CA

migraine

Phase I
www.amgen.com

AMG 334
(CGRP receptor antagonist mAb)

Amgen
Thousand Oaks, CA

migraine prevention

Phase II
www.amgen.com

AVP-825
(sumatriptan intranasal)

Avanir Pharmaceuticals
Aliso Viejo, CA

migraine

application submitted
www.avanir.com

AVP-923
(dextromethorphan/quinidine
fixed-dose combination)

Avanir Pharmaceuticals
Aliso Viejo, CA

migraine
(see also Alzheimer's, Parkinson's)

Phase II
www.avanir.com

Botox®
onabotulinumtoxinA

Allergan
Fort Worth, TX

chronic migraine prevention
(adolescents)
(see also pain, spasticity)

Phase III
www.allergan.com

Headache

Product Name

Sponsor

Indication

Development Phase

DFN-02	Dr. Reddy's Laboratories <i>Telangana, India</i>	acute migraine	Phase III www.drreddys.com
DFN-11 (drug-device combination)	Promius Pharma <i>Princeton, NJ</i>	acute migraine	application submitted www.promiuspharma.com
ELS-M11 (topical treatment)	Achelios Therapeutics <i>Chapel Hill, NC</i>	migraine	Phase I/II www.achelios.com
IPX232	Impax Laboratories <i>Hayward, CA</i>	migraine	Phase I completed www.impaxlabs.com
lasmiditan (serotonin-1F receptor agonist)	CoLucid Pharmaceuticals <i>Cambridge, MA</i>	migraine	Phase III www.colucid.com
LY2951742 (CGRP mAb)	Eli Lilly <i>Indianapolis, IN</i>	chronic cluster headache (see also pain)	Phase III www.lilly.com
		migraine	Phase II www.lilly.com
NXN-188 (nNOS inhibitor/5HT)	NeurAxon <i>Mississauga, Canada</i>	acute migraine	Phase II www.neuraxon.com

Headache

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Rizaport™ rizatriptan oral film	IntelGenx <i>Saint Laurent, Canada</i> RedHill Biopharma <i>Tel Aviv, Israel</i>	migraine	application submitted www.intelgenx.com www.redhillbio.com
Semprana™ dihydroergotamine	Allergan <i>Ft. Worth, TX</i>	acute migraine (adults)	application submitted www.allergan.com
SUD-001 (sumatriptan oral spray)	SUDA <i>Osborne Park, Australia</i>	migraine	Phase II completed www.sudaltd.com.au
TEV-48125 (CGRP receptor antagonist)	Teva Pharmaceuticals <i>North Wales, PA</i>	chronic and episodic migraine	Phase II www.tevapharm.com
TI-001 (oxytocin intranasal)	Trigemina <i>Moraga, CA</i>	high frequency migraine	Phase II www.trigemina.com
TNX-201 (cyclobenzaprine very low dose)	Tonix Pharmaceuticals <i>New York, NY</i>	episodic tension-type headache	Phase I www.tonixpharma.com
VVD-101 (aspirin/sumatriptan fixed-dose combination)	Vivid Pharma <i>Springfield, MO</i>	delayed alcohol-induced headache	Phase II

Huntington's Disease

Product Name

Sponsor

Indication

Development Phase

laquinimod

Teva Pharmaceuticals
North Wales, PA

Huntington's disease
(see also multiple sclerosis)

Phase II
www.tevapharm.com

MP-112

Marathon Pharmaceuticals
Northbrook, IL

Huntington's disease

Phase III
www.marathonpharma.com

OMS824
(PDE10 inhibitor)
ORPHAN DRUG

Omeros
Seattle, WA

Huntington's disease (Fast Track)

Phase II
www.omeros.com

PBT2
(metal protein-attenuating compound)
ORPHAN DRUG

Prana Biotechnology
Parkville, Australia

Huntington's disease

Phase II completed
www.pranabio.com

PF-02545920
(PDE10 inhibitor)
ORPHAN DRUG

Pfizer
New York, NY

Huntington's disease

Phase II
www.pfizer.com

pridopidine
ORPHAN DRUG

Teva Pharmaceuticals
North Wales, PA

Huntington's disease

Phase II/III
www.tevapharm.com

RP103
(cysteamine bitartrate
delayed release)
ORPHAN DRUG

Raptor Pharmaceuticals
Novato, CA

Huntington's disease
(see also genetic)

Phase III
www.raptorpharma.com

Huntington's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SD-809 (VMAT2 inhibitor) ORPHAN DRUG	Auspex Pharmaceuticals <i>La Jolla, CA</i>	chorea associated with Huntington's disease (see also Tourette's, other)	Phase III www.auspexpharma.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
adipose-derived mesenchymal stem cell therapy	American CryoStem <i>Eatontown, NJ</i>	multiple sclerosis	Phase I www.americancryostem.com
ALKS 8700 (monomethyl fumarate prodrug)	Alkermes <i>Waltham, MA</i>	multiple sclerosis	Phase I www.alkermes.com
anti-LINGO-1 (BIIB033)	Biogen <i>Cambridge, MA</i>	multiple sclerosis	Phase II www.biogen.com
ATX-MS-1467 (immune tolerizing agent)	EMD Serono <i>Rockland, MA</i>	multiple sclerosis	Phase I www.emdserono.com
AX-024 (Nck protein modulator)	Artax Biopharma <i>Cambridge, MA</i>	multiple sclerosis	Phase I www.artaxbiopharma.com
BAF312 (S1PR-1/5 receptor agonist)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	secondary-progressive multiple sclerosis	Phase III www.novartis.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BIIB061 (oral re-myelination)	Biogen <i>Cambridge, MA</i>	multiple sclerosis	Phase I www.biogen.com
daclizumab high-yield process (human IgG1 mAb)	AbbVie <i>North Chicago, IL</i> Biogen <i>Cambridge, MA</i>	relapsing-remitting multiple sclerosis	application submitted www.abbvie.com www.biogen.com
Gilenya [®] fingolimod	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	multiple sclerosis (pediatric) (see also other)	Phase III www.novartis.com
GZ402668 (anti-CD52 mAb)	Genzyme <i>Cambridge, MA</i>	relapsing multiple sclerosis	Phase I www.genzyme.com
idebenone	Santhera Pharmaceuticals <i>Liestal, Switzerland</i> National Institute of Neurological Disorders and Stroke <i>Bethesda, MD</i>	primary-progressive multiple sclerosis (see also muscular dystrophy)	Phase II www.santhera.com
INT131 (PPAR-gamma agonist/modulator)	InteKrin Therapeutics <i>Redwood City, CA</i>	multiple sclerosis	Phase II www.intekrin.com

