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Cancer Care: Latest and Greatest from DC

IRA Orphan "Fix" Bill, IRA Impacts, CMS AA Pilot Slows, FDA on ClinTrials & The Moonshot

Relevant Companies

Our Take & Next Up

FDA wants global oncology clinical trials, with confirmatory trials enrolled prior to accelerated approval. In other cancer news, CMS is looking to make changes to its oncology care model (EOM) enrolling oncology practices, per CMMI comments at a recent value-based cancer care meeting, by potentially adjusting the benchmarks. CMMI is also slowing down a new payment pilot for drugs approved via accelerated approval (mandated by a Biden Exec Order), given new data that sponsors in oncology are completing post-approval studies at high rates. Lastly, IRA is the elephant in the room – CMS chose the top ten drugs for '26 Part D negotiation based on scripts (not gross spend), a process that minimized oncolytic exposure. Imbruvica (JNJ/ABBV) is the sole cancer medicine on the list and the Orphan Drug bill to fix the IRA provision (See below) has a $5 B price tag, a large portion of the cost being due to Imbruvica, and the bill is unlikely to pass anytime soon. Cancer Moonshot slowly trudges along with CMS coverage of patient navigation services and cancers that primarily impact women (endometrial cancer) expected to get a boost in funding from the White House Women's Health Research Initiative announced this week. See below for details.

Key Points

CMS ACCELERATED APPROVAL PAYMENT REFORM SLOWS

CMMI is slowing its accelerated approval payment pilot (likely start 2025-26) given that sponsors are completing confirmatory trials. CMMI is currently reviewing both the FDA's new authorities FDORA (formal expedited withdrawal, post-approval reporting, accelerated approval council) and the shifting mix of products that are granted accelerated approval. The delay might be partly informed by CMS data released in October: post market studies are largely being completed for most new drugs in Medicare. The time to complete confirmatory trials for Part B drugs for oncology indications has remained below five years (60 months) since 2014. OCE's Project Confirm, which provides data management for accelerated approvals, found that 8% of AAs granted for new therapies led to withdrawal.

Adjustments to the Enhancing Oncology Model (EOM) are likely as CMMI acknowledges flaws. Financial solvency remains a major issue for independent and community specialty practices as oncology margins decline. The low enrollment and lack of participation for EOM (particularly from smaller practices), the second generation pilot to OCM, was publicly noted by CMMI at the Association for Value-Based Cancer Care (here). The agency has not yet announced changes but is expected to improve EOM, which could include benchmark or provider reimbursement changes.

FDA – ACCELERATED APPROVAL IN ONCOLOGY

Today, the FDA Oncologic Drug Advisory committee (ODAC) is discussing two cancer therapies, and their AA post-market requirements. Folotyn and Beleodaq sponsor (Acrotech) failed to complete confirmatory trials for accelerated approval (AA) status. We expect ODAC (here) to discuss the risk of incomplete confirmatory trials and provide recommendations to the agency.

Oncology Center of Excellence (OCE) sends a clear message: confirmatory trials must be enrolled & ongoing at time of accelerated approval. The FDA is looking at both the confirmatory trials and the accelerated approval request as a package. Dr. Richard Pazdur, OCE director, noted that the FDA is in a period of "tough love" and the agency will not provide an accelerated approval if confirmatory trials are not underway despite a demonstration of safety and efficacy. As a reminder, the FDA has several new statutory authorities over the accelerated approval process including a formal expedited withdrawal process due to the passage of FDORA in December 2022.

FDA is also focused on global clinical trials as key – aka the ORBIS program. The Oncology Center of Excellence (OCE) current projects (Community, Facilitate, ORBIS, Confirm, and Catalyst) aim to increase public engagement (Community), facilitate international harmonization (ORBIS), and assist with novel therapy development in smaller companies (Catalyst).

ELEPHANT IN THE ROOM – FOR ONCOLYTICS

Companies will have to come up with a small molecule development strategy to achieve ROI in 9 years. Companies are already publicly discussing delaying smaller (oncology) indications due to the IRA. Recall Genentech's Alexander Hardy noted at the time of ASCO that going after a prostate cancer indication first may precede ovarian cancer. He noted that ovarian could get an approval three years sooner but the co would delay ovarian to be launched three years later, in tandem with prostate cancer approval. Novartis and others have made similar public comments.

IRA marches on with continued uncertainty over negotiation pricing framework. Despite holding several HHS listening sessions for the negotiated drugs, CMS has not been transparent about the implementation process. CMS is not expected to follow a typical value-based pricing framework (as seen with ICER) as they incorporate non-traditional factors like ingredients and R&D costs.

As a reminder, Imbruvica (ABBV, JNJ) is the only oncologic drug to be negotiated in 2026. CMS will offer its first price in February 2024 and announce a final price in September 2024. CMS will not have to publicly justify their final price until March 2025. Every one of its indications is orphan.

A new orphan bill ($5 B cost, roughly) would "fix" the orphan indication exclusion in IRA … while it should pass through Congress, it is unlikely to in the near-term. The bill would (1) allow an exemption no matter how many orphan designations a co has (now limited to one orphan designation) and (2) if a second non-orphan designation triggers IRA then the clock starts ticking at the time of the second non-orphan indication.

CMS likely used script volume versus gross spending to arrive at Top 10 list, reducing oncology exposure. Xtandi was not included in the list, much to the surprise of most when CMS unveiled the top ten for 2026 negotiation. There is speculation that the agency purposely analyzed prescription volume – which swept in insulin and Stelara – and were largely unanticipated in year one.

CAR-T ENTHUSIASM FROM SCOTT GOTTLIEB

CAR-T (GILD, JNJ, BMY, NVS) enthusiasm anew from Dr. Scott Gottlieb, but we believe manufacturing and other factors will be a near-term headwind. At the annual Association for Value-Based Cancer Care (AVBCC) conference (here), Dr. Scott Gottlieb, former FDA commissioner, noted the progress of current CAR-T therapies and the tailwinds that are expected to improve the cell market. Dr. Gottlieb is writing a book on the topic.

Tailwinds, per Scott Gottlieb, include: improvements in sequencing at scale during COVID (with improving genomic tech), the well understood biology of cancer which make it ripe for targeted therapies, and the opportunity for novel regulatory review methods that may improve access for patients. We, however, anticipate manufacturing issues for CAR-T to persist as it will take time for capacity to be built from the current investments into manufacturing.

CANCER MOONSHOT

Investments in women's cancer (including endometrial cancer) are expected from the recently announced Initiative on Women's Health Research (here). Research on women's health (including endometrial cancer) has been chronically underfunded. The initiative directs the agencies to provide recommendations within 45 days on ways the agencies can advance women's health research.

Additionally, as a part of Cancer Moonshot, the Administration is putting in place billing codes for oncology patient navigation services (here). CMS will implement standardized billing codes that allow insurers to provide coverage for patient navigators. This builds on an earlier announcement that CMS will cover principal illness navigation service for Medicare beneficiaries.

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