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J.P. Morgan Notebook Day 1: No Big Deals, But Plenty Of Pipeline, Commercial Highlights

by Mandy Jackson

Daily round-up from the J.P. Morgan Healthcare Conference in San Francisco: There was no big M&A, but biopharma CEOs discussed deals they're pursuing. Also, Novartis's Zolgensma reimbursement insights, Sarepta's third DMD filing, Bristol's post-merger progress and more highlights.

Should We Expect No Big Deals This Year?

The overarching theme so far at the J.P. Morgan Healthcare Conference this year is a big question: "Where are the deals?"

There were several partnerships announced as the meeting kicked off on 13 January, such as [Biogen](#)'s agreement to license a neuroscience drug candidate from [Pfizer Inc.](#), but there were no big acquisitions revealed on day one of the conference.

That's in stark contrast to last year's J.P. Morgan meeting when [Eli Lilly & Co.](#) announced the \$8bn acquisition of [Loxo Oncology Inc.](#), which came within days of [Bristol-Myers Squibb Co.](#)'s announcement that it would pay \$74bn for [Celgene Corp.](#) (Also see "[Lift-Off For Lilly In Cancer Genetics With Loxo Buy](#)" -

Deal Watch: Pfizer, Biogen Partnership One Of Many As JPM Gets Underway

By Joseph Haas

13 Jan 2020

Although there was no major acquisition starting out the J.P. Morgan Healthcare Conference, the deal-making flow has been steady. Pfizer licensed a neurodegenerative disease candidate to Biogen, while also selling Axsome rights to reboxetine in narcolepsy and fibromyalgia. Biogen signed a neurology R&D

Scrip, 7 Jan, 2019.)

Bristol-Myers CEO Giovanni Caforio said on 13 January at this year's J.P. Morgan conference that the company remains committed to business development to expand its pipeline even after digesting its Celgene acquisition. Those deals are likely to be smaller transactions and partnerships, however.

collaboration with CAMP4 as well.

[Read the full article here](#)

"We really believe in the importance of business development," Caforio said during the Q&A session following his presentation at the meeting, noting that the history of both Bristol and Celgene has been to complement internal innovation with external assets.

He said Bristol's business development focus primarily will be "small science deals" going forward, including opportunities reviewed during J.P. Morgan that may strengthen the company's early pipeline.

"When we believe there is great science that complements ours, we do have capacity to do those deals," Caforio said.

[Merck & Co. Inc.](#) opted for a fireside chat with CEO Ken Frazier and executive vice president/president of Merck Research Laboratories Roger Perlmutter instead of a 25-minute presentation by the CEO. When asked about business development, Frazier reminded the audience that the company doesn't see a lot of value in big M&A deals, but intended to continue with bolt-on acquisitions and smaller transactions.

"We think that business development will remain for us a very important element going forward," the CEO said. "What's the next great opportunity in science and how can we get a hold of it in a way that creates the most value for shareholders? As we think about bolt-on acquisitions, we are looking for those in a financially disciplined way."

He noted that Merck did more than 80 deals in 2019 and spent about \$8bn, including the pending \$2.7bn acquisition of [ArQule Inc.](#) that was announced in December. (Also see "[Merck & Co. Joins Competitive BTK Research Space With \\$2.7bn ArQule Buy](#)" - Scrip, 9 Dec, 2019.)

Biogen CEO Michel Vounatsos left the door open for the company to enter into transactions of all sizes, noting that as of the end of 2019 it had about \$16bn in financial capacity for deals, including \$6bn on hand that could be leveraged with \$10bn in debt.

Through 2024, the company expects to have \$51bn in financial capacity for deals based on its current portfolio, but not including any sales from the Alzheimer's therapy aducanumab, which

Biogen plans to submit for US Food and Drug Administration approval imminently based largely on a Phase III program in which only one of two studies met the primary endpoint. (Also see ["Biogen's Big Day Arrives, But Aducanumab Results Don't Answer Key Question"](#) - Scrip, 5 Dec, 2019.)