Multiple Sclerosis

Product Name

Sponsor

Indication

Development Phase

laquinimod	Teva Pharmaceuticals <i>North Wales, PA</i>	relapsing-remitting multiple sclerosis (Fast Track) (see also Huntington's)	Phase III www.tevapharm.com
		progressive multiple sclerosis (Fast Track)	Phase II www.tevapharm.com
masitinib (tyrosine kinase inhibitor)	AB Science USA <i>Chatham, NJ</i>	progressive multiple sclerosis	Phase II/III www.ab-science.com
MEDI-551 (anti-CD19 mAb)	MedImmune <i>Gaithersburg, MD</i>	multiple sclerosis (see also other)	Phase I www.medimmune.com
MN-166 (ibudilast)	MediciNova <i>La Jolla, CA</i>	progressive multiple sclerosis (see also ALS)	Phase II www.medicinova.com
MRF-008 (guanabenz)	Myelin Repair Foundation <i>Saratoga, CA</i> National Institute of Neurological Disorders and Stroke <i>Bethesda, MD</i>	relapsing-remitting multiple sclerosis	Phase I www.myelinrepair.org
NeuroVax™ immunostimulant vaccine ORPHAN DRUG	Immune Response BioPharma <i>Atlantic City, NJ</i>	secondary-progressive multiple sclerosis (Fast Track)	Phase II www.immuneresponsebiopharma.com

Multiple Sclerosis

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ocrelizumab (anti-CD20 mAb)	Genentech <i>South San Francisco, CA</i>	primary-progressive multiple sclerosis, relapsing multiple sclerosis	Phase III www.gene.com
ofatumumab (CD20 mAb)	GlaxoSmithKline <i>Research Triangle Park, NC</i>	multiple sclerosis (see also other)	Phase II www.gsk.com
PF530 (interferon beta-1b biosimilar)	Pfenex <i>San Diego, CA</i>	multiple sclerosis	Phase I www.pfenex.com
PF-06342674 (IL-7 receptor inhibitor)	Pfizer <i>New York, NY</i>	multiple sclerosis	Phase I www.pfizer.com
ponesimod (S1P1 receptor agonist)	Actelion Pharmaceuticals <i>South San Francisco, CA</i>	relapsing multiple sclerosis	Phase II www.actelion.com
rHlgM22 (myelin protein stimulant)	Acorda Therapeutics <i>Ardsley, NY</i>	multiple sclerosis	Phase I www.acorda.com
RPC1063 (S1P1 agonist)	Receptos <i>San Diego, CA</i>	relapsing multiple sclerosis	Phase III www.receptos.com
RNS60 (charge-stabilized nanostructure saline product)	Revalesio <i>Tacoma, WA</i>	relapsing-remitting multiple sclerosis	Phase II www.revalesio.com

Multiple Sclerosis

Product Name

Sponsor

Indication

Development Phase

Tcelna®
imilecleucel-T

Opexa Therapeutics
The Woodlands, TX

secondary-progressive multiple
sclerosis (Fast Track)

Phase II
www.opexatherapeutics.com

Trimesta™
estriol succinate

Synthetic Biologics
Rockville, MD

relapsing-remitting multiple sclerosis

Phase II
www.syntheticbiologics.com

Tysarbi®
natalizumab

Biogen
Cambridge, MA

secondary-progressive multiple
sclerosis
(see also stroke)

Phase III
www.biogen.com

vatelizumab
(anti-VLA 2 mAb)

Genzyme
Cambridge, MA

multiple sclerosis

Phase II
www.genzyme.com

VAY736
(BAFF-R modulator)

Novartis Pharmaceuticals
East Hanover, NJ

relapsing-remitting multiple sclerosis

Phase II
www.novartis.com

VX15
(CD100 antigen inhibitor)

Vaccinex
Rochester, NY

multiple sclerosis

Phase I
www.vaccinex.com

XP23829
(NF-kappa B inhibitor)

XenoPort
Santa Clara, CA

relapsing multiple sclerosis

Phase I
www.xenoport.com

Muscular Dystrophy

Product Name

Sponsor

Indication

Development Phase

AAV1-FS344
(gene therapy-delivered
myostatin inhibitor)
ORPHAN DRUG

Milo Biotechnology
Cleveland, OH

Becker muscular dystrophy
(see also other)

Phase II/III
www.milobiotechnology.com

Duchenne muscular dystrophy

Phase I/II
www.milobiotechnology.com

ACE-083
(recombinant fusion protein)

Acceleron Pharma
Cambridge, MA

muscular dystrophy
(see also other)

Phase I
www.acceleronpharma.com

ATYR1940
(histidine-tRNA ligase modulator)
ORPHAN DRUG

aTyr Pharma
San Diego, CA

facioscapulohumeral muscular
dystrophy

Phase I/II
www.atypharma.com

BMN 044
(antisense RNA modulator)
ORPHAN DRUG

BioMarin Pharmaceutical
San Rafael, CA

Duchenne muscular dystrophy
(EXON 44)

Phase II
www.bmrn.com

BMN 045
(antisense RNA modulator)
ORPHAN DRUG

BioMarin Pharmaceutical
San Rafael, CA

Duchenne muscular dystrophy
(EXON 45)

Phase II
www.bmrn.com

Carmeseal MD™
P-188 NF
ORPHAN DRUG

Phrixus Pharmaceuticals
Ann Arbor, MI

Duchenne muscular dystrophy

Phase II
www.phrixuspharmaceuticals.com

Muscular Dystrophy

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
drisapersen (RNA interference) ORPHAN DRUG	BioMarin Pharmaceutical <i>San Rafael, CA</i>	Duchenne muscular dystrophy (EXON 51) (Fast Track)	application submitted www.bmrn.com
EryDex dexamethasone erythrocyte (encapsulated)	EryDel <i>Urbino, Italy</i>	Duchenne muscular dystrophy (see also genetic)	Phase I www.erydel.com
eteplirsen ORPHAN DRUG	Sarepta Therapeutics <i>Cambridge, MA</i>	Duchenne muscular dystrophy (EXON 51) (Fast Track)	Phase III www.sarepta.com
HT-100 (halofuginone) ORPHAN DRUG	Akashi Therapeutics <i>Cambridge, MA</i>	Duchenne muscular dystrophy (Fast Track)	Phase II www.akashirx.com
idebenone ORPHAN DRUG	Santhera Pharmaceuticals <i>Liestal, Switzerland</i>	Duchenne muscular dystrophy (Fast Track) (see also multiple sclerosis)	Phase III www.santhera.com
ISIS-DMPKRx (antisense) ORPHAN DRUG	Biogen <i>Cambridge, MA</i> Isis Pharmaceuticals <i>Carlsbad, CA</i>	myotonic dystrophy, type 1	Phase I www.biogen.com www.isispharm.com
MP-104 (steroid receptor agonist) ORPHAN DRUG	Marathon Pharmaceuticals <i>Northbrook, IL</i>	Duchenne muscular dystrophy (pediatric) (Fast Track)	Phase III www.marathonpharmaceuticals.com

