Legislation That Is Actually Moving

FDA Reforms, VALID Act & ARPA-H Likely Pass by Oct 1

Today the House Energy & Commerce Committee voted unanimously to advance their FDA User Fee Legislative Package (here). It now heads to the House floor following unanimous approval by the full E&C committee and the health subcommittee (last week). House leadership is maintaining momentum to ensure passage prior to the October deadline. A key rider, the VALID Act, is still expected in the final package. Portions of Pandemic Preparedness & Cures 2.0 could also make it in.

- **VALID Act: Laboratory Developed Tests (LDTs) language is still missing, but still expected for inclusion.** During committee markup, Representative DeGette (D-CO) commented on the need for oversight of laboratory testing and noted the inclusion of the VALID Act in the Senate version. The House is likely to adopt the VALID Act provisions before final passage. Senate provisions detail the premarket, abbreviated premarket, and supplemental application review process, provide exemptions (low-risk, humanitarian use, custom and low volume), and describe the technology certification pathway for moderate risk products to be certified to offer multiple tests.

- **Senate FDA Version: The Senate put out their User Fee package yesterday (here)**. While similar to the House package, the Senate HELP Committee text lacks provisions on clinical trial diversity, generic approval flexibilities, accelerated approval reform, device cyber security, and use of RWE (real-world evidence). While the Senate version may end up adopting additional amendments in markup, we expect a more robust House version language to prevail as the two bills are conferenced.

- **ARPA-H: The House today voted through an amendment to codify ARPA-H.** It would be an independent agency within HHS and the Director would be Presidentially-appointed and Senate confirmed. This would entail a public-private partnership model and helps life sciences, tools, digital health and medical technologies, including emerging technologies. We believe ARPA-H is a slam dunk, and the committee voted 53-3 to push ARPA-H forward.

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 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

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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.** 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 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.

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

- **OUR TAKE / NEXT STEPS:** We think the entire FDA User Fee/VALID Act/ARPA-H package will pass by Oct 1, 2022 (expiration date). The bill heads to the House floor for a full vote, while the

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Senate version awaits a markup in the coming weeks. Elements of the *Pandemic Preparedness Act* and Cures 2.0 are included here, and more may come into play. The package would likely all pass together ahead of the election.