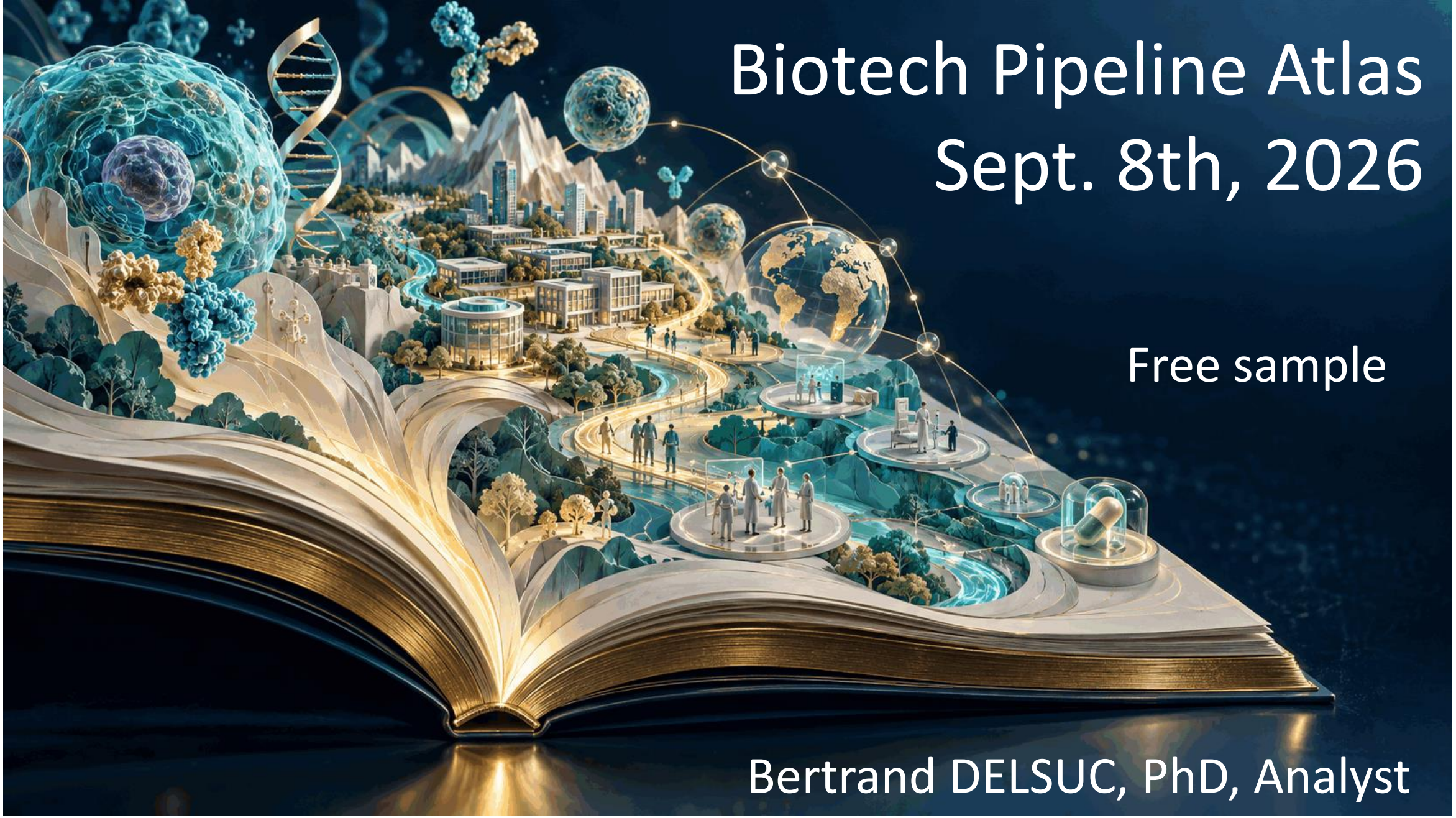

Biotellytics

Disclaimer

- The official release date of this document is September 8th, 2026
- All the companies covered in this document have been reviewed in 2026
- The data are up to date, as of September 4th, 2026, but the pipeline of many companies has been reviewed over the course of August, and sometimes earlier, thus some updates are missing
- The user should always check the accuracy of the data of this document, a 100% accuracy is not warranted
- The level of details may vary from one company to another
- There was no emphasis on the partner's list, so many partner names are probably missing
- The concise style of the notes may miss important information
- This document is delivered as is, without any further update due
- The data in this document should not be construed as reflecting the personal opinion of the author
- The author of this document is not responsible for any investment or business decision taken, based on data from this document

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Introduction

- This document covers the pipeline of 324 biopharma companies, listed in the US, some of which were acquired since the initiation of the data collection, as indicated in the slides
- The aim of this document is to quickly get a sense of what each company does, and have an idea of the status of each program
- This document does not cover all the business aspects of each company, in a comprehensive manner
- The slide order was established by market cap, some time in January 2026, along with manual additions along the way (some transitions may look weird☺)
- AI helped to compile those data, but only minimally (still, cover image credits: chatGPT). The data were mainly compiled and curated by the author!
- This document is delivered as a PPTX file, that you can easily update yourself, for your own purpose and interest – a 'companion' XLSX file is also provided, giving an example of how to use this document as an input for AI (e.g. to generate a catalyst calendar)
- You can use this document for personal use. You can even make it better! Please credit the source, if you use its data publicly.
- Please **read the disclaimer**, for other important considerations