Muscular Dystrophy

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
omigapil (GAPDH inhibitor) ORPHAN DRUG	Santhera Pharmaceuticals <i>Liestal, Switzerland</i>	congenital muscular dystrophy (pediatric)	Phase I www.santhera.com
PF-06252616 (myostatin inhibitor) ORPHAN DRUG	Pfizer <i>New York, NY</i>	Duchenne muscular dystrophy (Fast Track)	Phase II www.pfizer.com
SRP-4053 (genetic transcription inhibitor)	Sarepta Therapeutics <i>Cambridge, MA</i>	Duchenne muscular dystrophy (EXON 53) (pediatric)	Phase I/II www.sarepta.com
tadalafil	Eli Lilly <i>Indianapolis, IN</i>	Duchenne muscular dystrophy (pediatric)	Phase III www.lilly.com
Translarna™ ataluren ORPHAN DRUG	PTC Therapeutics <i>South Plainfield, NJ</i>	Duchenne muscular dystrophy (ages 5 years and older) (Fast Track)	application submitted www.ptcbio.com
VBP15 (glucocorticoid receptor agonist)	ReveraGen BioPharma <i>Silver Spring, MD</i>	Duchenne muscular dystrophy	Phase I www.reveragen.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB-001 (topical patch)	Frontier Biotechnologies <i>Nanjing, China</i>	chronic low back pain	Phase II www.frontierbiotech.com

Pain			
<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AF 219 (P2X3 purinoreceptor antagonist)	Afferent Pharmaceuticals <i>San Mateo, CA</i>	osteoarthritis pain	Phase II completed www.afferentpharma.com
AGN-214868 (SNARE protein antagonist)	Allergan <i>Fort Worth, TX</i>	postherpetic neuralgia	Phase II www.allergan.com
ALO-02 (oxycodone-naltrexone core) (abuse resistant)	Pfizer <i>New York, NY</i>	severe pain	application submitted www.pfizer.com
Amiket™ amitriptyline/ketamine	Immune Pharmaceuticals <i>New York, NY</i>	neuropathic pain (Fast Track)	Phase II www.immunepharmaceuticals.com
Ampligen® rintatolimod ORPHAN DRUG	Hemispherx Biopharma <i>Philadelphia, PA</i>	chronic fatigue syndrome	application submitted www.hemisphrx.net
APD371 (cannabinoid receptor CB2 agonist)	Arena Pharmaceuticals <i>San Diego, CA</i>	chronic pain	Phase I www.arenapharm.com
ARA 290 (non-erythropoietic peptide) ORPHAN DRUG	Araim Pharmaceuticals <i>Tarrytown, NY</i>	neuropathic pain in sarcoidosis (Fast Track)	Phase II www.araimpharma.com
ATx08-001 (PPAR-gamma agonist)	Aestus Therapeutics <i>East Windsor, NJ</i>	postherpetic neuralgia	Phase II completed www.aestustherapeutics.com

Pain <u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ATx09-002 (glycogen phosphorylase inhibitor)	Aestus Therapeutics <i>East Windsor, NJ</i>	lower back pain	Phase I completed www.aestustherapeutics.com
AV-101 (NMDA receptor antagonist)	VistaGen Therapeutics <i>South San Francisco, CA</i>	neuropathic pain	Phase I completed www.vistagen.com
AXS-02 (zoledronic acid) ORPHAN DRUG	Axsome Therapeutics <i>New York, NY</i>	complex regional pain syndrome (Fast Track)	in clinical trials www.axsome.com
AYX1 (early growth response protein 1 inhibitor)	Adynxx <i>San Francisco, CA</i>	prevention of the transition to persistent or chronic pain (Fast Track)	Phase II www.adynxx.com
AZD5213 (histamine-3 receptor antagonist)	AstraZeneca <i>Wilmington, DE</i>	neuropathic pain (see also Tourette's)	Phase II www.astrazeneca.com
Belbuca™ buprenorphine buccal film	BioDelivery Sciences <i>Raleigh, NC</i> Endo Pharmaceuticals <i>Malvern, PA</i>	moderate to severe chronic pain	application submitted www.bdsi.com www.endo.com
BI 1026706 (oral analgesic)	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	osteoarthritis pain	Phase I www.boehringer-ingelheim.com
Botox® onabotulinumtoxinA	Allergan <i>Fort Worth, TX</i>	osteoarthritis pain (see also headache, spasticity)	Phase II www.allergan.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BuTab ER enteric-coated buprenorphine	Relmada Therapeutics <i>New York, NY</i>	chronic pain	Phase I www.relamada.com
Butrans® Transdermal System CIII buprenorphine transdermal system	Purdue Pharma <i>Stamford, CT</i>	severe chronic pain (ages 7 - 16 years)	Phase III www.purduepharma.com
C746 (cytokine inhibitor)	Cosmederm Bioscience <i>San Diego, CA</i>	postherpetic neuralgia	Phase I www.cosmederm.com
cebranopadol	Actavis <i>Parsippany, NJ</i> Grünenthal <i>Aachen, Germany</i>	chronic nociceptive pain, neuropathic pain	Phase II www.actavis.com www.grunenthal.com
Celebrex® celecoxib	Pfizer <i>New York, NY</i>	chronic pain	application submitted www.pfzier.com
CEP-33237 (hydrocodone extended release) (abuse resistant)	Teva Pharmaceuticals <i>North Wales, PA</i>	chronic pain	application submitted www.tevapharm.com
clonidine topical gel	BioDelivery Sciences International <i>Raleigh, NC</i>	painful diabetic neuropathy (Fast Track)	Phase III www.bdsi.com
CMX-020 oral (arachidonic acid analog)	Cmxtwenty <i>Milwaukee, WI</i>	neuropathic pain	Phase I www.cmxtwenty.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CNTX-4975 (synthetic capsaicin)	Centrexion <i>Baltimore, MD</i>	painful intermetatarsal neuroma (Morton's neuroma)	Phase II www.centrexion.com
CNV1014802 (Nav1.7 sodium channel inhibitor) ORPHAN DRUG	Biogen <i>Cambridge, MA</i>	trigeminal neuralgia	Phase II www.biogen.com
COL-172 (oxymorphone extended release) (abuse resistant)	Collegium Pharmaceutical <i>Canton, MA</i>	pain (Fast Track)	Phase I www.collegiumpharma.com
CR845 (opioid kappa receptor agonist)	Cara Therapeutics <i>Shelton, CT</i>	chronic pain	Phase II www.caratherapeutics.com
Cymbalta [®] duloxetine	Eli Lilly <i>Indianapolis, IN</i>	fibromyalgia (adolescents)	Phase III www.lilly.com
DA-9801 (nerve growth factor stimulant)	Dong-A ST <i>Seoul, South Korea</i>	painful diabetic neuropathy	Phase II completed www.en.donga-st.com
d-methadone (REL-1017)	Relmada Therapeutics <i>New York, NY</i>	painful diabetic neuropathy	Phase I/II www.relmada.com
Dex-SL (sublingual) (dexmedetomidine)	RecroPharma <i>Malvern, PA</i>	chronic pain	Phase II www.recropharma.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
DS-1971	Daiichi Sankyo <i>Parsippany, NJ</i>	chronic pain	Phase I www.dsi.com
DSP-2230 (voltage-gated sodium channel inhibitor)	Sunovion Pharmaceuticals <i>Marlborough, MA</i>	neuropathic pain	Phase I www.sunovion.com
Egalet-001 (morphine extended release) (abuse resistant)	Egalet <i>Malvern, PA</i>	chronic lower back pain (Fast Track)	Phase III www.egalet.com
Egalet-002 (oxycodone extended release) (abuse resistant)	Egalet <i>Malvern, PA</i>	chronic pain	Phase III www.egalet.com
Eladur® bupivacaine transdermal ORPHAN DRUG	Durect <i>Cupertino, CA</i> Impax Laboratories <i>Hayward, CA</i>	postherpetic neuralgia	Phase II www.durect.com www.impaxlabs.com
ELI-200 (opioid/naltrexone) (abuse resistant)	Elite Pharmaceuticals <i>Northvale, NJ</i>	chronic pain	Phase I www.elitepharma.com
ELI-201 (oxycodone/naltrexone) (abuse resistant)	Elite Pharmaceuticals <i>Northvale, NJ</i>	chronic pain	Phase I www.elitepharma.com

