Neurology

Effect-Size Predictions

Using Data-free Virtual Clinical Trials

94% accuracy across 122 Neurology trials

Overall: 96% across all 604 validated trials

BioinvestGPT

Evidence Dossier

April 8, 2026

Platform Credentials at a Glance

94%

Prospective Prediction Accuracy

Across 122 validated trials (of 604 total) | Two-sided p < 0.0001

EA **100% Ex-Ante**
Every prediction certified and sent to clients before trial results were known

RG **Industrial Rigor**
Validated against real FDA/EMA clinical readouts

EP **Empirical Certainty**
Statistically significant with two-sided p < 0.0001

Data-Free	White-Box	Head-to-Head	122 of 604
Zero-Patient Input	Root Cause Explained	vs. Standard of Care	Two-Sided p < 0.0001 ● LIVE

STRATEGIC VALUE PILLARS

Zero-Data Advantage

NO DATA SHARING
Predictions require **zero proprietary patient data**, eliminating **IP liability** and privacy friction.

2M VIRTUAL PATIENTS
Pre-built, **clinically validated** virtual cohorts delivering rigorous **effect-size predictions** across all major indications.

Eliminating Blind Spots

SURROGATE DETECTION
Simultaneously predicts early surrogates (**ORR/PFS**) and final efficacy (**OS**) to expose **hidden discordances**.

FUTURE-PROOFING
Benchmarks against **competitor** pipeline drugs to ensure **superiority at launch**, not just at trial start.

Identify Biological Root Cause

BEYOND BLACK-BOX
Every prediction is backed by **biological root cause identification**, not opaque probability scores.

ACTIONABLE DRIVERS
Pinpoints specific **failure/success drivers: target selection, drug design, delivery method, or patient stratification.**

Effect-Size Portfolio Gating Spectrum

Neurology

122 of 604 validated trials

HAZARD RATIO GO/NO-GO THRESHOLD SPECTRUM

GO	**Strongly Superior**	HR < 0.3	*Transformative clinical benefit — megablockbuster potential*
GO	**Moderately Superior**	HR 0.3–0.6	*Commercially sufficient — blockbuster potential*
	HR = 0.6 THRESHOLD		
NO-GO	**Weakly Superior**	HR 0.6–0.8	*Marginal benefit — likely insufficient for market differentiation*
NO-GO	**Non-Superior**	HR 0.8–1.0	*No meaningful advantage over current standard of care*
NO-GO	**Inferior to SoC**	HR 1.0–1.2	*Patient harm risk — trial likely to fail primary endpoint*

Configurable Threshold

Clients may select any HR value as the GO/NO-GO boundary based on their commercial case. We use HR = 0.6 as the default threshold for effect-size GO/NO-GO prospective predictions. GO = commercially sufficient effect size; NO-GO = commercially insufficient. See Trial Detail pages for each ex-ante validated prediction.

EX-ANTE PREDICTION ACCURACY

SCALE

100 Drug Assets

95.9% First-in-Class ratio

COVERAGE

66 Sponsors

Public biopharma footprint

LEAD TIME

196 Days

Average advance prediction window

Full dataset: 96.0% (604 trials) | Filtered: 94.3% (122 trials) | Delta: -1.8pp

Predictive Performance Intelligence

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122 prospectively validated GO/NO-GO predictions

– –IMPAIRMENT NO-GO correct rate

Negative Predictive Value (NPV)

99%

89/90 NO-GO predictions validated

`TN / (TN + FN)`

99% of BioinvestGPT NO-GO predictions are prospectively independently validated, signaling **cost savings** from lackluster drugs. A NO-GO signal reliably identifies commercially insufficient FIC/BIC assets before Phase III spend, **preventing impairment write-downs**.

++RELIABILITY GO/NO-GO correct rate

Accuracy

94%

115/122 validated prospective predictions

`(TP + TN) / (TP + TN + FP + FN)`

94% of BioinvestGPT GO/NO-GO predictions are correct for any-stage FIC/BIC assets, **nearly 10 times higher** than the industry's current best level (5–10%). BioinvestGPT prospectively predicts both drug successes and failures with **near-perfect reliability**.

++REVENUE GO correct rate

Positive Predictive Value (PPV)

81%

26/32 GO predictions validated

`TP / (TP + FP)`

81% of BioinvestGPT GO predictions are prospectively independently validated as commercially sufficient drugs. A GO signal identifies **blockbuster FIC/BIC assets** with high reliability, enabling **revenue-maximizing** portfolio predictions.

++RELIABILITY GO/NO-GO bias-free rate

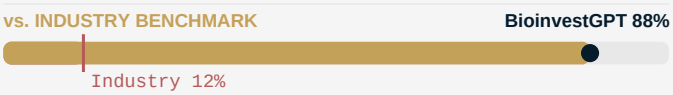
F1-Score

88%

Harmonic mean of Precision and Recall

`2×TP / (2×TP + FP + FN)`

Both GO and NO-GO predictions are almost **equally likely to be correct**, even though **blockbuster drugs are much rarer**. The model does not simply guess "failure" — it **makes the right call on both sides**.



++RELIABILITY GO/NO-GO reliability rate

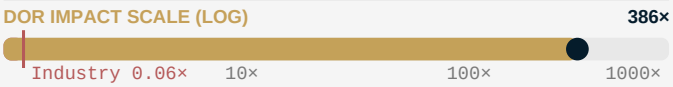
Diagnostic Odds Ratio (DOR)

386×

386× more likely correct than incorrect

`(TP × TN) / (FP × FN)`

BioinvestGPT GO/NO-GO predictions is **386 times more likely** to **correctly identify blockbuster assets** (true positives) and **rule out commercially insufficient assets** (true negatives), compared with the chance of making an incorrect prediction.



* The F1-Score and DOR for current drug GO/NO-GO prospective prediction approach is estimated based on the industry 10% nomination rate (boehringer-ingelheim.com/science-innovation/human-health-innovation/drug-discovery).

Therapeutic Area Breakdown

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Exhibit 1

Prediction accuracy remains consistently high across all therapeutic domains

BioinvestGPT's validation dataset spans 1 major therapeutic area, demonstrating that predictive accuracy is not an artifact of concentration in any single domain.

1. Source: BioinvestGPT validated prediction database. Classification based on clinical readout outcomes.
2. Therapeutic area mapping uses standardized groupings across 1 domain. Accuracy = (TP + TN) / Total.

Sponsors Coverage Breakdown

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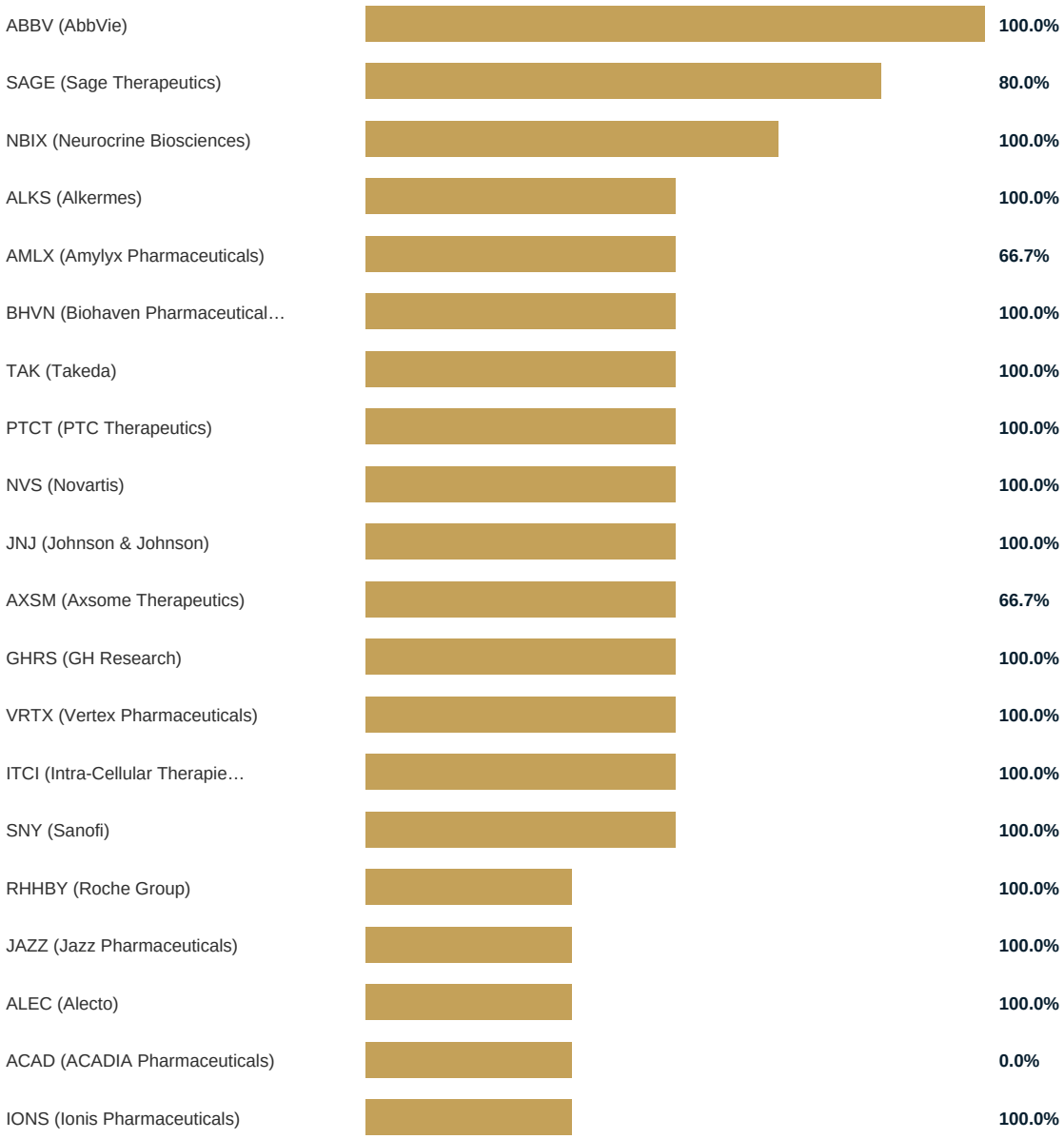
Exhibit 2

Validation spans 66 public biopharma sponsors across all market capitalizations

Coverage spans large-cap through small-cap sponsors, confirming model generalizability is not concentrated in any single company cohort.



Total unique sponsors: 66 | Average accuracy across top sponsors: 90.7%

1. Search sponsor name on data.bioinvestgpt.com to access the company-specific audited timestamped ex-ante predictions and readout validations.

Drug Innovation & Modality Coverage Breakdown

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Exhibit 3

Validated across 100 distinct drug assets spanning 8 modalities

DRUG CLASSIFICATION

FIRST-IN-CLASS

117

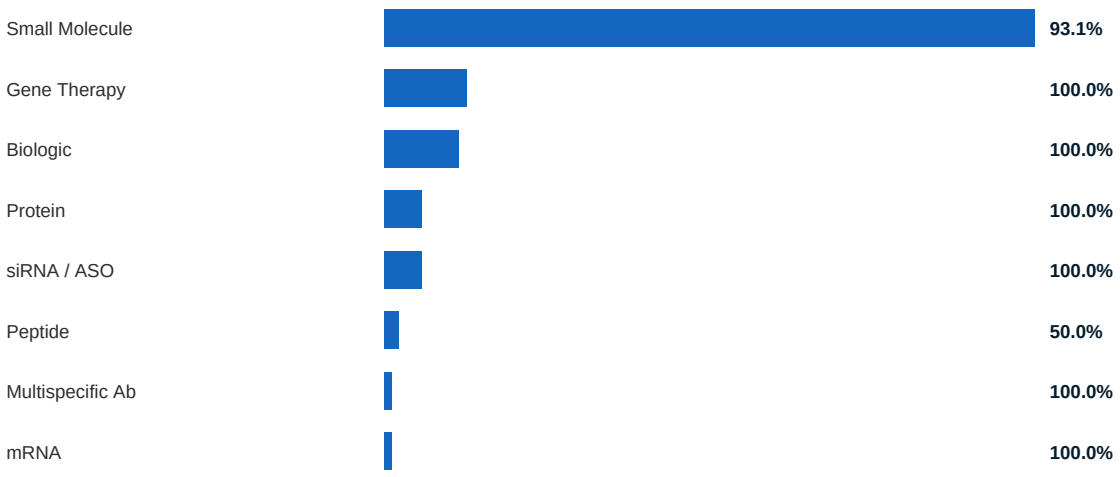
94.3% accuracy

BEST-IN-CLASS

5

94.3% accuracy

DRUG MODALITY DISTRIBUTION

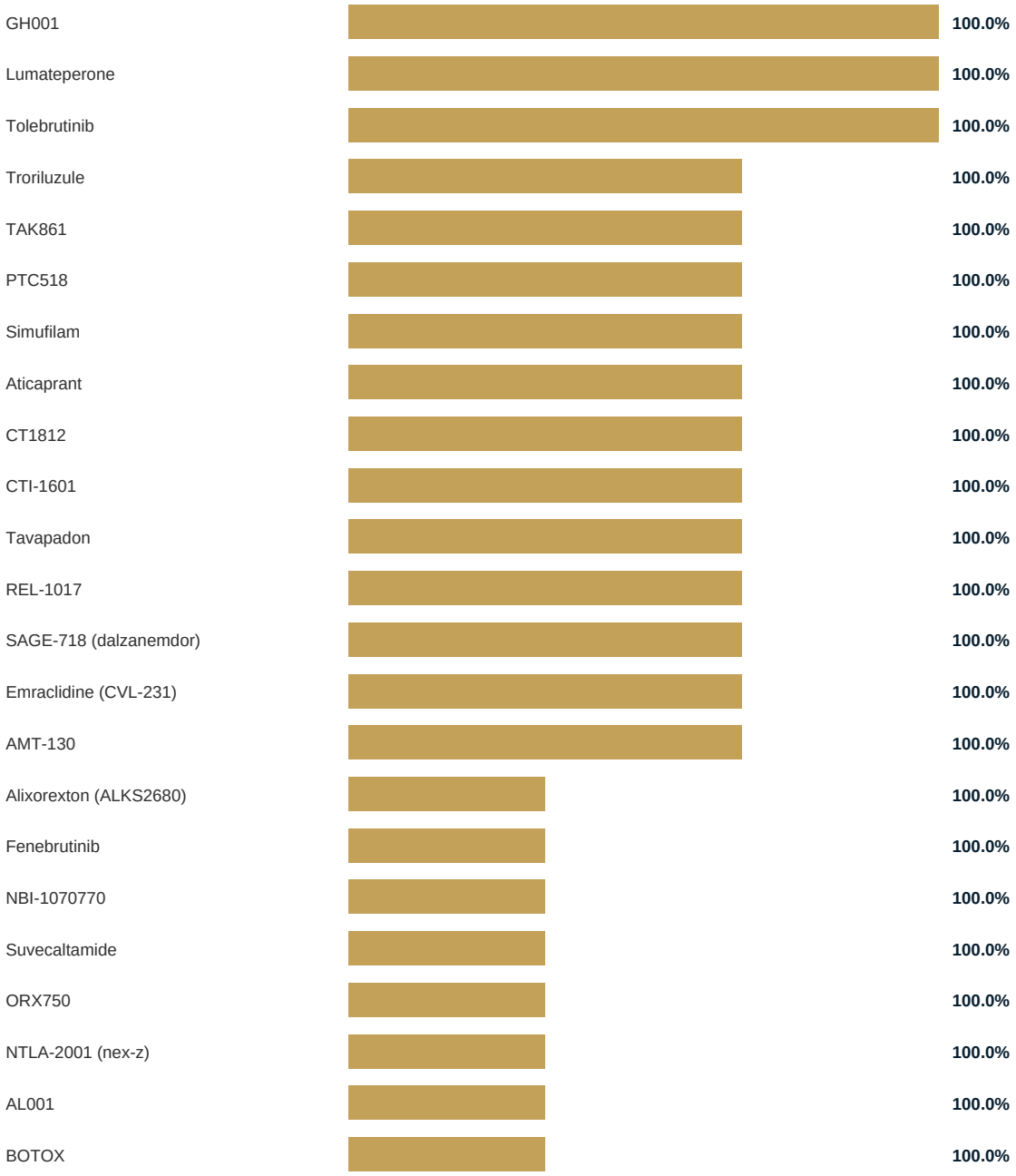


Drug Assets Coverage Breakdown

Neurology

Exhibit 4

Validated across 100 distinct drug assets with consistently high prediction accuracy



Total unique drug assets: 100 | Average accuracy across top drugs: 100.0%

1. Search drug name on data.bioinvestgpt.com to access the drug-specific audited timestamped ex-ante predictions and readout validations.