Biogen (BIIB) - Pipeline summary 1/3

Mkt cap ($): 32.6 B
as of 4 Sep 2026
Source: Stock Analysis

+ pipeline from Apellis Pharmaceuticals acquired Mach'26

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Lecanemab (LEQEMBI)	AD	Anti-Aβ mAb	Biologic (mAb)	Marketed
Omaveloxolone (SKYCLARYS)	Friedreich ataxia	Nrf2 activator	Small molecule	Marketed
Zuranolone (ZURZUVAE)	Postpartum depression	GABA A receptor PAM	Small molecule	Marketed
Nusinersen (SPINRAZA)	Spinal muscular atrophy (SMA)	ASO increasing splicing efficiency of SMN2 pre-mRNA by promoting ex7 inclusion for full-length SMN prod.	RNA (ASO)	Marketed
Tofersen (QALSODY)	SOD1-ALS	ASO binding to SOD1 mRNA (RNase H-mediated degradation)	RNA (ASO)	Marketed
Dimethyl fumarate (TECFIDERA)	MS	Enhancer of Nrf2 transcriptional pathway	Small molecule	Marketed
Diroximel fumarate (VUMERITY)	MS	MMF produg acting as Nrf2 activator	Small molecule	Marketed
Natalizumab (TYSABRI)	MS	mAb antagonist of α4 subunit of α4β1+α4β7 integrin receptors	Biologic (mAb)	Marketed
Interferon beta-1a (AVONEX)	MS	Immunomodulator	Biologic (protein)	Marketed
Peginterferon beta-1a (PLEGRIDY)	MS	Immunomodulator	Biologic (protein)	Marketed
Ranibizumab biosimilar (BYOOVIZ)	Ophthalmology (biosimilar)	Anti-VEGF-A mAb	Biologic (mAb)	Marketed
Lecanemab SC-AI (LEQEMBI IQLIK)	Early AD (Maintenance)	Anti-Aβ mAb	Biologic (mAb)	Marketed
Lecanemab SC-AI (LEQEMBI IQLIK)	Early AD (Initiation)	Anti-Aβ mAb	Biologic (mAb)	Registration (PDUFA mid-end-May'26, EU decision Q2-Q3'26)
Nusinersen high-dose (HD SPINRAZA)	Spinal muscular atrophy (SMA)	SMN2 splice modulator	RNA (ASO)	Marketed (US approval end-March'26 [vs PDUFA early Apr'26])
Lecanemab (LEQEMBI)	Preclinical AD	Anti-Aβ mAb	Biologic (mAb)	Phase 3 (primary completion Q4'28)
Dapirolizumab pegol	SLE (PHOENYCS FLY)	Pegylated humanized Fc-free anti-CD40L Fab fragment	Biologic (mAb)	Phase 3 (primary completion mid'28, 1st ph3 PHOENYCS GO toplined +ve Sept'24 / prez @ EULAR'25 & Lancet publi Jun'26)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

BIIB pipeline

Biogen (BIIB) - Pipeline summary 2/3

Mkt cap ($): 32.6 B
as of 4 Sep 2026
Source: Stock Analysis

+ pipeline from Apellis Pharmaceuticals acquired Mach'26

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Litifilimab	SLE	Anti-BDCA2 mAb	Biologic (mAb)	Phase 3 (2x ph3 readouts by YE'26➔Q4'26)
Litifilimab	Cutaneous lupus erythematosus	BDCA2 mAb	Biologic (mAb)	Phase 3 (readout mid'27➔H1'27, add. ph2 data mid-H1'26, 52wk ph2 data @ EADV'26)
Felzartamab	AMR	Anti-CD38 mAb	Biologic (mAb)	Phase 3 (readout mid'27➔H1'27)
Felzartamab	IgA nephropathy	Anti-CD38 mAb	Biologic (mAb)	Phase 3 (primary completion mid'27)
Felzartamab	Primary membranous nephropathy	Anti-CD38 mAb	Biologic (mAb)	Phase 3 (primary completion YE'28)
Omaveloxolone (SKYCLARYS)	Friedreich ataxia (Pediatric)	Nrf2 activator	Small molecule	Phase 3 (primary completion Q4'27)
Zorevunersen (STK-001)	Dravet syndrome	ASO targeting SCN1A	RNA (ASO)	Phase 3 (ph1/2 results in NEJM March'26, readout mid'27➔Q3'27)
Diranersen (BIIB080)	Early AD (CELIA)	ASO targeting Tau	RNA (ASO)	Phase 2 (-ve topline May'26 [dose response effect on CDR-SB @ W76] but 'slowing of decline across all doses, particularly @ LD 60mg Q24W, reductions in CSF tau & tau pathology by PET across all doses, Biogen intending to go into ph3, data @ AAIC Jul'26: 26% lower clinical decline on CDR-SB / 42% on ADAS-Cog13 / 50% on MMSE / 30% on modified iADRS / 23% on ADCOMS vs PBO at 18mo [mostly nominal sig.], lower benefit for 115mg Q6M & 115mg Q3M, mean reductions 50-65% vs bsln in CSF tau)
BIIB122	Parkinson's disease	LRRK2 inhibitor	Small molecule	Phase 2b (-ve topline May'26, dev. discontinued in PD)
BIIB091	MS	Peripheral BTK inhibitor	Small molecule	Phase 2 (readout Q2'26)
Felzartamab	Late MVI (kidney transplant)	Anti-CD38 mAb	Biologic (mAb)	Phase 2 (pot. regis. trial 2026)
Felzartamab	Graves' Disease	Anti-CD38 mAb	Biologic (mAb)	Phase 2 (FPI H2'26➔Q2'26)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

