

2017 Medicines in Development for Alzheimer's Disease

Alzheimer's Disease

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABBV-8E12 (anti-tau antibody)	AbbVie North Chicago, IL	Alzheimer's disease	Phase II www.abbvie.com
AC-1204 (glucose stimulant)	Accera Broomfield, CO	mild to moderate Alzheimer's disease	Phase III www.accerapharma.com
ACI-24 (anti-Abeta vaccine)	AC Immune Lausanne, Switzerland	Alzheimer's disease in Down syndrome patients	Phase I www.acimmune.com
ACI-35 (anti-pTau vaccine)	AC Immune Lausanne, Switzerland Janssen Research & Development Raritan, NJ	mild to moderate Alzheimer's disease	Phase I www.acimmune.com www.janssen.com
aducanumab (BIIB037) (amyloid beta mAb)	Biogen Cambridge, MA Neurimmune Zurich, Switzerland	Alzheimer's disease (Fast Track)	Phase III www.biogen.com
AGB101 (levetiracetam low-dose)	AgenBio 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 (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 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	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 University of Kansas Kansas City, KS	Alzheimer's disease	Phase I/II www.ausiopharma.com

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AVN-101 (serotonin 6 receptor antagonist)	AllaChem Hallandale Beach, FL Avineuro Pharmaceuticals San Diego, CA	Alzheimer's disease	Phase I completed www.allachem.com www.avineuro.com
AVN-322 (serotonin 6 receptor antagonist)	AllaChem Hallandale Beach, FL Avineuro Pharmaceuticals San Diego, CA	Alzheimer's disease	Phase I completed www.allachem.com www.avineuro.com
AVP-786 (dextromethorphan analogue/ultra-low dose quinidine)	Avanir Pharmaceuticals Aliso Viejo, CA Concert Pharmaceuticals Lexington, MA	agitation in Alzheimer's disease (Fast Track)	Phase III www.avanir.com www.concertpharma.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

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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, prodromal Alzheimer's disease	Phase II www.boehringer-ingelheim.com
BIIB076 (anti-tau antibody)	Biogen Cambridge, MA	Alzheimer's disease	Phase I www.biogen.com
BIIB092 (anti-extracellular tau antibody)	Biogen Cambridge, MA	Alzheimer's disease	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

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CAD106 (amilomotide) (VLP immunotherapy vaccine)	Novartis East Hanover, NJ	Alzheimer's disease	Phase II/III www.novartis.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 completed www.coriumgroup.com
CPC-201 (donepezil/solifenacin combination)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase II www.chasepharmaceuticals.com
CPC-212 (next-generation acetylcholinesterase inhibitor)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase I completed www.chasepharmaceuticals.com
CPC-250 (next-generation acetylcholine- sterase inhibitor)	Allergan Parsippany, NJ Chase Pharmaceuticals Washington, DC	Alzheimer's disease	Phase I completed www.chasepharmaceuticals.com
crenezumab (anti-amyloid beta antibody)	Genentech South San Francisco, CA	Alzheimer's disease	Phase III www.gene.com

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CSP-1103 (amyloid beta-protein inhibitor)	CereSpir New York, NY	mild cognitive impairment in patients at risk for Alzheimer's disease	Phase II www.cerespir.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	Phase II www.cogrx.com
E2027 (PDE9 inhibitor)	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	Phase III www.biogen.com www.eisai.com
EVT302 (MAO-B inhibitor)	Evotec Princeton, NJ	Alzheimer's disease	Phase II completed www.evotec.com
gantenerumab (amyloid beta-protein inhibitor)	Genentech South San Francisco, CA	Alzheimer's disease	Phase III www.gene.com

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GC021109 (purinoceptor P2Y6 agonist)	GliaCure Boston, MA	Alzheimer's disease (Fast Track)	Phase I www.gliacure.com
HSRx-888 (donepezil/food-based compound)	HSRx Biopharmaceutical Tucson, AZ	Alzheimer's disease	Phase II www.hsrxbiopharmaceutical.com
immune globulin/albumin	Grifols USA Los Angeles, CA	mild to moderate Alzheimer's disease	Phase II/III www.grifols.com
INP-102 intranasal	Impel NeuroPharma Seattle, WA	Alzheimer's disease	Phase 0 www.impelnp.com
intepirdine (RVT-101) (serotonin 6 receptor antagonist)	Axovant Sciences New York, NY	mild to moderate Alzheimer's disease (adjunctive treatment)	Phase III www.axovant.com
IONIS-MAPTRx (tau-targeting protein)	Ionis Pharmaceuticals Carlsbad, CA	mild Alzheimer's disease	Phase I/II www.ionispharma.com
JNJ-54861911 (BACE inhibitor)	Janssen Research & Development Raritan, NJ	asymptomatic patients (amyloid-positive) at risk for Alzheimer's disease	Phase II/III www.janssenrnd.com
JOT106 (oral capsule of trans-resveratrol)	Jupiter Orphan Therapeutics Jupiter, FL	Alzheimer's disease	Phase II www.jupiterorphan.com

