

Biogen's big bet on gene therapy starts to go south



Amy Brown

Biogen paid almost \$1bn two years ago for Nightstar, for two clinical-stage gene therapies for rare eye diseases that cause blindness. One of the two, BIIB112 for X-linked retinitis pigmentosa, looks to be going nowhere. The [Xirus study](#) failed its primary endpoint, which measured vision improvements via a technique called MAIA microperimetry, Biogen said today. Positive trends were observed across several clinically relevant prespecified secondary endpoints, it added, saying the future of the programme had yet to be decided. With nothing available to treat this genetic condition, which typically causes blindness at 40, and no safety concerns with BIIB112, there are perhaps motivations for a resurrection. However, investors with long memories will recall [previous concerns about lack of efficacy with the project](#). Results from a phase 3 trial of the second Nightstar asset, now dubbed BIIB111, are due before the end of this quarter, in the rare inherited retinal disease choroideremia. Investors would rather not have to start worrying about Biogen's deal-making prowess ahead of the US aducanumab decision, although another big test is looming: readout is imminent from the phase 3 Waterfall trial of SAGE-217, another asset gained via a handsome payment.

Paying handsomely: a selection of Biogen's biggest deals

Deal date	Deal description	Target	Deal focus/outcome	Deal Value (\$m)
Nov 2020	In-licensed	SAGE-217/SAGE-324 (Sage Therapeutics)	Depression/neurological conditions; ph3 data pending	\$875m up front (up to \$1.5bn in total)
Aug 2020	In-licensed	DNL151 (Denali)	Parkinson's disease; late-stage development to start YE 2021	\$560m up front (up to \$2.2bn in total)
Jun 2019	Acquisition	Nightstar Therapeutics	Gene therapy; BIIB112 (cotoretigene toliparvovec) failed ph2/3; BIIB111 (timrepigene emparvovec) readout due Q2	\$800m
Nov 2018	Acquisition of ~45% stake	Samsung Bioepis	Biosimilars; work ongoing	\$677m
Apr 2018	Collaboration	Ionis	Neurological diseases, includes BIIB094 in ph2 for Parkinson's disease.	\$375m up front (plus \$625m equity investment)
May 2017	In-licensed	BIIB093 (Remedy Pharmaceuticals)	Ph3 for large hemispheric infarction (ischaemic stroke)	\$120m up front
Apr 2017	In-licensed	Gosuranemab (Bristol Myers Squibb)	Ph2 for Alzheimer's disease (failed in progressive supranuclear palsy)	\$300m up front; up to \$410m in milestones
Feb 2015	Acquisition	Convergence Pharmaceuticals	Pain; lead asset BIIB074 poised to start ph3	\$200m up front; up to \$475m in milestones
Sep 2013	Collaboration	Ionis	Neurological disorders; tofersen in ph3 for ALS	\$100m upfront (in 2018 Biogen paid a further \$35m for a global license)

Source: Evaluate Pharma.

This story has been corrected to say that results from BII111 are due in the second quarter.

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Evaluate HQ
[44-\(0\)20-7377-0800](#)

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