BIIB pipeline

Biogen (BIIB) - Pipeline summary 3/3

Mkt cap ($): 32.6 B
as of 4 Sep 2026
Source: Stock Analysis

+ pipeline from Apellis Pharmaceuticals acquired Mach'26

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Salanersen (BIIB115)	Spinal muscular atrophy	ASO targeting SMN	RNA (ASO)	Phase 1 (data mid-Q1'26)
Felzartamab	Lupus nephritis	Anti-CD38 mAb	Biologic (mAb)	Phase 1
BIIB142	Autoimmune diseases	IRAK4 degrader	Small molecule (TPD)	Phase 1 (primary completion Q3'26)
BIIB145	Autoimmune diseases	BTK degrader	Small molecule (TPD)	Phase 1 (primary completion Q1'27)
BIIB144	Immunology	Anti-CD40 mAb	Biologic (mAb)	Preclinical (Ph1-ready as of Jul'26)
BIIB147	ALS	Stathmin-2 ASO	RNA (ASO)	Preclinical (Ph1-ready as of Jul'26)
Alloy-BIIB ASOs	Various	Alloy's AntiClastic ASOs against undisclosed genomic sequences	RNA (ASO)	Preclinical
3 PC programs from RayThera	Inflammatory diseases	Possibly TNFa inh. / STAT6 inh. / CCR4 inh.	Small molecule	Preclinical (ph1 FPI Q3'26 from RayThera's lead asset)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

BIIB pipeline

Cytokinetics (CYTK) – Pipeline summary

Mkt cap ($): 10.1 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Aficamten (MYQORZO)	Obstructive hypertrophic cardiomyopathy (oHCM) - (SEQUOIA-HCM)	Cardiac myosin inhibitor	Small molecule	Marketed (Approved in US / EU / CN, DE launch Q2'26, US & CN launches Jan'26, JP oHCM ph3 CAMELLIA-HCM by Bayer fully enrolled) – Bayer (JP) / Sanofi (CN)
Aficamten (MYQORZO)	Obstructive hypertrophic cardiomyopathy (oHCM) - (MAPLE-HCM)	Cardiac myosin inhibitor	Small molecule	Registration (sNDA filed, PDUFA 14 Nov'26, label expansion superiority to beta-blocker, ph3 H2H metoprolol w/ +ve topline May'25)
Aficamten (MYQORZO)	Non-obstructive hypertrophic cardiomyopathy (nHCM) - (ACACIA-HCM)	Cardiac myosin inhibitor	Small molecule	Phase 3 (+ve topline May'26: hit on both co-primaries KCCQ-CSS & pVO2 @W36, no new safety signal, LEVF<50 in 10% & treatment interruptions due to LVEF<40% in 3%, prez @ ESC'26 & NEJM publi Aug'26: LSM change in KCCQ-CSS @W36: 11.4 [9.6-13.2] afi vs 8.4 [6.6-10.2] PBO ie 3.0 [0.5-5.5] PBO-adj diff p=0.021 / LSM change in pVO2 @W36 0.64ml/kg/min [0.32-0.95] afi vs -0.03 [-0.35-0.28] PBO ie 0.67 [0.22-1.1] PBO-adj diff p=0.003)
Aficamten (MYQORZO)	Pediatric oHCM (CEDAR-HCM)	Cardiac myosin inhibitor	Small molecule	Phase 2/3 (ado coh. LPI Q4'26)
Ulacamten (CK-586)	HFpEF (LVEF ≥ 60%) - (AMBER-HFpEF)	Cardiac myosin inhibitor w/ shorter t1/2 vs aficamten	Small molecule	Phase 2 (coh. 1 LPI Q1'26, coh. 2 LPI YE'26, coh. 3 LPI 2027?)
CK-089	Muscular Dystrophy	Fast skeletal muscle troponin activator	Small molecule	Phase 1 (primary completion initially planned in 2025)
Omecamtiv mecarbil	HFrEF (LVEF < 30% within 6mo, NT-proBNP ≥ 1000pg/mL) - (COMET-HF)	Cardiac myosin activator	Small molecule	Phase 3 (confirmatory trial primary completion H2'27 – enriched – n=1800, based on GALACTIC-HF 1st ph3 prev. ran by Amgen in n>8800 & with HR 0.92 [0.86-0.99] on composite endpoint of '1st HF event or CV death')

