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BIO Notebook: FDA User Fees For AI Regulation, Partnering Strategy Evolves, And More

by Scrip Team

Insights from Day Two of the BIO International Convention in San Diego include the evolving pros and cons of partnering, user fees potentially supporting the FDA's AI ambitions, and J&J's view on dealmaking in 2024.

The Pros And Cons Of Flying Solo With Ex-US Launches

There is plenty of money to be made in the international markets for US biotechs, but deciding to go it alone or find a partner abroad remains a tricky choice.

Industry veteran Russell Cox, now CEO of *Epirium Bio Inc.* , said during a BIO 2024 panel that the strategy for a small biotech had been to sell the European rights to a drug to fund its US operations. However, if a firm's investors are on board and the cash is flowing, launches overseas without a partner are becoming more common, especially in the rare disease space.

“Those markets are a little more concentrated, the patients are easier to get to and it's actually a lot more efficient than you think,” he said. “It's becoming less company-specific and much more of a global approach.”

Cox said the rule of thumb for the licensing-out option was “don't ever give it up unless there's somebody who can do it way better than you.”

A prospective partner has to have “the capabilities, the infrastructure and the relationships because nobody's gonna love your baby as much as you,” he added.

Tolga Tanguler, chief commercial officer at *Alnylam Pharmaceuticals Inc.*, said biotechs that are thinking about taking their product aboard alone have to "start early in engaging the regulatory authorities to tell your story, because a lot of companies see it as a bit of an afterthought, 'let's get the US right and figure out the rest latter,' but then it's a little too late."

He said Japan was "an incredibly attractive market and they're willing to talk to you a lot about the costs, they have different payer models in Japan but again, you have to have those conversations early on," Tanguler said.

Tanguler added that the biggest challenge "by far" was hiring the right people on the ground who know the dynamics of the local market.

"If you don't have the right talent to engage and tell your story effectively, you have to start thinking about and looking for a partner because it's easier said than done," he said.

– *Kevin Grogan*

AI And FDA: A Risk-Based Approach And New Industry User Fees?

The US Food and Drug Administration likely will need a risk-based approach to reviewing the artificial intelligence aspects of drug applications as the technology becomes more pervasive in regulatory submissions, Center for Drug Evaluation and Research Director Patrizia Cavazzoni said at the BIO 2024 FDA town hall.

As the use of AI in applications increases it will become both economically unrealistic and infeasible from a time perspective to do otherwise, Cavazzoni said, highlighting the costs of the high-performance computers needed to bring large data sets in house and the constraints of the current review clocks.

Cavazzoni reiterated comments made earlier this year about the FDA needing a way to potentially work with industry or others to make AI "realistic from an economic standpoint." She may be foreshadowing a potentially hot topic in the next round of user fee negotiations with industry. (Also see "*Cost Of Reviewing Drugs With AI Is 'Prohibitive' For US FDA*" - Pink Sheet, 7 May, 2024.)

One lingering question is what FDA commitments would entice industry to support the agency's AI infrastructure. Another is how AI, which is used in a wide range of ways by a wide range of products FDA regulates, could be incorporated into the user fee programs, which are segmented by product type, including drugs, devices and biosimilars.

The FDA, of course, is not just reviewing AI in industry applications, but also using it in its own work.

Center for Biologics Evaluations and Research Director Peter Marks said his center recently used AI to analyze submissions with very large databases, such as applications with whole genome sequencing data.

“To organize and understand,” this data “we have to use AI,” Marks said.

CBER also is using AI to help identify adverse event reports that can benefit from a human review, Marks said.

The FDA is exploring many other potential uses for AI, but “you just want to make sure that at the end of the day anything you do with AI actually is real and it’s not a hallucination.”

– *Sarah Karlin-Smith*

J&J’s Head Dealmaker Nauman Shah Sees Robust Activity In 2023

Nauman Shah, global head of business development at *Johnson & Johnson* Innovative Medicine, pushed back when asked during a 3 June fireside chat at the BIO International Convention why biopharma mergers and acquisitions are down so far in 2024 relative to 2023 when expectations were for increased activity this year, with the executive suggesting that plenty of dealmaking is taking place, including within the walls of J&J.

The executive put 2024 dealmaking into context relative to the robust level of transactions announced last year, particularly towards the end of 2023.

"If you think about 2023, '23 was actually a pretty fantastic year from a deal activity standpoint,” Shah said. “We saw the industry close $159bn worth of deals executed, which was actually higher if you look out over a five-, six-year horizon, the highest since 2019, so pre-pandemic levels. And 2019 was extraordinary, because it was a record year, given the *Celgene Corporation* deal, given the *Allergan plc* deal,” executed by *Bristol Myers Squibb Company* for $74bn and by *AbbVie Inc.* for $63bn, respectively. (Also see "*With Celgene Acquisition Closed, Bristol Faces Major Milestones*" - Scrip, 21 Nov, 2019.)

Shah noted that J&J also was busy in 2023, completing 10 M&A and other types of deals in December alone, and started 2024 with the $2bn acquisition of antibody-drug conjugate developer *Ambrx, Inc.* announced in January. (Also see "*J&J Makes Its Biggest Bet Yet On Antibody-Drug Conjugates With Ambrx Buyout*" - Scrip, 8 Jan, 2024.) And he asserted that while the dollar value of M&A deals has come down in 2024, there is plenty of activity around smaller transactions.

“The overall volume has come down, whether that's because people have deployed a lot of capital in '23 and maybe that's part of the reason they've stepped back or whether it's just that

there aren't a lot of the late-stage assets currently available. It could be a combination of things," Shah said. "But I can tell you, while I can't speak for other companies, we as Johnson and Johnson, and specifically on the pharmaceutical side, continue to be extraordinarily active."

In May, the company announced two M&A deals for bispecific antibody developers in the immunology space. J&J is paying $1.25bn for a company spun out of *Numab Therapeutics AG* and $850m for Proteologix. (Also see "*J&J Continues Atopic Dermatitis Effort With Numab Deal*" - Scrip, 28 May, 2024.)

– *Mandy Jackson*