Pain			
<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ELI-202 (opioid/naltrexone) (abuse resistant)	Elite Pharmaceuticals <i>Northvale, NJ</i>	chronic pain	Phase I www.elitepharma.com
EMA401 (angiotensin type 2 receptor antagonist)	Spinifex Pharmaceuticals <i>Preston, Australia</i>	postherpetic neuralgia	Phase I www.spinifexpharma.com
fulranumab (nerve growth factor inhibitor)	Janssen Research & Development <i>Raritan, NJ</i>	osteoarthritis pain	Phase III www.janssenrnd.com
FX005 (sustained release p38 inhibitor)	Flexion Therapeutics <i>Burlington, MA</i>	end-stage osteoarthritis pain	Phase II www.flexiontherapeutis.com
FX006 (sustained release steroid)	Flexion Therapeutics <i>Burlington, MA</i>	moderate to severe osteoarthritis pain	Phase III www.flexiontherapeutis.com
GDC-0276 (Nav1.7 inhibitor)	Genentech <i>South San Francisco, CA</i> Xenon Pharmaceuticals <i>Burnaby, Canada</i>	chronic pain	Phase I www.gene.com www.xenon-pharma.com
GLYX-13 (rapastinel)	Naurex <i>Evanston, IL</i>	neuropathic pain	Phase II www.naurex.com
HLT Patch (lidocaine/tetracaine topical patch)	Nuvo Research <i>Ontario, Canada</i>	musculoskeletal pain	Phase II www.nuvoresearch.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
hydromorphone (intrathecal administration)	Mallinckrodt Pharmaceuticals <i>St. Louis, MO</i>	chronic pain	Phase III www.mallinckrodt.com
IMC-1 (celecoxib/famciclovir combination)	Innovative Med Concepts <i>Tuscaloosa, AL</i>	fibromyalgia	Phase II completed www.innovativemedconcepts.com
KIT-301 (naproxen/antihypertensive combination)	Kitov Pharmaceuticals <i>Washington, DC</i>	osteoarthritis pain	Phase II completed www.kitovpharma.com
KRN5500 (protein synthesis inhibitor) ORPHAN DRUG	DARA BioSciences <i>Raleigh, NC</i>	painful chronic chemotherapy- induced peripheral neuropathy (Fast Track)	Phase II www.darabio.com
LevoCap ER levorphanol	Relmada Therapeutics <i>New York, NY</i>	chronic pain	Phase II www.relamada.com
LY2951742 (CGRP mAb)	Eli Lilly <i>Indianapolis, IN</i>	osteoarthritis pain (see also headache)	Phase II www.lilly.com
Lyrica® pregabalin	Pfizer <i>New York, NY</i>	peripheral neuropathic pain (see also epilepsy)	Phase III www.pfizer.com
Lyrica® CR pregabalin controlled release	Pfizer <i>New York, NY</i>	fibromyalgia, postherpetic neuralgia	Phase III www.pfizer.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MDT-15 pellets	Medtronic Spinal and Biologics <i>Minneapolis, MN</i>	pain associated with lumbosacral radiculopathy and sciatica	Phase II www.medtronic.com
mGlu2/3 receptor agonist	Eli Lilly <i>Indianapolis, IN</i>	chronic pain	Phase I www.lilly.com
mirogabalin (CACNA2D1 protein modulator)	Daiichi Sankyo <i>Parsippany, NJ</i>	fibromyalgia	Phase III www.daiichisankyo.com
neridronic acid (bone resorption factor inhibitor)	Grünenthal <i>Aachen, Germany</i>	complex regional pain syndrome	Phase II/III www.grunenthal.com
neublastin (BG00010)	Biogen <i>Cambridge, MA</i>	neuropathic pain	Phase II www.biogen.com
NKTR-171 (sodium channel antagonist)	Nektar Therapeutics <i>San Francisco, CA</i>	neuropathic pain	Phase I www.nektar.com
NKTR-181 (mu-opioid analgesic)	Nektar Therapeutics <i>San Francisco, CA</i>	chronic pain (Fast Track)	Phase III www.nektar.com
NXN-462 (nNOS inhibitor)	NeurAxon <i>Mississauga, Canada</i>	neuropathic pain	Phase I completed www.neuraxon.com
O-desmethyltramadol	Syntrix Biosystems <i>Auburn, WA</i>	chronic pain	Phase II www.syntribio.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OLT1177 (topical gel)	Olatec Industries <i>Rye Brook, NY</i>	osteoarthritis pain	Phase II
oxycodone controlled-release tablets (tamper resistant)	Purdue Pharma <i>Stamford, CT</i>	chronic pain (adolescents)	Phase III completed www.purduepharma.com
palmitoylethanolamide	Prismic Pharmaceuticals <i>Scottsdale, AZ</i>	severe chronic pain	in clinical trials www.primicpharma.com
PF329 (hydromorphone ER) (abuse resistant)	Signature Therapeutics <i>Palo Alto, CA</i>	chronic pain	Phase I www.signaturerx.com
PF-05089771 (Nav 1.7 modulator)	Pfizer <i>New York, NY</i>	chronic neuropathic pain	Phase II www.pfizer.com
PF-06372865 (GABA A agonist)	Pfizer <i>New York, NY</i>	chronic pain	Phase II www.pfizer.com
PTI-202 (hydromorphone controlled release) (abuse resistant)	Durect <i>Cupertino, CA</i> Pain Therapeutics <i>Austin, TX</i>	moderate to severe chronic pain	Phase I www.durect.com www.paintrials.com
Remoxy [®] oxycodone extended release	Durect <i>Cupertino, CA</i> Pain Therapeutics <i>Austin, TX</i>	moderate to severe chronic pain	Phase III www.durect.com www.paintrials.com