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

CYTK pipeline

Zai Lab (ZLAB) – Pipeline summary 1/3

Mkt cap ($): 2.9 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Repotrectinib (AUGTYRO)	ROS1+ NSCLC, NTRK+ solid tumors	ROS1/TRK inhibitor	Small molecule	Marketed (CN rights, CN approval Jan'26 in NTRK+) - BMS (ex-CN)
Omadacycline (NUZYRA)	ABSSSI, CABP	Antibiotic (aminomethylcycline)	Small molecule	Marketed (CN rights) - Paratek (ex-CN)
Tumor Treating Fields (OPTUNE)	Glioblastoma (GBM)	Tumor Treating Fields device	Other	Marketed (CN rights) - NovoCure (ex-CN)
Ripretinib (QINLOCK)	4L GIST	KIT/PDGFRA switch-control inhibitor	Small molecule	Marketed (CN rights) - Deciphera (ex-CN)
Efgartigimod (VYVGART)	gMG, CIDP	FcRn blocker	Biologic (mAb)	Marketed (CN rights) – argenx (ex-CN)
Sulbactam-durlobactam (XACDURO)	HABP/VABP due to Acinetobacter complex	β-lactam/β-lactamase inhibitor combo	Small molecule	Marketed (CN rights) – Entasis (ex-CN)
Niraparib (ZEJULA)	Ovarian cancer	PARP inhibitor	Small molecule	Marketed (CN rights) – GSK (ex-CN)
Xanomeline-trospium (KarXT)	Schizophrenia - **(CN)**	M1/M4 agonist + peripheral anticholinergic	Small molecule	Marketed (CN rights, CN approval Dec'25 based on ph1-PK bnridge & CN ph3 by ZLAB, CN launch Q2'26) – BMS (ex-CN)
Tisotumab vedotin (Tivdak)	2L+ R/M Cervical cancer	Tissue factor ADC	Biologic (ADC)	Marketed (CN rights, CN approval Jun'26) – Pfizer/Genmab (ex-CN)
Tumor Treating Fields (OPTUNE)	1L Pancreatic cancer	Tumor Treating Fields device	Other	Registration (pot. CN approval H2'27) - NovoCure (ex-CN)
Bemarituzumab	1L Gastric/GEJ cancer (FORTITUDE-101)	Anti-FGFR2b antibody	Biologic (mAb)	Phase 3 (Amgen trial, +ve OS topline mid'25 but OS HR degradation [N.S.] @ ESMO'25) – Amgen (ex-CN)
Xanomeline-trospium (KarXT)	Psychosis in Alzheimer's disease (ADEPT-1/2/3/4)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion Q4'26 for ADEPT-1/2/4 & Q3'27 for OLE) - BMS (ex-CN)
Xanomeline-trospium (KarXT)	Schizophrenia (EMERGENT TEEN)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion YE'29) - BMS (ex-CN)
Xanomeline-trospium (KarXT)	Manic episodes in Bipolar Disorder Type I (BALSAL-1/2)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion Q4'26) - BMS (ex-CN)
Xanomeline-trospium (KarXT) + SOC	Manic episodes in Bipolar Disorder Type I (NCT07140913)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion mid'27, adjunct to Lithium, Valproate, or Lamotrigine) - BMS (ex-CN)
Xanomeline-trospium (KarXT)	Alzheimer's disease (MINDSET-1 & 2)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion Q3'28) - BMS (ex-CN)
Xanomeline-trospium (KarXT) mono or + KarEC	Autism-Related Irritability (NCT07284745 & NCT07285798)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, FPI Q3'26, primary completion Q3'29) - BMS (ex-CN)
Xanomeline-trospium (KarXT) + KarEC	Schizophrenia, Autism-Related Irritability (NCT07424404)	M1/M4 agonist + peripheral anticholinergic	Small molecule	Phase 3 (BMS trial, primary completion 2030) - BMS (ex-CN)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