Temporal Integrity: Prospective Proof

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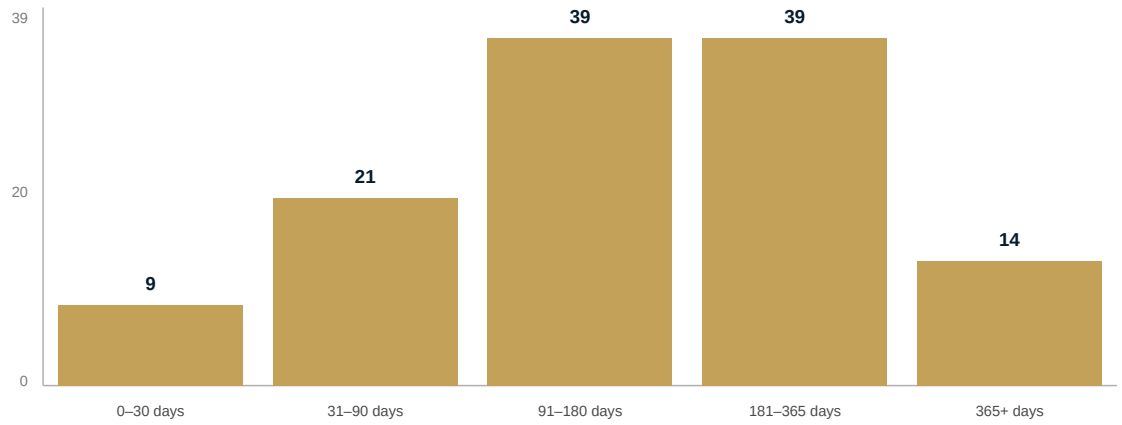
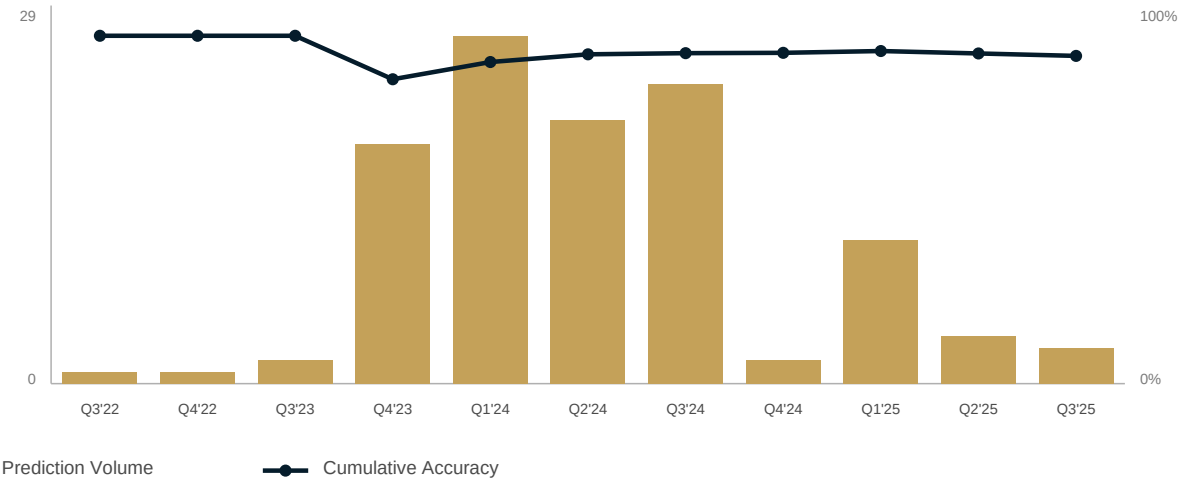
Exhibit 5

100% of predictions issued before clinical readout with median lead time of 196 days

LEAD TIME DISTRIBUTION



QUARTERLY PREDICTION VOLUME & CUMULATIVE ACCURACY



All 122 predictions were certified ex-ante, with an average lead time of 196 days before clinical readout. This temporal integrity is cryptographically verifiable and eliminates any possibility of post-hoc fitting.

Competitive Benchmark Analysis

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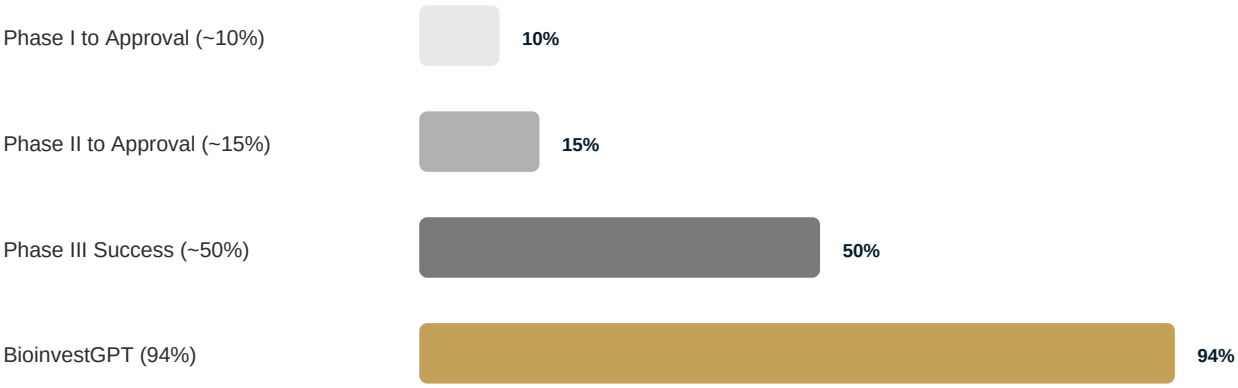
Exhibit 6

BioinvestGPT demonstrates multi-order-of-magnitude improvement over industry baselines

CLINICAL DEVELOPMENT SUCCESS RATES



QUANTITATIVE BENCHMARK

Metric	BioinvestGPT	Industry Avg	Context
Accuracy	94.3%	~10-15%	Phase I-to-approval historical rate
Precision (PPV)	81.3%	~11%	Avg drug approval probability
NPV	98.9%	~60%	Combined Phase I–III attrition detection
F1-Score	88.1%	12%	Based on industry 10% nomination rate
DOR	385.7x	0.06x	Based on industry 10% nomination rate
Lead Time	196 days	0 days	Post-hoc industry standard

Note: Industry success rates measure population-level probability of a drug advancing through clinical development phases. BioinvestGPT predicts phase-agnostic effect-size-based clinical benefit using HR = 0.6 as the GO/NO-GO threshold to classify commercially sufficient vs. insufficient drugs, with all predictions certified ex-ante. The comparison illustrates the magnitude of predictive alpha, not a direct methodological equivalence.

REDIRECTABLE R&D CAPITAL

$26.7B

89 of 90 NO-GO predictions validated × $300M avg trial cost

CAPTURED PORTFOLIO VALUE

$26.0B

26 of 32 GO predictions validated × $1B avg revenue

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
1756	**Tolebrutinib** **NCTID: NCT04411641** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: CNS-penetrant BTK inhibitor** **Area: Nervous System Diseases** **Indication: Nonrelapsing Secondary Progressive Multiple Sclerosis (NRSPMS)** **Human Patients: 1131** **Sponsor: Sanofi (SNY)**	Date: 19 Aug 2024 Quarter: Q4'24 Batch 12 Technical: FAILURE (statistically insignificant clinical benefit in 6-month CDP against placebo) Commercial: FAILURE (commercially insufficient clinical benefit in 6-month CDP inferior to siponimod)	Readout: 24 Dec 2025 **Lead Time: 492 days in advance** Interpretation: tolebrutinib achieved 31% placebo-adjusted reduction in 6-month (CDP); non-superior to siponimod's 29-33% placebo-adjusted reduction in 6-month CDP in the EXPAND trial NCT01665144 (see add'l link below); FDA issued CRL rejecting Sanofi's NDA for tolebrutinib for NR-SPMS	Correct Prediction Conclusion: statistically significant and commercially insufficient Classification: **True Negative (TN)**
1909	**Alixorexton (ALKS2680)** **NCTID: NCT06555783** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: OX2R agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy Type 2** **Human Patients: 80** **Sponsor: Alkermes (ALKS)**	Date: 8 Feb 2025 Quarter: Q2'25 Batch 9 Technical: SUCCESS (statistically significant clinical benefit in MWT for NT2 compared with placebo featuring a mild positive dose-response relationship and a moderate positive dose-toxicity relationship) Commercial: SUCCESS (commercially sufficient clinical benefit in MWT for NT2; non-inferior non-superior to ALKS2680/TAK861 in the best-case scenarios of optimal dosing regimens)	Readout: 12 Nov 2025 **Lead Time: 277 days in advance** Interpretation: Alixorexton 14mg/18mg met the primary endpoint in MSL on MWT at week 8 ($p<0.05$) and Alixorexton 18mg met the key secondary endpoint in ESS ($p<0.05$)	Correct Prediction Conclusion: statistically significant (featuring a mild positive dose-response relationship) and commercially sufficient (no approved treatment so far) Classification: **True Positive (TP)**
2048	**Fenebrutinib** **NCTID: NCT04544449** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: CNS-penetrant BTK inhibitor** **Area: Nervous System Diseases** **Indication: Primary Progressive Multiple Sclerosis** **Human Patients: 985** **Sponsor: Roche Group (RHHBY)**	Date: 28 Jul 2025 Quarter: Q4'25 Batch 4 Technical: SUCCESS (statistically significant yet moderate clinical benefit in 12-week CDP compared with placebo) Commercial: SUCCESS (commercially sufficient yet moderate clinical benefit in 12-week CDP moderately superior to ocrelizumab)	Readout: 10 Nov 2025 **Lead Time: 105 days in advance** Interpretation: fenebrutinib met the primary endpoint of delay in the onset of composite confirmed disability progression over a period of at least 120 weeks of treatment; being numerically superior to ocrelizumab	Correct Prediction Conclusion: statistically significant and commercially sufficient (ocrelizumab is the only SoC) Classification: **True Positive (TP)**
1662	**NBI-1070770** **NCTID: NCT06267846** **Phase: Phase 2** **Class: Best-In-Class** **Modality: small molecule** **MoA: oral anti-NMDAR-NR2B NAM** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 73** **Sponsor: Neurocrine Biosciences (NBIX)**	Date: 6 Jul 2024 Quarter: Q3'25 Batch 10 Technical: FAILURE (statistically maybe significant yet very weak additive clinical benefit in MADRS) Commercial: FAILURE (commercially insufficient additive clinical benefit in MADRS inferior to lumateperone)	Readout: 10 Nov 2025 **Lead Time: 492 days in advance** Interpretation: NBI-1070770 failed to meet the primary efficacy endpoint of MADRS compared with placebo	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1138	**Suvecaltamide** **NCTID: NCT05642442** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: t-type calcium channel antagonist** **Area: Nervous System Diseases** **Indication: Moderate to Severe Residual Tremor in Parkinson's Disease** **Human Patients: 169** **Sponsor: Jazz Pharmaceuticals**	Date: 21 Nov 2023 Quarter: Q2'25 Batch 3 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 6 Nov 2025 **Lead Time: 716 days in advance** Interpretation: suvecaltamide failed to meet the primary efficacy endpoint of TETRAS composite outcome score compared with placebo (P=0.2363)	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	(JAZZ)			
1907	**ORX750** **NCTID: NCT06752668** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: OX2R agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy and Idiopathic Hypersomnia** **Human Patients: 96** **Sponsor: Centessa Pharmaceuticals (CNTA)**	Date: 8 Feb 2025 Quarter: Q2'25 Batch 9 Technical: SUCCESS (statistically significant clinical benefit in MWT for NT1 NT2 and IH compared with placebo; featuring a differential positive dose-response relationship (NT1 >> IH > NT2) and a differential positive dose-toxicity relationship (NT2 > IH) Commercial: SUCCESS (commercially sufficient clinical benefit in MWT for NT1 NT2 and IH in observance of differential therapeutic windows (narrow range of high dose for NT2 medium range of medium dose for IH broad range of low dosage for NT1); non-superior to ALKS2680/TAK861 in the best-case scenarios of optimal dosing regimens)	Readout: 5 Nov 2025 **Lead Time: 270 days in advance** Interpretation: ORX750 1.5mg for NT1 achieved a >20 minute change from baseline in mean sleep latency compared with placebo on the MWT at Week 2 (p=0.0026). ORX750 4mg for NT2 achieved a >10 minute change from baseline in mean sleep latency compared with placebo on the MWT at Week 2 (p=0.0193). ORX750 2mg for IH achieved placebo-adjusted improvement in mean sleep latency compared with placebo on the MWT at Week 2 (p=0.0213)	Correct Prediction Conclusion: statistically significant (stronger efficacy in NT1 than in NT2/IH) and commercially probably sufficient Classification: **True Positive (TP)**
1116	**NTLA-2001 (nex-z)** **NCTID: NCT04601051** **Phase: Phase 1** **Class: First-In-Class** **Modality: gene therapy** **MoA: NP-delivered CRISPR-Cas9 reducing TTR production** **Area: Nervous System Diseases** **Indication: Patients With Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN) and With Transthyretin Amyloidosis-Related Cardiomyopathy (ATTR-CM)** **Human Patients: 72** **Sponsor: Intellia Therapeutics (NTLA)**	Date: 23 Jul 2023 Quarter: 2H'23 Batch 11 Technical: partial FAILURE (statistically insignificant clinical benefit in 6MWT for cardiomyopathy or in 10MWT for polyneuropathy) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 27 Oct 2025 **Lead Time: 827 days in advance** Interpretation: nex-z phase 3 clinical trials in ATTR-CM (MAGNITUDE) and ATTR-PN (MAGNITUDE-2) have been paused due to grade 4 hepatotoxicity in a patient	Correct Prediction Conclusion: statistically insignificant and commercially probably insufficient Classification: **True Negative (TN)**
579	**AL001** **NCTID: NCT04374136** **Phase: Phase 3** **Class: First-In-Class** **Modality: monoclonal antibody (mAb)** **MoA: anti-SORT1 inhibitor** **Area: Nervous System Diseases** **Indication: Frontotemporal Dementia due to a progranulin gene mutation (FTD-GRN)** **Human Patients: 110** **Sponsor: Alecto (ALEC)**	Date: 28 Jul 2025 Quarter: Q4'25 Batch 3 Technical: FAILURE (statistically insignificant clinical benefit in CDR + NACC FTLD-SB compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in CDR + NACC FTLD-SB)	Readout: 21 Oct 2025 **Lead Time: 85 days in advance** Interpretation: AL001 failed to meet the primary endpoint of slowing FTD-GRN progression	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1940	**BOTOX** **NCTID: NCT05216250** **Phase: Phase 2** **Class: First-In-Class** **Modality: biologic toxin** **MoA: botulinum toxin type A** **Area: Nervous System Diseases** **Indication: Upper Limb Essential Tremor** **Human Patients: 174** **Sponsor: AbbVie (ABBV)**	Date: 24 Feb 2025 Quarter: Q2'25 Batch 12a Technical: FAILURE (statistically maybe significant yet very weak clinical benefit in TREDS-R compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in TREDS-R/TETRAS UL inferior to propranolol)	Readout: 6 Oct 2025 **Lead Time: 224 days in advance** Interpretation: BOTOX achieved -1 placebo-adjusted improvement in TREDS-R total unilateral score (-2.61 vs -1.61 p=0.029); which is smaller than the 0.5-SD MCID threshold (>=4.2 point for TREDS-R)	Correct Prediction Conclusion: statistically significant weak clinical benefit and commercially probably insufficient (below-MCID improvement) Classification: **True Negative (TN)**
2047	**Carbetocin Nasal Spray**	Date: 28 Jul 2025	Readout: 24 Sep 2025	Missed Prediction