"The Biogen team is continuously screening opportunities from small to large size," Vounatsos said during the Q&A session following the company's presentation.

[Gilead Sciences Inc.](#) CEO Dan O'Day – in his first presentation at the company's helm from the J.P. Morgan podium – emphasized many times Gilead's investments in both internal and external innovation. O'Day said the company will pursue M&A "from a position of strength and with a sense of urgency," considering everything from early-stage collaborations to commercial-stage assets to expand Gilead's research and development pipeline as well as boost revenue growth.

That said, both O'Day and chief financial officer Andrew Dickinson noted the unique nature of the company's partnership with [Galapagos NV](#), which the companies expanded last year to give Gilead access to more of Galapagos's inflammation and fibrosis development programs. (Also see ["\\$5bn Galapagos Deal Won't Be Last For Gilead, Says O'Day"](#) - Scrip, 15 Jul, 2019.)

"It is a really thoughtful partnership," Dickinson said. "We can't do a lot of those, but we would like to do at least one more." He noted that Gilead is most interested in small- to medium-sized bolt-on transactions, and it has a fiduciary obligation to look at larger transactions, but said the company doesn't see big M&A deals adding enough value over time.

Zolgensma Reimbursement: VBR Is In, Annuity Payment Is Out

Only one patient with spinal muscular atrophy (SMA) treated with Zolgensma in the US has not been reimbursed since the expensive gene therapy launched in mid-2019. [Novartis AG's AveXis Inc.](#) president David Lennon talked about the launch of Zolgensma and market access during an interview at the J.P. Morgan Healthcare conference on 13 January.

Even though Zolgensma has been widely reimbursed so far, some patients have had to go through several rounds of back-and-forth with payers to secure reimbursement. Those are generally not newly diagnosed patients, for which the turnaround time from initiation to treatment, particularly for newborns, has been typically less than two weeks, according to Lennon.

"There's another population, which skews to the older population that has been treating with Spinraza currently and often there is push-back on different elements," Lennon said. Some of the requirements payers have in place include formal assurance that patients are going to stop taking Biogen's Spinraza (nusinersen) or more information from physicians around the

justification for initiating treatment.

Zolgensma launched in June with a \$2.1m price tag as the first potential one-time gene therapy for SMA. (Also see "[*It's Official: Novartis SMA Gene Therapy Zolgensma Is World's Most Expensive Drug*](#)" - Scrip, 24 May, 2019.) Novartis offered payers a value-based reimbursement option and a five-year annuity payment model for those that might rather pay in installments as opposed to fronting the full cost.

Not a single payer has taken Novartis up on the extended payment plan, however. In Europe, where Zolgensma is not yet approved and where governments are looking at contracts for a group of patients at one time, the idea seems to have more traction, Lennon said.

"This isn't the use case for it," Lennon said of the annuity payments in the US. "I think you need something bigger or a more urgent bolus of patients." If Zolgensma eventually secures FDA approval for Type 2 SMA as Novartis hopes, that could represent a more relevant test case, he said. "Right now, we are talking about a few hundred patients, five to 10 patients per a big plan. It's just not a big enough number."

US payers, however, have been receptive to the value-based reimbursement plan, in which Novartis agrees to pay a rebate if the drug doesn't perform as expected. The outcome the rebate is tied to is death or permanent ventilation. Novartis has not yet had to pay a rebate linked to a disappointing outcome, Lennon pointed out.

Novartis did not disclose the latest revenue for Zolgensma, which will be provided later in January when fourth quarter financials are released. Zolgensma generated \$160m in the third quarter, a strong launch. (Also see "[*2019 Drug Launches: New Specialty And Rare Disease Blockbusters Take Shape*](#)" - Scrip, 31 Dec, 2019.)

For Sarepta, Another Exon-Skipping Drug

Sarepta has initiated a rolling submission for a third exon-skipping drug casimersen with the US FDA, CEO Doug Ingram said during the company's 13 January presentation. The candidate, like Sarepta's other two exon-skipping drugs, would be targeted to a small subset of patients with Duchenne muscular dystrophy, this time for children who are exon 45 amenable.

