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Normal service resumes in amyotrophic lateral sclerosis



[Madeleine Armstrong](#)

Two recent FDA approvals in amyotrophic lateral sclerosis bucked the usual trend of disappointments in the neurodegenerative disease. But it was back to business as usual this week, with two stumbles in quick succession from Wave Life Sciences and Apellis. In fact, a look at [an Evaluate Vantage analysis of the late-stage ALS pipeline](#), carried out almost a year ago, shows that over half of these projects have since failed. Some developers are pressing on, with Prilenia and Clene still seeing a future for their assets. Such an optimistic stance is no doubt helped by the fact that Biogen and Ionis's Qalsody (tofersen) got accelerated approval for Sod1-mutated ALS despite the antisense therapy [failing its phase 3 trial, Valor](#). Perhaps the most optimistic of all is Brainstorm Cell Therapeutics, which after [FDA knockbacks](#) chose to file its project Nurown under protest, setting up an advisory committee meeting; a date has not yet been set. No such drama from Wave and Apellis, which have thrown in the towel on WVE-004 and Empaveli respectively. Notably one dose of WVE-004 performed statistically worse than placebo, echoing findings with [Biogen and Ionis's similarly-acting BIIB078 last year](#).

Late-stage ALS projects and recent failures

Project	Mechanism	Company	Status
Phase 3			
Masican (masitinib)	CD117, FGFR3 & PDGFR antagonist	AB Science	AB19001 ends Dec 2023
Jacifusen (ION363)	Fused in sarcoma antisense	Ionis	NCT04768972 ends Jun 2025
Phase 2/3			
SLS-005 (IV trehalose)	Natural disaccharide	Seelos Therapeutics	Healey ALS Platform Trial - Regimen E* ; data due Q4 2023
MN-166 (ibudilast/Ketas)	Phosphodiesterase inhibitor	Medicynova	Combat-ALS completes Dec 2023
Recent failures			
Empaveli	Complement factor C3 inhibitor	Apellis/Sobi	Meridian failed May 2023; discontinued
WVE-004**	C9orf72 antisense	Wave Life Sciences	Focus-C9 failed May 2023; discontinued
Reldesemtiv	Troponin activator	Cytokinetics	Courage-ALS failed Mar 2023; discontinued
Pridopidine	Sigma-1 receptor agonist	Prilenia	Healey ALS Platform Trial - Regimen D* failed Feb 2023; Prilenia still seeking path forward
CNM-Au8	Elemental gold nanocrystals	Clene Nanomedicine	Healey ALS Platform Trial - Regimen C* failed Oct 2022; Clene still seeking path forward
Verdiperstat	Myeloperoxidase enzyme inhibitor	Biohaven	Healey ALS Platform Trial - Regimen B* failed Sep 2022
Zilucoplan	Complement factor C5 inhibitor	UCB	Healey ALS Platform Trial - Regimen A* failed Mar 2022

**Investigator-sponsored trial; **Had been in development for C9orf72-associated ALS. Source: Evaluate Pharma & [clinicaltrials.gov](#).*

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