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	NCTID: NCT06173531 **Phase: Phase 3** **Class: First-In-Class** **Modality: peptide** **MoA: intranasally delivered peptide as a synthetic analog of oxytocin** **Area: Nervous System Diseases** **Indication: Hyperphagia in Prader-Willi Syndrome (PWS)** **Human Patients: 170** **Sponsor: ACADIA Pharmaceuticals (ACAD)**	Quarter: Q4'25 Batch 4 Technical: SUCCESS (statistically significant clinical benefit in HQCT compared with placebo) Commercial: SUCCESS (commercially sufficient clinical benefit in HQCT slightly-to-moderately superior to diazoxide choline)	**Lead Time: 58 days in advance** Interpretation: Carbetocin failed to meet the primary efficacy endpoint compared with placebo	Note: due to suboptimal disease modeling of PWS that has been permanently improved Conclusion: statistically insignificant and commercially insufficient Classification: **False Positive (FP)**
1886	**ZYN002** **NCTID: NCT04977986** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: cannabidiol gel** **Area: Nervous System Diseases** **Indication: Fragile X Syndrome** **Human Patients: 215** **Sponsor: Harmony Biosciences (HRMY)**	Date: 26 Jan 2025 Quarter: Q2'25 Batch 5 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in ABC-C FXS only for patients with complete FMR1 methylation) Commercial: SUCCESS (commercially sufficient yet weak clinical benefit in ABC-C FXS only for patients with complete FMR1 methylation due to no FDA approved drug on the market)	Readout: 24 Sep 2025 **Lead Time: 241 days in advance** Interpretation: ZYN002 failed to meet the primary efficacy endpoint compared with placebo	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
2013	**Zilganersen (ION373)** **NCTID: NCT04849741** **Phase: Phase 3** **Class: First-In-Class** **Modality: antisense oligonucleotide (ASO)** **MoA: degrades GFAP mRNA** **Area: Nervous System Diseases** **Indication: Alexander Disease (AxD)** **Human Patients: 54** **Sponsor: Ionis Pharmaceuticals (IONS)**	Date: 7 May 2025 Quarter: Q3'25 Batch 6 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in 10MWT compared with placebo) Commercial: partial SUCCESS (commercially maybe sufficient yet weak clinical benefit in 10MWT at most near the minimal clinically important difference MCID)	Readout: 22 Sep 2025 **Lead Time: 138 days in advance** Interpretation: Zilganersen achieved a statistically significant improvement in 10MWT at week 61 (mean difference 33.3% p=0.0412)	Correct Prediction Conclusion: statistically borderline significant and commercially maybe sufficient (depending on whether 10MWT improvement exceeds MCID) Classification: **True Negative (TN)**
1908	**Alixorexton (ALKS 2680)** **NCTID: NCT06358950** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: OX2R agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy Type 1** **Human Patients: 92** **Sponsor: Alkermes (ALKS)**	Date: 8 Feb 2025 Quarter: Q2'25 Batch 9 Technical: SUCCESS (statistically significant clinical benefit in MWT for NT1 compared with placebo featuring a positive dose-response relationship) Commercial: SUCCESS (commercially sufficient clinical benefit in MWT for NT1 non-inferior non-superior to ORX750/TAK861 in the best-case scenarios of optimal dosing regimens)	Readout: 7 Sep 2025 **Lead Time: 211 days in advance** Interpretation: Alixorexton met the primary endpoint in MSL on MWT at week 6 (24 min for 4mg with p=0.01; 26 minutes for 6mg with p<0.0001; 28 minutes for 8mg with p<0.0001) and the key secondary endpoint in ESS (-6.4 for 4mg with p=0.01; -8.7 for 6mg with p<0.0001; -8.3 for 8mg with p<0.0001)	Correct Prediction Conclusion: statistically significant and commercially sufficient (no approved treatment so far) Classification: **True Positive (TP)**
1375	**AMX0035** **NCTID: NCT06122662** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: sodium phenylbutyrate + taurursodiol** **Area: Nervous System Diseases** **Indication: Progressive Supranuclear Palsy** **Human Patients: 110** **Sponsor: Amylyx Pharmaceuticals (AMLX)**	Date: 26 Apr 2025 Quarter: Q2'25 Batch 14e Technical: FAILURE (statistically maybe significant yet very weak clinical benefit in PSPRS compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in PSPRS though no FDA approved drug is available)	Readout: 27 Aug 2025 **Lead Time: 123 days in advance** Interpretation: AMX0035 failed to meet any primary or secondary endpoint; further development discontinued	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
1500	**Troriluzule** **NCTID: NCT04641143** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: riluzole prodrug** **Area: Mental Disorders** **Indication: Obsessive Compulsive Disorder** **Human Patients: 700** **Sponsor: Biohaven Pharmaceuticals (BHVN)**	Date: 6 Mar 2024 Quarter: Q2'24 Batch 11 Technical: FAILURE (statistically insignificant additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 11 Aug 2025 **Lead Time: 523 days in advance** Interpretation: troriluzole failed to achieve the efficacy primary endpoint	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1499	**Troriluzule** **NCTID: NCT04693351** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: riluzole prodrug** **Area: Mental Disorders** **Indication: Obsessive Compulsive Disorder** **Human Patients: 589** **Sponsor: Biohaven Pharmaceuticals (BHVN)**	Date: 6 Mar 2024 Quarter: Q2'24 Batch 11 Technical: FAILURE (statistically insignificant additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 11 Aug 2025 **Lead Time: 523 days in advance** Interpretation: troriluzole failed to achieve the efficacy primary endpoint	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1990	— **NCTID: NCT05686239** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: Inidascamine (RL-007)** **Area: Nervous System Diseases** **Indication: Cognitive Impairment Associated With Schizophrenia** **Human Patients: 242** **Sponsor: ATAI Life Sciences (ATAI)**	Date: 24.Apr.25 Quarter: Q3'25 Batch 2 Technical: FAILURE (statistically maybe significant yet very weak additive clinical benefit in MCCB compared with placebo) Commercial: FAILURE (commercially insufficient additive clinical benefit in MCCB inferior to KarXT)	Readout: 25 Jul 2025 Lead Time: — Interpretation: inidascamine failed to achieve the primary endpoint in MCCB score at week 6	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1911	**TAK861** **NCTID: NCT06470828** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: OX2R agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy Type 1** **Human Patients: 168** **Sponsor: Takeda (TAK)**	Date: 8 Feb 2025 Quarter: Q2'25 Batch 11 Technical: SUCCESS (statistically significant clinical benefit in MWT for NT1 compared with placebo featuring a positive dose-response relationship) Commercial: SUCCESS (commercially sufficient clinical benefit in MWT for NT1 non-inferior non-superior to ORX750/ALKS2680 in the best-case scenarios of optimal dosing regimens)	Readout: 14 Jul 2025 **Lead Time: 156 days in advance** Interpretation: TAK861 met all primary and secondary endpoints	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1910	**TAK861** **NCTID: NCT06505031** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: OX2R agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy Type 1** **Human Patients: 105** **Sponsor: Takeda (TAK)**	Date: 8 Feb 2025 Quarter: Q2'25 Batch 11 Technical: SUCCESS (statistically significant clinical benefit in MWT for NT1 compared with placebo featuring a positive dose-response relationship) Commercial: SUCCESS (commercially sufficient clinical benefit in MWT for NT1 non-inferior non-superior to ORX750/ALKS2680 in the best-case scenarios of optimal dosing regimens)	Readout: 14 Jul 2025 **Lead Time: 156 days in advance** Interpretation: TAK861 met all primary and secondary endpoints	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1692	**BPL-003**	Date: 24 Apr 2025	Readout: 1 Jul 2025	Correct Prediction

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	NCTID: NCT05870540 **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: intranasal 5-MeO-DMT** **Area: Mental Disorders** **Indication: Treatment Resistant Depression** **Human Patients: 196** **Sponsor: atai Life Sciences (ATAI)**	Quarter: Q3'25 Batch 2 Technical: SUCCESS (statistically significant clinical benefit in MADRS compared with placebo) Commercial: partial SUCCESS (commercially sufficient clinical benefit non-superior-non-inferior to GH001; moderately superior to esketamine + SNRI/SSRI; yet moderately inferior to CYB003)	**Lead Time: 68 days in advance** Interpretation: BPL003 (8mg) achieved statistically significant -12.1 points MADRS reduction at Day 29 (p = 0.0025); superior to esketamine that achieved placebo-adjusted -9 points MADRS reduction at Day 8 (see add'l link below); yet non-superior to –15.5 points MADRS reduction at Day 8 by GH001 (p<0.0001)	Conclusion: statistically significant and commercially sufficient (superior to esketamine) Classification: **True Positive (TP)**
1300	**XPro1595** **NCTID: NCT05318976** **Phase: Phase 2** **Class: First-In-Class** **Modality: recombinant protein biologic** **MoA: biologic soluble TNFalpha neutralizer** **Area: Nervous System Diseases** **Indication: Early Alzheimer's Disease With Biomarkers of Inflammation** **Human Patients: 208** **Sponsor: Inmune Bio (INMB)**	Date: 21 Aug 2024 Quarter: Q1'25 Batch 11 Technical: partial SUCCESS (statistically maybe significant yet moderate clinical benefit in EMACC for neuroinflammation-biomarker-stratified mild AD patients) Commercial: partial SUCCESS (commercially maybe sufficient yet moderate clinical benefit in CDR/ADCS-ADL at most numerically superior to lecanemab for neuroinflammation-biomarker-stratified mild AD patients)	Readout: 30 Jun 2025 **Lead Time: 313 days in advance** Interpretation: XPro1595 did not meet the primary cognitive endpoint EMACC in the mITT population; yet a clinical benefit in EMACC (effect size 0.27) is observed for early Alzheimer's patients with two or more biomarkers of inflammation at baseline	Correct Prediction Conclusion: statistically insignificant and commercially probably insufficient Classification: **True Negative (TN)**
492	**ARCT810** **NCTID: NCT05526066** **Phase: Phase 2** **Class: First-In-Class** **Modality: mRNA medicine** **MoA: LNP-delivered OTC** **Area: Nervous System Diseases** **Indication: Ornithine Transcarbamylase Deficiency (OTCD)** **Human Patients: 8** **Sponsor: Arcturus Therapeutics (ARCT)**	Date: 14 Feb 2024 Quarter: Q4'24 Batch 8 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 30 Jun 2025 **Lead Time: 502 days in advance** Interpretation: ARCT810 achieved -2.5 µmol/L/day glutamine levels and +14.7% RUF (p=0.026)	Correct Prediction Conclusion: statistically significant and commercially probably sufficient (no FDA approved drug for OTCD) Classification: **True Positive (TP)**
1875	**ST920** **NCTID: NCT04046224** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV2/6-delivered GLA gene therapy** **Area: Nervous System Diseases** **Indication: Fabry Disease** **Human Patients: 33** **Sponsor: Sangamo Therapeutics (SGMO)**	Date: 19 Jan 2025 Quarter: Q2'25 Batch 3 Technical: FAILURE (statistically maybe significant yet very weak clinical benefit in eGFR compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in eGFR inferior to pegunigalsidase alfa)	Readout: 24 Jun 2025 **Lead Time: 156 days in advance** Interpretation: ST920 achieved a positive yet statistically insignificant eGFR slope at week 52	Correct Prediction Conclusion: statistically maybe insignificant and commercially TBD Classification: **True Negative (TN)**
1438	**COMP360** **NCTID: NCT05624268** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: 5HT2AR agonist** **Area: Mental Disorders** **Indication: treatment-resistant depression (TRD)** **Human Patients: 255** **Sponsor: Compass Pathways (CMPS)**	Date: 11 Feb 2025 Quarter: Q2'25 Batch 1 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to SoC and to 5-MeO-DMT)	Readout: 23 Jun 2025 **Lead Time: 132 days in advance** Interpretation: COMP360 achieved statistically significant -3.6 points MADRS reduction at Week 6(p <0.001); inferior to esketamine that achieved placebo-adjusted -9 points MADRS reduction at Day 8 (see add'l link below)	Correct Prediction Conclusion: statistically significant and commercially insufficient Classification: **True Negative (TN)**
1905	**VTX3232**	Date: 8 Feb 2025	Readout: 17 Jun 2025	Correct Prediction