"It is our goal to obtain that approval in 2020," Ingram said. If successful, he noted, "We will be among that very rare club of biotechs that have three or more internally developed and FDA approved therapies." Sarepta's exon-skipping drugs, including the first one Exondys (eteplirsen) and the second one Vyondys (golodirsen), have not been without controversy, however, due to limited efficacy data. The three drugs together address about 30% of the DMD patient population.

The FDA approval of Vyondys in December was a bit of a surprise, as it represented a big reversal on the part of the agency, which had issued a complete response letter due to renal safety and then reversed its decision.

Presenting to the J.P. Morgan audience, Ingram thanked FDA for working with the company to resolve their questions – and also restated that the company hadn't overstepped the mark or pressured the regulator to change its mind.

Surprise As FDA Approves Sarepta's Duchenne Drug Vyondys 53

By **Andrew McConaghie**

13 Dec 2019

The FDA has overcome doubts about the drug's safety and reversed its decision in just four months.

[Read the full article here](#)

Sarepta already is gaining some commercial momentum. The company announced that Exondys generated more than \$100m in the fourth quarter and around \$381m in 2018.

Investors, however, are more interested in Sarepta's gene therapy SRP-9001. The therapy has produced positive nine-month functional data from the first four-patient cohort, significantly impacting biomarkers for the disease, including a 96% expression of micro-dystrophin measured by signal intensity, as well as encouraging improvements of the physical function of the first four boys in the trial.

Results from SRP-9001's first placebo-controlled trial will be ready in early 2021, with what Ingram, Sarepta and the Duchenne community hope will be a major step forward in halting the life-limiting disease.

"This is what the revolution looks like," Ingram said. The gene therapy initiative was also recently boosted by a new ex-US commercial deal with Roche.

Sarepta also announced at the J.P. Morgan meeting another coup – Gilead's ex-CEO John Martin joined its board. Ingram told *Scrip* that he had reached out to Martin to help advise the company, saying his experience in launching curative treatments in hepatitis C would be invaluable to Sarepta.

"He has been involved in so many transformative moments in medicine ... but he has never joined any other company board before," Ingram said. "But if you are going to bet on any company, why wouldn't it be Sarepta?"

Bristol-Myers Happy With Post-Merger Progress

Bristol-Myers CEO Caforio's 2019 presentation at the J.P. Morgan Healthcare Conference in San

Francisco occurred just days after the company announced that it would pay \$74bn for Celgene and his 2020 presentation on 13 January came about two months after the companies closed their transaction. (Also see "[Bristol Values Celgene's Hematology, Immunology Portfolio At \\$74bn, But Does It Price In Risk?](#)" - Scrip, 3 Jan, 2019.)

And now that Celgene has been integrated into Bristol-Myers, Caforio told the audience, "I feel better about our opportunity at Bristol-Myers Squibb today than I felt one year ago when we announced the deal. The company truly is well positioned today and in the future."

Caforio outlined progress in Bristol's pipeline during the past year – including for the Celgene assets – and noted the eight launches that the company anticipates over the next 24 months. Among those are new indications for key products, including first-line lung cancer indications for the blockbuster combination of PD-1 inhibitor Opdivo (nivolumab) and CTLA4-inhibitor Yervoy (ipilimumab) based on the CheckMate-227 and CheckMate-9LA studies. (Also see "[An Early Surprise Win For BMS's Opdivo/Yervoy In Lung Cancer](#)" - Scrip, 22 Oct, 2019.)

Celgene made progress on several late-stage assets prior to its merger into Bristol, such as a new drug application (NDA) resubmission to the US FDA for S1P receptor modulator ozanimod in multiple sclerosis.

