Monday, February 24, 2020

On February 21, 2020, the Personalized Medicine Coalition (PMC) released its annual “Personalized Medicine At FDA: The Scope and Significance of Progress in 2019” (Report) that monitors FDA approvals of precision medicine therapies and diagnostic assays.

Notable Developments for 2019

Several milestones were noted for 2019 - ranging from new drug approvals to the first FDA qualified digital technology software.

1. While the total percentage of personalized medicines approved by FDA decreased from 2018 (25% in 2019 versus 42% in 2018) there were a significant number of approvals for the treatment of non-cancer disease, including the approval of Zolgensma™ (onasemnogene abeparvovec-xioi) for the treatment of spinal muscular atrophy. This gene therapy corrects bi-allelic mutations in the SMN1 gene. Report at pages 6 and 7.

2. Eleven of the 44 new therapeutic drugs approved by FDA were personalized medicines. Seven of the 11 were approved for indications outside the field of oncology. Report at pages 6 and 7.
3. FDA approved many new indications for existing, approved personalized medicines, providing new treatment options for many patients. Report at page 8.

4. Several biosimilars for personalized medicines were approved. Report at page 8.

5. Five of the 12 newly approved personalized therapies reverse previously unmitigated root causes of certain congenital diseases. There are now therapies for disease where no prior treatment was available. Report at page 9.

6. In 2019, FDA's Center for Devices and Radiological Health (CDRH) approved or cleared 7 new or expanded in vitro diagnostic tests that are important to personalized medicines. Report at page 12 and 13.

7. One of the approved in vitro diagnostic tests is a new type of diagnostic that uses a blood sample rather than a tissue sample, a so-called “liquid biopsy.” Patients unable to tolerate a tissue biopsy or who cannot provide a sufficient sample now only need provide a blood sample. Report at page 13.

8. OsiriX CDE Software Module™ (a biomarker test for brain injury) is the first technology software qualified by FDA for a personalized medicine test type. Report at page 13.

Progress and Challenges

According to the PMC, much of the progress over the last several years can be attributed to the commitment of scientists and FDA toward moving from a one-size to fit all to a personalized approach. “This progress is largely due to the commitment to personalized medicine in the biopharmaceutical and diagnostic industries as well as at FDA.” Report at page 14.

Technologies Advancing Personalized Medicine: A Briefing on Capitol Hill

The PMC cautions that to ensure continued progress, policy makers must continue to favor policies that encourage advancement in the field. Report at page 14.

To further support and maintain momentum in the field, the PMC has co-organized a bicameral, bipartisan Congressional Personalized Medicine Caucus on February 26, 2020 in Washington, DC. The meeting will explore what personalized medicine is and why it matters, with an emphasis on the clinical and economic benefits of earlier disease detection and improved prevention strategies. Registration details here.

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