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	NCTID: NCT06556173 **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: CNS-penetrant NLRP3 inhibitor** **Area: Nervous System Diseases** **Indication: early Parkinson's Disease (PD)** **Human Patients: 11** **Sponsor: Ventyx Biosciences (VTYX)**	Quarter: Q2'25 Batch 9 Technical: FAILURE (statistically insignificant clinical benefit in MDS-UPDRS compared with placebo featuring moderate toxicity) Commercial: FAILURE (commercially insufficient clinical benefit in MDS-UPDRS compared with levodopa/tavapadon monotherapy)	**Lead Time: 129 days in advance** Interpretation: VTX3232's improvement in MDS-UPDRS Parts II and III combined score at week 6 was neither disclosed nor statistically significant; non-superior to -10.2 improvement in MDS-UPDRS total score achieved by Levodopa at week 26 (see add'l link below)	Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1262	**Iluzanebart (VGL101)** **NCTID: NCT05677659** **Phase: Phase 2** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: TREM2 agonist** **Area: Nervous System Diseases** **Indication: Adult-Onset Leukoencephalopathy With Axonal Spheroids and Pigmented Glia** **Human Patients: 20** **Sponsor: Vigil Neuroscience (VIGL)**	Date: 12 May 2024 Quarter: Q1'25 Batch 5 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 4 Jun 2025 **Lead Time: 388 days in advance** Interpretation: Iluzanebart failed to achieve statistically significant clinical benefit in primary efficacy endpoints	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1313	**Govorestat (AT007)** **NCTID: NCT05397665** **Phase: Phase 2 Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: Aldose Reductase inhibitor** **Area: Nervous System Diseases** **Indication: Hereditary Neuropathy Caused by SORD Deficiency** **Human Patients: 56** **Sponsor: Applied Therapeutics (APLT)**	Date: 2 Nov 2023 Quarter: 2H'23 Batch 27 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 18 May 2025 **Lead Time: 563 days in advance** Interpretation: govorestat failed to deliver statistically significant 10MWRT improvement at month 12 compared with placebo	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
557	**PTC518** **NCTID: NCT05358717** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: HTT mRNA degradation** **Area: Nervous System Diseases** **Indication: Huntington's Disease** **Human Patients: 252** **Sponsor: PTC Therapeutics (PTCT)**	Date: 5 Mar 2024 Quarter: Q2'24 Batch 10 Technical: SUCCESS (statistically significant clinical benefit in lowering tHTT level) Commercial: FAILURE (commercially insufficient clinical benefit in UHDRS-TFC score)	Readout: 5 May 2025 **Lead Time: 426 days in advance** Interpretation: PTC518 failed to deliver statistically significant improvement in the efficacy endpoint cUHDRS at month 12	Correct Prediction Conclusion: statistically significant (mHTT) and commercially insufficient (UHDRS) Classification: **True Negative (TN)**
1591	**REC-994** **NCTID: NCT05085561** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: superoxide dismutase small molecule mimetic** **Area: Nervous System Diseases** **Indication: Cerebral Cavernous Malformation (CCM)** **Human Patients: 62** **Sponsor: Recursion Pharmaceuticals (RXRX)**	Date: 11 May 2024 Quarter: Q3'24 Batch 5 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit with a negative dose-response relationship) Commercial: FAILURE (commercially insufficient clinical benefit with inferior-to-microsurgery/radiosurgery efficacy)	Readout: 5 May 2025 **Lead Time: 359 days in advance** Interpretation: REC994 failed to achieve statistically significant improvement in efficacy whose further development has thus been discontinued	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
1868	**IMU-838** **NCTID: NCT05054140** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: DHODH inhibitor and NURR1 activator** **Area: Nervous System Diseases** **Indication: Progressive Multiple Sclerosis (PMS)** **Human Patients: 450** **Sponsor: Immunic (IMUX)**	Date: 14 Jan 2025 Quarter: Q2'25 Batch 2 Technical: FAILURE (statistically insignificant clinical benefit in EDSS/CDP compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in EDSS/CDP inferior to tolebrutinib)	Readout: 30 Apr 2025 **Lead Time: 106 days in advance** Interpretation: IMU838 failed to achieve statistically significant EDSS improvement compared with placebo	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1958	**Adjunctive KarXT** **NCTID: NCT05145413** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: fixed-dose combination of xanomeline and trospium chloride KarXT** **Area: Nervous System Diseases** **Indication: treatment-resistant schizophrenia** **Human Patients: 396** **Sponsor: Bristol-Myers Squibb (BMS)**	Date: 2 Apr 2025 Quarter: Q2'25 Batch 14b Technical: SUCCESS (statistically significant additive clinical benefit in PANSS compared with placebo) Commercial: SUCCESS (commercially sufficient additive clinical benefit in PANSS moderately superior to lumateperone)	Readout: 22 Apr 2025 **Lead Time: 20 days in advance** Interpretation: KarXT + SoC failed to deliver additional clinical benefit in PANSS total score at week 6 compared with SoC alone	Missed Prediction Note: due to suboptimal disease modelling of schizophrenia not uncontrollable by standard therapies; whic… Conclusion: statistically insignificant and commercially insufficient Classification: **False Positive (FP)**
1725	**Simufilam** **NCTID: NCT05026177** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: FLNA inhibitor** **Area: Nervous System Diseases** **Indication: Mild-to-Moderate Alzheimer's Disease** **Human Patients: 1125** **Sponsor: Cassava Sciences (SAVA)**	Date: 6 Aug 2024 Quarter: - Batch - Technical: FAILURE (statistically maybe significant yet very weak clinical benefit in ADAS-COG12/ADCS-ADL23 against placebo) Commercial: FAILURE (commercially insufficient clinical benefit in ADAS-COG12/ADCS-ADL23 inferior to lecanemab)	Readout: 25 Mar 2025 **Lead Time: 231 days in advance** Interpretation: simufilam failed to meet primary efficacy endpoints ADAS-COG12 and ADCS-ADL at week 76	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1658	**OAV101 (AVXS-101)** **NCTID: NCT05089656** **Phase: Phase 3** **Class: First-In-Class** **Modality: gene therapy** **MoA: rAAV9-mediated gene therapy with SMN1 transgene** **Area: Nervous System Diseases** **Indication: Type 2 Spinal Muscular Atrophy (SMA)** **Human Patients: 127** **Sponsor: Novartis Pharmaceuticals (NVS)**	Date: 1 Jul 2024 Quarter: Q3'24 Batch 14 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit in HFMSE at week 52) Commercial: FAILURE (commercially insufficient clinical benefit in HFMSE at week 52 inferior to nusinersen)	Readout: 19 Mar 2025 **Lead Time: 261 days in advance** Interpretation: OAV101 achieved 1.88 point placebo-adjusted improvement in HFMSE score (p=0.0074); inferior to nusinersen's 4.9 point placebo-adjusted improvement in HFMSE score (see add'l link below)	Correct Prediction Conclusion: statistically significant and commercially insufficient (1.88 point is below the 3-point minimal threshold of clinically meaningful improvement in HFMSE score (DOI -10.1007/s40120-024-00653-2) Classification: **True Negative (TN)**
1506	**Aticaprant** **NCTID: NCT05550532** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: selective KOR antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder (MDD)** **Human Patients: 444** **Sponsor: Johnson & Johnson (JNJ)**	Date: 18 Mar 2024 Quarter: Q2'24 Batch 12 Technical: partial SUCCESS (statistically maybe significant yet very weak additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit inferior to quetiapine or lumateperone)	Readout: 6 Mar 2025 **Lead Time: 353 days in advance** Interpretation: aticaprant discontinued further development due to insufficient efficacy	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
1430	**Aticaprant** **NCTID: NCT05455684** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: selective KOR antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder (MDD)** **Human Patients: 513** **Sponsor: Johnson & Johnson (JNJ)**	Date: 19 Jan 2024 Quarter: Q2'24 Batch 12 Technical: partial SUCCESS (statistically maybe significant yet very weak additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit inferior to quetiapine or lumateperone)	Readout: 6 Mar 2025 **Lead Time: 412 days in advance** Interpretation: aticaprant discontinued further development due to insufficient efficacy	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1622	**Ulixacaltamide** **NCTID: NCT06087276** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: T-type calcium channels blocker** **Area: Nervous System Diseases** **Indication: Essential Tremor** **Human Patients: —** **Sponsor: Praxis Precision Medicines (PRAX)**	Date: 15 May 2024 Quarter: Q3'24 Batch 12 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 28 Feb 2025 **Lead Time: 289 days in advance** Interpretation: ulixacaltamide discontinued further development due to insufficient efficacy at pre-define interim analysis	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1663	**AXS07 (meloxicam and rizatriptan)** **NCTID: NCT05550207** **Phase: Phase 3** **Class: Best-In-Class** **Modality: small molecule** **MoA: COX antagonist and 5HT1B/5HT1D agonist** **Area: Nervous System Diseases** **Indication: Migraine** **Human Patients: 100** **Sponsor: Axsome Therapeutics (AXSM)**	Date: 6 Aug 2024 Quarter: Q3'24 Batch 13 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit superior to meloxicam/rizatriptan monotherapy)	Readout: 24 Feb 2025 **Lead Time: 202 days in advance** Interpretation: AXS07 achieved >24-hour sustained pain relief for 31.2% more patients than after oral CGRPs ($p<0.001$) and statistically significant mTOQ4 total score superior to oral CGRP inhibitors ($p<0.001$)	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1877	**SPN820 (NV-5138)** **NCTID: NCT05066672** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: mTORC1 activator** **Area: Mental Disorders** **Indication: treatment-resistant depression** **Human Patients: 400** **Sponsor: Supernus Pharmaceuticals (SUPN)**	Date: 19 Jan 2025 Quarter: Q2'25 Batch 4 Technical: FAILURE (statistically insignificant clinical benefit in MADRS compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit in MADRS inferior to lumateperone)	Readout: 18 Feb 2025 **Lead Time: 30 days in advance** Interpretation: SPN820 failed to achieve statistical significance in MADRS total score	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1841	**ALTO-300** **NCTID: NCT05922878** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: MT1/MIT2 agonist and 5HT2C antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 200** **Sponsor: Alto Neuroscience (ANRO)**	Date: 9 Nov 2024 Quarter: Q1'25 Batch 9 Technical: partial SUCCESS (statistically maybe significant yet weak (additive) clinical benefit in MADRS) Commercial: FAILURE (commercially insufficient additive clinical benefit in MADRS inferior to lumateperone)	Readout: 12 Feb 2025 **Lead Time: 95 days in advance** Interpretation: ALTO-300 passed futility test yet failed to achieve sufficient efficacy at interim analysis; an increase of 50 biomarker positive patients are needed in the final analysis whose topline results are expected in mid-2026	Correct Prediction Conclusion: statistically maybe significant (yet weak efficacy) and commercially maybe insufficient Classification: **True Negative (TN)**
1265	**GH001**	Date: 5 Jun 2024	Readout: 3 Feb 2025	Correct Prediction

