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JP Morgan day two roundup – quiet, but no bad news

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Investors seem to have come to terms with the fact that this year the JP Morgan healthcare conference is unlikely to be a rabble-rouser. Day two of the confab saw the clearing of rainy skies and a recovery in the Nasdaq biotech index from Monday's slide, as little stand-out news emerged.

Nektar was the winner of the day, adding another $1.7bn to its bloated market cap on the basis of a smattering of new data. Sage Therapeutics and Bluebird Bio, another two very closely watched small drug developers, also took their turn to bang the drum, while Axovant's beleaguered chief executive left observers with no illusions about his feelings. *EP Vantage* rounds up the big news of the day.

Blossoming potential

Despite **Nektar** being on the verge of filing a novel opioid for approval in chronic pain, the group's Q&A session was dominated by its much earlier-stage immuno-oncology asset, NKTR-214. In the past few months this has become one of the hottest pipeline projects in this space, based on its potential to turn "cold" tumours "hot" (*SITC – Nektar's plan to make cold tumours blossom, November 14, 2017*).

The hope is that NKTR-214 will enable patients with PD-1 negative tumours – a substantial majority – to receive treatment with checkpoint inhibitors. Yesterday the company presented data from the Pivot trial of NKTR-214 plus Opdivo, most of which had already been seen, but gave incremental updates in a couple of tumour types that confirmed very impressive response rates.

Chief executive Howard Robin did not hold back with some very excitable predictions of great things to come from NKTR-214, and investors obliged once again. The company's market cap has surged in the last few months and yesterday its shares jumped another 19%, taking Nektar's valuation close to $11bn. With much phase I data due over the remainder of 2018, this stock remains one to watch.

Another company riding high is **Sage Therapeutics**, which has seen its market value balloon on the back of a series of successful trials of its novel depression agents. Chief executive John Jonas addressed investors at a rapid pace, and with a packed year ahead that will yield more important data readouts, see multiple trial initiations and the filing of its lead asset, Sage will need to keep moving at a fair lick.

Investors are already betting that things will go well – the company's market cap, which has almost tripled since the beginning of November, currently sits at $7bn. Interesting snippets to emerge at JP Morgan included confirmation that work with SAGE-217 in biopolar depression will start this year, while work in essential tremor will be refocused on a new agent, SAGE-324.

Many investors will be disappointed if either Sage or Nektar are found presenting next year. However, with the latest quarterly M&A data showing few signs of a rebound, these closely-watched bid targets will need to make a strong case for these kind of valuations.

The same applies to **Bluebird bio**, and its effusive chief executive, Nick Leschly, also delivered a typically upbeat presentation, predicting that the company will file three projects for approval by the end of 2019. Its gene therapy Lentiglobin will go to regulators this year in beta-thalassaemia, followed next year by filings for a second gene therapy Lenti-D, for a rare childhood neurological disease, and the Celgene-partnered multiple myeloma CAR-T, bb2121.

The focus of the company is now on delivery, he stated, and naturally thoughts are turning to pricing. Notably, Mr Leschly claimed not to be a fan of strategies based on "what can the market bear or what can we get away with", and later went on to commend Spark's risk-sharing strategy for Luxturna.

Having just raised a huge $651m in an oversubscribed share sale, Mr Leschly can afford some hubris – he boasted that the company's $1.6bn cash runway will last until 2021. And of course as another hotly tipped takeover stock these issues might not concern him for much longer. Celgene is considered a natural suitor, though others would no doubt be interested.

Elsewhere

CAR-T competitor Juno also presented on day two, and highlighted its own anti-BCMA CAR-T therapy JCARH125, which it hopes to move into the clinic in multiple myeloma shortly. Chief executive Hans Bishop said Juno had been encouraged by the success of similar agents, and that they see potential in earlier lines of therapy with their asset, which has been developed with durable remissions in mind.

Still, with Celgene and Bluebird well ahead in BCMA targeting, Juno's plans for a swift clinical programme will need to proceed without a hitch to stay in this game. The same issue of timeliness applies for JCAR017, which Juno now refers to as liso-cel, and was the main focus of the presentation.

The company believes liso-cel could be approved by the end of this year and Mr Bishop stressed the agent's relatively benign tolerability profile and potential for use in an outpatient setting. Juno believes this could be an important differentiator but again the company is well behind Novartis and Gilead, both of which are in the launch phase with their CD19-targeted assets, Kymriah and Yescarta.

Biontech became the second private mRNA specialist to present to a packed room, following Moderna yesterday, with interest no doubt piqued by last week's massive $270m fund raising. The series A round is thought to be the biggest ever for a European biotech.

While the presentation focused on its mRNA candidates, the group also snuck in news that clinical trials of its CAR-T candidate for ovarian cancer will not start until 2019 – it previously said they would be under way in first half of 2017, so it represents a significant delay to its work in cell therapy (*Biontech deal sees Roche bet on mRNA, September 21, 2016*)

As Biontech is a privately owned company there was no immediate indication of whether this is a huge disappointment, or just normal pipeline prioritisation, but it could well be a sign that the preclinical research did not go as smoothly as expected. Investors are likely focused on near-term catalysts like phase II data from a head and neck cancer vaccine – in any case, as most of the progress in CAR-T has so far been in haematological cancers, Biontech's focus on a solid tumour means this delay may not put it too far behind any potential competitors.

Bad to worse

Finally, Axovant's conference went from bad to worse, and chief executive David Hung clearly did not relish the opportunity to stand up and explain himself in front of investors, who had witnessed his company crash to cash value on Monday.

According to reports he took only 11 minutes to talk through the abandoning of Alzheimer's project intepirdine and outline plans to license in a new project, before exiting the stage. In the Q&A session he then had to apologise for an "embarrassing" typo in a press release about a follow on asset nelotanserin. The bets are on as to whether Mr Hung, in an Axovant hat or any other, will be presenting at next year's conference.

An EP Vantage staff report, with reporting by Madeleine Armstrong in San Francisco. For live updates from the JP Morgan healthcare conference in San Francisco on January 9-12 follow @ByMadeleineA on Twitter. To contact the writers of this story email news@epvantage.com.

See our full coverage from the JP Morgan conference here.