

Biogen and Ionis stumble again



[Madeleine Armstrong](#)

Chalk up yet another disappointment in amyotrophic lateral sclerosis, and another for Ionis and Biogen. Today the partners said they were discontinuing BIIB078 after it failed to show a clinical benefit in a phase 1 trial. There is not much to go on, but worryingly patients receiving a high dose, 90mg, had worsening disease versus placebo; those on 60mg showed no benefit. Stifel analysts reckon this suggests dose-dependent toxicity, which could spell trouble for Ionis's antisense approach - especially after its Roche-partnered Huntington's project tominersen was [linked with a similar phenomenon](#). And Biogen is particularly exposed to this technology, having paired up with Ionis on various CNS antisense projects. The Sod1 antisense tofersen is still in play in presymptomatic ALS, but [its future looks dim after the failure of the Valor trial last year](#). While BIIB078 and tofersen were designed for specific genetic subtypes of ALS, the partners also have a potentially universal therapy, BIIB105, in development; and Ionis has a wholly owned phase 3 asset, jacifusen (ION363), for ALS with mutations in the *FUS* gene. Still, data on the latter are some way off. More immediately, an [adcom this week](#) will discuss the approval chances of Amylyx's AMX0035.

Biogen/Ionis partnered projects

Project	Indication	Description	Status
Tofersen (BIIB067)	Sod1-associated ALS	Sod1 antisense	Failed ph3 Valor in symptomatic pts; Atlas in presymptomatic subjects ends Aug 2026
BIIB078 (IONIS-C9Rx)	C9orf72-associated ALS	C9orf72 antisense	Failed ph1 ; project discontinued
BIIB080 (IONIS-MAPTRx)	Alzheimer's	Tau antisense	Ph1/2 completes May 2022
BIIB101 (ION464)	Parkinson's & MSA	Alpha-synuclein antisense	Ph1 Horizon completes Jul 2022
ION582	Angelman syndrome	UBE3A-ATS antisense	Ph1/2 Halos completes Jul 2023
BIIB094 (ION859)	Parkinson's	LRRK2 antisense	Ph1 Reason completes Sep 2023
BIIB105 (ION541)	ALS	Ataxin 2 antisense	Ph1 completes Dec 2024

MSA= multiple system atrophy. Source: Ionis website, Evaluate Pharma & clinicaltrials.gov.

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