CAPITOL STREET

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Relief Rally: Controversial Biologics Leader Is Leaving FDA

Center for Biologics (CBER) New Leader Announcement Imminent As Pre-Mid Term Agency Shuffle Takes Place

Relevant Companies

REGENXBIO, Capricor Therapeutics, SOLID BIOSCIENCES, disc medicine, Scholar Rock, ATARA BIO, Replimune, ultragenyx, uniQure, moderna

Our Take & Next Up

Good news for biopharma. CBER Director Vinay Prasad is (once again) leaving the FDA at the end of April, providing more certainty for orphan and ultra rare product reviews. Despite having had the support of FDA Commissioner Makary and HHS Secretary RFK, Jr, the controversial Center for Biologics Director has ruffled too many patient groups and innovators in an election year. His departure comes after concerns over the myriad rejections of rare disease therapies, reports of personal review interference, abrasive management style and staff treatment, as well as commuting from the west coast on the government's dime. We have previously written on the dysfunction at the FDA and CRLs (here and here).

Key Points

Investors, providers, patients, and biopharma will breathe a sigh of relief. Companies with medicines that faced Prasad's wrath will likely benefit, given this development. We expect some of the 'surprise' CRLs may become more of a conversation, in the same way the Moderna flu shot reversal took shape in February.

The new CBER leader will be announced before Prasad's departure in late April, and we doubt it will be an academic. Vinay Prasad will leave the FDA (per WSJ here), leaving multiple roles as Chief Medical and Scientific Officer and as CBER Director. FDA Commissioner Makary announced the move as planned, given Prasad's one year leave from the U of California system.

The controversies surrounding unmet need product rejections angered patients and companies, particularly those in the rare disease space. Prasad garnered criticism due to interference in review decisions and public comments on pending reviews. A sampling of complete response letters (CRLs) under his watch include:

- RGX-121 (RGNX) - Hunter syndrome
- Deramiocel (CAPR) - Cardiomyopathy associated with DMD
- SGT-003 (SLDB) - DMD

- EBVALLO (ATRA) - Epstein-Barr virus positive post-transplant lymphoproliferative disease (EBV+ PTLD)
- UX111 (RARE) - Sanfilippo syndrome type A (MPS IIIA)
- Bitopertin (IRON) - erythropoietic protoporphyria (EPP) reviewed under CNPV
- AMT-130 (QURE) - Huntington's disease.
- Apitegromab (SRRK) - SMA
- RP1 (REPL) - advanced melanoma

Going forward, we view FDA reviews of novel, ultra rare and orphan therapies to be more smooth sailing, per the FDA novel pathways unveiled over the last year. FDA Commissioner Makary is likely to make pathways and approvals smoother, and follow newly-released guidance in an attempt to bring consistency to product reviews. See our analysis of the plausible mechanism pathway and clinical trial policies here.

- Plausible Mechanism Framework
- Commissioner's National Priority Voucher (CNPV) pilot program
- Rare Disease Evidence Principles (RDEP)

HHS has restructured of late due to other controversies: Deputy Secretary Jim O'Neill was reassigned from HHS due to anti-vax policy with regard to the childhood schedule. The broader restructuring included the elevation of Medicare director Chris Klomp as the new HHS chief counselor and reassignment of General Counsel Mike Stuart. Kyle Diamantas and Grace Graham become senior counselors for the FDA, and John Brooks is now senior counselor for CMS.

Remember, the Center for Drugs (CDER) also lacks a permanent director. Acting Director Tracy Beth Høeg, who is a sports medicine physician with no FDA or drug development experience stated her top priorities include evaluating the safety of antidepressants taken by pregnant women and scrutinizing RSV immunizations for infants. Høeg is a sports medicine physician and Ph.D. epidemiologist with little regulatory experience. Recent reports note her intention to bring another MAHA-like figure into FDA reviews: Adam Urato, a maternal-fetal medicine specialist and critic of antidepressant safety.

As a reminder, we have said that could see HHS Secretary RFK Jr depart post-midterm elections. It's that time for agency leaders to leave. RFK Jr. has some MAHA "wins" to celebrate: reducing the number of recommended childhood vaccines, revamping the ACIP, creating a new food pyramid that sets the framework for child and adult nutrition programs, pushing to remove fluoride from the water supply. RFKJ likely stays through the election. Potential replacements include FDA commissioner Makary and CMS administrator Oz. Dr. Oz intends to serve the entire Trump term, stating that he is a "lifer" at the administration.

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