ZLAB pipeline

Zai Lab (ZLAB) – Pipeline summary 2/3

Mkt cap ($): 2.9 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Tumor Treating Fields (OPTUNE)	Brain metastases from NSCLC (METIS)	Tumor Treating Fields device	Other	Phase 3 (Novocure trial, +ve topline March'24, US filing Q4'25) - NovoCure (ex-CN)
Efgartigimod SC (VYVGART)	Myositis incl. IMNM, DM, ASys (ALKIVIA)	FcRn blocker	Biologic (mAb)	Phase 2/3 (argenx trial, +ve ph3 topline Aug'26 in IMNM & IMNM+DM pops - ~+14/+15 pbo-adj improv. on TIS @ W52, regis. in CN but subtype TBD for CN NDA) - argenx (ex-CN)
Efgartigimod (VYVGART)	Ocular MG (ADAPT OCULAR)	FcRn blocker	Biologic (mAb)	Phase 3 (argenx trial, +ve topline end-Feb'26) – argenx (ex-CN)
Efgartigimod (VYVGART)	Seronegative gMG (ADAPT SERON)	FcRn blocker	Biologic (mAb)	Phase 3 (argenx trial, +ve topline Aug'25, US approval May'26) – argenx
Efgartigimod (VYVGART)	Sjogren's disease (UNITY)	FcRn blocker	Biologic (mAb)	Phase 3 (argenx trial, readout 2027➔H2'27) - argenx (ex-CN)
Efgartigimod (VYVGART)	TED (UplighTED-1/2)	FcRn blocker	Biologic (mAb)	Phase 3 (argenx trial, futile Dec'25) - argenx (ex-CN)
Efgartigimod (VYVGART)	LN, LMN	FcRn blocker	Biologic (mAb)	Phase 2 (ZLAB trials, indications disconstinued, lack of benefit) - argenx (ex-CN)
Zocilurtatug pelitecan (ZL-1310)	Relapsed ES-SCLC (DLLEVATE)	DLL3 TopoI ADC	Biologic (ADC)	Phase 3 (FPI H2'26, IA 2027, primary completion mid'28)
Zocilurtatug pelitecan (ZL-1310) tarlatamab ± durvalumab	1L & 2L+ ES-SCLC (doublet in 2L+, triplet in 1L+) - (DeLLphi-313)	DLL3 TopoI ADC (+ DLL3 TCE!)	Biologic (ADC)	Phase 1 (FPI Apr'26, primary completion Q2'28, DLL3 TCE+DLL3 ADC combo, Part 1 = dose-esc in 2L+ / Part 2 = exp. In 2L/2L+ / Part 3 = zoci+tarla+durva triplet combo in 1L) – Amgen
Zocilurtatug pelitecan (ZL-1310) mono & combos (+ atezo ± Pt-chemo)	ES-SCLC (1L induction, 1L maintenance, 2L+)	DLL3 TopoI ADC	Biologic (ADC)	Phase 1 (multiple prior data supporting ph3 advancement, Part 1 mono dose-esc in 2L+ [0.8-2.8mpk] @ ENA'25, 54% icORR in 2L+ @ AACR'26 from Part 2 exp. [1.2 & 1.6mpk], data w/ atezo ± chemo in 1L H2'26 ➔ 1L ph3 go/no-go Q4'26, add. data @ ESMO'26 from doublet combo dose-esc Part 1B & from triplet combo dose-esc Part 1C, Part 3 = doublet exp. at 1.6mpk)
Zocilurtatug pelitecan (ZL-1310) + obrixtamig ± atezo	1L ES-SCLC, poorly differentiated NECs	DLL3 TopoI ADC (+ DLL3 TCE!)	Biologic (ADC)	Phase 1/2 (BI ph1/2 of DLL3 TCE+DLL3 ADC!, FPI 2026, Part 1 = doublet dose-esc in NECs, Part 2 = zoci+obrix+atezo in 1L ES-SCLC) - Boehringer Ingelheim
Zocilurtatug pelitecan (ZL-1310)	Other NECs incl. epNEC	DLL3 TopoI ADC	Biologic (ADC)	Phase 1/2 (38% cORR in epNEC ph1 @ AACR'26 ➔transition into regis. trial by YE'26 in 2L+ epNEC)
Tisotumab vedotin (Tivdak)	Cervical cancer (1L r/m combo)	Tissue factor ADC	Biologic (ADC)	Phase 2 (Pfizer trial, InnovaTV 205 discontinued May'26 by Pfizer/Genmab, strategic decision) - Pfizer/Genmab (ex-CN)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

ZLAB pipeline

Zai Lab (ZLAB) – Pipeline summary 2/3

Mkt cap ($): 2.9 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Povetacicept	IgA nephropathy (RAINIER)	BAFF/APRIL inhibitor (TACI-Fc fusion protein)	Biologic (fusion protein)	Phase 3 (Vertex trial but CN contrib. by ZLAB, +ve ph3 W36 topline from Vertex global ph3 March'26, PDUFA 30 Nov'26 for AA, CN filing date by ZLAB?) - Vertex (ex-CN)
Povetacicept	PMN (OLYMPUS)	BAFF/APRIL inhibitor (TACI-Fc fusion protein)	Biologic (fusion protein)	Phase 2/3 (Vertex trial but CN contrib. by ZLAB, transition ph2➔ph3 started as of May'26 [vs mid'26], primary completion YE'28) - Vertex (ex-CN)
Povetacicept	Generalized Myasthenia Gravis (ETNA)	BAFF/APRIL inhibitor (TACI-Fc fusion protein)	Biologic (fusion protein)	Phase 2 (Vertex POC trial, primary completion Q1'29) - Vertex (ex-CN)
ZL-1109 (elegrobart, VRDN-003)	TED (Active/Chronic) - **(CN)**	Anti-IGF-1R antibody	Biologic (mAb)	Phase 3 (CN, LPI Q3'26, topline 2027 [primary completion Q1'27], +ve global ph3s topline REVEAL-1/2 by Viridian H1'26 in Active & Chronic TED) – Viridian (ex-CN)
ZL-1108 (veligrotug)	Active/Chronic TED	Full antagonist anti-IGF-1R mAb (IV Q3W)	Biologic (mAb)	Phase 3 (CN rights via Zenas, Viritian trial w/ +ve topline Sept'24 for THRIVE [active] & Dec'24 for THRIVE-2 [chronic] ➔ US approval Jun'26, seemingly removed from ZLAB pipe, assumedly to prioritize next-gen ZL-1109/elegrobart) – Viridian (ex-CN)
ZL-1218	Solid tumors	Anti-CCR8 mAb	Biologic (mAb)	Phase 1
ZL-1503	Moderate-to-severe atopic dermatitis	IL-13 x IL-31R bsAb	Biologic (bsAb)	Phase 1a/b (Part A SAD data [4 DLs] in HVs H2'26 from global portion, Part B MAD data [2 DLs] in AtD H1'27)
ZL-2105	Immunology	undisclosed	Undisclosed	Preclinical
ZL-1222	Solid tumors	HLE Fc-silent PD-1 vHH mAB x reduced potency hIL-12 mutein fusion protein	Biologic (fusion protein)	Preclinical (FIH likely 2027➔global ph1 FPI 2027)
ZL-6201	Solid tumors incl. sarcoma	LRRC15-targeted TopoI ADC DAR8 cleavable tripeptide linker	Biologic (ADC)	Phase 1 (global, POC data 2027➔dose-esc data H1'27)
ZL-6301	Solid tumors	ROR1-targeted TopoI ADC	Biologic (ADC)	Preclinical
ZL-1311	Solid tumors incl. Gastric/GEJ Cancer	Fc-silent 2+1 MUC17 vHH x CD3 TCE	Biologic (bsAb)	Preclinical (IND filing by YE'26 & global ph1 FPI H1'27)
ZL-6202	Solid tumors	undisclosed	Undisclosed	Preclinical
ZL-1223	Solid tumors	undisclosed	Undisclosed	Preclinical