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	NCTID: NCT05800860 **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: psychedelic 5-MeO-DMT** **Area: Mental Disorders** **Indication: treatment-resistant depression** **Human Patients: 80** **Sponsor: GH Research (GHRS)**	Quarter: Q3'24 Batch 14 Technical: partial SUCCESS (statistically significant yet moderate clinical benefit) Commercial: partial SUCCESS (commercially maybe sufficient yet moderate clinical benefit)	**Lead Time: 243 days in advance** Interpretation: GH001 achieved statistically significant placebo-adjusted –15.5 points MADRS reduction at Day 8 (p<0.0001); superior to esketamine that achieved placebo-adjusted -9 points MADRS reduction at Day 8 (DOI-10.1001/jamapsychiatry.2017.3739)	Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1636	**Secukinumab** **NCTID: NCT05722522** **Phase: Phase 3** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: anti-IL17A inhibitor** **Area: Nervous System Diseases** **Indication: Rotator Cuff Tendinopathy** **Human Patients: 33** **Sponsor: Novartis (NVS)**	Date: 1 Jul 2024 Quarter: Q3'24 Batch 14 Technical: FAILURE (statistically insignificant additive clinical benefit in WORC-PSD as an adjunctive therapy to SoC) Commercial: FAILURE (commercially insufficient additive clinical benefit in WORC-PSD as an adjunctive therapy to SoC)	Readout: 31 Jan 2025 **Lead Time: 214 days in advance** Interpretation: secukinumab hasn't met the efficacy threshold and further development discontinued without disclosure of data	Correct Prediction Conclusion: statistically probably insignificant and commercially insufficient Classification: **True Negative (TN)**
1264	**GH001** **NCTID: NCT05804708** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: psychedelic 5-MeO-DMT** **Area: Mental Disorders** **Indication: Postpartum Depression** **Human Patients: 10** **Sponsor: GH Research (GHRS)**	Date: 5 Jun 2024 Quarter: Q3'24 Batch 14 Technical: partial SUCCESS (statistically significant yet moderate clinical benefit) Commercial: partial SUCCESS (commercially maybe sufficient yet moderate clinical benefit)	Readout: 10 Jan 2025 **Lead Time: 219 days in advance** Interpretation: GH001 achieved statistically significant of –35.4 points MADRS reduction at Day 8 (p<0.0001; placebo data not disclosed)	Correct Prediction Conclusion: statistically significant and commercially maybe sufficient (pending placebo data disclosure to evaluate efficacy competitiveness) Classification: **True Positive (TP)**
1263	**GH001** **NCTID: NCT05839509** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: psychedelic 5-MeO-DMT** **Area: Mental Disorders** **Indication: Bipolar II Disorder** **Human Patients: 6** **Sponsor: GH Research (GHRS)**	Date: 5 Jun 2024 Quarter: Q3'24 Batch 14 Technical: partial SUCCESS (statistically significant yet moderate clinical benefit) Commercial: partial SUCCESS (commercially maybe sufficient yet moderate clinical benefit)	Readout: 10 Jan 2025 **Lead Time: 219 days in advance** Interpretation: GH001 achieved statistically significant -16.8 points MADRS reduction at Day 8(p=0.0099; superior to -8 points MADRS reduction at Day 8 achieved by quetiapine; though absolute MADRS reduction values are not directly comparable due to varying placebo effects especially for psychiatric disorders	Correct Prediction Conclusion: statistically significant and commercially maybe sufficient (pending placebo data disclosure to evaluate efficacy competitiveness) Classification: **True Positive (TP)**
1487	**DNL343** **NCTID: NCT05842941** **Phase: Phase 2 Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: transferrin-receptor-mediated BBB-crossing EIF2beta activator** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis** **Human Patients: 249** **Sponsor: Denali Therapeutics / AbbVie (DNLI / ABBV)**	Date: 27 Feb 2024 Quarter: Q4'24 Batch 2 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 6 Jan 2025 **Lead Time: 314 days in advance** Interpretation: DNL343 failed to achieve its primary efficacy endpoint	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1576	**Navacaprant (NMRA-335140)** **NCTID: NCT06029426** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule**	Date: 14 Aug 2024 Quarter: Q4'24 Batch 2 Technical: FAILURE (statistically maybe significant yet very weak clinical benefit)	Readout: 2 Jan 2025 **Lead Time: 141 days in advance** Interpretation: navacaprant failed to meet the primary efficacy endpoint MADRS total score compared to	Correct Prediction Conclusion: statistically insignificant and commercially insufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	MoA: KOR antagonist **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 332** **Sponsor: Neumora Therapeutics (NMRA)**	Commercial: FAILURE (commercially insufficient clinical benefit non-superior non-inferior to aticaprant; yet inferior to lumateperone)	placebo; non-superior to aticaprant that achieved borderline statistical significance in MADRS total score in phase 2 trial (p=0.044)	Classification: **True Negative (TN)**
898	**AXS-05** **NCTID: NCT05557409** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: NMDAR antagonist & sigma-1 receptor agonist** **Area: Nervous System Diseases** **Indication: Alzheimer's Disease Agitation** **Human Patients: 350** **Sponsor: Axsome Therapeutics (AXSM)**	Date: 6 Jul 2024 Quarter: Q3'24 Batch 13 Technical: SUCCESS (statistically significant clinical benefit in CMAI) Commercial: SUCCESS (commercially sufficient clinical benefit in CMAI definitely non-inferior to and maybe superior to brexpiprazole)	Readout: 30 Dec 2024 **Lead Time: 177 days in advance** Interpretation: AXS05 failed to meet any primary and secondary efficacy endpoints	Missed Prediction Note: due to suboptimal drug MoA modelling that has been permanently improved Conclusion: statistically insignificant and commercially insufficient Classification: **False Positive (FP)**
1854	**Suzetrigine (VX-548)** **NCTID: NCT06176196** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: oral Nav1.8/SCN10A inhibitor** **Area: Nervous System Diseases** **Indication: Painful Lumbosacral Radiculopathy** **Human Patients: 218** **Sponsor: Vertex Pharmaceuticals (VRTX)**	Date: 7 Dec 2024 Quarter: Q1'25 Batch 13 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in NPRS at week 12 against placebo) partial FAILURE (statistically insignificant clinical benefit in DSIS at week 12 against placebo) Commercial: FAILURE (commercially insufficient clinical benefit in NPRS/DSIS/TEAE non-superior to NSAIDs and inferior to epidural steroid injection)	Readout: 19 Dec 2024 **Lead Time: 12 days in advance** Interpretation: suzetrigine achieved non-superior-non-inferior-to-placebo efficacy in NPRS at week 12 (-2.02 vs -1.98 without disclosing p-value); DSIS data not disclosed	Correct Prediction Conclusion: statistically insignificant (NPRS) and commercially insufficient Classification: **True Negative (TN)**
1368	**Prasinezumab** **NCTID: NCT04777331** **Phase: Phase 2** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: anti-alpha-synuclein** **Area: Nervous System Diseases** **Indication: Early Parkinson's Disease** **Human Patients: 586** **Sponsor: Prothena / Roche (PRTA/ RHHBY)**	Date: 30 Nov 2023 Quarter: Q3'24 Batch 17 Technical: FAILURE (commercially insufficient clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 19 Dec 2024 **Lead Time: 385 days in advance** Interpretation: prasinezumab failed to meet any primary and secondary efficacy endpoints	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1619	**CT1812** **NCTID: NCT05225415** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: allosteric sigma-2 receptor antagonist** **Area: Nervous System Diseases** **Indication: Dementia With Lewy Bodies** **Human Patients: 130** **Sponsor: Cognition Therapeutics (CGTX)**	Date: 15 May 2024 Quarter: Q3'24 Batch 10 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit despite tolerable toxicity) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 18 Dec 2024 **Lead Time: 217 days in advance** Interpretation: CT1812 met the safety primary endpoint with trends only of cognitive/functional improvements	Correct Prediction Conclusion: statistically significant (safety) and commercially probably insufficient (efficacy) Classification: **True Negative (TN)**
1567	**minzasolmin (UCB0599)** **NCTID: NCT04658186** **Phase: Phase 2** **Class: First-In-Class**	Date: 26 Apr 2024 Quarter: Q4'24 Batch 7 Technical: partial SUCCESS (statistically maybe significant yet	Readout: 16 Dec 2024 **Lead Time: 234 days in advance** Interpretation: minzasolmin failed to meet any primary and secondary	Correct Prediction Conclusion: statistically insignificant and commercially insufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Modality: small molecule **MoA: ASYN-misfolding inhibitor** **Area: Nervous System Diseases** **Indication: Early-stage Parkinson's Disease** **Human Patients: 496** **Sponsor: UCB Biopharma (UCB)**	very weak clinical benefit in MDS-UPDRS) Commercial: FAILURE (commercially insufficient clinical benefit with inferior-to-levodopa efficacy in MDS-UPDRS)	efficacy endpoints	Classification: **True Negative (TN)**
1409	**CTI-1601** **NCTID: NCT05579691** **Phase: Phase 2** **Class: First-In-Class** **Modality: fusion protein biologic** **MoA: TAT-delivered FXN recombinant fusion protein** **Area: Nervous System Diseases** **Indication: Friedreich Ataxia** **Human Patients: 28** **Sponsor: Larimar Therapeutics (LRMR)**	Date: 7 Jan 2024 Quarter: Q1'24 Batch 14 Technical: partial SUCCESS (statistically significant yet weak clinical benefit due to TAT delivery) Commercial: FAILURE (commercially insufficient clinical benefit inferior to omaveloxolone)	Readout: 16 Dec 2024 **Lead Time: 344 days in advance** Interpretation: nomlabofusp achieved early trends yet very weak improvements in mFARS	Correct Prediction Conclusion: statistically maybe significant yet weak (mFARS) and commercially probably insufficient Classification: **True Negative (TN)**
551	**Dazucorilant** **NCTID: NCT05407324** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: lipid selective glucocorticoid receptor antagonist** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis (ALS)** **Human Patients: 249** **Sponsor: Corcept Therapeutics (CORT)**	Date: 14 Apr 2024 Quarter: Q4'24 Batch 9 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 11 Dec 2024 **Lead Time: 241 days in advance** Interpretation: dazucorilant failed to achieve its primary efficacy endpoint	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1762	**Neflamapimod** **NCTID: NCT05869669** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: p38alpha inhibitor** **Area: Nervous System Diseases** **Indication: Dementia With Lewy Bodies** **Human Patients: 160** **Sponsor: CervoMed (CRVO)**	Date: 19 Aug 2024 Quarter: Q4'24 Batch 12 Technical: partial FAILURE (statistically maybe significant yet very weak additive clinical benefit in CDR-SB) Commercial: FAILURE (commercially insufficient additive clinical benefit in MSD-UPDRS3)	Readout: 10 Dec 2024 **Lead Time: 113 days in advance** Interpretation: neflamapimod failed to meet the primary efficacy endpoint CDR-SB; no data disclosed for MSD-UPDRS3	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1456	**Tavapadon** **NCTID: NCT04223193** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: a selective D1R/D5R partial agonist** **Area: Nervous System Diseases** **Indication: early Parkinson's Disease** **Human Patients: 304** **Sponsor: AbbVie (ABBV)**	Date: 14 Feb 2024 Quarter: Q3'24 Batch 8 Technical: SUCCESS (statistically significant clinical benefit) Commercial: partial SUCCESS (commercially sufficient additive clinical benefit superior to levodopa for patients who receive tavapadon + MAOB inhibitor combotherapy) partial FAILURE (commercially insufficient clinical benefit non-superior to levodopa for patients who receive tavapadon monotherapy)	Readout: 9 Dec 2024 **Lead Time: 299 days in advance** Interpretation: tavapadon monotherapy achieved -9.1 improvement (p< 0.0001) in MDS-UPDRS Parts II and III combined score at week 26; non-superior to -10.2 improvement in MDS-UPDRS total score achieved by Levodopa at week 26 (no data announced for tavapadon + MAOA combotherapy)	Correct Prediction Conclusion: statistically significant and commercially insufficient (as monotherapy) Classification: **True Negative (TN)**
1679	**REL-1017** **NCTID: NCT06011577** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: GLUN1-GLUN2D**	Date: 14 Jul 2024 Quarter: Q3'24 Batch 17 Technical: FAILURE (statistically insufficient additive clinical benefit) Commercial: FAILURE (commercially	Readout: 9 Dec 2024 **Lead Time: 148 days in advance** Interpretation: REL-1017 will be discontinued for lack of efficacy	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	antagonist **Area: Mental Disorders** **Indication: major depressive disorder (MDD)** **Human Patients: 340** **Sponsor: Relmada Therapeutic (RLMD)**	insufficient additive clinical benefit)		
383	**REL-1017** **NCTID: NCT04855747** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: GLUN1-GLUN2D antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder Depression (MDD)** **Human Patients: 340** **Sponsor: Relmada Therapeutics (RLMD)**	Date: 14 Jul 2024 Quarter: Q3'24 Batch 17 Technical: FAILURE (statistically insignificant additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 4 Dec 2024 **Lead Time: 143 days in advance** Interpretation: REL-1017 is found unlikely to meet the primary efficacy endpoint of adding significant clinical benefit in MADRS10 score	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
568	**PTC857** **NCTID: NCT05349721** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: ALOX15 inhibitor** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis (ALS)** **Human Patients: 307** **Sponsor: PTC Therapeutics (PTCT)**	Date: 6 Aug 2024 Quarter: Q4'24 Batch 1 Technical: FAILURE (statistically insignificant additive clinical benefit in ALSFRS-R) Commercial: FAILURE (commercially insufficient additive clinical benefit in ALSFRS-R)	Readout: 26 Nov 2024 **Lead Time: 112 days in advance** Interpretation: PTC857 failed to achieve any primary or secondary efficacy endpoints	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1621	**Taldefgrobep alfa** **NCTID: NCT05337553** **Phase: Phase 3** **Class: First-In-Class** **Modality: recombinant protein biologic** **MoA: selective ACVR2B antagonist** **Area: Nervous System Diseases** **Indication: Spinal Muscular Atrophy (SMA)** **Human Patients: 269** **Sponsor: Biohaven (BHVN)**	Date: 15 May 2024 Quarter: Q3'24 Batch 11 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit in MFM32)	Readout: 25 Nov 2024 **Lead Time: 194 days in advance** Interpretation: taldefgrobep alfa + SoC failed to achieved the MFM32 primary efficacy endpoint at week 48 compared with SoC alone	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
580	**AL002** **NCTID: NCT04592874** **Phase: Phase 2** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: anti-TREM2 mAb activator** **Area: Nervous System Diseases** **Indication: Alzheimer's Disease** **Human Patients: 381** **Sponsor: Alector / AbbVie (ALEC/ABBV)**	Date: 6 Aug 2024 Quarter: Q4'24 Batch 3 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to lecanemab)	Readout: 25 Nov 2024 **Lead Time: 111 days in advance** Interpretation: AL002 failed to meet primary efficacy endpoints CDR-SB	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1724	**Simufilam** **NCTID: NCT04994483** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: misfolded FLNA inhibitor** **Area: Nervous System Diseases**	Date: 6 Aug 2024 Quarter: Q4'24 Batch 4 Technical: FAILURE (statistically maybe significant yet very weak clinical benefit in ADAS-COG12/ADCS-ADL23 against placebo)	Readout: 25 Nov 2024 **Lead Time: 111 days in advance** Interpretation: simufilam failed to meet primary efficacy endpoints ADAS-COG12 and ADCS-ADL at week 52	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Indication: Alzheimer's Disease **Human Patients: 804** **Sponsor: Cassava Sciences (SAVA)**	Commercial: FAILURE (commercially insufficient clinical benefit in ADAS-COG12/ADCS-ADL23 inferior to lecanemab)		
1383	**SAGE-718 (dalzanemdor)** **NCTID: NCT05107128** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: NMDAR PAM** **Area: Nervous System Diseases** **Indication: Huntington's Disease** **Human Patients: 189** **Sponsor: Sage Therapeutics (SAGE)**	Date: 20 Apr 2024 Quarter: Q3'24 Batch 1 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 20 Nov 2024 **Lead Time: 214 days in advance** Interpretation: dalzanemdor failed to meet the primary efficacy endpoint	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1709	**NGN101** **NCTID: NCT05228145** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV9-delivered CLN5 trans gene** **Area: Nervous System Diseases** **Indication: CLN5 Batten Disease** **Human Patients: 6** **Sponsor: Neurogene (NGNE)**	Date: 31 Jul 2024 Quarter: Q4'24 Batch 10 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in UBDRS or visual acuity) Commercial: FAILURE (commercially insufficient clinical benefit in UBDRS or visual acuity)	Readout: 18 Nov 2024 **Lead Time: 110 days in advance** Interpretation: NGN101's further development has been discontinued	Correct Prediction Conclusion: statistically probably insignificant and commercially probably insufficient Classification: **True Negative (TN)**
1710	**NGN-401** **NCTID: NCT05898620** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV9-delivered miRNA-regulated MECP2 gene therapy** **Area: Nervous System Diseases** **Indication: Rett Syndrome** **Human Patients: 16** **Sponsor: Neurogene (NGNE)**	Date: 31 Jul 2024 Quarter: Q4'24 Batch 10 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 11 Nov 2024 **Lead Time: 103 days in advance** Interpretation: low-dose NGN401 achieved 2-score CGI-I from baseline; high-dose NGN401 achieved 33.3% SAE (1/3)	Correct Prediction Conclusion: statistically probably significant (yet weak overall clinical benefit considering both efficacy and toxicity) and commercially probably insufficient Classification: **True Negative (TN)**
1457	**Emraclidine (CVL-231)** **NCTID: NCT05227703** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: a selective CNS-penetrant CHRM4 PAM** **Area: Nervous System Diseases** **Indication: schizophrenia** **Human Patients: 372** **Sponsor: AbbVie (ABBV)**	Date: 14 Feb 2024 Quarter: Q3'24 Batch 8 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 11 Nov 2024 **Lead Time: 271 days in advance** Interpretation: emraclidine failed to achieved the primary efficacy endpoint PANSS at week 6	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1458	**Emraclidine (CVL-231)** **NCTID: NCT05227690** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: a selective CNS-penetrant CHRM4 PAM** **Area: Nervous System Diseases** **Indication: schizophrenia** **Human Patients: 372** **Sponsor: AbbVie (ABBV)**	Date: 14 Feb 2024 Quarter: Q3'24 Batch 8 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 11 Nov 2024 **Lead Time: 271 days in advance** Interpretation: emraclidine failed to achieved the primary efficacy endpoint PANSS at week 6	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
357	**Lumateperone** **NCTID: NCT04959032** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: 5HT2AR antagonist** **Area: Nervous System Diseases** **Indication: Schizophrenia relapse prevention** **Human Patients: 228** **Sponsor: Intra-Cellular Therapies (ITCI)**	Date: 23 Sep 2022 Quarter: - Batch - Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 5 Nov 2024 **Lead Time: 774 days in advance** Interpretation: Lumateperone achieved a 22.2% placebo-adjusted lower relapse of schizophrenia (16.4% vs 38.6%; p=0.0002)	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1569	**Bepranemab** **NCTID: NCT04867616** **Phase: Phase 2** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: anti-tau mAb inhibitor** **Area: Nervous System Diseases** **Indication: Alzheimer's Disease** **Human Patients: 466** **Sponsor: UCB Biopharma (UCB)**	Date: 26 Apr 2024 Quarter: Q3'24 Batch 3 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit in CDR-SB for early AD patients) Commercial: FAILURE (commercially insufficient clinical benefit with inferior-to-donanemab/lecanemab in CDR-SB for early AD patients)	Readout: 31 Oct 2024 **Lead Time: 188 days in advance** Interpretation: Bepranemab did not meet the primary efficacy endpoint CDR-SB.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1788	**Trontinemab (RO7126209)** **NCTID: NCT04639050** **Phase: Phase 1 Phase 2** **Class: Best-In-Class** **Modality: bispecific antibody** **MoA: bispecific anti-abeta and anti-TfR1 antibody** **Area: Nervous System Diseases** **Indication: Mild to Moderate Alzheimer's Disease** **Human Patients: 285** **Sponsor: Roche Group (RHHBY)**	Date: 15 Sep 2024 Quarter: Q4'24 Batch 17b Technical: SUCCESS (statistically significant clinical benefit in brain amyloid load reduction) Commercial: FAILURE (commercially insufficient clinical benefit in CDR-SB/ADAS-Cog13 inferior to lecanemab)	Readout: 30 Oct 2024 **Lead Time: 45 days in advance** Interpretation: trontinemab achieved strong effect in amyloid load reduction at week 28 (-107 CL at 36 mg/kg vs -3 CL in placebo); no data available for CDR-SB/ADAS-Cog13	Correct Prediction Conclusion: statistically significant (in amyloid beta reduction) and commercially TBD (wait for CDR-SB/ADAS-Cog13 data) Classification: **True Positive (TP)**
1696	**aldesleukin** **NCTID: NCT06096090** **Phase: Phase 2** **Class: First-In-Class** **Modality: peptide** **MoA: low-dose recombinant IL2** **Area: Nervous System Diseases** **Indication: Alzheimer's Disease** **Human Patients: 40** **Sponsor: Coya Therapeutics (COYA)**	Date: 24 Jul 2024 Quarter: Q3'24 Batch 19 Technical: FAILURE (statistically insignificant clinical benefit in ADAS-Cog13 or CDR-SB) Commercial: FAILURE (commercially insufficient clinical benefit in ADAS-Cog13 or CDR-SB)	Readout: 29 Oct 2024 **Lead Time: 97 days in advance** Interpretation: aldesleukin failed to meet the exploratory efficacy endpoints ADAS-Cog14 or CDR-SOB	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1202	**Ganaxalone** **NCTID: NCT05323734** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: 3beta-methylated synthetic analog of allopregnanolone** **Area: Nervous System Diseases** **Indication: Tuberous Sclerosis Complex related Epilepsy** **Human Patients: 128** **Sponsor: Marinus Pharmaceuticals (MRNS)**	Date: 14 Jul 2024 Quarter: Q3'24 Batch 16 Technical: partial SUCCESS (statistically maybe significant yet very weak additive clinical benefit in seizure frequency) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 24 Oct 2024 **Lead Time: 102 days in advance** Interpretation: ganaxolone did not meet the primary efficacy endpoint of seizure frequency despite numerical improvement (p=0.09)	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1718	**ALTO-100** **NCTID: NCT05712187** **Phase: Phase 2** **Class: First-In-Class**	Date: 6 Aug 2024 Quarter: Q4'24 Batch 2 Technical: FAILURE (statistically maybe significant yet very weak	Readout: 22 Oct 2024 **Lead Time: 77 days in advance** Interpretation: ALTO100 did not meet the primary efficacy endpoint MADRS	Correct Prediction Conclusion: statistically insignificant and commercially insufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Modality: small molecule **MoA: BDNF activator** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 301** **Sponsor: Alto Neuroscience (ANRO)**	(additive) clinical benefit in MADRS) Commercial: FAILURE (commercially insufficient (additive) clinical benefit in MADRS inferior to lumateperone)		Classification: **True Negative (TN)**
1374	**AMX0035** **NCTID: NCT05676034** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: sodium phenylbutyrate + taurursodiol** **Area: Nervous System Diseases / Genetic Diseases** **Indication: Wolfram Syndrome** **Human Patients: 12** **Sponsor: Amylyx Pharmaceuticals (AMLX)**	Date: 13 Dec 2023 Quarter: Q1'24 Batch 10 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 17 Oct 2024 **Lead Time: 309 days in advance** Interpretation: AMX0035 achieved +36.7 min*ng/mL C-Peptide Response as the primary efficacy endpoint; and phase 2a safe and 100% participants meeting prespecified responding criteria (24 week -0.26% HbA1c; +7.6% target glucose range; +0.05 vision acuity; 62.5% CGIC and 75% PGIC).	Correct Prediction Conclusion: statistically probably significant and commercially probably sufficient Classification: **True Positive (TP)**
477	**VK0214** **NCTID: NCT04973657** **Phase: Phase 1** **Class: First-In-Class** **Modality: small molecule** **MoA: TRbeta agonist** **Area: Nervous System Diseases** **Indication: Adrenomyeloneuropathy Form (AMN) of X-linked Adrenoleukodystrophy (X-ALD)** **Human Patients: 36** **Sponsor: Viking Therapeutics (VKTX)**	Date: 12 Oct 2022 Quarter: - Batch - Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 9 Oct 2024 **Lead Time: 728 days in advance** Interpretation: VK0214 achieved 37.9% placebo-adjusted VLCFA reduction at day 28 (p = 0.0105) and 24.9% placebo-adjusted LDL-C reduction at day 28 (p<0.0001)	Correct Prediction Conclusion: statistically significant and commercially sufficient (no FDA approved drug for X-ALD) Classification: **True Positive (TP)**
1382	**SAGE-718 (dalzanemdor)** **NCTID: NCT05619692** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: NMDAR PAM** **Area: Nervous System Diseases** **Indication: Alzheimer's Diseas** **Human Patients: 174** **Sponsor: Sage Therapeutics (SAGE)**	Date: 20 Apr 2024 Quarter: Q3'24 Batch 1 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 8 Oct 2024 **Lead Time: 171 days in advance** Interpretation: dalzanemdor failed to meet the primary efficacy endpoint.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
528	**Apitegromab** **NCTID: NCT05156320** **Phase: Phase 3** **Class: First-In-Class** **Modality: monoclonal antibody** **MoA: latent myostatin mAb inhibitor** **Area: Nervous System Diseases** **Indication: Spinal Muscular Atrophy (SMA)** **Human Patients: 188** **Sponsor: Scholar Rock Holding (SRRK)**	Date: 6 Aug 2024 Quarter: Q4'24 Batch 3 Technical: FAILURE (statistically maybe significant yet very weak additive clinical benefit in HFMSE total score) Commercial: FAILURE (commercially insufficient additive clinical benefit in HFMSE total score non-superior to nusinersen/risdiplam monotherapy)	Readout: 7 Oct 2024 **Lead Time: 62 days in advance** Interpretation: apitegromab achieved 1.8 point HFMSE score difference at week 52 for the combined 10mg/kg and 20mg/kg populations (p=0.0192) yet only 1.4 point HFMSE score difference at week 52 for the 20mg/kg population (p=0.1149).	Correct Prediction Conclusion: statistically significant (yet weak benefit with negative dose-response relationship) and commercially insufficient (1.8 point is below the 3-point minimal threshold of clinically meaningful improvement in HFMSE score doi.org/10.1007/s40120-024-00653-2) Classification: **True Negative (TN)**
1455	**tavapadon** **NCTID: NCT04201093** **Phase: Phase 3** **Class: First-In-Class**	Date: 14 Feb 2024 Quarter: Q3'24 Batch 8 Technical: SUCCESS (statistically significant clinical benefit)	Readout: 26 Sep 2024 **Lead Time: 225 days in advance** Interpretation: tavapadon achieved -11.5 to -12 improvement (p< 0.0001)	Overall Correct Prediction Conclusion: statistically significant and commercially probably insufficient as monotherapy