Pain <u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
resiniferatoxin (TRPV-1 receptor agonist) ORPHAN DRUG	Sorrento Therapeutics <i>San Diego, CA</i>	intractable cancer pain	Phase I/II www.sorrentotherapeutics.com
rexlemestrocel-L (cell replacement)	Mesoblast <i>New York, NY</i>	pain associated with moderate disc degeneration	Phase II www.mesoblast.com
S-718632 (hydrocodone extended release) (abuse resistant)	Egalet <i>San Diego, CA</i> Shionogi <i>Otsuka, Japan</i>	chronic pain	Phase I www.egalet.com www.shionogi.co.jp
Sativex® nabiximols	GW Pharmaceuticals <i>Cambridge, United Kingdom</i> Otsuka America Pharmaceutical <i>Princeton, NJ</i>	cancer pain (Fast Track) (see also spasticity)	Phase III www.gwpharm.com www.otsuka.com
SoluMatrix® Meloxicam cyclo-oxygenase 2 inhibitor	Iroko Pharmaceuticals <i>Philadelphia, PA</i>	osteoarthritis pain	application submitted www.iroko.com
SoluMatrix® Naproxen cyclo-oxygenase inhibitor	Iroko Pharmaceuticals <i>Philadelphia, PA</i>	osteoarthritis pain	Phase II www.iroko.com
SPI-205 (nerve growth factor modulator)	Spectrum Pharmaceuticals <i>Henderson, NV</i>	chemotherapy-induced neuropathy	Phase I www.sppirx.com

Pain

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
tanezumab (nerve growth factor inhibitor)	Eli Lilly <i>Indianapolis, IN</i> Pfizer <i>New York, NY</i>	osteoarthritis pain	Phase III www.lilly.com www.pfizer.com
		cancer pain	Phase II www.lilly.com www.pfizer.com
TD-9855 (norepinephrine/serotonin reuptake inhibitor)	Theravance <i>South San Francisco, CA</i>	chronic pain	Phase I www.theravance.com
tetrodotoxin (TTX) (sodium channel antagonist)	WEX Pharmaceuticals <i>Vancouver, Canada</i>	chemotherapy-induced severe neuropathic pain	Phase II www.wexpharma.com
TNX-102 SL (cyclobenzaprine very low dose)	Tonix Pharmaceuticals <i>New York, NY</i>	fibromyalgia	Phase II www.tonixpharma.com
TPH-023 (flurbiprofen tape)	Teikoku Pharma USA <i>San Jose, CA</i>	chronic low back pain	Phase II www.teikokuusa.com
TRV 734 (opioid mu receptor agonist)	Trevena <i>King of Prussia, PA</i>	moderate to severe chronic pain	Phase I www.trevenainc.com
TV-45070 topical (sodium channel inhibitor)	Teva Pharmaceuticals <i>North Wales, PA</i>	neuropathic pain, osteoarthritis pain	Phase II www.tevapharm.com

Pain			
<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
V116517	Purdue Pharma <i>Stamford, CT</i>	chronic pain	Phase II completed www.purduepharma.com
V158866 (fatty acid amide hydrolase inhibitor)	Vernalis <i>Berkshire, United Kingdom</i>	neuropathic pain due to spinal cord injury	Phase II www.vernalis.com
VBV-129	ViroBay <i>Menlo Park, CA</i>	neuropathic pain	Phase I www.virobayinc.com
VM202 (modified hepatocyte growth factor gene therapy)	ViroMed <i>Seoul, South Korea</i>	painful diabetic neuropathy (see also ALS)	Phase II www.viomed.co.kr
XTAMPZA ER™ oxycodone extended release (abuse resistant)	Collegium Pharmaceuticals <i>Canton, MA</i>	chronic low back pain (Fast Track)	application submitted www.collegiumpharma.com
Zavryl™ ibuprofen/phosphatidylcholine	PLx Pharma <i>Houston, TX</i>	osteoarthritis pain	Phase II www.plxpharma.com
ZTlido™ lidocaine transdermal patch	Scilex Pharmaceuticals <i>Malvern, PA</i>	postherpetic neuralgia	Phase I www.scilexpharma.com

Parkinson's Disease

Product Name

Sponsor

Indication

Development Phase

AAV2-GDNF

National Institute to Neurological
Disorders and Stroke
Bethesda, MD
University of California
San Francisco, CA
UniQure
Lexington, MA

Parkinson's disease

Phase I
www.unique.com

ADS-5102
(amantadine ER)
ORPHAN DRUG

Adamas Pharmaceuticals
Emeryville, CA

levodopa-induced dyskinesia in
Parkinson's disease

Phase III
www.adamapharma.com

APL-130277
(apomorphine sublingual film)

Cynapsus Therapeutics
Toronto, Canada

Parkinson's disease

Phase III
www.cynapsus.ca

Apokyn®
apomorphine for continuous
subcutaneous infusion
ORPHAN DRUG

US WorldMeds
Louisville, KY

idiopathic Parkinson's disease

Phase III
www.usworldmeds.com

AQW051
(alpha7 nicotine acetylcholine
receptor partial agonist)

