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January 13, 2014

Alnylam lives the biotech dream as big pharma returns to RNAi

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It was abundantly clear that Merck & Co's $1.1bn purchase of Sirna Therapeutics was going nowhere fast, so the group's sale of this asset for a mere $175m to Alnylam will do little more than confirm that the 2006 acquisition had been severely overpriced.

Far more surprising is the other deal that Alnylam has pulled off, attracting a $700m investment from Sanofi in what looks like the biggest endorsement of RNAi since big pharma effectively exited the space in 2010. Alnylam's chief executive, John Maraganore, is officially living the biotech dream, having bought low from one big pharma and sold high to another in a single day.

Sanofi had earlier picked up rights to Alnylam's lead project, patisiran, for treating the rare disease transthyretin-familial amyloid polyneuropathy, in Japan and the Asia-Pacific region. Today's alliance expands this to all territories outside the US and Western Europe.

The news comes after an amazing 12 months for Alnylam, whose stock ended Friday up 228% in the past year despite a relatively early-stage pipeline. The group's RNAi technology is clearly convincing investors where others have struggled, and Alnylam raised $174m a year ago on the back of the enthusiasm.

Problem solved?

Its success has owed much to overcoming a major obstacle. RNAi – RNA interference – involves the use of short RNA molecules to bind to specific mRNA sequences and thus silence gene expression, and the problem has been how to deliver RNA fragments into the cytoplasm.

But Alnylam made a major breakthrough with subcutaneous delivery this year (*Alnylam's subcutaneous success drives shares to record high, July 12, 2013*). Indeed, the company says it has solved the problem as far as delivery into the liver is concerned.

Sanofi also gets rights to three more Alnylam projects, plus an option to pick up ex-US/Western Europe rights to any of Alnylam's rare genetic disease assets.

In return it has cut Alnylam a $700m cheque for new equity amounting to a 12% stake, at a 21% premium to Friday's close. Alnylam finished the third quarter with $194m in the bank, and boasts that the added cash will transform it, securing it beyond several product launches.

The $700m windfall is a huge endorsement for RNAi, which was once seen as a highly promising area, with Merck & Co spending $1.1bn to buy Sirna, and Roche shelling out some $500m to emulate this effort. But such early enthusiasm hit the buffers three years ago, when one by one the big pharmas involved in RNAi said enough was enough.

Roche offloaded its work to what is now Arrowhead Research for nothing more than a 10% equity stake plus milestones and royalties; Novartis declined to take an option over Alnylam's IP; Pfizer and Abbott Laboratories exited some work in the area. Were it not for the Sanofi deal, today's sale of the Sirna asset would have completed the rout.

Huge discount

Alnylam has picked up Sirna from Merck for just $25m in cash plus $150m in equity, as well as $105m in milestones and single-digit royalties.

On a call today Alnylam insisted that Merck had done "tremendous work" with Sirna since its acquisition, but said that key staff tended to leave when big pharmas just bought up assets. Thus Sirna's sale at a huge discount simply showed that the ownership model did not work, it opined.

For Alnylam to have pulled this off at the same time as securing $700m from Sanofi looks like one of shrewdest biotech moves of recent times. The fact that orphan diseases are concerned must also have helped.

Alnylam was up 44% in early trade today, and the enthusiasm also infected other RNAi stocks: Arrowhead was up 11% and Tekmira Pharmaceuticals put on 23%. If Sanofi's bet really does mark the turnaround in the sorry fortunes of the technology, Dicerna Pharmaceuticals, an extremely early-stage RNAi company, might have timed its proposed $69m float to perfection.

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