

## Agios's pricing dilemma



[Elizabeth Cairns](#)



### How can the company wring the most value from Pyrukynd?

Agios has a newly approved drug and a problem. Its pyruvate kinase R activator mitapivat has just got the nod from the FDA as a therapy for the rare disease pyruvate kinase deficiency, and will go on sale as Pyrukynd at \$334,880 a year.

The trouble is the pill is also in late-stage development for thalassemia and the more common blood disorder sickle cell disease. Should it gain approval here too, a high price in the latter indication will be hard to justify, which means one of two possibilities – neither of which offers much comfort to Agios and its investors.

Pyrukynd is the first therapy to be approved by the FDA for PKD, which affects around 3,000 people in the US. Around 2,000 patients have thalassemia, but sickle cell has around 100,000 US patients.

The pill is in two phase 3 trials in alpha and beta thalassemia, and a phase 2/3 in sickle cell. Should these lead to approval, Agios has some decisions to make.

#### To cut or not to cut

On a conference call today Agios's chief commercial officer, Richa Poddar, said the company expected to maintain the same price for thalassemia as for PKD. For sickle cell the calculation is harder.

Two sickle cell products are already on sale in the US. Global Blood Therapeutics' Oxbritya carries a list price of \$125,000 per year; Novartis's Adakveo comes in at around \$88,000. Were Agios to carry over the PKD pricing to this disease, uptake could be poor in the absence of materially stronger supporting data.

Ms Poddar said sickle cell pricing would depend on the pivotal [Rise Up study](#), which is assessing two endpoints: haemoglobin response and the annualised rate of vaso-occlusive crises. Counterintuitively, hitting just one of these could result in a higher price.

"Given the size of the [sickle cell] patient population, we can take a price discount but still have overall value creation for Pyrukynd across all three indications," Ms Poddar said. "If we see only one of the endpoints like haemoglobin ... we could go for a salvage patient population and maintain price across all three indications."

But if Agios does have to price Pyrukynd lower in sickle cell than in PKD and thalassemia, unfortunate sales dynamics could play out in the market.

One possibility is that the price for the other conditions is dragged down. An Agios spokesperson told *Evaluate*

Vantage last month that “the assumption among investors is that, if the price goes down for [sickle cell disease], that would be across the indications”.

There is another prospect. An obvious temptation might be to prescribe the pill off-label for the rarer conditions, using the cheaper sickle cell pricing.

Today is a vindication for Agios’s refocusing as a rare disease player ([Agios gets \\$1.8bn and a new focus, December 21, 2020](#)). Now it must wait and see what the data show.

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