Vanda Pharmaceuticals
Washington, DC

levodopa-induced dyskinesia in
Parkinson's disease

Phase II completed
www.vandapharmaceuticals.com

autologous stem cell therapy

NeuroGeneration
Los Angeles, CA

Parkinson's disease

Phase I completed
www.neurogeneration.com

Parkinson's Disease

Product Name

Sponsor

Indication

Development Phase

AVP-923
(dextromethorphan/quinidine
fixed-dose combination)

Avanir Pharmaceuticals
San Diego, CA

levodopa-induced dyskinesia in
Parkinson's disease
(see also Alzheimer's, headache)

Phase II
www.avanir.com

BIIB054
(alpha-synuclein modulator)

Biogen
Cambridge, MA

Parkinson's disease

Phase I
www.biogen.com

CVT-301
(levodopa inhalation)

Acorda Therapeutics
Ardsley, NY

Parkinson's disease

Phase III
www.acorda.com

D1 potentiator

Eli Lilly
Indianapolis, IN

Parkinson's disease

Phase I
www.lilly.com

dipraglurant-IR

Addex Therapeutics
Geneva, Switzerland

levodopa-induced dyskinesia in
Parkinson's disease

Phase II
www.addextherapeutics.com

eltoprazine
(serotonin 1A/1B partial agonist)

Amarantus BioScience
San Francisco, CA

levodopa-induced dyskinesia in
Parkinson's disease

Phase II
www.amarantus.com

fipamezole

Santhera Pharmaceuticals
Liestal, Switzerland

Parkinson's disease (Fast Track)

Phase II
www.santhera.com

GM6
(peptide therapeutic)

Genervon Biopharmaceuticals
Pasadena, CA

Parkinson's disease
(see also ALS, stroke)

Phase II
www.genervon.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IPX203 (carbidopa/levodopa fixed-dose combination)	Impax Pharmaceuticals <i>Hayward, CA</i>	Parkinson's disease	Phase II www.impaxlabs.com
IPX231	Impax Pharmaceuticals <i>Hayward, CA</i>	Parkinson's disease	Phase I completed www.impaxlabs.com
IRX4204 (retinoid X receptor agonist)	Io Therapeutics <i>Santa Ana, CA</i>	early Parkinson's disease	Phase I completed www.io-therapeutics.com
KW-6002 (istradefylline)	Kyowa Hakko Kirin America <i>Princeton, NJ</i>	Parkinson's disease (adjunctive treatment)	application submitted www.kyowa-kirin.com
LY03003 (rotigotine controlled release)	Luye Pharma <i>Princeton, NJ</i>	early Parkinson's disease	Phase I www.luye.cn/en
OS-320 (amantadine ER)	Osmotica Pharmaceutical <i>Marietta, GA</i>	levodopa-induced dyskinesia in Parkinson's disease	Phase III www.osmotica.com
P2B-001 (pramipexole/rasagiline)	Phase Two B <i>Rehovot, Israel</i>	Parkinson's disease	Phase II/III www.phase2b.com
PF-06649751	Pfizer <i>New York, NY</i>	Parkinson's disease	Phase I www.pfizer.com

Parkinson's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Posiphen® R-phenserine	QR Pharma <i>Berwyn, PA</i>	Parkinson's disease (see also Alzheimer's)	Phase II www.qrpharma.com
PRX002/RG7935 (alpha-synuclein inhibitor)	Prothena Biosciences <i>South San Francisco, CA</i> Roche <i>New York, NY</i>	Parkinson's disease	Phase I www.prothena.com www.roche.com
rasagiline transdermal patch	Teikoku Pharma USA <i>San Jose, CA</i>	Parkinson's disease	Phase I completed www.teikokuusa.com
SYN120 (serotonin 2A/6 receptor antagonist)	Biotie Therapies <i>South San Francisco, CA</i>	Parkinson's disease dementia	Phase II www.biotie.com
tozadenant (adenosine A2A receptor antagonist)	Biotie Therapies <i>South San Francisco, CA</i>	Parkinson's disease	Phase II/III www.biotie.com
V 81444 (adenosine A2A receptor antagonist)	Vernalis <i>Berkshire, United Kingdom</i>	Parkinson's disease	Phase I/II completed www.vernalis.com
VY-AADC01 (gene therapy)	Voyager Therapeutics <i>Cambridge, MA</i> University of California <i>San Francisco, CA</i>	Parkinson's disease	Phase I www.voyagertherapeutics.com

Parkinson's Disease

Product Name

Sponsor

Indication

Development Phase

Xadago®
safinamide

Newron Pharmaceuticals
Bresso, Italy
Zambon Pharma
Bresso, Italy

early-stage Parkinson's disease
(add-on therapy to dopamine,
mid-to late-stage Parkinson's
disease (add-on therapy to levodopa)

application submitted
www.newron.com
www.zambonpharma.com

XP21279
(dopamine receptor agonist)

XenoPort
Santa Clara, CA

Parkinson's disease

Phase II
www.xenoport.com

Spasticity

Product Name

Sponsor

Indication

Development Phase

baclofen extended release

Sun Pharma Advanced Research
Mumbai, India

muscle spasticity in multiple sclerosis

Phase III
www.sunpharma.in

Botox®
onabotulinumtoxinA
ORPHAN DRUG

Allergan
Fort Worth, TX

lower limb spasticity (pediatric),
upper limb spasticity (pediatric
and adults)
(see also headache, pain)

Phase III
www.allergan.com

Dysport®
abobotulinumtoxinA

Ipsen Biopharmaceuticals
Basking Ridge, NJ

upper limb spasticity (adults)
(see also Tourette's)

application submitted
www.ipsenus.com

focal spasticity of the lower and
upper limbs (pediatric)

Phase III
www.ipsenus.com

OS-440
(arbaclofen extended release)

Osmotica Pharmaceutical
Marietta, GA

muscle spasticity in multiple sclerosis

Phase III
www.osmotica.com

Spasticity

Product Name

Sponsor

Indication

Development Phase

Sativex®
nabiximols

GW Pharmaceuticals
Cambridge, United Kingdom
Otsuka America Pharmaceutical
Princeton, NJ

muscle spasticity in multiple sclerosis
(see also pain)

Phase II
www.gwpharm.com
www.otsuka.com

Xeomin®
incobotulinumtoxinA

Merz Pharmaceuticals
Greensboro, NC

lower limb spasticity in patients
with cerebral palsy

Phase III
www.merzusa.com

post-stroke spasticity

Phase III
www.merzusa.com

Spinal Cord Injury

Product Name

Sponsor

Indication

Development Phase

AC105
(nervous system modulator)

