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JP Morgan day three roundup – Trump reminds the sector who's in charge

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Biotech investors should have been pleased with the chunky research deal delivered by Vertex on day three of the JP Morgan conference, but instead the US president-elect spoiled the party. Donald Trump's vigorous pledge to act on drug prices ended the Nasdaq Biotechnology Index's 2017 rally and hit pharma stocks elsewhere, reminding the sector what the real story is going to be this year.

News from elsewhere did manage to filter through, however, and Mylan's embattled chief executive uttered what could become a profound prediction for the coming year. A development setback with mRNA from Curevac served as a reminder of likely slow progress in this hot space, while other stock market darlings, Bluebird Bio, Kite Pharma and Editas, all highlighted approaching crucial clinical milestones.

The new normal

"If anyone's walking away from this conference thinking it's business as usual, that's a mistake." So said the embattled Mylan chief executive, Heather Bresch, arguably providing the quote that could come define this year's JP Morgan conference.

She was talking more about the need for US business models to change rather than specifically referring to the comments president-elect Donald Trump had made an hour or so before, ravaging healthcare stocks. However both proclamations highlight gathering momentum for real action on US drug prices, the prospect of which is looking increasingly hard to brush off (*Fickle Trump erases biopharma's golden day, January 12, 2017*).

Ms Bresch said more than incremental changes in pricing models were needed to achieve this, and that "truly rethinking the business model" would be required. This has to happen because the system as it stands is not sustainable, she said, adding that a "market-based solution" would be preferred. It did not seem possible that drug pricing could become a more dominant theme for the sector this year – but it just did.

Takeda becomes Maverick's wingman

Merck and Vertex provided the biggest deal news of the day, although Takeda announced a fairly chunky early-stage move with the freshly formed Maverick Therapeutics (*JP Morgan – Vertex and German Merck find their perfect fit, January 12, 2017*).

The T-cell specialist, spun out of Harpoon Therapeutics last year, has developed a way of creating antibodies designed to be active only in the tumour microenvironment. This should eliminate off-site toxicity and allow its therapies to hit previously intractable targets, according to the California biotech.

As part of the deal Takeda has invested $125m in Maverick – a sum that includes an up-front fee and equity and R&D payments – and has an option to buy the company after five years.

Curevac setback

Two days after the mRNA megastar Moderna finally lifted the lid on some of its work, its lower-profile but more clinically advanced rival Curevac slipped out a major clinical setback in its conference presentation. Its lead project, the mRNA-based prostate cancer vaccine CV9104, failed in a phase IIb trial – pertinent news now that Moderna has unveiled a focus on vaccines.

The companies' technologies are very different, of course, but Curevac's hypothesis that mRNA-based cancer vaccines will need to be used in conjunction with checkpoint inhibitors perhaps illuminates the path that Moderna might take in its collaboration with Merck in this space. With reports of safety setbacks at Moderna also emerging this week, the Curevac news serves to remind that progress in this area is very likely to be slow.

Approaching top line

The most important aspect of Kite's JP Morgan discussion concerned the pivotal cohort of KTE-C19's Zuma-1

trial, which has so far only yielded three-month data. The last of 51 DLBCL patients completed six-month follow-up on January 7, but in the meantime Kite started a rolling US filing in the broader setting of B-cell non-Hodgkin's lymphoma.

The group also decided to increase enrolment into this already complex trial by around 50%, as a result of which six-month results – vital for its rolling BLA – will concern 101 lymphoma patients. It said the six-month data would be toplined in a press release in the first quarter, before presentation hopefully at Asco.

The risk of responding patients relapsing remains a major threat to the dataset, though Kite stressed the delayed efficacy it has seen, meaning that conversely some partially responding patients could in time go into full remission. Among incremental early R&D updates the company revealed that a third-generation, humanised – rather than murine or fully human – anti-CD19 CAR-T project would feature an on-off switch licensed from Cell Design Labs last year.

Bluebird sings its mojo

Bluebird bio delivered an energetic presentation and breakout – its chief executive, Nick Leschly, described four programmes "charging towards the net" and laid out a timetable for clinical data readouts this year.

An update on the phase III beta-thalassemia study of its gene therapy Lenti-D will be provided at EHA in June, and patient numbers will be small although the impact of a new manufacturing process will be closely scrutinised. A further update on the programme will come later in the year at Ash, when data from new sickle cell patients will also be released.

The big event, however, will be Asco in June, when an update on its Celgene-partnered BMCA CAR therapy bb2121 will be presented; Mr Leschly said he was "cautiously very optimistic", pointing out that more data on durability were crucial, as well treating more patients. It remains unclear why the therapy was driving such impressive efficacy without the toxicity seen with other CAR-T projects – "we have hypotheses but not an answer", he said. An update on a next-generation CAR, bb21217, which uses culture with a PI3k inhibitor in manufacturing to yield cells with a younger phenotype, should also emerge this year.

Editas brushes off patent concerns

The gene editing company Editas unsurprisingly found itself defending intellectual property at its breakout session – the risk of years of onerous and costly legal battles remains the biggest fear for the handful of companies working with Crispr. Executives insisted that they did not view the IP landscape as "binary" – "no one piece of IP or even family of components do we see as fundamentally binary – for us or our competitors," its finance chief, Andrew Hack, told delegates.

Crispr-derived therapeutics have yet to enter the clinic – at least in the US or Europe – so this argument remains to be tested. So executives were understandably keen to steer the talk towards developmental work and their technologies – chief executive Katrine Bosley highlighted a second gene editing system, cpf1, which she described as potentially even more specific than Cas9, and which could increase the scope of products the company could generate.

In terms of specific clinical work the company confirmed that an IND for the LCA10 programme in inherited eye diseases would be filed by the end of the year.

This story was amended to correct detail regarding Kite's six-month dataset.

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.