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KPAX002-2 (proprietary version of methylphenidate)	K-PAX Pharmaceuticals Mill Valley, CA	Alzheimer's disease	Phase II 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
LY3002813 (N3pG-amyloid beta antibody)	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com
LY3202626 (BACE inhibitor)	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase II www.lilly.com
LY3303560 (tau antibody)	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com

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M1 agonist (selective M1 receptor agonist)	Allergan Parsippany, NJ Heptares Therapeutics Hertfordshire, United Kingdom	cognitive impairment in Alzheimer's disease	Phase I www.allergan.com
MEDI1814 (anti-amyloid beta 42 mAb)	Eli Lilly Indianapolis, IN MedImmune Gaithersburg, MD	Alzheimer's disease	Phase I www.lilly.com www.medimmune.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	psychosis in Alzheimer's disease	Phase II www.tvm-lifescience.com
MSDC-0160 (mTOT modulator)	Metabolic Solutions Development Kalamazoo, MI	Alzheimer's disease	Phase II completed www.msdrx.com
NBXT-001+Nobilis™ inhalation device (NMDA receptor antagonist)	Nobilis Therapeutics Portland, OR	Alzheimer's disease	Phase I www.nobilistx.com
neflamapimod (VX-745) (p38 mitogen-activated protein kinase inhibitor)	EIP Pharma Cambridge, MA	Alzheimer's disease	Phase II www.eippharma.com

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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	mild to moderate Alzheimer's disease	Phase III www.archerpharma.com www.nilvad.eu
NPT088 (GAIM Ig fusion targeting amyloid- β , tau, a-synuclein)	Proclara Biosciences Cambridge, MA	Alzheimer's disease	Phase I www.proclarabio.com
Nuplazid® pimavanserin	ACADIA Pharmaceuticals San Diego, CA	psychosis and agitation in Alzheimer's disease	Phase II www.acadia-pharm.com
PF-05251749 (casein kinase 1 delta/epsilon)	Pfizer New York, NY	Alzheimer's disease	Phase I www.pfizer.com
PF-06648671 (gamma secretase complex modulator)	Pfizer New York, NY	Alzheimer's disease	Phase I www.pfizer.com
PF-06751979 (enzyme inhibitor)	Pfizer New York, NY	Alzheimer's disease	Phase I www.pfizer.com

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pioglitazone (low-dose) (PPAR γ agonist)	Takeda Pharmaceuticals Skokie, IL Zinfandel Pharmaceuticals Chapel Hill, NC	mild cognitive impairment in Alzheimer's disease	Phase III www.takeda.com
piromelatine (melatonin agonist)	Neurin Pharmaceuticals Zug, Switzerland	mild dementia in Alzheimer's disease	Phase II www.neurin.com
Posiphen [®] R-phenserine	QR Pharma Berwyn, PA	mild to moderate Alzheimer's disease	Phase I/II www.qrpharma.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 I www.gene.com
RVT-103+RVT-104 (QAAM+cholinesterase inhibitor)	Axovant Sciences New York, NY	Alzheimer's disease	Phase I www.axovant.com
SAR228810 (anti-protofibrillar AB mAb)	Sanofi US Bridgewater, NJ	Alzheimer's disease	Phase I www.sanofi.com

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selective BACE 1 inhibitor	Eli Lilly Indianapolis, IN	Alzheimer's disease	Phase I www.lilly.com
solanezumab (amyloid beta protein inhibitor)	Eli Lilly Indianapolis, IN	preclinical Alzheimer's disease	Phase III www.lilly.com
SUVN-502 (serotonin 6 receptor antagonist)	Suven Life Sciences Hyderabad, India	moderate Alzheimer's disease (adjunctive treatment)	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	cognitive disorder 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/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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TPI 287 (next-generation taxane)	Cortice Biosciences New York, NY	mild to moderate Alzheimer's disease	Phase I www.corticebiosciences.com
UB-311 (anti-amyloid endobody 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
verubecestat (MK-8931) (BACE1 protein inhibitor)	Merck Kenilworth, NJ	Alzheimer's disease	Phase III www.merck.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 August 30, 2017**. 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.

Definitions

Phase 0 – First-in-human trials conducted in accordance with FDA’s 2006 guidance on exploratory Investigational New Drug (IND) studies designed to speed up development of promising drugs by establishing very early on whether the agent behaves in human subjects as was anticipated from preclinical studies.

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.