Acorda Therapeutics
Ardsley, NY

spinal cord injury (Fast Track)

Phase II
www.acorda.com

AST-OPC1
(human embryonic stem cell (hESC)
derived cell therapy)

Asterias Biotherapeutics
Menlo Park, CA

spinal cord injury

Phase I/II
www.asteriasbiotherapeutics.com

BA-210
(Rho GTP-binding protein-inhibitor)
ORPHAN DRUG

BioAxone BioSciences
Cambridge, MA

acute spinal cord injury (Fast Track)

Phase II
www.bioaxonebio.com

HuCNS-SC®
adult neural stem cell therapy

StemCells
Newark, CA

cervical and thoracic spinal
cord injury

Phase II
www.stemcellsinc.com

Spinal Cord Injury

Product Name

Sponsor

Indication

Development Phase

NSI-566
(spinal cord-derived neural stem cell therapy)

Neuralstem
Germantown, MD

chronic spinal cord injury
(see also ALS, stroke)

Phase I
www.neuralstem.com

SUN13837
(fibroblast growth factor receptor)

Daiichi Sankyo
Parsippany, NJ

spinal cord injury

Phase II
www.dsi.com

Stroke

Product Name

Sponsor

Indication

Development Phase

3K3A-APC
(recombinant mutant form of human wild-type activated protein C)

ZZ Biotech
Houston, TX

acute ischemic stroke

Phase II
www.zzbiotech.com

Brilinta®
ticagrelor

AstraZeneca
Wilmington, DE

reduction of vascular events in acute ischemic stroke or transient ischemic attack

Phase III
www.astrazeneca.com

Cirara™
RP-1127

Remedy Pharmaceuticals
New York, NY

severe ischemic stroke
(see also brain injury)

Phase II
www.remedypharmaceuticals.com

CNTO 0007
(cell replacement therapy)

Janssen Research & Development
Raritan, NJ

ischemic stroke

Phase I completed
www.janssenrnd.com

dalfampridine

Acorda Therapeutics
Ardsley, NY

chronic post-stroke walking deficits

Phase III
www.acorda.com

Stroke

Product Name

Sponsor

Indication

Development Phase

Durlaza™
aspirin controlled release

New Haven Pharmaceuticals
Branford, CT

stroke prevention

application submitted
www.newhavenpharma.com

EG-1962
(nimodipine microparticles)
ORPHAN DRUG

Edge Therapeutics
Berkeley Heights, NJ

prevention of cerebral ischemia
following aneurysmal subarachnoid
hemorrhage

Phase I/II
www.edgetherapeutics.com

GM6
(peptide therapeutic)

Genervon Biopharmaceuticals
Pasadena, CA

acute ischemic stroke (Fast Track)
(see also ALS, Parkinson's)

Phase II
www.genervon.com

MP-124
(PARP inhibitor)

Mitsubishi Tanabe Pharma
Jersey City, NJ

acute ischemic stroke

Phase I
www.mt-pharma-america.com

MultiStem®
allogeneic stem cell therapy

Athersys
Cleveland, OH

ischemic stroke

Phase II
www.athersys.com

NA-1
(PDZ domain inhibitor)

NoNO
Toronto, Canada

stroke

Phase II
www.nonoinc.ca

NSI-566
(spinal cord-derived neural
stem cell therapy)

Neuralstem
Germantown, MD

ischemic stroke
(see also ALS, spinal cord injury)

Phase I/II
www.neuralstem.com

PF-05230907

Pfizer
New York, NY

intracerebral hemorrhage

Phase I
www.pfizer.com

Stroke

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ponezumab (PF-04360365) (beta amyloid inhibitor)	Pfizer <i>New York, NY</i>	cerebral amyloid angiopathy	Phase II www.pfizer.com
Pradaxa [®] dabigatran	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	stroke (secondary prevention)	Phase III www.boehringer-ingelheim.com
Sanguinate [™] PEGylated hemoglobin and carbon monoxide	Prolong Pharmaceuticals <i>South Plainfield, NJ</i>	reduction or prevention of delayed cerebral ischemia following subarachnoid hemorrhage	Phase II www.prlongpharma.com
SB-623 (cell replacement therapy)	Sunovion Pharmaceuticals <i>Marlborough, MA</i>	chronic stroke	Phase II www.sunovion.com
sodium nitrite	Hope Pharmaceuticals <i>Scottsdale, AZ</i>	reversal of cerebral vasospasm after subarachnoid hemorrhage	Phase II www.hopepharm.com
Stemedyne [™] -MSC mesenchymal stem cell therapy	Stemmedica Cell Technologies <i>San Diego, CA</i>	ischemic stroke	Phase II www.stemedica.com
Tysarbi [®] natalizumab	Biogen <i>Cambridge, MA</i>	acute ischemic stroke (see also multiple sclerosis)	Phase II www.biogen.com

Tourette's Syndrome

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AZD5213 (histamine-3 receptor antagonist)	AstraZeneca <i>Wilmington, DE</i>	Tourette's syndrome (see also pain)	Phase II www.astrazeneca.com
CPP-115 (4-aminobutyrate transaminase inhibitor)	Catalyst Pharmaceuticals <i>Coral Gables, FL</i>	Tourette's syndrome (see also epilepsy)	Phase I www.catalystpharma.com
Dysport® abobotulinumtoxinA	Ipsen Biopharmaceuticals <i>Basking Ridge, NJ</i>	Tourette's syndrome (see also spasticity)	Phase II www.ipsenus.com
ecopipam	Psydon Pharmaceuticals <i>Germantown, MD</i>	Tourette's syndrome	Phase II www.psydonrx.com
NBI-98854 (VMAT2 inhibitor)	Neurocrine Biosciences <i>San Diego, CA</i>	Tourette's syndrome (see also other)	Phase I www.neurocrine.com
SD-809 (VMAT2 inhibitor) ORPHAN DRUG	Auspex Pharmaceuticals <i>La Jolla, CA</i>	Tourette's syndrome (adolescents) (see also Huntington's, other)	Phase I www.auspexpharma.com
SNC-102 (acamprosate controlled release)	Synchroneuron <i>Duxbury, MA</i>	Tourette's syndrome (see also other)	Phase II www.synchroneuron.com
vigabatrin (CCP-109)	Catalyst Pharmaceutical Partners <i>Coral Gables, FL</i>	Tourette's syndrome	Phase I/II www.catalystpharma.com

Other

Product Name

Sponsor

Indication

Development Phase

AAV1-FS344
(gene therapy-delivered
myostatin inhibitor)

