

Alnylam: The \$12bn company with the \$2bn war chest



[Jonathan Gardner](#)

When a company ticks off as many items from its to-do list as Alnylam has in the past two weeks, it is almost inevitable that a massive fundraising follows. The RNAi specialist announced a \$675m share sale today, to support, among other things, the commercial and payer liaison capabilities that would be necessary following approval of amyloidosis agent patisiran.

Alnylam has many irons in the fire, of course, and the \$1.8bn cash pile that it will have amassed could erode quickly in the midst of a commercial launch, multiple phase III trials and a rich mid-stage pipeline. It may not be long until investors start looking eagerly to the day that the group can begin using its own revenue stream to fund operations.

Alnylam shares sank 5% in early trading today following the announcement. It is seldom a good time to ask investors for more money, but as shares were at a record high on Friday, it made sense to top up the war chest.

Good news, but ...

While there is mostly good news surrounding Alnylam right now, among the uncertainties is how the phase III trial of the Medicines Company-partnered cholesterol-lowering project inclisiran will ultimately be supported. Medicines Company has long said it does not intend to fully finance the phase III Orion programme – launch of which was announced last week – a partnership has yet to emerge and year-end deal signing has been targeted.

Nonetheless, advancing this agent into phase III can be seen as good news for Alnylam amidst a plentiful period.

The big event of the past two weeks was full readout of data from the Apollo trial of Alnylam's patisiran in hereditary ATTR amyloidosis, which clearly suggest that it will have an efficacy edge over Ionis's inotersen ([Alnylam confirms amyloidosis advantage, November 3, 2017](#)). The Massachusetts-based company plans on submitting a new drug application to the US FDA by the end of 2017, trailing inotersen only slightly, and just today announced that the European Medicines Agency has granted an accelerated review to its marketing application.

But good news arrived on several other agents. The Medicines Company-partnered cholesterol-lowering project inclisiran began its first phase III trial, along with the wholly-owned givosiran in acute hepatic porphyrias – the latter of these two is expected to report data by mid-2018, with US FDA submission targeted for the end of 2018.

Haemophilia project fitusiran, dosing on which was suspended in September because of a fatal thrombotic event, has now been cleared for a phase II open label extension and initiation of a phase III trial by the end of 2017. The new safety protocols include physician and patient education on reducing the use of factor replacement therapies while undergoing treatment with fitusiran, which aims to reduce the body's production of antithrombin to enable coagulation.

What's next

That is a full plate for any company, but even more so for a group that has not reached commercial stage, which helps explain the magnitude of the fundraising.

On top of that, launch of patisiran may not necessarily be straightforward, necessitating some careful work with payers as this agent approaches the market – Alnylam has a co-development deal with Sanofi on this project. While RNA and DNA therapeutics are an emerging part of the treatment landscape, payers remain wary of their costs – even if, in the case of hereditary ATTR amyloidosis, liver transplant is in the offing for many patients, and many other complications require frequent treatment.

The price probably will not be known until after regulators give their blessing, but work with payers will need to

begin before any such decision. Alnylam will need a skilled team in place to navigate launch – success of which could give investors some hope that one day they will no longer be asked to withstand another half-billion-dollar-plus cash call.

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