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# Outside Oncology: 6 Updates From Roche's Pharma Investor Day

by Jessica Merrill

Management updated investors on M&A strategy, US drug pricing reform and R&D investment.

Much of the focus of *Roche Holding AG*'s 14 September investor overview was on Roche's cancer pipeline, and how the company plans to maintain its leadership position in oncology. But the company's top leaders also addressed other topics involving business strategy and the macro environment. (Also see "*Roche Gives The Stage To A New Generation Of Cancer Drugs*" - Scrip, 15 Sep, 2021.) Here are six other takeaways from the meeting.

## 1. US Drug Price Reform Outlook

Roche took a cautiously optimistic tone about the outcome of potential drug pricing reform legislation in the US. Some drug industry leaders have become increasingly wary that the US Congress could pass drug price reform legislation as part of a budget reconciliation bill that would give the government more leeway to negotiate Medicare drug prices more directly with manufacturers. (Also see "*Pharma On Its Back Foot As US Drug Price Reform Advances*" - Scrip, 8 Sep, 2021.) Pharmaceuticals CEO Bill Anderson, however, pointed out that more details remain to be sorted out before any final legislation is passed.

"We remain optimistic that when the senators and representatives actually get down to drafting legislation, there will be enough calm minds that appreciate the value of innovation that we will end up with something we can live with," he said. "It may cost us something, it almost certainly will, but that's okay. We can live with that."

He said Roche stands ready to engage with legislators in "constructive conversations" about how to lower patient out-of-pocket costs and make the US drug system more sustainable.

## 2. Investing In mRNA

Following the rapid and successful development of mRNA-based vaccines for COVID-19, many

big pharma leaders are pivoting to bulk up their technology platforms in the emerging field. Roche isn't known as a traditional vaccines manufacturer, but chief medical officer Levi Garraway said the company is interested in mRNA and is exploring the area in preclinical development, both in terms of generating new potential therapeutics and exploring ways of inhibiting mRNA.

"There is a lot we are looking at," Garraway insisted, even though the company hasn't been at the forefront of the rush to invest in mRNA therapeutics.

Anderson said the company's strategy is to focus on the disease that needs to be met and then find the modality to best address it.

"Sometimes, we get asked questions like 'What about this modality? Why aren't you there?' And the answer usually is 'well, because we have something else that we think is better,'" Anderson said.

### 3. Deals Slowdown

Roche hasn't completed a single late-stage pharmaceutical deal so far in 2021 – and CEO Anderson assured investors that the slowdown in late-stage deal activity is okay. Last year, the company completed five late-stage deals, he said.

"Why is that? Because we found five late-stage deals that were both strategically important, great science that we liked, but also they made sense financially," Anderson said. "So far in 2021, we haven't seen one that met all those criteria, and we don't feel pressure to push the button on deals that frankly don't make financial sense even if they look good strategically and scientifically."

Roche remains committed primarily to early-stage deals, Anderson added. "We don't believe in waiting until Phase III and then rolling the dice with big transactions. We don't think that's worked very well for large companies in the past," he said.

### 4. R&D Spend

After Roche announced a 19% R&D spending increase in the first half of 2021, analysts were curious to hear more about the company's thoughts on R&D spending going forward. Anderson said R&D spend will continue to increase faster than spend in sales and marketing, general and administration and operations. "We're not going to continue to increase R&D spend by 19%," he added. That increase was related to some one-time factors including increased investment in COVID-19 therapeutics and the maturing pipeline. (Also see "*Roche R&D Spending Surge Reflects In-House Innovation, M&A Prices*" - Scrip, 27 Jul, 2021.)

"We are not increasing our investment because we think productivity is going down," Anderson

added. The aim is to increase productivity fueled by other factors like technology, which has been a successful strategy for the company in the past, both in pharmaceuticals and diagnostics.

## 5. Wait-And-See On Gantenerumab

Investors eagerly awaiting an update on an accelerated regulatory strategy for the Alzheimer's therapy gantenerumab will have to wait. The company declined to comment on any ongoing conversations with the US Food and Drug Administration about a potential accelerated approval based on surrogate endpoints, following the agency's approval of *Biogen, Inc.*'s similar drug Aduhelm (aducanumab) earlier this year. Unlike fellow competitor *Eli Lilly and Company*, which said it will seek accelerated approval for its donanemab, Roche has so far declined to commit. (Also see "*Roche Deals With Huge Gantenerumab Curiosity At H1 Update*" - Scrip, 23 Jul, 2021.)

"We are not going to comment on any ongoing negotiations with health authorities for any reason," Garraway said of the gantenerumab strategy. "It is encouraging and good that the FDA is showing openness to looking at drugs in Alzheimer's disease and trying to find ways to get them to patients faster," he said. However, he added, "we want to deliver medicines to patients that have a comprehensive dataset with a robust safety and efficacy profile," suggesting the company may wait until the Phase III clinical trials testing gantenerumab read out next year.

## 6. What About That Spark Gene Therapy?

Roche is poised to make a decision by the end of the year on the optimal dose and immunomodulatory regimen to bring forward into Phase III for its hemophilia A gene therapy, gained through the acquisition of Spark in 2019. The deal caught the attention of the US Federal Trade Commission at the time, given the portfolio overlap with Roche's blockbuster hemophilia treatment Hemlibra (emicizumab), though it eventually cleared the anti-trust review. (Also see "*Roche/Spark Deal Clears FTC In A Sigh Of Relief For Pharma Dealmakers*" - Scrip, 16 Dec, 2019.)

However, it's now been nearly two years since the deal closed and there has not been a substantial update on the development plan. While that seems like a relatively lengthy delay for the Phase III trial, the broader field of gene therapy has also faced renewed safety scrutiny. Adeno-associated virus (AAV) vector-based gene therapies were just the subject of a two-day review by the US Food and Drug Administration's Cellular, Tissue and Gene Therapies Advisory Committee following the emergence of clinical toxicities across the field, including deaths. (Also see "*Gene Therapy: AAV Doses Should Not Be Subject To Fixed Upper Limit, US FDA Panel Says*" - Pink Sheet, 8 Sep, 2021.)

Roche's hemophilia A gene therapy is an AAV-vector based gene therapy, but management said the safety profile continues to be acceptable at the doses studied, while in clinical testing the therapy resulted in a 91% reduction in annualized bleeding rate and a 97% reduction in annualized infusion rate compared to the year prior to the administration.

"We are learning how to manage gene therapy, almost like a disease-by-disease basis," global head neuroscience, immunology, ophthalmology, infectious and rare diseases clinical development Paulo Fontoura said. "We are learning together with the FDA." Different viral vectors appear to have different safety profiles and new and better viral vectors are still needed, he said. Dosing and routes of administration are also challenges. The key to continue to advance gene therapies in development will be safety monitoring, he added.