

Agios gets \$1.8bn and a new focus



[Elizabeth Cairns](#)



Selling its cancer drugs to Servier leaves Agios more reliant on mitapivat.

Sometimes reasonably okayish is good enough. Seemingly inspired by mitapivat's decent but by no means outstanding performance in sickle cell anaemia at this year's Ash meeting, Agios Pharmaceuticals has jettisoned its oncology franchise in favour of focusing its efforts on mitapivat and its other rare disease assets.

Servier has snapped up the cancer business for \$1.8bn, \$1.2bn of which Agios will put towards share buybacks, helping explain why Agios's investors sent the company's stock up 26% in early trade today. But the pressure on mitapivat has now increased sharply, and it will have to impress when a new tranche of sickle cell data emerge next year.

Agios's oncology business is led by its IDH-1 inhibitor Tibsovo, launched in 2018 for acute myeloid leukaemia. There are also three other candidates: vorasidenib, in phase III for glioma, and two other research-stage projects. Servier will pay a 5% royalty on sales of Tibsovo in the US and 15% on sales of vorasidenib – and US approval of this IDH 1 and 2 inhibitor will also trigger a \$200m milestone payment.

At nearly \$2bn this is the biggest oncology deal Servier has made this year. Its acquisition of Baxalta's Precision Biosciences-partnered Car-T portfolio, and takeover of Symphogen, are either smaller or of unknown value. The French group won out over "17 or 18" other companies for the privilege of buying Agios's cancer portfolio, management said on a call.

The deal is also worth more than half of Agios's pre-deal market cap. Agios says that after handing over \$1.2bn to its shareholders it will have enough cash to see it through to 2025, at which point it hopes to turn its first profit.

Blood

In the meantime it is left with mitapivat, from which an early cut of phase III data were reported at Ash. The project was left standing by Forma's rival FT-4202 ([Forma gets an early edge over Agios in sickle cell disease, December 7, 2020](#)). Full data from this trial are expected early next year.

At least mitapivat did better in the niche disease of pyruvate kinase deficiency, hitting in Activate, one of its pivotal trials ([Agios gets a pre-Ash boost, December 1, 2020](#)). The other pivotal study, Activate-T, is testing the drug in more severely affected patients who regularly receive blood transfusions. This is set to report in the first quarter of 2021 and Agios believes that approval will follow, perhaps in 2022.

Mitapivat is also being developed in beta-thalassemia, with a study planned to start next year.

True, Agios has other non-oncology drugs. AG-946 is an oral activator of wild-type and mutated pyruvate kinase R enzymes that entered a study in healthy volunteers in the third quarter of 2020. The initial focus here is sickle cell disease, judging by clinicaltrials.gov.

But mitapivat must make a clean sweep of all its mid and late-stage trials for Agios's reorganisation to be worthwhile. The clinical readouts due at the beginning of next year will be vital to the company's future.

Agios's pipeline...			
Project	Mechanism	Indication(s)	Trials
Mitapivat	Pyruvate kinase R stimulant	PK deficiency	Activate Ph3 trial hit last month; Activate-T data expected Q1 2021; approval expected 2022
		Thalassaemia	Ph2 study in adults with non-transfusion-dependent α - or β -thalassemia ongoing; ph3 in transfusion-dependent and non-transfusion dependent α and β -thalassemia planned for 2021
		Sickle cell anaemia	Interim ph2 data reported at Ash 2020; full data expected Q1 2021
AG-946	Pyruvate kinase R stimulant	Sickle cell anaemia	First-in-human trial ongoing
...and its ex-pipeline			
Vorasidenib	IDH 1 and 2 inhibitor	IDH-mutant low-grade glioma	Ph3 Indigo study ongoing; primary completion 2024
AG-270	Methionine adenosyltransferase 2A inhibitor		Ph1 trial in methylthioadenosine phosphorylase-deleted NSCLC and pancreatic cancer ongoing; primary completion 2023
AG-636	Dihydroorotate dehydrogenase inhibitor	Non-Hodgkin lymphoma	Ph1 trials ongoing

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