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Modality: small molecule **MoA: a selective D1R/D5R partial agonist** **Area: Nervous System Diseases** **Indication: early Parkinson's Disease** **Human Patients: 522** **Sponsor: AbbVie (ABBV)**	Commercial: partial SUCCESS (commercially insufficient additive clinical benefit superior to levodopa for patients who receive tavapadon + MAOB inhibitor combotherapy); partial FAILURE (commercially insufficient clinical benefit non-superior to levodopa for patients who receive tavapadon monotherapy)	in MDS-UPDRS Parts II and III combined score at week 26; numerically slightly superior to -10.8 improvement in MDS-UPDRS total score achieved by Levodopa at week 26 (doi/full/10.1056/NEJMoa033447); thus commercially insufficient because levodopa is a generic.	(combotherapy data TBD) Classification: **True Negative (TN)**
1607	**PBFT02** **NCTID: NCT04747431** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV1-mediated GRN gene therapy** **Area: Nervous System Diseases** **Indication: Frontotemporal Dementia and Progranulin Mutations (FTD-GRN)** **Human Patients: 15** **Sponsor: Passage Bio (PASG)**	Date: 13 May 2024 Quarter: Q3'24 Batch 8 Technical: partial SUCCESS (statistically significant yet weak clinical benefit in CDR + NACC FTLD) Commercial: partial SUCCESS (commercially maybe sufficient clinical benefit in CDR + NACC FTLD)	Readout: 16 Sep 2024 **Lead Time: 126 days in advance** Interpretation: PBFT02 increased CSF PGRN expression up to 6-10 fold exceeding the range found in healthy adult controls; yet plasma PGRN expression remained below healthy reference levels.	Correct Prediction Conclusion: statistically significant and commercially TBD Classification: **True Positive (TP)**
1364	**Luvadaxistat** **NCTID: NCT05182476** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: DAAO inhibitor** **Area: Nervous System Diseases** **Indication: Schizophrenia** **Human Patients: 216** **Sponsor: Neurocrine Biosciences (NBIX)**	Date: 30 Nov 2023 Quarter: Q3'24 Batch 13 Technical: partial SUCCESS (statistically maybe significant yet weak additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 12 Sep 2024 **Lead Time: 287 days in advance** Interpretation: uvadaxistat failed to meet primary endpoint.	Overall Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1628	**fosgonimeton (ATH-1017)** **NCTID: NCT04488419** **Phase: Phase 2 Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: HGF/MET agonist** **Area: Nervous System Diseases** **Indication: Alzheimer's Disease** **Human Patients: 554** **Sponsor: Athira Pharma (ATHA)**	Date: 24 Jun 2024 Quarter: Q3'24 Batch 14 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to lecanemab)	Readout: 3 Sep 2024 **Lead Time: 71 days in advance** Interpretation: none of the primary and secondary endpoints were met; with numerical treatment effect for APOE4 carriers.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1244	**PRAX-562** **NCTID: NCT05818553** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: anti-SCN8A antagonist** **Area: Nervous System Diseases** **Indication: Developmental and Epileptic Encephalopathies** **Human Patients: 20** **Sponsor: Praxis Precision Medicines (PRAX)**	Date: 5 Jan 2024 Quarter: Q2'24 Batch 11 Technical: SUCCESS (statistically significant clinical benefit more for SCN8A-mutant patients than for SCN2A-mutant patients) Commercial: SUCCESS (commercially sufficient clinical benefit for SCN8A-mutant patients)	Readout: 3 Sep 2024 **Lead Time: 242 days in advance** Interpretation: 46% placebo-adjusted reduction in monthly motor seizure and thus primary endpoint met (whether efficacy is better for SCN8A-DEE is pending for further data).	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1428	**Tolebrutinib** **NCTID: NCT04410991** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: CNS-penetrant BTK inhibitor** **Area: Nervous System Diseases**	Date: 24 Jan 2024 Quarter: Q1'24 Batch 15 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to teriflunomide)	Readout: 2 Sep 2024 **Lead Time: 222 days in advance** Interpretation: superiority primary endpoint (Tolebrutinib vs teriflunomide in annualized replace rate reduction) not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Indication: Relapsing Forms of Multiple Sclerosis (RMS) **Human Patients: 899** **Sponsor: Sanofi (SNY)**			
1427	**Tolebrutinib** **NCTID: NCT04410978** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: CNS-penetrant BTK inhibitor** **Area: Nervous System Diseases** **Indication: Relapsing Forms of Multiple Sclerosis (RMS)** **Human Patients: 974** **Sponsor: Sanofi (SNY)**	Date: 24 Jan 2024 Quarter: Q1'24 Batch 15 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to teriflunomide)	Readout: 2 Sep 2024 **Lead Time: 222 days in advance** Interpretation: superiority primary endpoint (Tolebrutinib vs teriflunomide in annualized replace rate reduction) not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1661	**NBI-1117568** **NCTID: NCT05545111** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: oral CHRM4 agonist** **Area: Nervous System Diseases** **Indication: Schizophrenia** **Human Patients: 213** **Sponsor: Neurocrine Biosciences (NBIX)**	Date: 6 Jul 2024 Quarter: Q3'24 Batch 13 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in PANSS at week 6 against placebo featuring moderate toxicity) Commercial: FAILURE (commercially insufficient clinical benefit in PANSS and toxicity inferior to KarXT yet non-inferior/non-superior to emraclidine)	Readout: 28 Aug 2024 **Lead Time: 53 days in advance** Interpretation: achieved 7.5 point placebo-adjusted improvement in PNASS score; inferior to the SoC KarXT that showed 11.6-point placebo-adjusted improvement in phase 3 studies.	Correct Prediction Conclusion: statistically significant and commercially insufficient Classification: **True Negative (TN)**
1510	**CT1812** **NCTID: NCT03507790** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: allosteric sigma-2 receptor antagonist** **Area: Nervous System Diseases** **Indication: Mild to Moderate Alzheimer's Disease** **Human Patients: 153** **Sponsor: Cognition Therapeutics (CGTX)**	Date: 18 Mar 2024 Quarter: Q2'24 Batch 12 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit despite tolerable toxicity) Commercial: FAILURE (commercially insufficient clinical benefit inferior to donanemab/lecanemab)	Readout: 29 Jul 2024 **Lead Time: 133 days in advance** Interpretation: safe yet none of the key exploratory efficacy endpoints was met (consistent trend only).	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1638	**BLZ945** **NCTID: NCT04066244** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: CSF1R inhibitor** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis (ALS)** **Human Patients: 28** **Sponsor: Novartis (NVS)**	Date: 1 Jul 2024 Quarter: - Batch - Technical: FAILURE (statistically insignificant additive clinical benefit with moderate toxicity to accelerate ALS disease progression) Commercial: FAILURE (commercially insufficient additive clinical benefit in ALSFRS-R)	Readout: 26 Jul 2024 **Lead Time: 25 days in advance** Interpretation: not disclosed yet trial terminated after internal review of efficacy and toxicity available data	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1379	**SAGE-324** **NCTID: NCT05173012** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: steriod GABAAR PAM** **Area: Nervous System Diseases** **Indication: Essential Tremor** **Human Patients: 147** **Sponsor: Sage Therapeutics**	Date: 15 Dec 2023 Quarter: Q1'24 Batch 10 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 24 Jul 2024 **Lead Time: 222 days in advance** Interpretation: endpoints not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	(SAGE)			
1404	**ION582** **NCTID: NCT05127226** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: small interfering RNA (siRNA) antisense oligonucleotide** **MoA: intrathecal anti-UBE3A-AS ASO** **Area: Nervous System Diseases** **Indication: Angelman Syndrome** **Human Patients: 51** **Sponsor: Ionis Pharmaceuticals (IONS)**	Date: 3 Jan 2024 Quarter: Q2'24 Batch 8 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit non-superior non-inferior to GTX102)	Readout: 22 Jul 2024 **Lead Time: 201 days in advance** Interpretation: 97% patients have improvements in SAS-CGI-C endpoint and consistent improvements in multiple functions; advance to pivotal trial alone by Ionis.	Correct Prediction Conclusion: statistically significant and commercially insufficient Classification: **True Positive (TP)**
1664	**LX2006** **NCTID: NCT05445323** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAVrh10-based gene therapy that deliver human FXN gene to cardiac cells** **Area: Nervous System Diseases** **Indication: Friedreich's Ataxia** **Human Patients: 9** **Sponsor: Lexeo Therapeutics (LXEO)**	Date: 7 Jul 2024 Quarter: Q3'24 Batch 13 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit in cardiac fibrosis/cardiac arrhythmia/exercise tolerance despite significantly increasing FXN expression level) Commercial: FAILURE (commercially insufficient clinical benefit in cardiac fibrosis/cardiac arrhythmia/exercise tolerance)	Readout: 15 Jul 2024 **Lead Time: 8 days in advance** Interpretation: 11.4% LVMI reduction at month 12; which falls in the lower end of the minimal clinically meaningful improvement threshold of 10-15% reduction in LVMI over 6 to 12 months.	Correct Prediction Conclusion: statistically maybe significant yet weak clinical benefit and commercially insufficient Classification: **True Negative (TN)**
908	**AMT-130** **NCTID: NCT05243017** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV gene therapy that delivers anti-HTT miRNA** **Area: Nervous System Diseases** **Indication: Early Manifest Huntington's Disease** **Human Patients: 15** **Sponsor: uniQure (QURE)**	Date: 13 May 2024 Quarter: Q3'24 Batch 8 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit in UHDRS) Commercial: FAILURE (commercially insufficient clinical benefit in UHDRS)	Readout: 9 Jul 2024 **Lead Time: 57 days in advance** Interpretation: externally statistically significant less cUHDRS (-0.2 vs -1 at Month 24 for high-dose only (p=0.007) but not low-dose (p=0.21); no statistical improvement in motor or cognitive function even for high-dose group.	Correct Prediction Conclusion: still statistically maybe significant (not strictly placebo-controlled) and commercially insufficient (no motor cognitive function improvement) Classification: **True Negative (TN)**
909	**AMT-130** **NCTID: NCT04120493** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV gene therapy that delivers anti-HTT miRNA** **Area: Nervous System Diseases** **Indication: Early Manifest Huntington's Disease** **Human Patients: 36** **Sponsor: uniQure (QURE)**	Date: 13 May 2024 Quarter: Q3'24 Batch 8 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit in UHDRS) Commercial: FAILURE (commercially insufficient clinical benefit in UHDRS)	Readout: 9 Jul 2024 **Lead Time: 57 days in advance** Interpretation: externally statistically significant less cUHDRS (-0.2 vs -1 at Month 24 for high-dose only (p=0.007) but not low-dose (p=0.21); no statistical improvement in motor or cognitive function even for high-dose group.	Correct Prediction Conclusion: still statistically maybe significant (not strictly placebo-controlled) and commercially insufficient (no motor cognitive function improvement) Classification: **True Negative (TN)**
934	**Buntanetap** **NCTID: NCT05357989** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: neurotoxic protein synthesis inhibitor** **Area: Nervous System Diseases** **Indication: Parkinson's Disease** **Human Patients: 523** **Sponsor: Annovis Bio (ANVS)**	Date: 24 Mar 2024 Quarter: Q2'24 Batch 13 Technical: FAILURE (statistically maybe significant yet very weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 2 Jul 2024 **Lead Time: 100 days in advance** Interpretation: primary efficacy endpoint not met in presentation rather than press release (x.com/adamfeuerstein/status/1808471091066581487)	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
1246	**WVE-003** **NCTID: NCT05032196** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: stereopure antisense oligonucleotide** **MoA: SNP3 mtHTT mRNA oligonucleotide** **Area: Nervous System Diseases** **Indication: Huntington's Disease** **Human Patients: 47** **Sponsor: Wave Life Sciences (WVE)**	Date: 21 Nov 2023 Quarter: 2H'23 Batch 28 Technical: partial SUCCESS (statistically significant clinical benefit in reducing mHTT protein level); partial FAILURE (statistically insignificant clinical benefit in modifying disease progression) Commercial: FAILURE (commercially insufficient clinical benefit in modifying disease progression despite significant reduction in mHTT protein)	Readout: 25 Jun 2024 **Lead Time: 217 days in advance** Interpretation: -46% mean reduction in mHTT yet insignificant improvement for TMS.	Correct Prediction Conclusion: statistically significant in mHTT and commercially insufficient in TMS Classification: **True Negative (TN)**
776	**JZP385** **NCTID: NCT05122650** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: t-type calcium channel antagonist** **Area: Nervous System Diseases** **Indication: Essential Tremor** **Human Patients: 420** **Sponsor: Jazz Pharmaceuticals (JAZZ)**	Date: 21 Nov 2023 Quarter: Q1'24 Batch 1 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 20 Jun 2024 **Lead Time: 212 days in advance** Interpretation: endpoints not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
557	**PTC518** **NCTID: NCT05358717** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: HTT mRNA degradation** **Area: Nervous System Diseases** **Indication: Huntington's Disease** **Human Patients: 252** **Sponsor: PTC Therapeutics (PTCT)**	Date: 5 Mar 2024 Quarter: Q2'24 Batch 10 Technical: SUCCESS (statistically significant clinical benefit in lowering tHTT level) Commercial: FAILURE (commercially insufficient clinical benefit in UHDRS-TFC score)	Readout: 20 Jun 2024 **Lead Time: 107 days in advance** Interpretation: 43% mHTT lowering for 10mg; TMS (2.0 points worsening for 5mg and 1.3 points worsening for 10mg vs. 4.9 points worsening for placebo).	Correct Prediction Conclusion: statistically significant and commercially insufficient (TMS and UHDRS scores are both functional endpoints; pending for UHDRSdata) Classification: **True Negative (TN)**
1464	**TSHA-102** **NCTID: NCT06152237** **Phase: Phase 1** **Class: First-In-Class** **Modality: AAV9-based gene therapy** **MoA: scAAV9-delivered miniMECP2 gene therapy** **Area: Nervous System Diseases** **Indication: Rett Syndrome** **Human Patients: 6** **Sponsor: Taysha Gene Therapies (TSHA)**	Date: 14 Feb 2024 Quarter: Q2'24 Batch 2 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 18 Jun 2024 **Lead Time: 125 days in advance** Interpretation: weak efficacy in seizure reduction (none is seizure free without anti-seizure medication).	Correct Prediction Conclusion: statistically maybe significant and commercially insufficient Classification: **True Negative (TN)**
353	**Lumateperone** **NCTID: NCT05061706** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: D1R/D2R/D4R/5TH2AR antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 480** **Sponsor: Intra-Cellular Therapies (ITCI)**	Date: 9 Jan 2024 Quarter: Q2'24 Batch 5 Technical: SUCCESS (statistically significant additive clinical benefit) Commercial: SUCCESS (commercially sufficient additive clinical benefit)	Readout: 18 Jun 2024 **Lead Time: 161 days in advance** Interpretation: 4.5 point placebo-adjusted reduction in MADRS.	Correct Prediction Conclusion: statistically significant and commercially sufficient Classification: **True Positive (TP)**
1593	**Soticlestat**	Date: 11 May 2024	Readout: 17 Jun 2024	Correct Prediction