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

ZLAB pipeline

uniQure (QURE) – Pipeline summary

Mkt cap ($): 3.1 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
AMT-130, ifezuntirgene inilparvovec	Huntington's disease	AAV gene therapy delivering microRNA to lower huntingtin (HTT) expression	Gene therapy (AAV)	Registration (initially assumed as pivotal then not then yes, +ve ph1/2 3y topline Sept'25 ➔ FDA Type A meeting minutes received early-March'26 where FDA confirmed that data from Ph1-2s vs external control are not sufficient for a filing, FDA "strongly recommending" to conduct a prospective double-blind sham surgery RCT ➔ FDA staff changes ➔ Type B meeting Q2'26 to discuss pot. design approaches ➔ Jun'26 Type B meeting outcomes [NB not minutes]: FDA accepting a BLA filing based on 3y data from ph1-2s & accepting concurrent control on SOC rx rather than sham procedure for the confirmatory trial ➔ US BLA resubmission early-Sept'26 – Priority Review requested, UK & EU filings also early-Sept'26, 4y topline Sept'26 from first 2 coh. [n=12 HD & n=12 LD])
AMT-162	ALS (SOD1) – (EPISOD1)	AAV gene therapy (gene silencing for SOD1)	Gene therapy (AAV)	Phase 1/2 (recruitment voluntary paused following Sept'25 IDMC recom. after 1 TR-SAE/DLT, data from n=5 still being collected)
AMT-191	Fabry disease	AAV gene therapy delivering GLA (α-galactosidase A) gene	Gene therapy (AAV)	Phase 1/2 (updated efficacy data at WORLDSymp'26, add. dosing in MD / HD coh. paused pending eval. of asymptomatic Gr3 liver enzyme elevs in n=2 from MD coh. further confirmed as DLTs, Jun'26 update in n=11 with 3 to 18mo+ FU: dose-dpdt elevations in all n=11 across 3 DLs with α-Gal A activity ranging 1-16x above mean normal range at LD & 14.5-230x at MD & 59-144x at HD / plasma lyso-Gb3 levels stable across all DLs regardless of ERT / ERT withdrawn in all n=11 / no TRSAEs at LD-MD & no add. SAEs at HD beyond those in n=2 prev. reported in Sept'25 / add. dosing at MD & HD remaining paused as per protocol & pending further eval. of asymptomatic liver enzyme elevations in n=2 at MD further confirmed as DLTs – both resolved after immunosuppression as per protocol)
AMT-260	Refractory Mesial Temporal lobe epilepsy (TLE)	AAV gene therapy (CNS gene modulation)	Gene therapy (AAV)	Phase 1/2 (data update Jun'26 from DL1 coh. [no DL2 data as prev. planned]: meaningful reductions in disabling seizures during M4-6 ranging 79-100% in n=3 & 'variable changes' ranging 33% decrease to 36% increase in n=3, DL2 LPI Q3'26, next update H1'27 incl. DL2)
AMT-240	Autosomal Dominant Alzheimer's Disease	AAV gene therapy aimed at silencing APOE	Gene therapy (AAV)	Preclinical
Etranacogene dezaparvovec, AMT-061 (HEMGENIX)	Hemophilia B	AAV gene therapy delivering FIX (factor IX) gene	Gene therapy (AAV)	Marketed - CSL Behring

