House FDA User Fee Package Released

Diagnostic Regulation Missing, Accelerated Approval Reforms, Generic Clarity

House Energy and Commerce leadership released this evening FDA User Fee Legislative Package (here). Health Subcommittee will introduce the "Food and Drug Amendments of 2022" this week, which will be marked up next week. The "extra" policies here largely track the trends seen in User Fee hearings in both House and Senate. We expect a few additional policies to be tacked on with passage by Oct 1, 2022.

WHATS NOT IN

- **DIAGNOSTICS: Laboratory Developed Tests (LDTs) language (VALID Act) is missing, but there is still time for inclusion and passage in 2022.** While regulating diagnostics have enjoyed bipartisan support, the absence at the House MDUFA hearing was commented on by Rep. Bucshon (R-IN) and the subsequent absence in the user fee package is not unexpected. The Senate HELP Committee has taken the lead on Diagnostics reform, we still expect discussion & possible passage in 2022.

- **DRUGS**: **Not included here, but Congress may reverse a federal court's decision (CPRX) that orphan drug exclusivity applies to an entire disease and cannot be limited to a specific indication or population.** At a Senate hearing last week, the directors of FDA's centers for drugs and biologics said they are eager to work with Sens. Baldwin (D-WI.) and Cassidy (R-LA) on legislation to address the issue. A ruling in September 2021 by the U.S. Court of Appeals for the 1th Circuit stated that FDA violated the Orphan Drug Act when it granted Jacobus Pharmaceuticals orphan exclusivity for use of Ruzurgi amifampridine to treat children with Lambert-Eaton myasthenic syndrome (LEMS). The court ruled that a prior orphan exclusivity granted to Catalyst Inc. (CPRX) for its Firdapse amifampridine for adults covered all indications for the disease (Source: *Biocentury*).

- **CANCER SCREENING**: **Medicare Multi-Cancer Early Detection Screening Coverage Act of 2021 (GRAIL EXAS).** Medicare is not currently allowed to cover cancer screening. A bipartisan and bicameral bill would allow coverage has been introduced. If could catch a ride with the UFAs this fall.

WHATS IN

- **OVERALL: FDA User fees policy relating to Drugs, Medical Devices, Generics, and Biosimilars are included in the package. This includes PDUFA VII, MDUFA V, GDUFA III, BsUFA III**. All programs will be funded 2023-2027 with the existing fee structure and Congressional reporting requirements. Commitments on product review timelines, hiring estimates, and program enhancements are taken directly from the FDA's performance goal letter.

- **INFECTIOUS DISEASE DRUGS**: **Allows for biological products to qualify as Qualified Infectious Disease Product (QIDP) and allows for priority review** of innovative biological antifungal such products if such products require clinical data to demonstrate safety or effectiveness. The policy does not extend QIDP exclusivity to biological products, however.

- **ORPHAN Reauthorizes orphan drug grants through 2027** & **asks FDA to report on orphan progress.** Bill expands uses of such grants to include the development of regulatory science and

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manufacturing and controls related to individualized medical products to treat those with rare diseases or conditions. Requires HHS to submit a report summarizing FDA's activities relating to designating, approving, and licensing drugs used to treat rare diseases no later than 4 years after enactment. Requires FDA to study processes for evaluating drugs for rare diseases in the United States and the European Union. Requires FDA to convene public meetings to solicit input from stakeholders regarding approaches to improving engagement with rare disease condition patients, patient groups, and experts.

- **CELL & GENE THERAPIES**: Requires FDA to convene a public workshop on best practices on generating scientific data necessary to facilitate development of human cell-, tissue-, and cellular-based medical products, and the latest scientific information about such products.

- **GENERICS: Generic manufacturers will receive more color on ingredients and flexibilities in approvals**. FDA would provide generic sponsors, upon request, information regarding any differences in ingredients between their generic drug and the reference listed drug to which they are compared. This is expected to simplify the application process by taking the guesswork out of the generic formulation. Generic drug approval will be allowed even if its proposed labeling differs from that of the brand drug if the differences are limited to FDA-approved changes made within 90 days.

- **MEDICAL DEVICES: Manufacturers will be required to develop processes to address cybersecurity vulnerabilities**. Manufacturers must provide a software bill of materials in their labeling, and submit this information to the FDA in premarket submissions. Failure to comply will be a prohibited act and 510(k) clearance may be denied if security info is inadequate.

CLINICAL TRIALS: ACCELERATED APPROVAL, DIVERSITY & MODERNIZATION

- **DRUGS: Clinical trial diversity provides a reasonable equity approach & echoes FDA Commissioner Califf sentiment.** Manufacturers will be required to submit a diversity action plan that includes the sponsor's goals for enrollment in the clinical trial, rationale for such goals, and an explanation for how the sponsor intends to meet such goals. Diversity plans will be required to be published no later than 2024. FDA will also be required to provide guidance on the enactment of plans and considerations for decentralized CTs, and host public workshops to enhance clinical trial diversity.

- **DRUGS**: **Accelerated approval reform gives FDA ability to withdraw approvals, request status reports.** FDA would be authorized to require post-approval studies, which may be supported by real-world evidence, to be underway at the time of approval and allows the FDA to withdraw approvals where sponsors fail to conduct studies with due diligence (after a public meeting, dialogue with FDA commissioner). The bill also codifies labeling requirements for accelerated approval and information on surrogate endpoints and requires more frequent reports on post-approval study progress. We note this was a key issue for E&C Democrats, but it stops short of setting limitations on how long drugs can stay on the market as suggested in Rep Pallone's (D-NJ) bill.

- **DRUGS & DEVICES: Real-World Evidence includes lessons learned from PHE.** FDA will be required to issue guidance addressing the use of real-world evidence and real-world data, including that obtained for drugs and devices authorized for emergency use during the PHE. This requirement is specifically intended to support drug and device approvals and clearances, which signals to the FDA and manufacturers that real-world evidence may become increasingly important in submission packages.

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- **DRUGS & DEVICES: Modernization of the FDA trials with cell-based assays, organ chips and microphysiological systems, and sophisticated computer modeling**. The new modalities move away from animal based pre-clinical models.

- **OUR TAKE / NEXT STEPS:** We find this bipartisan package to be very level-headed**.** We think the entire package will pass by Oct 1, 2022 (avoids the pink slips at the FDA). (1) VALID Act diagnostic testing regulation, (2) Cancer screening coverage, as well as (3) orphan drug reforms are still possible for inclusion. We see elements of the *Pandemic Preparedness Act* and Cures 2.0 here. Mark-ups are scheduled for next week, where amendments may be brought up.

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