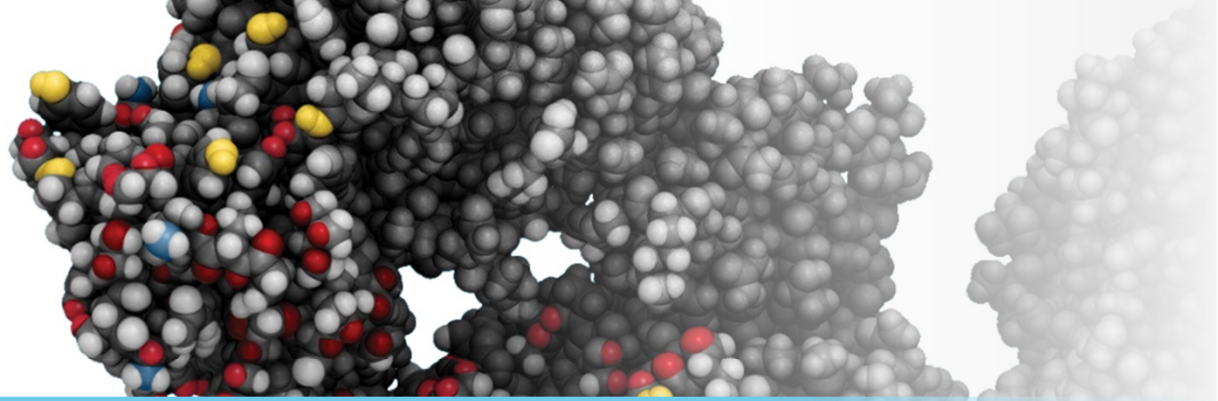
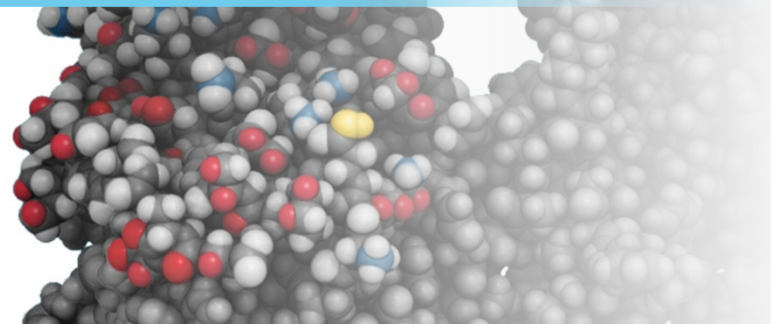
Morgan Lewis



BLOCKBUSTER BIOLOGICS REVIEW ISSUE 22

Legislative and Regulatory Updates



"March-In" Rights

- **On December 8, 2023, the National Institute of Standards and Technology (NIST) released a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights**
  - "March-in" rights were created under the University and Small Business Patent Procedures Act of 1980 sponsored by Sens. Birch Bayh and Bob Dole (also known as the Bayh-Dole Act), in which the government allows recipients of federal research funding to retain rights to inventions conceived or reduced to practice under a federal funding agreement. The government, however, retains certain rights and imposes certain obligations on the contractor, including the authority to "march-in."
  - To date, the US government has never granted a request for "march-in" rights. There have been at least six requests made to NIH to use its "march-in" rights (against CellPro, Inc., Norvir/ritonavir, Xalatan/latanoprost, Fabrazyme/agalsidase beta, Norvir/ritonavir, and Xtandi/enzalutamide), all arguing that the drugs are too expensive. In each case, the NIH found that lowering prices was not an appropriate use of "march-in" rights.
  - The goal of the Biden administration with the Draft Interagency Guidance Framework is to reduce prescription prices by allowing the US government to consider the reasonableness of the price of a product covered by technology that was developed using federal funding (a sponsored patent) as a factor in assessing whether to exercise "march-in" rights.

Biologics Surge at FDA in 2023

A review of 2023 approvals and FDA review activities by the Pink Sheet suggests that 2023 continued biologics programs' climb toward toppling the traditionally dominant small molecule programs. Among other developments, the report describes that:

- Novel biologic approvals (34) continue to approach (but not yet quite reach) parity with new molecular entities (38) in 2023;
- Recent surges in activity in CBER are driving these gains in novel biologics approvals;
- Looking toward FDA's various expected programs (e.g., breakthrough/RMAT designation and accelerated approval), biologics applications are outpacing small molecule programs in terms of the proportion of development programs qualifying and utilizing expedited programs and pathways; and
- Among novel biologics approvals in 2023, 1/3 were antibody products, with cell and gene therapies and vaccines making up the next largest cohorts of product classes.

Continued FDA Emphasis on Advanced Manufacturing

- In December 2023, FDA published a draft guidance announcing its new Advanced Manufacturing Technologies (AMT) Designation Program, working to facilitate the development and accelerate approval of products manufactured using designated AMTs.
  - Designation under this program requires the incorporation of a novel manufacturing technology (or an established technology in a novel way) to improve manufacturing while maintaining or improving drug quality.
  - Benefits of designation include enhanced interactions with the agency and prioritized review of applications incorporating designated AMTs.
  - Additional information regarding this program can be found at this *As Prescribed* post.
- The forecast for 2024 includes continued emphasis on advanced manufacturing technologies, as FDA continues to implement related provisions from FDORA and PREVENT Pandemics Acts legislation passed in late 2023, including provisions related to "platform technologies," for which guidance is expected from the agency.

FDA START Pilot – a/k/a "Operation Warp Speed for Rare Disease"

- FDA is accepting applications through March 1, 2024 to join the Support for clinical Trials Advancing Rare disease Therapeutics (START) pilot program.
- Inspired by the rapid development of therapeutics during the pandemic under the government's Operation Warp Speed for Rare Disease, the program is intended to support and further accelerate rare disease therapeutic development; development programs accepted into this pilot are promised more frequent communication from FDA to provide "a mechanism for addressing clinical development issues."
- Eligible programs must have an active investigational new drug application (IND) in place. In addition:
  - In CBER, eligible products must be gene or cellular therapies intended to address an unmet need as a treatment for a rare or serious condition, likely to lead to significant disability or death within the first year of life.
  - In CDER, eligible products must be intended to treat rare neurodegenerative conditions.
  - Additional information, including eligibility information, is available here.

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