

## Nightstar gives Biogen a cheap way to expand in gene therapy



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### Biogen's \$877m buyout of Nightstar Therapeutics provides yet more evidence that gene therapy is reaching critical mass.

Another week, another gene therapy buyout. This time it is the turn of Nightstar Therapeutics, a UK company that recently listed on Nasdaq, to be bought out, in a move that will see Biogen bolster its presence in gene therapies to treat rare eye diseases.

The deal follows Biogen's 2015 ophthalmology tie-up with AGTC and 2016 collaboration with Regenxbio, though the former was terminated in December after a clinical trial failure. For Nightstar's backers it represents a decent though not spectacular return: the \$25.50 takeout price is 82% above where Nightstar had floated in 2017, but 14% below where it had peaked six months ago.

If nothing else, this shows that if sellers can swallow their pride, and ignore what their companies were theoretically worth when overblown investor exuberance peaked, deals will get done. Nightstar's founding investor, Syncona, is well in the money, for instance, today claiming a 4.5-fold return on its original investment of £56.4m (\$74.4m).

The takeout also vindicates Syncona's pursuit of Nasdaq as an exchange for its portfolio companies ([Interview – Syncona's uncompromising message to the European markets, October 24, 2017](#)). Nightstar would likely have struggled to reach a similar investor following and valuation had it been listed on a European stock market.

That said, the 82% premium to the IPO price is not especially demanding, and does not even put Nightstar in the top 10 takeouts of biotechs that were floated in the past five years ([Takeover flurry aside, progress of recent biotech floats underwhelms, March 4, 2019](#)).

## Acquisitions of biotechs that floated in the past 5 years

Target	Takeover date	Acquiring company	Takeout valuation	Premium to IPO price
Loxo	Jan 2019	Lilly	\$8.0bn	1,708%
Avexis	Apr 2018	Novartis	\$8.7bn	990%
Kite	Aug 2017	Gilead	\$11.9bn	959%
Auspex	Mar 2015	Teva	\$3.2bn	742%
Advanced Accelerator Applications	Oct 2017	Novartis	\$3.9bn	413%
ZS Pharma	Nov 2015	Astrazeneca	\$2.7bn	400%
Spark	Feb 2019	Roche	\$4.8bn	398%
Colucid	Mar 2017	Lilly	\$960m	365%
Neuroderm	Jun 2017	Mitsubishi Tanabe	\$1.1bn	290%
Juno	Jan 2018	Celgene	\$9.0bn	263%
Ignyta	Dec 2017	Roche	\$1.7bn	195%
Armo	May 2018	Lilly	\$1.6bn	194%
Vitae	Sep 2016	Allergan	\$639m	163%
Nightstar	Mar 2019	Biogen	\$877m	82%
Clementia	Feb 2019	Ipsen	\$1.3bn	67%

For Biogen the clear attraction was an acquisition that brought two clinical assets and could still be done at below \$1bn. Nightstar's lead gene therapy, NSR-REP1, is in phase III studies for choroideraemia, while the second asset, NSR-RPGR, is in phase I for X-linked retinitis pigmentosa.

There has been interest in gene therapies for diseases of the eye, with [Johnson & Johnson paying \\$100m](#) in January for rights to Meiragtx projects for treating achromatopsia and X-linked retinitis pigmentosa. Being a distinct, immune-privileged compartment makes the eye relatively amenable to gene therapy.

Of course, Biogen has already been active here, through deals with Regenxbio and AGTC. [The former focused on rights](#) to AAV vectors for treating two unspecified rare genetic vision disorders. The latter is being dissolved after the [failure of an X-linked retinoschisis trial](#) in December.

### Expectations meet reality

It has not all been plain sailing for Nightstar. In September phase I/II data in X-linked retinitis pigmentosa suggested lack of efficacy with low NSR-RPGR doses, and inflammation problems in higher-dose cohorts.

This kicked off a share selloff that brought Nightstar's stock down to a level where Biogen felt comfortable pulling the acquisition trigger. On an analyst call today Biogen insisted that it had seen a sufficient signal of early efficacy.

Both Nightstar assets could be launched in the early 2020s; NSR-REP1 should yield phase III data in the second half of next year, while NSR-RPGR is in a phase II/III dose-expansion trial, but needs a second pivotal study, which has yet to start.

Biogen said the takeover made a good fit with its current focus, given that the retina is part of the CNS. Still, the group remains hugely exposed to next year's pivotal readout of aducanumab in Alzheimer's disease, and Nightstar does little to take the heat off this exceedingly risky catalyst.

But, a week after Roche spent \$4.8bn on Spark Therapeutics, gene therapy has received another vote of confidence.

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