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	NCTID: NCT04940624 **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: CH24H inhibitor** **Area: Nervous System Diseases** **Indication: Dravet Syndrome** **Human Patients: 144** **Sponsor: Takeda Pharmaceutical / Ovid Therapeutics (TAK / OVID)**	Quarter: Q3'24 Batch 5 Technical: partial SUCCESS (statistically maybe significant yet weak additive clinical benefit to SoC) Commercial: FAILURE (commercially insufficient additive clinical benefit with inferior-to-cannabidiol-or-fenfluramine efficacy in seizure frequency reduction)	**Lead Time: 37 days in advance** Interpretation: primary endpoint not met despite meeting several secondary endpoints.	Conclusion: statistically insignificant and inferior to SoC Classification: **True Negative (TN)**
965	**Efgartigimod** **NCTID: NCT05633407** **Phase: Phase 2** **Class: First-In-Class** **Modality: antibody fragment biologic** **MoA: anti-FcRn antibody fragment** **Area: Nervous System Diseases** **Indication: Postural Orthostatic Tachycardia Syndrome** **Human Patients: 53** **Sponsor: argenx (ARGX)**	Date: 4 Jul 2023 Quarter: 2H'23 Batch 9 Technical: partial SUCCESS (statistically significant yet weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 17 Jun 2024 **Lead Time: 349 days in advance** Interpretation: no clinically meaningful improvements compared to placebo on both total MaPS score and COMPASS31 and trial discontinued.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1381	**SAGE-718** **NCTID: NCT05358821** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: NMDAR PAM** **Area: Nervous System Diseases** **Indication: Huntington's Disease** **Human Patients: 69** **Sponsor: Sage Therapeutics (SAGE)**	Date: 20 Apr 2024 Quarter: Q3'24 Batch 1 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 11 Jun 2024 **Lead Time: 52 days in advance** Interpretation: only trends of functional improvement IN HD-CAB thus statistically insignificant.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1541	**SPN-817** **NCTID: NCT05518578** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: a novel synthetic form of Huperzine A; potentially as an acetylcholinesterase inhibitor and an NMDAR antagonist** **Area: Nervous System Diseases** **Indication: Resistant Epilepsy** **Human Patients: 35** **Sponsor: Supernus Pharmaceuticals (SUPN)**	Date: 11 Apr 2024 Quarter: Q2'24 Batch 15 Technical: partial SUCCESS (statistically maybe significant yet weak clinical benefit in motor seizure frequency reduction) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 23 May 2024 **Lead Time: 42 days in advance** Interpretation: 75% mean focal seizure reduction; 25% TEAE-related discontinuation; high efficacy and high toxicity.	Correct Prediction Conclusion: statistically significant yet commercially insufficient (high toxicity is usually not tolerated for anti-seizure medicine) Classification: **True Negative (TN)**
1566	**BIIB105** **NCTID: NCT04494256** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: small interfering RNA (siRNA) antisense oligonucleotide** **MoA: anti-ATXN2 ASO** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis and Participants With the ALS Ataxin-2 (ATXN2) Genetic Mutation** **Human Patients: 99** **Sponsor: Biogen / Ionis Pharmaceuticals (BIIB/IONS)**	Date: 23 Apr 2024 Quarter: Q3'24 Batch 3 Technical: FAILURE (statistically insignificant additive clinical benefit to SoC regardless of the CAG repeat expansion level in the ATXN2 gene) Commercial: FAILURE (commercially insufficient additive clinical benefit to SoC regardless of the CAG repeat expansion level in the ATXN2 gene)	Readout: 16 May 2024 **Lead Time: 23 days in advance** Interpretation: no reduction in ALS biomarkers; discontinued.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
935	**Buntanetap/Posiphen** **NCTID: NCT05686044** **Phase: Phase 2 Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: neurotoxic protein synthesis inhibitor** **Area: Nervous System Diseases** **Indication: Alzheimer Disease** **Human Patients: 320** **Sponsor: Annovis Bio (ANVS)**	Date: 24 Mar 2024 Quarter: Q2'24 Batch 13 Technical: FAILURE (statistically maybe significant yet very weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 29 Apr 2024 **Lead Time: 36 days in advance** Interpretation: key efficacy endpoints ADAS-COG11; ADCS-CGIC; ADCS-ADL not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
893	**JNJ-40411813** **NCTID: NCT04836559** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: mGluR2 PAM** **Area: Nervous System Diseases** **Indication: Focal Onset Seizures** **Human Patients: 110** **Sponsor: Johnson & Johnson / Addex Therapeutics (JNJ / ADXN)**	Date: 9 Apr 2024 Quarter: Q2'24 Batch 14 Technical: partial SUCCESS (statistically maybe significant yet very weak additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 29 Apr 2024 **Lead Time: 20 days in advance** Interpretation: primary endpoint not met; development discontinued	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1362	**NBI-1065845** **NCTID: NCT05203341** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: AMPAR PAM** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 183** **Sponsor: Neurocrine Biosciences (NBIX)**	Date: 30 Nov 2023 Quarter: - Batch - Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 23 Apr 2024 **Lead Time: 145 days in advance** Interpretation: significant MADRS for one dose (likely low dose) yet insignificant for another dose (likely high dose); anti-correlation between dosage and efficacy.	Correct Prediction Conclusion: overall statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1454	**Tavapadon** **NCTID: NCT04542499** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: a selective D1R/D5R partial agonist** **Area: Nervous System Diseases** **Indication: Parkinson's Disease** **Human Patients: 507** **Sponsor: AbbVie / Cerevel Therapeutics (ABBV / CERE)**	Date: 14 Feb 2024 Quarter: Q2'24 Batch 4 Technical: FAILURE (statistically insignificant additive clinical benefit) Commercial: FAILURE (commercially insufficient additive clinical benefit)	Readout: 18 Apr 2024 **Lead Time: 64 days in advance** Interpretation: on time 1.7 hours vs 0.6 hours ($p<0.0001$); key registrational endpoint MDS-UPDRS not met; key registrational endpoint off time reduction met yet fail to reach the minimally clinically meaningful threshold (-1.0 hour and -3.5 points [10.1002/mds.23638.]).	Correct Prediction Conclusion: overall statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1380	**SAGE-718** **NCTID: NCT05318937** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: NMDAR PAM** **Area: Nervous System Diseases** **Indication: Parkinson Disease Cognitive Dysfunction** **Human Patients: 86** **Sponsor: Sage Therapeutics (SAGE)**	Date: 15 Dec 2023 Quarter: Q1'24 Batch 10 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commerciall sufficient clinical benefit)	Readout: 17 Apr 2024 **Lead Time: 124 days in advance** Interpretation: WAIS-IV primary efficacy endpoint not met	Missed Prediction Note: due to suboptimal modelling of SAGE718 MoA that has been permanently corrected. Conclusion: statistically insignificant and commercially insufficient Classification: **False Positive (FP)**
352	**Lumateperone** **NCTID: NCT04985942** **Phase: Phase 3**	Date: 9 Jan 2024 Quarter: Q1'24 Batch 15 Technical: SUCCESS (statistically	Readout: 16 Apr 2024 **Lead Time: 98 days in advance** Interpretation: -4.9 placebo-adjusted	Correct Prediction Conclusion: statistically significant and commercially sufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Class: First-In-Class **Modality: small molecule** **MoA: D1R/D2R/D4R/5TH2AR antagonist** **Area: Mental Disorders** **Indication: Major Depressive Disorder** **Human Patients: 485** **Sponsor: Intra-Cellular Therapies (ITCI)**	significant additive clinical benefit) Commercial: SUCCESS (commercially sufficient additive clinical benefit)	delta in MADRS score (p<0.0001) & CGI-S (p<0.0001).	Classification: **True Positive (TP)**
1128	**GTX-102** **NCTID: NCT04259281** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: small interfering RNA (siRNA) antisense oligonucleotide** **MoA: intrathecal UBE3A-AS ASO** **Area: Nervous System Diseases** **Indication: Angelman Syndrome** **Human Patients: 74** **Sponsor: Ultragenyx Pharmaceutical (RARE)**	Date: 3 Jan 2024 Quarter: Q1'24 Batch 13 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 15 Apr 2024 **Lead Time: 103 days in advance** Interpretation: rapid and clinically significant improvement resulting a total MRDI response of +2.0 (p<0.0001) at Day 170 and +2.0 (p=0.0007) at Day 338.	Correct Prediction Conclusion: statistically significant and commercially sufficient (no SoC) Classification: **True Positive (TP)**
1268	**ALKS 2680** **NCTID: ISRCTN98204977** **Phase: Phase 1** **Class: First-In-Class** **Modality: small molecule** **MoA: HCRTR2 agonist** **Area: Nervous System Diseases** **Indication: Narcolepsy** **Human Patients: 147** **Sponsor: Alkermes (ALKS)**	Date: 5 Oct 2023 Quarter: 2H'23 Batch 21 Technical: partial SUCCESS (statistically significant clinical benefit in efficacy); partial FAILURE (statistically insignificant clinical benefit in toxicity worse for NT1 than for NT2) Commercial: FAILURE (commercially insufficient clinical benefit due to on-target toxicity non-superior to TAK994 or JZP441)	Readout: 10 Apr 2024 **Lead Time: 188 days in advance** Interpretation: safe and effective; advance to NT1/NT2 only; not IH	Overall Correct Prediction Conclusion: statistically significant and commercially TBD Classification: **True Negative (TN)**
1056	**serdexmethylphenidate (SDX)** **NCTID: NCT05668754** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: norepinephrine/dopamine reuptake inhibitors** **Area: Nervous System Diseases** **Indication: Idiopathic Hypersomnia** **Human Patients: 50** **Sponsor: Zevra Therapeutics (ZVRA)**	Date: 14 Feb 2024 Quarter: Q2'24 Batch 3 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 26 Mar 2024 **Lead Time: 41 days in advance** Interpretation: primary safety endpoint met and secondary efficacy endpoints meaningful.	Correct Prediction Conclusion: statistically significant and commercially sufficient to advance to phase 3 Classification: **True Positive (TP)**
896	**AXS-12 (reboxetine)** **NCTID: NCT05059223** **Phase: Phase 3** **Class: Best-In-Class** **Modality: small molecule** **MoA: selectively inhibitor of the reuptake of norepinephrine** **Area: Nervous System Diseases** **Indication: Narcolepsy** **Human Patients: 90** **Sponsor: Axsome Therapeutics (AXSM)**	Date: 1 Dec 2023 Quarter: Q1'24 Batch 2 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 25 Mar 2024 **Lead Time: 115 days in advance** Interpretation: 83% cataplexy attack reduction achieved by AXS12; superior to 49-69% cataplexy attack reduction achieved by the SoC Sodium oxybate (doi.org/10.1016/j.sleep.2003.11.002) primary and secondary endpoints met.	Correct Prediction Conclusion: statistical significant and commercially sufficient Classification: **True Positive (TP)**
1346	**Pimavanserin** **NCTID: NCT04531982** **Phase: Phase 3**	Date: 27 Nov 2023 Quarter: Q1'24 Batch 6 Technical: SUCCESS (statistically	Readout: 11 Mar 2024 **Lead Time: 105 days in advance** Interpretation: primary endpoint not	Missed Prediction Note: due to suboptimal modeling of drug MoA that has been

