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Pharma news over the Christmas period

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While 2017 closed in far ruder health for biotech investors than many had dared hope for, lingering doubts about the sustainability of the market for flotations persist, as do fears that shareholders are pricing biotechs out of the market for business development deals.

So it will come as a relief that the Christmas holidays, normally a quiet period, showed that some of these fears might be unfounded. Bankers worked hard to get several IPO documents filed, and there was no deal drought, with Mallinckrodt buying Sucampo just after Roche's surprise swoop on Ignyta (*Ignyta investors have the last laugh, December 22, 2017*).

December 29

As the clock ticked down to the New Year Valeant and Bill Ackman's activist Pershing Square fund felt enough festive spirit to propose ending shareholder litigation relating to accusations of insider trading around the time of Valeant's botched attempt to buy Allergan back in 2014.

Relevant Allergan investors are to receive $290m – the deal is subject to judicial approval – though Valeant will only foot a third of this; the remainder will fall to Pershing, which had amassed an Allergan stake around the time of the attempted takeover. No doubt the fact that the case was due to come to trial on January 30 had focused the parties' minds.

Four biotechs filed to go public, three on the back of assets licensed from other groups. Restorbio, a subsidiary of Puretech Health, filed to raise up to $85m in a Nasdaq IPO focused on age-related respiratory tract infections and its lead asset, RTB101/BEZ235, which started a phase II study in November.

Puretech had licensed BEZ235, which targets the rapamycin complex 1 (TORC1) pathway, last year from Novartis in a deal that effectively kicked off Restorbio. A follow-on TORC1 inhibitor is apparently at the discovery stage.

Solid Biosciences hopes to raise up to $100m to develop its Duchenne muscular dystrophy gene therapy SGT-001, currently in a phase I/II study. SGT-001 aims to restore functional dystrophin protein expression and holds US rare paediatric disease designation, and might thus also become eligible for a priority review voucher.

Armo Biosciences is the only one of the four prospective floaters with a focus on oncology: AM0010 is a pegylated IL-10 licensed from Ligand Pharmaceuticals in monotherapy and combo clinical trials in various cancer types, and has already featured at scientific meetings including AACR and Asco-GI. Armo's float seeks $86.3m.

December 28

A day before those three proposed IPOs Menlo Therapeutics, a biotech formerly known as Tigercat Pharma, announced a $97.8m float to develop serlopitant, an NK-1 receptor antagonist licensed from Merck & Co. The lead indications are pruritus, for which Menlo has started a phase II trial, and refractory chronic cough.

December 26

As for bigger deals, Mallinckrodt's $1.2bn Boxing Day acquisition of Sucampo includes two marketed products in the shape of Amitiza for constipation and the eye disease drug Rescula, along with two phase III rare disease projects. Any suggestion that the price tag is rich could be explained by Mallinckrodt's need to reduce reliance on its best seller, H. P. Acthar Gel, whose sales have recently fallen.

Sucampo believes that its first late-stage candidate, VTS-270, could be a disease-modifying therapy for Niemann-Pick disease, a fatal inherited lipid disorder whose symptoms are managed with Johnson & Johnson's Zavesca. The Mallinckrodt acquisition has raised hopes for the pivotal trial of VTS-270, which if positive will allow an FDA submission this year.

Reading out a little later will be a phase III study of CPP-1X in another orphan indication, familial adenomatous polyposis. The companies forecast peak sales of VTS-270 and CPP-1X at $150m and $300m respectively.

Mallinckrodt must believe that it can increase these figures to justify its outlay for Sucampo.

Sucampo's late-stage pipeline			
Project	**Indication**	**Trial**	**Primary completion**
VTS-270	Niemann-Pick disease, type C	NCT02534844	Mar 2018
CPP-1X/sulindac	Familial adenomatous polyposis	NCT01483144	End 2018

December 22

The run-up to Christmas saw continued uncertainty for Portola's blood-thinner antidote Andexxa, with the US FDA delaying the date for an approval decision from February 3 to May 4.

There appears to be nothing to the delay beyond a need for extra time to review additional data submitted by Portola. However, with the agency having already rejected Andexxa once, and the project carrying an NPV of $4.3bn, based on *EvaluatePharma's* sellside consensus, Portola's stock fell 11%.

Among 2017's CAR-T developments one of the most curious was the emergence of a Chinese company, Nanjing Legend, whose LCAR-B38M secured a prized multiple myeloma late-breaker at Asco. Still, despite the clinical data looking stellar, questions remained: how reliable was a trial conducted in Asia? How representative of the target population were these relatively early-stage patients?

These doubts were swept away when Johnson & Johnson handed over $350m for a share of global rights to LCAR-B38M. Nanjing's aim has been to start a clinical study in the US, so J&J will clearly help with this; but for the US group to have been enticed into doing a deal before seeing any western patient data is quite a coup.

On the same day Array Biopharma whetted bankers' appetites by setting up a subsidiary, Yarra – Array spelled backwards – to develop its p38 MAP kinase inhibitor ARRY-797, in phase II for the rare disease LMNA-related dilated cardiomyopathy. While Yarra is wholly owned by Array, it intends to seek outside equity financing.

One big biotech that could have done with a better end to 2017 is Celgene, and there had been hopes for new indications for its cash cow Revlimid. Unfortunately the failure of its Relevance trial has closed the door in first-line follicular lymphoma, which according to Leerink analysts could have added $1.3-1.4bn in sales.

Another label-expansion study due to report by the end of this quarter has thus become even more important: Augment is evaluating Revlimid plus Rituxan versus Rituxan alone in relapsed/refractory follicular lymphoma (*Celgene slide piles pressure on Revlimid, October 26, 2017*). This might have a greater chance of success, but it is a smaller opportunity, at only $600-700m a year.

It will not go unnoticed that Celgene kicks off next week's JP Morgan conference, biotech's annual tone-setting investor jamboree. After lowering long-term guidance Celgene ended December down 12% on the year; investors will look to its JP Morgan pre-announcement with hope that 2018 is not even tougher than 2017.

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