ARPA-H & FDA Life Sciences Outlook Bright

Aduhelm Prompts FDA Process Changes, Commissioner Wants to Regulate Dx Tests

Today, Capitol Street co-hosted with JPMorgan 41st annual Healthcare Conference a panel with FDA commissioner, Dr. Robert Califf, and ARPA-H director, Renee Wegrzyn. The leaders discussed life sciences and biopharma priorities for their respective agencies in a new year.

- **Inflation Reduction Act: FDA notes that innovation hangs in the balance with implementation uncertainty.** FDA Commissioner expects changes to how companies calculate long-term returns, but innovation is engrained in the American landscape. Dr. Califf noted that there is still unpredictability of how the law will be implemented, but acknowledged that "conditions of calculations on long-term return on investments" will change. Longer-term, he believes the culture of innovation and creativity remains important in the US.

- **Alzheimer's Disease**: **FDA remains optimistic about the future of Alzheimer's treatments, but agrees with investigation report.** Dr. Rob Califf notes the FDA needs to improve the process for confirmatory trials. We can also expect reform of FDA's advisory committee structure to improve review. Accelerated approvals continue to be supported by the public and FDA's close collaboration with companies is not surprising given the volume of data handled. He cautioned the audience that there is no substitute for good data.

- **FDA Commissioner wants to regulate diagnostic tests (LDT) despite VALID Act falling flat in 2022.** Dr. Califf noted he was largely satisfied with the FDA authorities and flexibilities in the omnibus, but the lack of diagnostic regulations was upsetting. The growth in funding for biologics reflects rapid development in that area. FDA is looking for feedback as they seek to regulate these industries. He applauded the shift away from toxicology animal models, but also warned that proposed models need to prove that they work. Dr. Namandjé N. Bumpus, FDA's Chief Scientist, will be guiding work on animal testing alternatives.

- **Diagnostic testing pitfalls remain but Liquid Biopsy testing is exciting.** Dr. Califf highlighted that the industry must practice caution in this realm (false positives and introduction of toxic chemicals). He stressed the importance of knowing long-term impacts on patients and how they may be impacted say 10 years down the line, but acknowledged that the "potential for diagnostics now is beyond anything that could have been imagined", and this made regulating a trial-and-error process, particularly during the pandemic.

- **Cell & gene therapy costs will come down as platforms become more generalizable** Treatment costs are expected to become more accessible as platforms become more generalizable and methods become more consistent. Dr. Califf noted that the FDA is working to guide this standardization and increasing the interchangeability of technologies. FDA has more dollars for cell and gene therapy review per the 2022 FDA user fee bill.

- **What is ARPA-H, a new agency with $2.5 B Funding?** ARPA-H Program Managers (PM) are expected to commit to a minimum term of 3 years as government employees. Dr. Renee Wegrzyn highlighted that this role may be the perfect fit for industry leaders and academics looking for a shift in their careers, and hoping to solve a specific problem in healthcare. The Omnibus added $1.5 B in funding to ARPA-H, bringing total funding up to $2.5 B. Funding will prioritize unique opportunities and solutions that cannot be helped by other research players (HHS, NIH).

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- **Cancer Moonshot chugs along.** ARPA-H was announced in the same breath as Cancer Moonshot and have been closely linked together. A Cancer Moonshot representative will be appointed at ARPA-H to advance Cancer Moonshot goals and bridge programs within the agency and NCI and devise high level solutions. For potential oncology advances, Dr. Califf focused on increasingly advanced technologies that could improve quality of care (digital pathology, AI systems).

- **Cross agency collaboration is tough but all strive to meet this goal (FDA, ARPA-H, CMS, NIH)**. Dr. Wegrzyn shared enthusiasm for collaborating with other federal agencies, remarking on the positive relationships with NCI and NCI Director Dr. Monica Bertagnolli. To maintain FDA relationships, ARPA-H is considering liaisons within ARPA-H to serve as intermediaries. She also highlighted an omnibus ARPA-H provision that allows the agency to compensate the FDA for working with the agency. FDA Commissioner, Dr. Califf, also expressed enthusiasm for collaboration with ARPA-H, and stated that the ARPA-H by design is expected to bring agencies together