Biologic license application (BLA)-supporting data for the CD19-targeting chimeric antigen receptor T-cell (CAR-T) therapy lisocabtagene maraleucel (JCAR017, liso-cel) in lymphoma were presented at the American Society for Hematology meeting in December – and

Caforio emphasized Bristol's commitment to the cell therapy space in oncology at the J.P. Morgan meeting, noting that more products and new indications are coming in this space. (Also see "[Bristol's CAR-T Strategy Comes Into Focus With Two Near-Term Filings](#)" - Scrip, 10 Dec, 2019.)

The company has filed its BLA for liso-cel and anticipates a BLA filing in the first half of 2020 for idecabtagene vicleucel (ide-cel, bb2121) against B-cell maturation antigen (BCMA) in multiple myeloma – a filing that must be expected sooner rather than later since executive vice president and president, hematology Nadim Ahmed said during the Q&A session following Caforio's presentation that Bristol will have two CAR-T therapies on the market by the end of this year.

The CAR-T programs have been high-profile assets under both Celgene and Bristol, but one asset

With Celgene Acquisition Closed, Bristol Faces Major Milestones

By **Mandy Jackson**

21 Nov 2019

Bristol bought Celgene following multiple pipeline setbacks, but the biotech giant made positive progress recently that the big pharma will be expected to continue.

[*Read the full article here*](#)

that's flown somewhat under the radar is Reblozyl (luspatercept), which is partnered with [Acceleron Pharma Inc.](#) and was approved last year for transfusion-dependent beta-thalassemia. (Also see "[Keeping Track: Biosimilars, Novel Approvals, And Ebola](#)" - Pink Sheet, 10 Nov, 2019.)

An NDA is pending now at FDA for a much larger indication – the treatment of adults with very low to intermediate risk myelodysplastic syndromes (MDS)-associated anemia who have ring sideroblasts and require red blood cell transfusions.

Ahmed said anemia is one of the biggest problems associated with MDS, because patients become transfusion-dependent. He noted that Bristol will capitalize on its experience in MDS – through Celgene's long-term participation in that market with its drug Vidaza (azacitadine) – and its relationships with treating physicians in this area to maximize Reblozyl's potential beyond the "smaller niche indication" of transfusion-dependent beta-thalassemia.

Teva's Prospects For An Opioid Litigation Settlement

[Teva Pharmaceutical Industries Ltd.](#) CEO Kare Schultz said during the company's breakout session that he is cautiously optimistic the company could finalize a broad opioid litigation settlement ahead of a big state case in New York that is headed to trial in mid-March. "There is kind of a deadline coming up because it would be advantageous for everybody to get the actual settlement done before the next state trial," Schultz said.

The uncertain cost of the company's potential liability in ongoing opioid litigation has been a big overhang for Teva, even as the company has made some progress on its efforts to make a turnaround. (Also see "[Teva Turnaround Hung Up By The Uncertain Cost Of Settling Opioid Cases](#)" - Scrip, 7 Nov, 2019.)

Last year, Teva proposed a national settlement framework that would allow the company to settle much of the ongoing litigation with some states' attorneys general. That proposal includes a \$250m upfront payment and an offer to donate \$23bn in supply of the opioid addiction treatment Suboxone (buprenorphine naloxone) tablets over 10 years. (Also see "[Teva Seeks Global Resolution Of Opioid Litigation As Bellwether Trial Is Halted](#)" - Pink Sheet, 21 Oct, 2019.)

"I think there's a lot of political momentum behind this because it's actually doing something really to try and improve the situation," Schultz added.

Teva did not update investors on the 2019 financials or outlook for 2020. The financial update is planned for 12 February. The company has said it hopes 2019 will be a trough year before it returns to growth in 2020, but there are a lot of elements of its business strategy that need to be in place to get there. One snag has been the slow launch of the CGRP drug Ajovy for migraine (fremanezumab), which has struggled against Eli Lilly & Co.'s Emgality (galcanezumab) and [Amgen Inc.](#)'s Aimovig (erenumab), both of which are available in an auto-injector.

