Cephalon closes the blast doors for a rocketing stem cell deal

The pharma industry waited nearly the entire year to strike the biggest licensing deal of 2010, and very few would have predicted it would be for stem cells. As such, Cephalon’s agreement with stem cell developer Mesoblast, worth potentially more than $2bn, has left some analysts rather startled.

Cephalon will pay a sizeable $130m upfront, and will also acquire a 20% stake in the Australian company at a decent 45% premium to Mesoblast’s 30-day share price average; the stock gained 21% today to a record high of A$4.05. The deal, which appears to have been facilitated by Mesoblast’s recent acquisition of US sister company Angioblast, will see Cephalon develop and commercialise Mesoblast’s stem cell therapies for cardiovascular and neurological disorders after completing phase IIa. However, achieving proof-of-concept and gaining a first regulatory approval in this notoriously high-risk arena are unquestionably massive hurdles (Behind disappointment stem cell therapy showing pluripotential, October 12, 2010).

**Specialist seeks commercial partner**

Since its 2004 inception, Mesoblast has established itself as a specialist in the isolation and engineering of adult mesenchymal precursor cells (MPC) – naturally-occurring, multipotent stem cells able to differentiate into any type of cell in the body, and help regenerate tissues.

The Antipodean developer originally focused on bone and joint disorders, while another company, Angioblast Systems, founded in the US by Mesoblast’s chief executive Silviu Itescu, researched stem cell therapy for cardiovascular disease.

Mesoblast originally licensed rights to Angioblast’s products; however, just a few weeks ago Mesoblast acquired Angioblast outright, to consolidate all their activities under one umbrella.

Given that Mesoblast needed a strong commercial partner, but did not have complete ownership of Angioblast’s candidates – something that may have put off potential suitors in this high-risk arena – it would seem that its acquisition of Angioblast was implicit in attracting Cephalon.

The short period between Mesoblast acquiring Angioblast and striking the deal with Cephalon suggests discussions between the companies must have been ongoing for some time; it seems likely that Cephalon was waiting until the merger with Angioblast was finalised before making its move.

**Largest in its space**

Under the terms of the agreement, which as Mr Itescu pointed out in a conference call yesterday, is the “largest transaction in the regenerative medicine space”, Mesoblast will develop its cardiac and neurological stem cell programmes through phase IIa proof-of-concept, after which Cephalon will take over development and commercialisation and could pay in excess of $1.7bn to Cephalon if all milestones are met. Cephalon will pay for half of all development activities, including pre-clinical work, for the neurological programme.

Despite a very favourable upfront payment, and the $220m for a 20% stake, the risk still weighs heaviest on Mesoblast, which needs to achieve the ever-elusive proof-of-concept for stem cell therapeutics before Cephalon even touches the candidates.

The most advanced therapy covered by the deal is Revascor, a drug in phase IIa trials for congestive heart failure and acute myocardial infarction. A 12-month safety study is ongoing and Mesoblast concedes it will not be clear on whether Revascor can be handed to Cephalon and moved into pivotal trials at least until these results are in.

Nevertheless, the Cephalon deal means funding for the bulk of this clinical work shouldn’t be a problem for Mesoblast, which already had a two-year cash runway before today’s event.

**Surprised**
Analysts at UBS who cover Cephalon were surprised by the deal, and reckoned investors would be particularly cautious given the big money and large risks involved; Cephalon shares dipped 3% in early trade today to $63.90.

The bank also sees high risks for heart failure research in general, an area which has seen its fair share of failed trials. Whilst crediting Cephalon with an ability of “finding diamonds in the rough”, the UBS research note today continued by saying “this is the first deal that we can remember where we are really scratching our head.”

At the very least this deal shows that faith in stem cells is still alive, and allows Mesoblast the opportunity to conduct more extensive early-stage research, although a similarly sized stem cell deal, Genzyme’s licensing of Osiris Therapeutics’ MPCs Prochymal and Chondrogen in late 2008 for $130m upfront, has failed to live up to expectations (Osiris left clinging to a niche after Prochymal failure, September 9, 2009).

While Genzyme's experience in this field will be of concern for Cephalon’s shareholders, perhaps Cephalon's management sees potential in Mesoblast’s cell lines that other observers have overlooked or discounted.