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Class: First-In-Class **Modality: small molecule** **MoA: 5HT2AR/5HT2CR antagonist** **Area: Nervous System Diseases** **Indication: Schizophrenia** **Human Patients: 454** **Sponsor: Acadia Pharmaceuticals (ACAD)**	significant additive clinical benefit) Commercial: SUCCESS (commercially sufficient additive clinical benefit non-inferior to KarXT)	met.	permanently corrected. Conclusion: statistically insignificant and commercially insufficient Classification: **False Positive (FP)**
342	**AMX0035** **NCTID: NCT05021536** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: sodium phenylbutyrate + taurursodiol** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis** **Human Patients: 600** **Sponsor: Amylyx Pharmaceuticals (AMLX)**	Date: 18 Feb 2024 Quarter: Q2'24 Batch 2 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 8 Mar 2024 **Lead Time: 19 days in advance** Interpretation: endpoints not met.	Missed Prediction Note: due to suboptimal modelling of ALS that has been permanently corrected. Conclusion: statistical insignificant and commercially insufficient Classification: **False Positive (FP)**
1431	**PQ912** **NCTID: NCT04498650** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: QPCT/QPCTL inhibitor** **Area: Nervous System Diseases** **Indication: Early Alzheimers Disease Mild Cognitive Impairment Due to AD** **Human Patients: 259** **Sponsor: Vivoryon Therapeutics (VVY)**	Date: 20 Jan 2024 Quarter: Q1'24 Batch 16 Technical: partial SUCCESS (statistically maybe significant yet very weak clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to donanemab or lecanemab)	Readout: 4 Mar 2024 **Lead Time: 44 days in advance** Interpretation: primary/secondary endpoint not met.	Overall Correct Prediction Conclusion: statistical insignificant and commercially insufficient Classification: **True Negative (TN)**
1341	**SAR443820** **NCTID: NCT05237284** **Phase: Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: RIPK1 inhibitor** **Area: Nervous System Diseases** **Indication: Amyotrophic Lateral Sclerosis** **Human Patients: 305** **Sponsor: Denali Therapeutics / Sanofi (DNLI/SNY)**	Date: 21 Nov 2023 Quarter: Q1'24 Batch 7 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 16 Feb 2024 **Lead Time: 87 days in advance** Interpretation: primary endpoint not met.	Correct Prediction Conclusion: statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1006	**4D-310** **NCTID: NCT05629559** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: gene therapy** **MoA: AAV GLA gene therapy** **Area: Nervous System Diseases** **Indication: Fabry Disease** **Human Patients: 18** **Sponsor: 4D Molecular Therapeutics (FDMT)**	Date: 2 Dec 2023 Quarter: Q1'24 Batch 8 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 12 Feb 2024 **Lead Time: 72 days in advance** Interpretation: weak efficacy in cardiac endpoints (minimal clinically important difference (MCID) no key renal endpoints with previous clinical hold due to toxcicity.	Correct Prediction Conclusion: more likely to be statistically insignificant and commercially insufficient Classification: **True Negative (TN)**
1409	**CTI-1601** **NCTID: NCT05579691** **Phase: Phase 2** **Class: First-In-Class** **Modality: fusion protein biologic**	Date: 7 Jan 2024 Quarter: Q1'24 Batch 14 Technical: partial SUCCESS (statistically significant yet weak clinical benefit due to TAT delivery)	Readout: 12 Feb 2024 **Lead Time: 36 days in advance** Interpretation: insignificant FXN levels	Correct Prediction Conclusion: statistically significant yet weak and commercially insufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	MoA: TAT-delivered FXN recombinant fusion protein **Area: Nervous System Diseases** **Indication: Friedreich Ataxia** **Human Patients: 28** **Sponsor: Larimar Therapeutics (LRMR)**	Commercial: FAILURE (commercially insufficient clinical benefit inferior to omaveloxolone)		Classification: **True Negative (TN)**
1284	**VX-548** **NCTID: NCT05558410** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: NaV1.8 inhibitor** **Area: Nervous System Diseases** **Indication: Acute Pain After an Abdominoplasty** **Human Patients: 1118** **Sponsor: Vertex Pharmaceuticals (VRTX)**	Date: 12 Oct 2023 Quarter: 2H'23 Batch 23 Technical: SUCCESS (statistically significant clinical benefit compared with placebo) Commercial: FAILURE (commercially insufficient clinical benefit due to non-superior-to-HB/APAP efficacy and inferior-to-HB/APAP toxicity leading to significant risk of post-marketing withdrawal)	Readout: 30 Jan 2024 **Lead Time: 110 days in advance** Interpretation: statistically significant than placebo; non-superior to HB/APAP in all endpoints.	Correct Prediction Conclusion: statistically significant and commercially insufficient Classification: **True Negative (TN)**
1285	**VX-548 HB/APAP** **NCTID: NCT05553366** **Phase: Phase 3** **Class: First-In-Class** **Modality: small molecule** **MoA: NaV1.8 inhibitor** **Area: Nervous System Diseases** **Indication: Acute Pain After a Bunionectomy** **Human Patients: 1075** **Sponsor: Vertex Pharmaceuticals (VRTX)**	Date: 12 Oct 2023 Quarter: 2H'23 Batch 23 Technical: SUCCESS (statistically significant clinical benefit compared with placebo) Commercial: SUCCESS (commercially sufficient clinical benefit due to superior-to-HB/APAP efficacy and non-inferior-to-HB/APAP toxicity)	Readout: 30 Jan 2024 **Lead Time: 110 days in advance** Interpretation: statistically significant than placebo; VX-548 is superior to HB/APAP inSPID48 for bunionectomy (P=0.0016) and non-superior in other endpoints.	Overall Correct Prediction Conclusion: statistically significant and overall commercially sufficient Classification: **True Positive (TP)**
1271	**PRX012** **NCTID: TBD** **Phase: Phase 1** **Class: Best-In-Class** **Modality: monoclonal antibody (mAb)** **MoA: amyloid beta mAb inhibitor** **Area: Nervous System Diseases** **Indication: Alzheimer Disease** **Human Patients: 100** **Sponsor: Prothena Corporation (PRTA)**	Date: 6 Oct 2023 Quarter: 2H'23 Batch 21 Technical: SUCCESS (statistically significant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit inferior to both donanemab and lecanemab)	Readout: 8 Jan 2024 **Lead Time: 94 days in advance** Interpretation: encouraging amyloid beta reduction without numbers (widely accepted strong sign of weak efficacy).	Correct Prediction Conclusion: commercial insufficient inferior to both donanemab and lecanemab Classification: **True Negative (TN)**
961	**ARGX-117** **NCTID: NCT05225675** **Phase: Phase 2** **Class: First-In-Class** **Modality: monoclonal antibody (mAb)** **MoA: C2 mAb inhibitor** **Area: Nervous System Diseases** **Indication: Multifocal Motor Neuropathy** **Human Patients: 54** **Sponsor: argenx (ARGX)**	Date: 30 Nov 2023 Quarter: Q1'24 Batch 4 Technical: SUCCESS (statistically significant clinical benefit) Commercial: SUCCESS (commercially sufficient clinical benefit)	Readout: 8 Jan 2024 **Lead Time: 39 days in advance** Interpretation: 91% placebo-adjusted reduction in the need for IVIg rescue.	Correct Prediction Conclusion: statistically significant and commercially sufficient (no approved SoC for reducing IVIg rescue Classification: **True Positive (TP)**
1259	**LP352** **NCTID: NCT05364021** **Phase: Phase 1 Phase 2** **Class: First-In-Class** **Modality: small molecule** **MoA: 5HT2C superagonist** **Area: Nervous System Diseases** **Indication: Developmental and**	Date: 2 Oct 2023 Quarter: 2H'23 Batch 19 Technical: FAILURE (statistically insignificant clinical benefit) Commercial: FAILURE (commercially insufficient clinical benefit)	Readout: 2 Jan 2024 **Lead Time: 92 days in advance** Interpretation: 32.5% motor seizable frequency reduction compared with placebo.	Missed Prediction Note: due to suboptimal MoA modelling that has been permanently corrected. Conclusion: statistically significant and commercially sufficient

Validated Trial Evidence: Filtered Registry (122 of 604 trials)

Filtered view: **Neurology**

#	Clinical Trial	Certified Prediction	Validation	Result
	Epileptic Encephalopathies **Human Patients: 52** **Sponsor: Longboard Pharmaceuticals (LBPH)**			Classification: **False Negative (FN)**

Appendix: Statistical Methodology & Glossary

KPI FORMULAS

Accuracy
`(TP + TN) / (TP + TN + FP + FN)`

Precision (PPV)
`TP / (TP + FP)`

NPV
`TN / (TN + FN)`

F1-Score
`2 × TP / (2 × TP + FP + FN)`

DOR
`(TP × TN) / (FP × FN)`

Sensitivity
`TP / (TP + FN)`

Specificity
`TN / (TN + FP)`

GLOSSARY

Ex-Ante / Prospective	Prediction issued before outcome is known; timestamped proof
TP	True Positive — predicted blockbuster drug-indication pair with moderately or strongly superior to SoC clinical benefit with HR < 0.6, confirmed by clinical readout
TN	True Negative — predicted non-blockbuster drug-indicatpion pair with weakly superior or inferior to SoC clinical benefit with HR > 0.6, confirmed by clinical readout
FP	False Positive — predicted blockbuster drug-indication pair with moderately or strongly superior to SoC clinical benefit with HR < 0.6, contradicted by clinical readout
FN	False Negative — predicted non-blockbuster drug-indicatpion pair with weakly superior or inferior to SoC clinical benefit with HR > 0.6, contradicted by clinical readout
FIC	First-In-Class — novel mechanism of action and indication pair, no approved precedent
BIC	Best-In-Class — improved agent within an established class
DOR	Diagnostic Odds Ratio — measures discriminative power
MoA	Mechanism of Action — how a drug exerts its therapeutic effect
SoC	Standard of Care — current best available treatment
NCTID	ClinicalTrials.gov trial identifier (e.g., NCT12345678)

DATA PROVENANCE

- All predictions in this dossier are sourced from the BioinvestGPT validated prediction database.
- Each prediction is independently verifiable via its unique index number on data.bioinvestgpt.com.
- Clinical readout outcomes are sourced from official company press releases, ClinicalTrials.gov results, and peer-reviewed literature.
- This dossier was generated on April 8, 2026.

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Clinical trials are inherently uncertain. Regulatory approval, commercial success, and patient outcomes depend on numerous factors beyond the scope of this analysis, including but not limited to: regulatory authority decisions, manufacturing capabilities, competitive dynamics, payer coverage, and post-marketing safety signals.

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