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Neurocrine voyages into gene therapy

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A collaboration over four gene therapy programmes substantially beefs up Neurocrine's pipeline, while Voyager Therapeutics gets a much-needed infusion of credibility.

After hopes of a swift approval for Voyager Therapeutics' Parkinson's disease gene therapy were dashed last year, the company was under pressure to lift investors' spirits. This was achieved today via a broad collaboration with Neurocrine Biosciences, which has bought rights to four projects for a chunky $165m up front, including a $50m equity investment pitched at a substantial premium.

That Neurocrine, flushed with cash and the successful launch of Ingrezza, would chose to pivot to such a high-risk space proves that gene therapy is one of the hottest tickets in town. The company's shares barely budged on the news, despite the announcement representing a substantial long-term investment in a technology that has much to prove.

Voyager stock predictably jumped, surging almost 50% to $11, around the price of the equity investment. A cash injection at a premium will always boost investor confidence, but other benefits to this deal are readily apparent.

The up-front fee means Voyager is financed for an additional two years, last until 2022, while the company will also get to access Neurocrine's experience in getting products through late-stage development and the regulatory process. Voyager's naivety was arguably laid bare here late last year, when the group had to admit that it was going to take much longer to get its Parkinson's project to market than initially hoped (*Voyager's Parkinson's gene therapy journey just got longer, November 8, 2018*).

Neurocrine will presumably be more inclined to set realistic expectations as it strives to be taken seriously as a commercial-stage drug developer. The 2017 launch of Ingrezza has beaten expectations so far – a rare example of a successful independent launch – while opicapone, an add-on therapy for Parkinson's disease, will be filed in the next few months.

Voyager's pipeline		
Project	**Technology**	**Indication**
Phase II		
VY-AADC02*	AAV gene therapy	Parkinson's disease
Preclinical		
VY-FXN01**	AAV gene therapy	Friedreich's ataxia
VY-SOD101	SOD 1 gene therapy	ALS
VY-HTT01^	AAV gene therapy	Huntington's disease
VY-TAU01^^	AAV gene therapy	Taupathies incl Alzheimer's disease
VY-NAV01	AAV gene therapy	Pain, chronic, severe
*Note: *covered by Neurocrine deal; **covered by Neurocrine deal, but Sanofi has ex-US option; ^Sanofi has ex-US option and option to co-promote in the US; ^^in collaboration with Abbvie. Source: company website.*		

Neurocrine has been describing itself as a movement disorder specialist for some time now, so the company is a plausible fit for Voyager programmes. As well as the lead Parkinson's project, VY-AADC, the deal covers a preclinical asset for Friedreich's ataxia, VY-FXN01, and two additional programs yet to be determined. Programmes in ALS and Huntington's are likely candidates.

True, Neurocrine has zero experience in the gene therapy realm, but the terms of the deal see Voyager undertaking all the development work, with the former picking up the cheques. This includes funding the pivotal VY-AADC programme, the phase I trial of VY-FXN01, and development of the undisclosed assets.

Voyager can opt in on the commercialisation of the two lead projects, or choose to receive royalties and milestones; the deal involves up to $1.7bn in potential payments across the four programmes.

Development milestones of up to $170m are attached to the Parkinson's project, the progress of which will be most closely followed by Neurocrine investors. The first of two placebo-controlled pivotal studies, Resolve-1 just got under way, and results are not expected until late in 2021. Executives were vague on when Resolve-2 might get under way, and it will be interesting to see whether Neurocrine's influence will prompt any substantial change in strategy here.

Why Neurocrine would chose to travel down the gene therapy route is another question that investors will be asking today. There is only one other Parkinson's gene therapy in the clinic, Axovant/Oxford Biomedica's AXO-Lenti-PD, so perhaps this was a move to prevent others getting in first. However, the failure of Ingrezza in Tourette's late last year was a painful setback, and this deal hardly slots in another near-term revenue opportunity.

Still, the company became profitable last year, and is sitting on a healthy pile of cash, so perhaps there is more to come from Neurocrine on the deal-making front.

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