Teva is developing an auto-injector version of Ajovy that it thinks will help level the playing field, but it is pending at FDA. Pricing competition in the space has been stiffer than Teva had originally expected, Schultz said. The pricing set by Amgen when it launched Aimovig was lower than Teva had expected, and then rebates have been higher with three players in the market. "That means that the value per patient is probably maybe half of what we thought it would be net three/four years ago, but on the other hand, the volume is significantly higher."

Vertex's Leiden Passes The Baton To Kewalramani

In a fitting presentation for [Vertex Pharmaceuticals Inc.](#)'s big transition year, outgoing CEO Jeffrey Leiden and incoming CEO and current chief medical officer Reshma Kewalramani addressed J.P. Morgan attendees together. It was a symbolic changing of the guard as Leiden wraps up his seven-year tenure as Vertex CEO and Kewalramani begins her leadership reign. The leadership change comes at a broader period of transition for Vertex, which aims to move beyond its core therapeutic area of cystic fibrosis.

"I cannot underestimate, and I don't think anybody does, the challenge of going from one medicine to many, one disease area, to many," Kewalramani. But she said Vertex is well positioned to take on the challenge, having now built out the pipeline partly through internal R&D and partly through business development into areas like sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes.

"These are all diseases of high unmet need. We understand the causal human biology. We have biomarkers that translate from bench to bedside. These are all programs with efficient development pathways, and they're all served by specialty markets," she said. "That's probably the most important thing to know about our pipeline."

She highlighted two programs in particular on two early-stage development programs. One is the type 1 diabetes program acquired with the \$950m acquisition of Semma Therapeutics in September, focused on developing insulin-producing beta cells grown from stem cells. (Also see "[Vertex Buys Semma, Gaining Cell Therapy-Based Type 1 Diabetes Treatments](#)" - Scrip, 3 Sep, 2019.) She said Vertex set an ambitious goal of bringing that product to the clinic in late 2020 or early 2021.

The other is a treatment for alpha-1 antitrypsin deficiency (AATD), a lung disease caused by a misfolded protein which Vertex initiated a Phase II trial for in December. The company's near-term priority, however, is executing on the launch of the new triple combination cystic fibrosis drug Trikafta. (Also see "[Keeping Track: Vertex' Trikafta Speeds To US Approval; New Indications For AZ's Farxiga, J&J's Stelara, GSK's Zexjula](#)" - Pink Sheet, 24 Oct, 2019.)

The US Election 2020 And What It Means For Pharma

In the Donald Trump era, many commentators throw up their hands and say it's impossible to

predict what will happen next week, let alone in the November 2020 US presidential election.

However, one biopharma veteran was happy to provide his prediction of the outcome, and how it might play out for the sector.

BIO chairman Jim Greenwood was one of a diverse panel discussing the US political climate at the Biotech Showcase, taking place in parallel to the J.P. Morgan conference in San Francisco.

Biopharma finds itself under attack from both sides of the political spectrum: Trump is threatening to introduce international reference pricing, and Democratic presidential candidates such as Bernie Sanders and Elizabeth Warren want to introduce federal government controls on drug prices and target pharma profits.

“If our assumptions are correct, the House is going to remain Democrat and the Senate is likely to remain Republican,” said Greenwood.

“Then regardless of who wins the presidential election, it will probably still be the Republican majority in the Senate that protects our industry from the most horrible of the proposals out there.”

Invoking Charles Dickens, he added that the sector was outdoing itself in innovation, but couldn't turn to either political party for support.

“We are living in the best of times and the worst of times,” Greenwood said, “the science is galloping and we've never been able to provide as much hope to patients as now, with gene therapy, cell therapy, CRISPR and all of that. At the same time, this is the worst political dilemma the industry has ever faced. It's really dire.”

Scrip Asks...What Does 2020 Hold For Biopharma? Part 3: Policy And Regulation

By **Eleanor Malone** and **Joseph Haas**

09 Jan 2020

The US election will intensify the spotlight on drug costs, regulators will have their hands full with advanced therapy filings, and the need to address rising infectious disease threats will mount. Biosimilars, cannabis and Brexit are among other hot topics for 2020.

[Read the full article here](#)