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

QURE pipeline

BioAge Labs (BIOA) – Pipeline summary

Mkt cap ($): 440.1 m
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
BGE-102	Obesity & CV risk factors	Oral CNS-penetrant NLRP3 inhibitor	Small molecule	Phase 1/2 (+ve ph1 MAD in obese & elevated hsCRP @ 120mg Jan'26 & add. ph1 MAD cohort in obese & elevated hsCRP Apr'26 ➔ ~85% hsCRP reduction from d7 over 2-3wks & normalized level 87-93% & similar 55-58% IL6 reduction [more noise vs PBO than hsCRP] at d21-d14 @60-120mg OD)
BGE-102	Pts w/ Elevated Cardiovascular Risk (QUELL-CV)	Oral CNS-penetrant NLRP3 inhibitor	Small molecule	Phase 2a (RCT, FPI Jun'26, topline H2'26 [YE'26] – PE = % change in hsCRP, pot. phase 3 FPI YE'27)
BGE-102 ± anti-VEGF	DME (QUELL-DME)	Oral CNS-penetrant NLRP3 inhibitor (potential for therapeutic retinal exposure)	Small molecule	Phase 1/2 (FPI mid'26, topline mid'27)
APJ agonist - Oral	Obesity	Oral APJ agonist	Small molecule	Preclinical (IND filing YE'26)
APJ agonist - Parenteral	Obesity	SC APJ agonist nanobody	Biologic (mAb)	Preclinical
LLY Program #1	Cardiometabolic Disease	mAb against undisclosed target	Biologic (mAb)	Preclinical – Eli Lilly
LLY Program #2	Cardiometabolic Disease	mAb against undisclosed target	Biologic (mAb)	Preclinical – Eli Lilly
NVS Target Discovery	'intersection of exercise & healthy aging'	Multiple targets	Undisclosed	Preclinical – Novartis

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

BIOA pipeline

Kailera Therapeutics (KLRA) – Pipeline summary 1/2

Mkt cap ($): 2.1 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
Ribupatide (KAI-9531, HRS9531)	Obesity w/o T2D (BMI ≥30, BMI ≥27 with a comorbidity) – (KaiNETIC-1 - Global)	GLP-1/GIP receptor dual agonist - injectable	Peptide	Phase 3 (4-6-8-10mg QW [1mg starting, Q4W titration block], topline 2028 [primary completion Q1'28], CN ph3 2-4-6mg QW [1mg starting, Q4W titration block] ➔ mean BW loss @W48 10.7-16.4-17.7% @2-4-6mg vs 1.4% PBO) – Hengrui Pharma (CN)
Ribupatide (KAI-9531, HRS9531)	Obesity w/ T2D (BMI ≥27) – (KaiNETIC-2 - Global)	GLP-1/GIP receptor dual agonist - injectable	Peptide	Phase 3 (4-6-8-10mg QW [1mg starting, Q4W titration block], topline 2028 [primary completion Q1'28]) – Hengrui Pharma (CN)
Ribupatide (KAI-9531, HRS9531)	Obesity w/o T2D (BMI ≥35) – (KaiNETIC-3 - Global)	GLP-1/GIP receptor dual agonist - injectable	Peptide	Phase 3 (H2H sema 2.4mg SC, 8-10mg QW [1mg starting, Q4W titration block], topline 2028 [primary completion Q1'28]) – Hengrui Pharma (CN)
Ribupatide (KAI-9531, HRS9531)	Obesity w/ & w/o T2D (BMI ≥35) – Global	GLP-1/GIP receptor dual agonist - oral	Peptide	Phase 3 (global ph3 program FPI H1'27 for oral ribu) – Hengrui Pharma (CN)
Ribupatide HD (KAI-9531, HRS9531)	Obesity w/o T2D (BMI ≥35) - US	GLP-1/GIP receptor dual agonist - injectable	Peptide	Phase 2b (RCT - 10-13-16-20mg x48wks, fully enrolled, readout 2027➔mid'27) – Hengrui Pharma (CN)
Oral ribupatide (KAI-9531-T, HRS9531 tablet)	Obesity - Global	GLP-1/GIP receptor dual agonist - oral	Peptide	Phase 2 (global Ph2 FPI 2026 with topline 2027➔global ph3 FPI H1'27, +ve topline Feb'26 from CN ph2 [mean BW loss @W26 6.7-11.9-11.4% @ 10-25-50mg *5-10-10mg starting doses - all Q4W titration block* vs 2.1% PBO - no plateau], CN ph3 FPI 2026) – Hengrui Pharma (CN)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

