

Pfizer's \$150m gene therapy Bamboo shoot



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So far the only big pharma group to have pursued gene therapy to the point of regulatory success is GlaxoSmithKline. But Pfizer is redoubling its efforts, paying \$150m up front for the 78% of Bamboo Therapeutics it did not already own.

This is despite the company, a spin-out from Asklepios BioPharmaceutical, not yet having any clinical-stage products. The deal is also notable for its rarity in this space. It appears that the Bamboo deal is the first time a big pharma has acquired a pure-play gene therapy company.

Pipeline

The focus of the deal is BMB-D001, Bamboo's gene therapy for Duchenne muscular dystrophy, which is scheduled to enter a phase I/II trial by the end of the year. BMB-D001 is a recombinant adeno-associated viral (rhAAV) vector that delivers a shortened but functional copy of the dystrophin gene.

It also has pre-clinical gene therapies for Friedreich's ataxia and the neurological disorder Canavan's disease. The Friedreich's programme uses an rhAAV vector to deliver a functional copy of the frataxin gene to patients with the movement disorder.

The Canavan's therapy has been in development for some years, according to Bamboo's website; an initial clinical trial of an AAV-delivered ASPA gene – which is mutated in Canavan's patients – was begun in 2001. The product was directly injected into the brains of 13 patients between 2001 and 2005.

Five-year data showed decreased brain atrophy, improvement in several areas of neurological function, and decreased seizure frequency. Bamboo is now working on a next-generation version which is still pre-clinical.

The company also manufactured the AAV vector that is being used in an NIH-sponsored phase I/II study in giant axonal neuropathy.

Pfizer's acquisition of this company is therefore a move to add gene therapies to its own pipeline, in contrast to its prior licensing-based efforts in this space. The group has a license to two clinical-stage gene therapies thanks to its tie-up with Spark Therapeutics. Recent results with the haemophilia B candidate SPK-9001 look good ([New data Spark haemophilia B gene therapy battle, June 17, 2016](#)).

First time

When it comes to gene therapy by far the most successful big pharma is GlaxoSmithKline. Its ex vivo cell-based gene therapy, the immunodeficiency treatment Strimvelis, in June became the second ever gene therapy to gain approval ([Therapy focus – Strimvelis approval could rejuvenate gene therapy arena, June 2, 2016](#)).

Glaxo has four other in-house clinical-stage gene therapies. Novartis and Merck & Co are the only other big pharmas with disclosed in-house gene therapy projects.

Licensing agreements are fairly typical of interactions between big pharma groups and gene therapy companies. Takeovers are rarer.

Selected big pharma gene therapy deals

Company	Project	Status	Strategy	Deal source	Deal value (\$m)	Deal date
Novartis	TherAtoH and CGF166	Both phase II	In-licensed	GenVec	214	Jan 2010
Pfizer	SPK-FIX and SPK-9001	Both phase II	In-licensed	Spark Therapeutics	280	Dec 2014
Sanofi	UshStat and StarGen	Both phase II	In-licensed	Oxford BioMedica	53	Apr 2009
Johnson & Johnson	Ad-REIC	Phase II	Company acquisition	Crucell	425	Mar 2011
Sanofi	VY-AADC01	Phase I	In-licensed	Voyager Therapeutics	845	Febr 2015
Amgen	AMT-090	Phase I	Company acquisition	Synergen	254	Dec 1994
Bristol-Myers Squibb	AAV-S100A1	Pre-clinical	In-licensed	uniQure	603	Apr 2015
Pfizer	BMB-D001	Pre-clinical	Company acquisition	Bamboo Therapeutics	645	Aug 2016

More than 20 years ago Amgen bought Synergen, and one of the projects it picked up was a research-stage glial cell derived neurotrophic factor gene therapy. Now known as AMT-090, this has clawed its way into phase I trials in Parkinson's disease – it is also in pre-clinical research for Huntington's – but this is hardly speedy work.

The other big pharma buy that transferred a gene therapy was Johnson & Johnson's acquisition of Crucell five years ago. But Crucell had two years earlier licensed rights to Ad-REIC, a prostate cancer gene therapy, to Japanese group Momotaro-Gene. J&J will simply collect royalties if the project makes it to market.

In any case neither of these acquisitions were focused on gene therapy. Amgen was taking out a rival biotech and J&J was more interested in Crucell's vaccines than its gene therapy. It is therefore not an exaggeration to say that Pfizer buying Bamboo is the first time a big pharma has acquired a gene therapy company.

Pfizer has not staked the family fortune on Bamboo, however. The deal is back end-loaded, with the contract specifying \$495m in milestones. Pfizer knows as well as anyone that despite two gene therapies having made it to market in Europe, this field still has a lot to prove.

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