

## Vertex gets more in vivo gene editing Verve



[Madeleine Armstrong](#)

Vertex's latest tie-up in in vivo gene editing, with Verve Therapeutics, raises plenty of questions. First, the companies are not saying what liver disease they are targeting – although this is not unusual for these types of deals, as shown by the table below. Next, the agreement could spur doubts about Vertex's other forays into in vivo gene editing including its most recent, [a collaboration with Mammoth Biosciences](#). Mammoth is developing "ultra-small" Crispr systems, while Verve traditionally uses more precise base editing, which avoids the potentially harmful double-stranded DNA breaks seen with Crispr/Cas9. However, yesterday's press release does not mention base editing – hopefully it will soon become clear whether Vertex and Verve are indeed using this technology, or something else. To complicate matters further, Verve licenses the base editing tech from Beam Therapeutics; that group's in vivo efforts are largely focused on liver diseases, while Verve, until now, has concentrated on cardiovascular projects, although its [agreement with Beam also covers two undisclosed targets](#). Verve, which took its lead asset, VERVE-101, into the clinic last week, wasted no time in carrying out a \$225m fundraising; its stock slumped 13% this morning.

## Selected in vivo gene editing deals

<b>Big partner</b>	<b>Small partner</b>	<b>Setting</b>	<b>Terms</b>	<b>Date</b>
Vertex	Verve	A single undisclosed liver disease	\$25m up front, \$35m equity, \$406m milestones	<a href="#">Jul 2022</a>
Novartis	Precision Biosciences	Diseases incl SCD & beta-thal	\$75m up front, \$1.4bn milestones	<a href="#">Jun 2022</a>
Pfizer	Beam	Three undisclosed rare genetic diseases of the liver, muscle and CNS	\$300m up front, \$1.05bn milestones	<a href="#">Jan 2022</a>
Bayer	Mammoth	Five indications with an initial focus on liver disease	\$40m up front, milestones >\$1bn	<a href="#">Jan 2022</a>
Moderna	Metagenomi	Undisclosed	Undisclosed	<a href="#">Nov 2021</a>
Vertex	Mammoth	Two undisclosed rare diseases	\$41m up front (includes convertible debt), \$650m milestones	<a href="#">Oct 2021</a>
Apellis	Beam	Six programmes focused on complement-driven diseases of the eye, liver & brain	\$50m up front, \$25m milestones, more if Apellis opts into any programme	<a href="#">Jun 2021</a>
Vertex	Moderna	Cystic fibrosis	\$75m up front, \$380m milestones	<a href="#">Sep 2020</a>
Novartis	Sangamo	3 neurodevelopmental targets, incl autism	\$75m up front, \$720m milestones	<a href="#">Jul 2020</a>
Regeneron	Intellia	5 more liver targets, incl haemophilia A & B (expanded collab)	\$70m up front, \$30m equity, milestones undisclosed	<a href="#">Jun 2020</a>
Biogen	Sangamo	Alzheimer's, Parkinson's, a neuromuscular disease and up to 9 CNS targets	\$125m up front, \$2.37bn milestones	<a href="#">Feb 2020</a>
Askbio (now Bayer)	Editas	Neurological diseases	Undisclosed	<a href="#">Oct 2019</a>
Novo Nordisk	Bluebird Bio (now with 2seventy bio)	Haemophilia A	Undisclosed	<a href="#">Oct 2019</a>
Vertex	Crispr Therapeutics	DMD & DM1	\$175m up front, up to \$1bn inclusive	<a href="#">Jun 2019</a>
Allergan	Editas	Up to 5 ocular programmes (led to LCA10 project EDIT-101, later handed back by Abbvie)	\$90m up front, undisclosed milestones	<a href="#">Mar 2017</a>
Regeneron	Intellia	Up to 10 targets, primarily focused on liver (led to ATTR amyloidosis project NTLA-2001)	\$75m up front, \$50m equity, undisclosed milestones	<a href="#">Apr 2016</a>
Bayer	Casebia JV with Crispr Therapeutics	Haemophilia, ophthalmology and autoimmune diseases (Bayer took step back from JV in Oct 2019)	\$300m to JV plus \$35m investment in Crispr	<a href="#">Dec 2015</a>
Vertex	Crispr Therapeutics	Cystic fibrosis (also led to ex vivo SCD project CTX001)	\$75m up front, \$30m equity, \$420m milestones on any project licensed in	<a href="#">Oct 2015</a>

Source: Evaluate Pharma & [clinicaltrials.gov](https://clinicaltrials.gov).

*The table in this story has been updated to include the Moderna/Metagenomi deal.*

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