KLRA pipeline

Kailera Therapeutics (KLRA) – Pipeline summary 2/2

Mkt cap ($): 2.1 B
as of 4 Sep 2026
Source: Stock Analysis

Program / Asset	Indication	Mechanism of Action	Modality	Stage
KAI-7535 (HRS-7535)	Obesity (BMI ≥30 or BMI ≥27+1 comorbidity incl. T2D)	GLP-1 receptor agonist - oral	Small molecule	Phase 2 (global ph2 FPI Apr'26 with topline 2027 [BW change @W44], 15mg start dose & more gradual [Q4W] titration vs CN studies / top dose 180mg with option to go at 360mg or remain at MTD, CN ph2 mean BW loss @W26 3-7.1-6.2-9.4% @ 30-60-120-180mg *30mg Q4W starting doses & titration block for all except 15mg Q2W for 30mg* vs 2.5% PBO, +ve CN ph3s topline Jul'26: HARBOR-1 in obesity ➔ mean BW loss @W44 9.5% at 120mg & 10.9% at 180mg vs 2.5% PBO [efficacy estimand] -- mean BW loss @W44 8% at 120mg & 9.8% at 180mg vs 2.4% PBO [treatment policy estimand] / OUTSTANDT-2 in T2D [non-in to dapa]: HbA1c reduction @ W32 1.58% at 30mg & 1.5% at 60mg & 1.68% at 90mg vs 1.28% dapa 10mg [efficacy estimand]) – Hengrui Pharma (CN)
KAI-4729 (HRS-4729)	Obesity	GLP-1/GIP/glucagon receptor tri-agonist - injectable	Peptide	Preclinical (global ph1 FPI 2026 with topline 2027, +ve CN SAD/MAD ph1 May'26 [1-4-8-12mg QW vs ribu 4mg QW vs PBO QW]: mean BW loss @ W12 "up to" 16% at 12mg vs 5.4% PBO) – Hengrui Pharma (CN)

Preclinical Ph1 Ph2 Ph3 Reg. Mkt

KLRA pipeline

Main abbreviations 1/2

- **General:**

+ve / -ve: positive/negative
FPI: Fist Patient In, extended to clinical trial initiation
LPI: Last Patient In, enrollment/dosing completion
Improv.: improvement(s)
RCT: Randomized Clinical Trial
HR: Hazard Ratio
ORR / DCR / CBR / PFS / OS: Overall Response Rate / Disease Control Rate / Clinical Benefit Rate (similar to DCR but usually requiring some durability criteria) / Progression-Free Survival / Overall Survival
CR / PR / SD / PD: Complete Response / Partial Response / Stable Disease, by RECIST 1.1 or other criteria where relevant
IST / IIT:Investigator-Sponsored Trial, Investigator-Initiated Trial (by academia, consortia, trial networks)
IND / CTA: Investigational New Drug/Clinical Trial Authorization
FDA: U.S. Food & Drug Administration
EMA: European Medicines Agency
CHMP: (EMA's) Committee for Medicinal Products for Human Use (provides recommendation for approval or not to the EC)
POC: Proof-Of-Concept
AEs / SAEs / TRAEs: (Treatment-Emergent) Adverse Events / Serious Adverse Events / Treatment-Related Adverse Events
Gr: Grade
DL: Dose Level
Dose-esc / Dose-exp.: Dose-escalation / Dose expansion
PDUFA: Prescription Drug User Fee Act target date for review decision
PC: PreClinical
OLE: Open-Label Extension
IV / SC / IP / IT / IVT: relates to the mode of administration, IntraVenous / SubCutaneous / IntraPeritoneal / IntraTumoral / IntraVitreal
IA / FA: Interim Analysis/Final Analysis
Pot.: potentially
Regis.: registrational (pivotal)
AA: Accelerated Approval
resub.: resubmission
add.: additional
BLA / NDA / MAA: Biologics License Application / New Drug Application/ Marketing Authorisation Application
MRD: Measurable Residual Disease (or Minimal Residual Disease)
CRL: Complete Response Letter
dev.: development
CS: CorticoSteroids (steroids, GCS: GlucoCorticoSteroids)
pop.: population

Main abbreviations 2/2

- **General:**

  DLT / MTD: Dose-Limiting Toxicities / Maximum Tolerated Dose
  RP2D / RP3D / RDE: Recommended Phase 2 or Phase 3 Dose / Recommended Dose for Extension
  PK / PD: PharmacoKinetics / PharmacoDynamics
  t1/2: half-life (+ Cmax, AUC, Ctrough, etc)
  NHP: Non-Human Primate
  Endpt / PE: endpoint / Primary Endpoint
  FIH: Fist-In-Human
  SAD / MAD: Single Ascending Dose / Multiple Ascending Dose
  IDMC / DSMB: Independent Data Monitoring Committee / Data & Safety Monitoring Board
  PRV: Priority Review Voucher
  CNPV: Commissioner's National Priority Voucher
  mpk: mg/kg
  manuf.: manufacturing
  OD / QxW / QxM : Once Daily/Every x weeks/Every x Months
  BID/TID: Twice Daily/Thrice Daily
  ADA: Anti-Drug Antibodies
  Reg.: Registration
  Inv. / BICR: Investigator / Blinded Independent Central Review
  FU / LTFU: Follow-Up / Long-Term Follow-Up
  eval.: evaluation
  PBO / PBO-adj: PlaceBO / Placebo-adjusted
  AdCom: (FDA) Advisory Committee
  coh.: cohort

- **Modalities:**

  OV: Oncolytic virus
  CAR-T: Chimeric Antigen Receptor T-cells
  RLT: RadioLigand Therapeutics
  mAb / bsAb: monoclonal Antibody / bispecific (monoclonal) antibody – extended to multispecific antibodies
  ADC: Antibody-Drug Conjugate
  TPD: Targeted Protein Degradation
  TIL: Tumor-Infiltrating Lymphocytes