Milo Biotechnology
Cleveland, OH

inclusion body myositis
(see also muscular dystrophy)

Phase I/II
www.milobiotechnology.com

ABT-RTA 408
(NF/E2 related factor 2 stimulant)

AbbVie
North Chicago, IL
Reata Pharmaceuticals
Irving, TX

mitochondrial myopathies
(see also genetic)

Phase II
www.abbvie.com
www.reatapharma.com

ACE-083
(recombinant fusion protein)

Acceleron Pharma
Cambridge, MA

inclusion body myositis
(see also muscular dystrophy)

Phase I
www.acceleronpharma.com

anti-eTau
(BMS 986168)

Bristol-Myers Squibb
Princeton, NJ

neurodegenerative disease

Phase I
www.bms.com

AZD3241
(myeloperoxidase inhibitor)

AstraZeneca
Wilmington, DE

multiple system atrophy

Phase II
www.astrazeneca.com

Bendavia™
(mitochondrial permeability transition
pore inhibitor)

Stealth BioTherapeutics
Newton, MA

mitochondrial myopathies

Phase II
www.stealthbt.com

Benlysta®
belimumab

GlaxoSmithKline
Research Triangle Park, NC

myasthenia gravis

Phase II
www.gsk.com

BYM338
(type II-B activin receptor
modulator mAb)

Novartis Pharmaceuticals
East Hanover, NJ

sporadic inclusion body myositis

Phase II/III
www.novartis.com

Other

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Cinryze® C1 esterase inhibitor (human)	Shire ViroPharma <i>Exton, PA</i>	neuromyelitis optica	Phase I www.shire.com
CK-2127107	Astellas Pharma US <i>Northbrook, IL</i> Cytokinetics <i>South San Francisco, CA</i>	skeletal muscle function improvement associated with neuromuscular dysfunction, muscle weakness and/or muscle fatigue	Phase I completed www.cytokinetics.com
ferric carboxymaltose	Luitpold Pharmaceuticals <i>Shirley, NY</i>	restless legs syndrome	Phase III www.luitpold.com
Firdapse™ amifampridine ORPHAN DRUG	Catalyst Pharmaceuticals <i>Coral Gables, FL</i>	Lambert-Eaton myasthenia syndrome	Phase III www.catalystpharma.com
Gilenya® fingolimod	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	chronic inflammatory demyelinating polyradiculoneuropathy (see also multiple sclerosis)	Phase III www.novartis.com
HT-3951 (monoamine oxidase B inhibitor)	Dart NeuroScience <i>San Diego, CA</i>	neurodegenerative disorders	Phase I www.dartneuroscience.com
JZP-110 (neurotransmitter modulator) ORPHAN DRUG	Jazz Pharmaceuticals <i>Palo Alto, CA</i>	excessive daytime sleepiness associated with narcolepsy	Phase III www.jazzpharma.com

Other

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
JZP-386 (dopamine receptor agonist)	Concert Pharmaceuticals <i>Lexington, MA</i> Jazz Pharmaceuticals <i>Palo Alto, CA</i>	narcolepsy	Phase I completed www.concertpharma.com www.jazzpharma.com
MEDI-551 (anti-CD19 mAb)	MedImmune <i>Gaithersburg, MD</i>	neuromyelitis optica (see also multiple sclerosis)	Phase II www.medimmune.com
NBI-98854 (VMAT2 inhibitor)	Neurocrine Biosciences <i>San Diego, CA</i>	tardive dyskinesia (Fast Track) (see also Tourette's)	Phase III www.neurocrine.com
ofatumumab (CD20 mAb)	GlaxoSmithKline <i>Research Triangle Park, NC</i>	neuromyelitis optica (see also multiple sclerosis)	Phase II www.gsk.com
Privigen [®] Immune globulin	CSL Behring <i>King of Prussia, PA</i>	chronic inflammatory demyelinating polyradiculoneuropathy	Phase III www.cslbehring.com
QPI 1007 (gene expression inhibitor)	Quark Pharmaceuticals <i>Fremont, CA</i>	non-arteritic anterior ischemic optic neuropathy	Phase I www.quarkpharma.com
RGN-352 (injectable thymosin beta-4)	RegeneRx Biopharmaceuticals <i>Rockville, MD</i>	central nervous system disorders	Phase I completed www.regenerx.com
SA237 (anti-IL-6 receptor mAb)	Chugai Pharma USA <i>Berkeley Heights, NJ</i>	neuromyelitis optica	Phase III www.chugai-pharm.com

Other

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SD-809 (VMAT2 inhibitor) ORPHAN DRUG	Auspex Pharmaceuticals <i>La Jolla, CA</i>	tardive dyskinesia (see also Huntington's, Tourette's)	Phase III www.auspexpharma.com
SNC-102 (acamprosate controlled-release)	Synchroneuron <i>Duxbury, MA</i>	tardive dyskinesia (see also Tourette's)	Phase II www.synchroneuron.com
Soliris [®] eculizumab ORPHAN DRUG	Alexion Pharmaceuticals <i>Cheshire, CT</i>	myasthenia gravis, neuromyelitis optica	Phase III www.alxn.com
TAK-137 (AMPA receptor modulator)	Takeda Pharmaceutical <i>Deerfield, IL</i>	neurological disorders	Phase I www.takeda.com
TAK-935 (steroid hydroxylase inhibitor)	Takeda Pharmaceutical <i>Deerfield, IL</i>	neurological disorders	Phase I www.takeda.com
Tardoxal [™] pyridoxal phosphate	Medicure Pharma <i>Somerset, NJ</i>	tardive dyskinesia (Fast Track)	Phase II www.medicure.com
TPI 287 (abeotaxane) ORPHAN DRUG	Cortice Biosciences <i>New York, NY</i>	corticobasal dementia, progressive supranuclear palsy (see also Alzheimer's, brain tumors)	Phase I www.corticebio.com
UX001 (aceneuramic acid extended release)	Ultragenyx Pharmaceutical <i>Novato, CA</i>	hereditary inclusion body myositis	Phase I/II www.ultragenyx.com

Other

Product Name

Sponsor

Indication

Development Phase

Zyrem®
sodium oxybate

Jazz Pharmaceuticals
Palo Alto, CA

narcolepsy (pediatric)

Phase III
www.jazzpharma.com

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 fewer than 200,000 people in the United States, or that affects more than 200,000 persons but is not expected to recover the costs of its development and marketing.

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.

The content of this report has been obtained through public, government and industry sources, and the Adis “R&D Insight” database based on the latest information.

Report current as of June 15, 2015. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States and abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. 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, at www.phrma.org.