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Interview – Alnylam needs to get the price right for patisiran

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Alnylam came a long way in 2017, vanquishing its amyloidosis rival Ionis and filing its lead project, patisiran, for approval. But the company still has several hurdles to clear if patisiran is to become a commercial success – and getting payers onside is no doubt one of the biggest obstacles.

Alnylam might become the latest group to go down the outcomes-based payment road, its chief operating officer, Yvonne Greenstreet, tells *EP Vantage*. Its fate is now in its own hands after it regained global rights to patisiran under a restructured deal with Sanofi, and is confident of taking on the hereditary amyloidosis market alone.

“We’re probably better focused on the rare disease space, where you require a much higher-touch approach with patients and you’ve got smaller numbers of physicians to address,” Ms Greenstreet says. She reckons that a 250-strong team will be needed to commercialise patisiran, comprising 75 employees in the US, the same number in Europe, and another 100 in the rest of the world.

Risk sharing?

Alnylam will no doubt be treading carefully as it sets patisiran’s price, but is keeping its cards close to its chest. Ms Greenstreet will only say that the RNAi project’s price will be in line with that of other rare disease therapies; meanwhile, analysts expect patisiran to cost $200,000-400,000 per patient per year.

Drug costs have become a political hot potato, and the advent of expensive new modalities like gene therapy and CAR-T has not helped the perception that biopharma is overcharging – although some argue that price rises for older drugs, rather than the cost of novel therapies, are to blame (*Vantage point – Biotech gets creative about the pay cheque, January 23, 2018*).

Still, companies including Glaxosmithkline, Novartis and Spark have been forced to get creative with payment models for gene and cell therapies. This approach is something that Alnylam might also consider, says Ms Greenstreet: “We haven’t priced patisiran yet, but we’re very committed to exploring performance-based arrangements. We’d like to be able to risk-share with payers.”

First Alnylam has to get approval for patisiran. This looks likely based on stellar data from the Apollo study. The project has US priority review status, with an approval decision expected by August 11. The EMA accepted Alnylam’s European application in January, and a CHMP opinion could come as early as July, Jefferies anlaysts speculate.

Taking heart

Much depends on how broad a label patisiran receives. Historically, hereditary transthyretin-mediated amyloidosis (hATTR) has been categorised depending on where the damage is most severe – into polyneuropathy for those with nervous system damage or cardiomyopathy for those with heart disease – and there have been questions over whether patisiran will get the go-ahead in patients with cardiac involvement.

Ms Greenstreet seems confident that this is no longer an issue: “Now it’s really quite clear that it’s one disease.” She adds that this, alongside “compelling” cardiac data from Apollo, should convince regulators “that the indication we should get is for the treatment of hATTR amyloidosis, full stop.”

Even if patisiran ends up with a narrower label, “maybe one that’s restricted to hATTR with polyneuropathy, we will definitely get these very exciting cardiac endpoints featured on our label”, Ms Greenstreet says.

Another question is how patisiran will compete with Ionis’s inotersen, which is set to reach the market first – inotersen also has US priority review status and a PDUFA date of July 6.

Patisiran is expected quickly to make up lost ground. Alnylam and Leerink analysts both reckon that it will ultimately capture 75% of the market. *EvaluatePharma* sellside consensus puts patisiran’s 2022 sales at $758m versus inotersen’s $497m.

Next generation

As well as expecting to have its hands full with the patisiran launch, Alnylam has various other projects on the go, including a next-generation amyloidosis candidate, ALN-TTRsc02, to which it also regained full rights under the restructured Sanofi deal.

ALN-TTRsc02 has the advantage of being given subcutaneously, while patisiran is administered intravenously. Alnylam also hopes that the new candidate could broaden the population addressed by its therapies, and has said it plans to test ALN-TTRsc02, which is slated to go into phase III development in late-2018, in other indications including wild-type ATTR.

Wild-type disease affects four times as many patients as hereditary amyloidosis, Leerink analysts estimate, adding that ALN-TTRsc02 will be less profitable than patisiran and could cannibalise the older product.

Still, it will be a while until Alnylam has to worry about this. For now, it needs to focus on getting the price – and the launch – of patisiran right.

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